Patient and Parent Guidebook on Muscular Dystrophy

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This book is dedicated to
the courageous families of patients
with muscular dystrophy.

For everything there is a season,
and a time for every matter under heaven:

A time to be born and a time to die;
A time to plant and a time to pluck what is planted;
A time to kill and a time to heal;
A time to breakdown and a time to build up;
A time to weep and a time to laugh;
A time to mourn and a time to dance;
A time to cast away stones and a time to gather stones together;
A time to embrace and a time to refrain from embracing;
A time to seek and a time to lose;
A time to keep and a time to cast away;
A time to rend and a time to sew;
A time to keep silence and a time to speak;
A time to love and a time to hate;
A time for war and a time for peace.

Ecclesiastes: Chapter 3, Verse 128

Until one is committed, there is hesitancy, the chance to draw back, always ineffectiveness. Concerning all acts of initiative (and creation), there is one elementary truth, the ignorance of which, kills countless ideas and splendid plans-that the moment one definitely commits oneself, then providence moves too.

All sorts of things occur to help one that would never otherwise have occurred. A whole stream of events issues from the decision raising in one's favour, all manner of unforeseen incidents and meetings and material assistance which no man could have dreamed would have come his way.

The Scottish Himalayan Expedition
By W. H. Murray

Whatever you can do or dream you can, begin it. Boldness has genius, power and magic in it. Begin it now.

GOETHE

Preface

Muscular dystrophy is a disease that progressively weakens not just the patient, but affects the entire family too in their day to day living. Muscular dystrophy patients are very special indeed. Despite the physical limitations due to weakening muscles, they have an extremely positive attitude towards life. They have an acceptance of reality that we all should learn from. They have the ability and courage to go through extreme physical pain and difficulty in attempting to improve their conditions through the prolonged rehabilitation process.

Whereas, as doctors our focus is and should be on the patient, what many of us don't realize is that the parents and families go through a lot of emotional and physical hardship as well. To have to manage just the routine activities of life of the patients is a very tiring and exhausting process. Having to support the medical treatments, investigations, rehabilitation, etc. takes enormous time and energy. Since muscular dystrophy patients are completely dependent, the families have a very important role to play in their daily lives as well as their medical treatments. This takes courage, caring and commitment along with determination and dedication.

Looking after these patients involves hard physical strenuous work, lots of time, a very positive attitude and a spiritual acceptance of this difficult reality. On top of all this, their having to hear from multiple doctors that there was nothing that could be done to treat the patients was frustrating and hurting. What can be worse than having to watch a family member slowly wither away and at the same time have to repeatedly hear that nothing can be done for them. (Of course, now with the availability of stem cell therapy, that is no longer true!).

The parents and the families often cannot even express their own emotional, mental and physical pains and suffering to anyone. Most parents have to give up many of their own work commitments and leisure activities just to look after the children. We therefore wrote this book for those courageous families that have to work 24X7 for several years trying to make the patients comfortable, pain free and as mobile and independent as possible.

Whilst writing this book we tried to think from the point of view of a patients parent or the patient themselves. What are the questions that they have in their minds that need to be answered, what are the areas where there is confusion in their minds, what information do they need to help them make informed decisions about future course of action. So this book has 4 sections. The first one is about the disease itself, the second is about various treatments that are already available , the third about recent advances and the fourth about some other supportive aspects of the problem. In the second section

about the treatment there are various multidisciplinary aspects covered that include the use of steroids, other medications, orthopedic management, respiratory care and psychological management. A interesting part of this book is the importance of yoga and diet in the overall management of muscular dystrophy. These two aspects along with the chapter on exercises and stretches and assistive devices is something parents can easily implement at home.

A lot of parents are also unclear about what are the future developments that are likely to be useful for their children. Whereas some of the newer drugs and gene therapy are still in the future, stem cell therapy is available in the present moment. Therefore a separate section has been devoted to the recent advances. There is a in-depth discussion of the role of stem cell therapy in muscular dystrophy where our results have been discussed focusing primarily on the improvements that have been seen and the safety aspects. Stem cell therapy, which is a part of the newly expanding field of regenerative medicine, is here to stay. There is a growing body of published scientific literature that is showing both efficacy and safety of this form of treatment. We can keep arguing about whether it is a proven or unproven form of treatment but the fact is that the improvements that have been reported from the centers doing this therapy are better than any other form of treatment currently available and almost certainly seem to be altering the natural history of the disease. When dealing with a progressively worsening disease with a well defined mortality such as Duchene muscular dystrophy,its important to realize that the risks of not doing anything can be greater than trying a treatment with reasonable safety and efficacy. These are however grey areas and it is hoped that this book helps parents and patients make these decisions with a clearer mind.

Whereas, we as doctors and therapists can spend some time with the patients during their treatment processes, it is the families that are with the patients through the entire day and night. It is our belief that if the family members understood the disease process and the treatment methods better they would be able to make a much bigger difference to the patient's lives than we doctors can. They would also be able to make informed choices about the various new treatment options that are now becoming available. This book was therefore written for this purpose. Whereas we have written an earlier book titled "Stem cell therapy and other recent advances in muscular dystrophy", we realized that the book (which was meant for medical doctors and therapists to read) was too technical for families and patients to understand.

This book is therefore, written in a very simple non technical manner. It is meant to help parents, siblings, spouses and the patients themselves to understand what this disease is all about and what are the simple things that they can do to make life a little easier for the patients. We salute the muscular dystrophy families and offer them this book to educate, empower, enrich and enlighten them on what best they can do together for these very special children and adults with muscular dystrophy.

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Contents

Introduction

Muscular dystrophy was first described in 1830's as a group of disorders. Around 50,000 -250,000 people are affected annually, but these numbers seem to be growing each year. It is more prevalent in some cultures. Both the sexes are affected but males are affected more than females. Many muscular dystrophies occur in childhood and others may occur as late as 60 years of age. Duchenne muscular dystrophy is the most common form and estimated at 1 in 3,500 male births while Becker's muscular dystrophy is 1 in 18,518 male births. Prevalence of all forms of Limb girdle muscular dystrophy ranges from 1 in 14,500 to 1 in 1,23,000. It is estimated that every year there are over 1600 children born with this condition in India.

Muscular dystrophy is recognized by progressive weakness and eventually degeneration of muscles. Muscular dystrophy can also affect the heart, lungs, eyes, spine, brain, endocrine system and gastrointestinal system. There are around 9 types of muscular dystrophy and each differs by the extent and distribution of muscle weakness, age of onset, severity of symptoms and family history.

Awareness about muscular dystrophy, its prevalence, understanding the cause, the problems, the possible way to minimize deformities, treatment options available and other aspects of this disorder are little understood by parents. Many a times, diagnosis gets delayed, because problems of a child are attributed to general weakness. As soon as the diagnosis is made, parents often give up on the child, because proper guidance about what to do next is not available. Sometimes, guidance comes too late. Many complications, which can be avoided, occur due to lack of timely intervention.

Hence, this book is intended to provide a step by step guideline for patients and caregivers of muscular dystrophy. Its main aim is to help them to understand the disease better by providing holistic information, so that they can be prepared for changes in their patients or themselves. This will also equip them with information about how to handle those changes, to a certain extent.

Here at NeuroGen Brain and Spine Institute, we are very aware that there is an increasing need for guidance of muscular dystrophy, as we have treated more than 400 patients of various kinds of muscular dystrophy.

So this book is written with an intention to provide simple and easy-to-follow text with clear illustrations. We hope that this helps the parents of children afflicted with muscular dystrophy and adult patients to cope with this condition better.

SECTION - A :

About Muscular Dystrophy

1.

Clinical Presentation and Diagnosis

What is muscular dystrophy?

Muscular dystrophy is a condition in which muscles start getting weaker day by day and the weakness increases over time. Hence it is known as a progressive condition. These disorders are generally passed on from generation to generation but can also occur in families with no previous history of this condition.

What causes muscular dystrophy?

The cause of muscular dystrophy is an abnormal gene or defective gene. Genes control the functions of each cell present in our body including the muscle cell. These genes also control the protein required for the muscles to function properly.

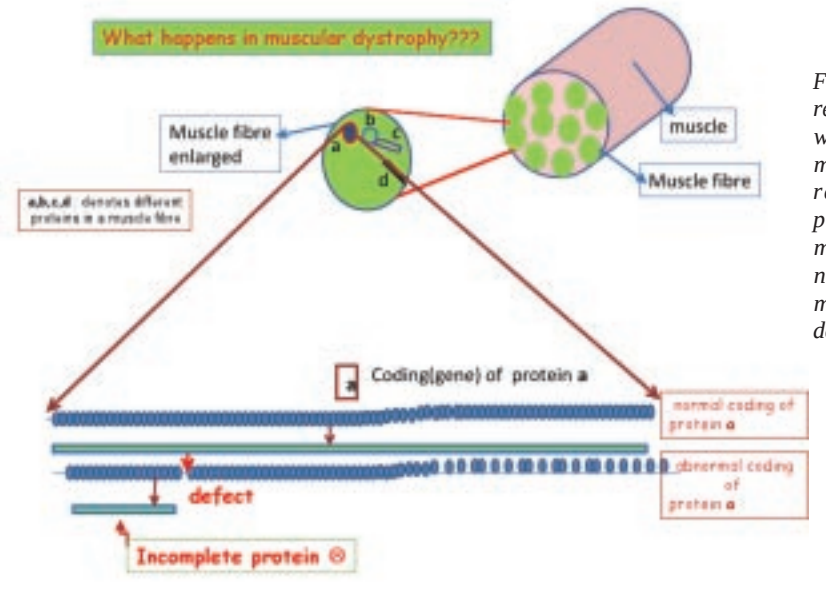


Fig. 1.1 - Diagrammatic representation of a muscle with a spindle/group of muscle fibres. a, b, c, d represents different proteins which form a muscle. An example of normal and abnormal muscle protein (due to defect in protein a)

What is an abnormal gene in muscular dystrophy?

In muscular dystrophy one of the proteins required for muscles is not produced properly. Here, either the protein is absent or present in very little quantity which leads to muscle damage and weakness. Depending upon the faulty gene or faulty protein, the type of muscular dystrophy is distinguished.

What are the early symptoms of muscular dystrophy?

Symptoms can be present from birth, early childhood or may appear from middle age or sometimes even later than that also. In young babies we see them as floppy and their milestones are delayed i.e. babies sit, crawl or walk much later than normal babies. But delayed milestones alone don't confirm the diagnosis of muscular dystrophy.

Which are the muscle groups involved in different type of muscular dystrophy ?

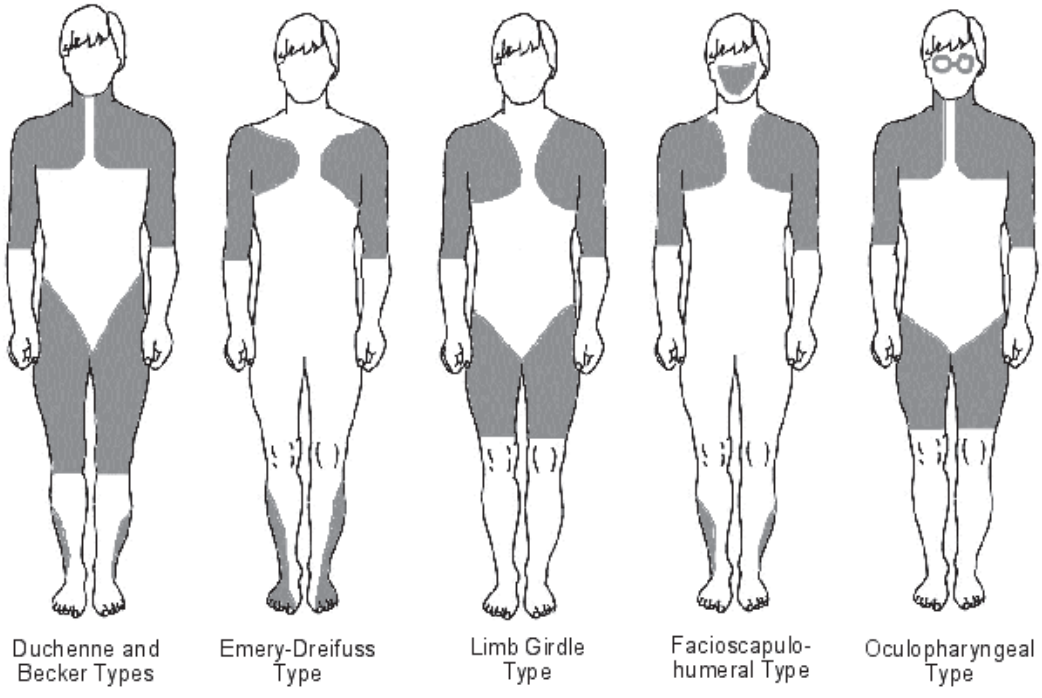


Fig. 1.2 Different muscle group is involved in different type of muscular dystrophy:

Common symptoms of muscular dystrophy:

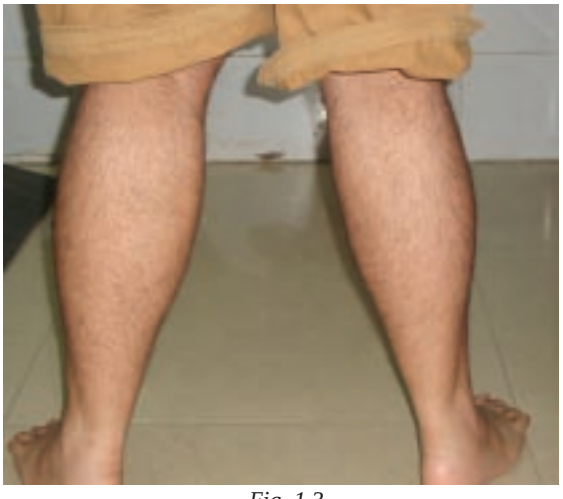


Fig. 1.3

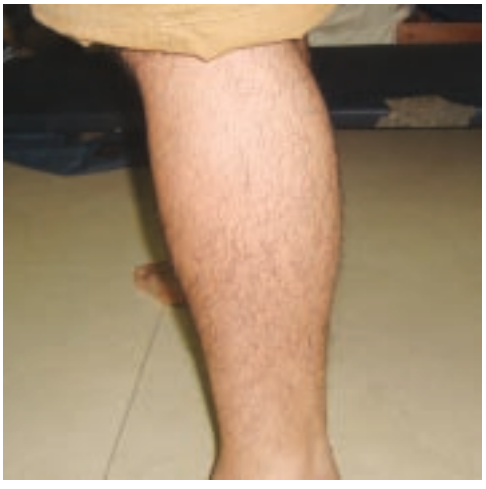


Fig. 1.4

Pseudo muscle hypertrophy (though the muscles appear big they are actually weak) e.g. large calf muscle

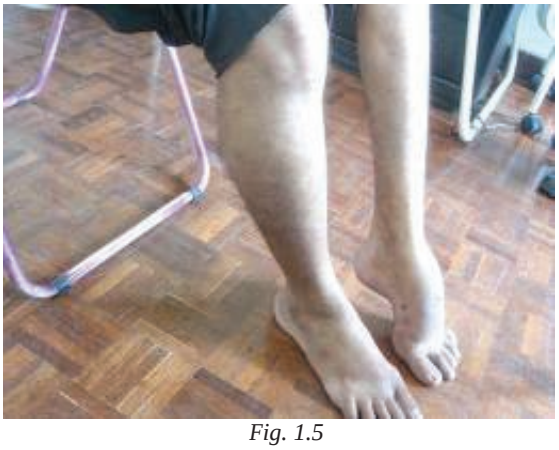


Fig. 1.5

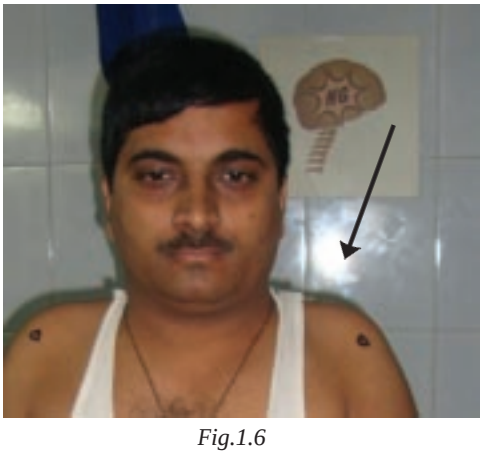


Fig.1.6

3. *Muscle wasting-thinning of the muscles*

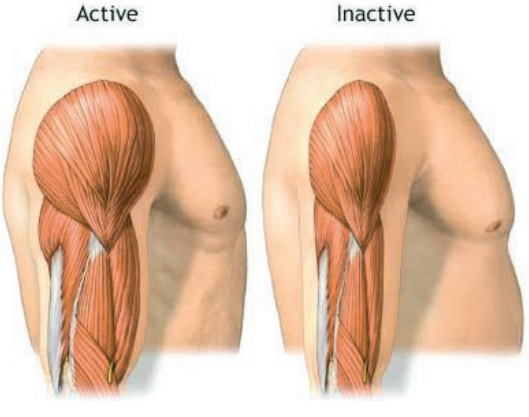


Fig. 1.7: Muscle weakness

6.

Fig. 1.8: Poor Balance

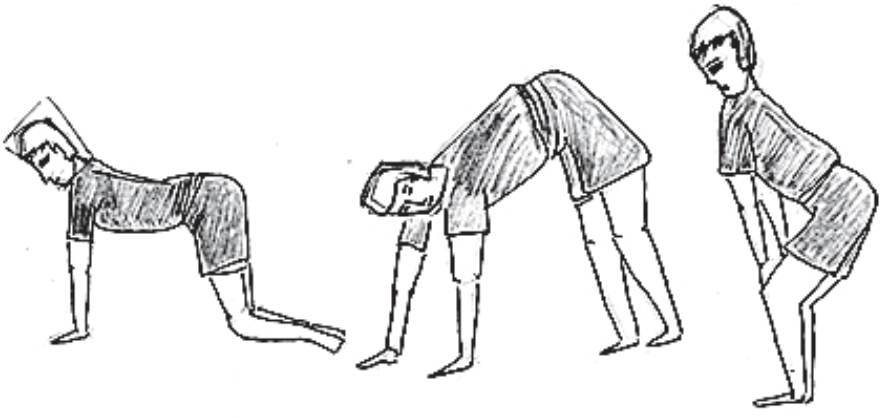


Fig 1.9: Difficulty in getting up from the floor or low seat.

Fig. 1.10

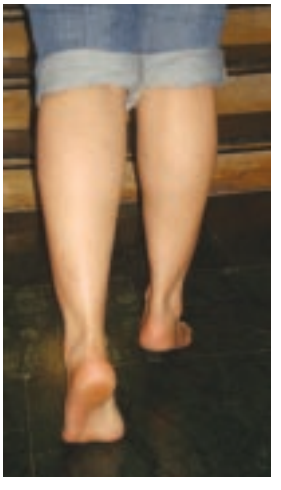


Fig. 1.11

Walking on toes

Fig. 1.12: Difficulty in Climbing stairs

Fig. 1.13: Waddling gait: walks clumsily and takes short steps due weak hip muscles

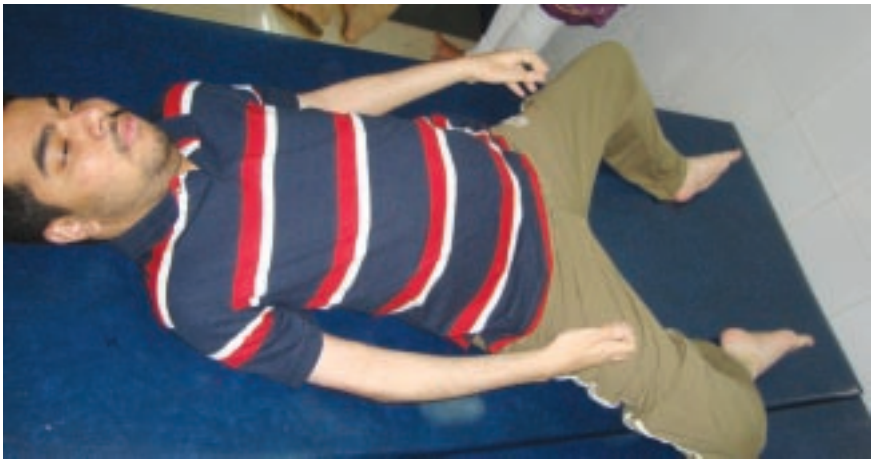


Fig. 1.14: Contractures: when joint become tight due to tightness of muscles or reduced muscle length

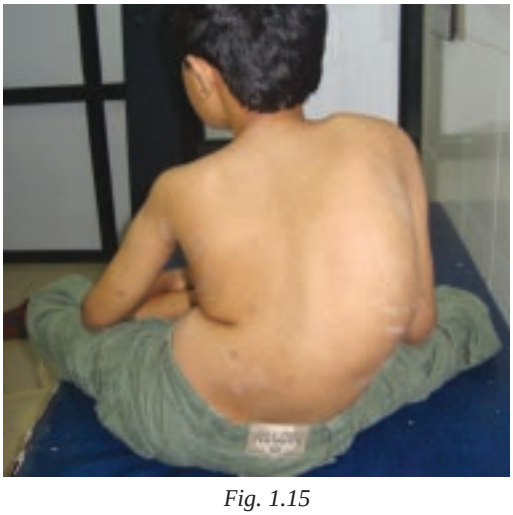


Fig. 1.15

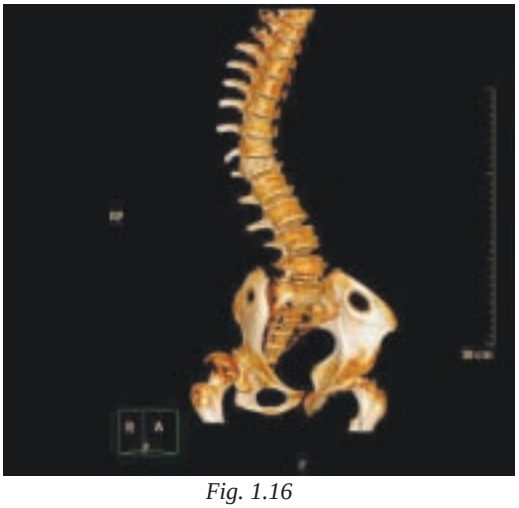


Fig. 1.16

Scoliosis: sideways bending of the spine or back bone

Fig. 1.17: Gower's sign: the child walks up his thighs with his hands.

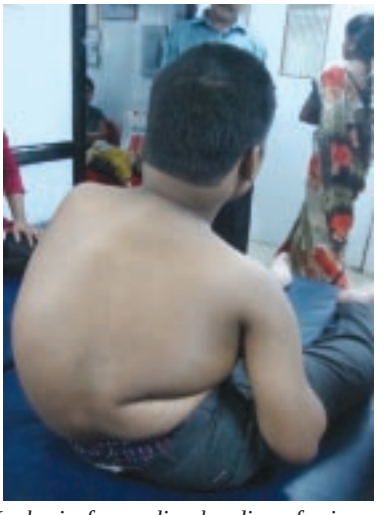


Fig. 1.18: Kyphosis: forwarding bending of spine or back bone.

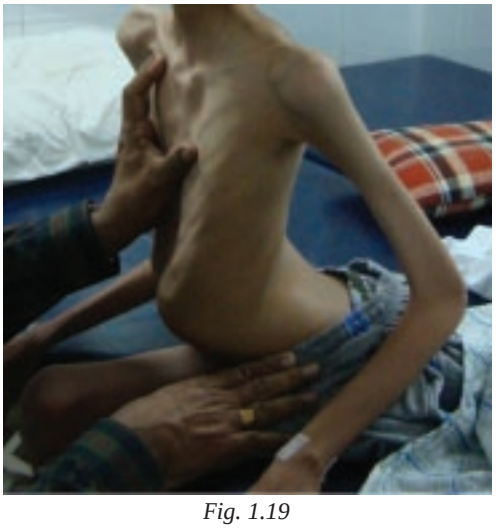


Fig. 1.19

Fig. 1.20

Lordotic posture: deep curve in the spine near the waist line which is due to weakness of abdominal muscles.

Few others Symptoms seen are :

- •. Speech delay
- • Frequent falls while walking

How do the various forms of muscular dystrophy differ from each other?

The most common forms of muscular dystrophy:

Type	When it starts?	Specific symptoms	Other body part involved
1. Duchenne Muscular Dystrophy (DMD)	Before 4 to 5 years of age	1. Weak hip and shoulder muscles. 2. Stops walking around the age of 10 to 12 years. 3. Kyphosis and scoliosis of spine. 4. Weak breathing muscles. 5. Affects males.	Cardiomyopathy (heart becomes big in size and weak in pumping action.)
2. Beckers Muscular Dystrophy (BMD)	Early childhood to adult	1. Weak hip and shoulder muscles 2. Can walk even beyond the age of 15 years. 3. Breathing muscles also become weak but at a very later stage.	Same as above
3. Limb Girdle Muscular Dystrophy (LGMD)	Early childhood to adult	1. Muscles of hip and shoulder become weak but slowly.	Same as above

Less common forms of muscular dystrophy:

Type	When it starts?	Specific symptoms	Other body part involved
4. Facioscapulohumeral Dystrophy (FSHD)	Before the age of 19 to 20	1. Weakness of shoulder, face and upper arm but slowly.	None
5. Congenital Muscular Dystrophy	At birth or within first few months	1. Low tone or floppy 2. Contractures. 3. Delayed milestones. 4. Weak breathing muscles.	Mentally retarded and problems with eyes.
6. Myotonic Dystrophy	Starts between 11 to 20 years of age	1. Weakness of shoulder, face and upper arm but slowly.	Mental retardation, cataracts, reduced size of testicles, heart problems.
7. Oculopharyngeal Dystrophy	Between 40 to 60 years	1. Slow weakness of the eye and throat muscles.	None
8. Emery Dreifuss Myopathy	Childhood to adults	1. Weakness of shoulder and upper arm. 2. Contractures.	Cardiomyopathy.
9. Distal Myopathy	40 -50 years	1. Weakness of hand, arm and foot muscles.	None.

How is muscular dystrophy diagnosed?

- **Clinical examination:** Clinical examination is done by the doctor / physician. Your child will be asked to run, jump and climb stairs and after this he will be asked to sit or get up from the floor. If he puts his hands on the knees and pushes himself up, it is a sign of muscular dystrophy known as Gower's sign. (Fig. 1.17)
- **Blood test:** Blood sample is taken and checked for CPK level (i.e. Creatine Phospho Kinase, which is a chemical substance released in blood by a damaged muscle). Usually in muscular dystrophy, the level is 10 times more than the normal.

  Normal CPK levels:

  Male: 38 - 174 units / L

  Female: 96 - 140 units / L
- **Muscle biopsy:** Under anesthesia, a small piece of muscle is taken from the bulkiest muscle and examined under a microscope.
- **Electromyogram (EMG):** A thin-needle is inserted through the muscle to be tested and electrical activity is studied. This also can give a clue whether the muscle is damaged or not.

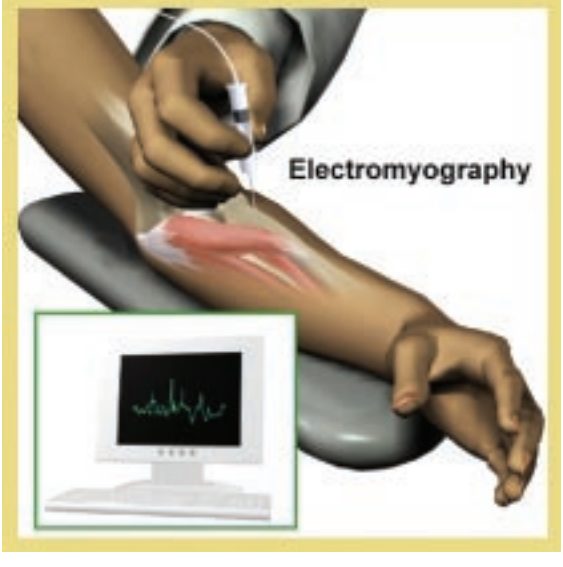


Fig. 1.21: EMG procedure

- **Carrier analysis:** In few cases, the mother is the carrier of the faulty genes which means that the mother must have passed the faulty gene or disease to the child. However, the mother is not always a carrier. It could be a fresh mutation. If mother is found to be the carrier, genetic counseling should be advised for other female relatives of the child.
- **Genetic testing:** Blood samples are examined for mutations in the gene. Genetic testing is compulsory once muscular dystrophy is confirmed by doing muscle

biopsy. Genetic testing for DMD / BMD is commonly available in India. For LGMD or FSHD it is available in very few centers. It is important for several reasons to have the genetic confirmation of the diagnosis as it will help to determine if the patient is eligible for any clinical trials and also helps the family to take decision regarding prenatal diagnosis and future planning of pregnancies.

- **MRI or Magnetic Resonance Imaging:** MRI is a painless and non surgical procedure which is done to examine the muscle quality, size or any abnormalities in the size. It monitors the fatty replacement of the muscle tissue as well as progression of the disease.

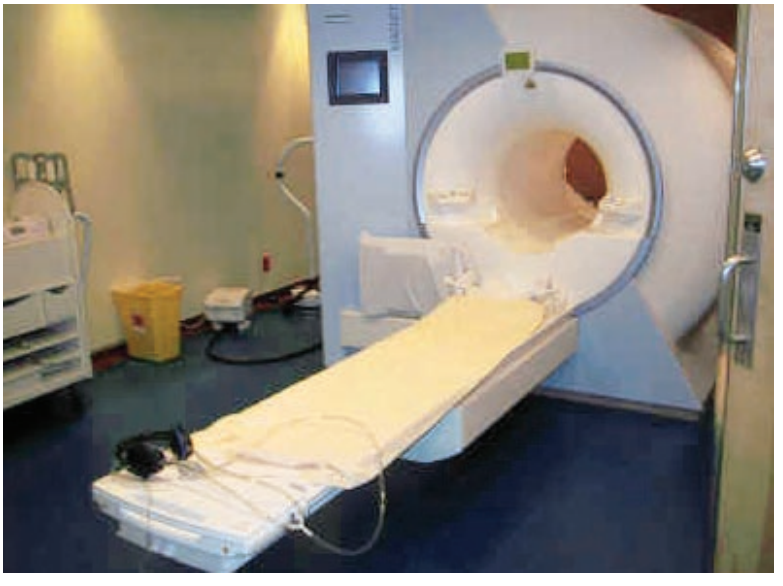


Fig. 1.22: MRI machine

What next after the diagnosis?

Once the diagnosis is confirmed it is important that the patient / parents approach an appropriate team of specialists who can guide in the management of the condition. The team may comprise of a physician, neurologist, pediatrician, orthopedic surgeon, neurosurgeon, physiotherapist, occupational therapist, speech therapists, social worker, dietician and psychologist. These specialists can give proper advice regarding the outcome and treatment options.

What are the treatment options for muscular dystrophy?

Till date, no cure is available for muscular dystrophy, although, medicines like steroids can delay the progress of the disease. Stem cell therapy and rehabilitation are found to be very beneficial in improving the condition. These treatments certainly ensure the patients an improved quality of life and increased life expectancy.

2:

Complications

The signs & symptoms differ a lot depending on the type of muscular dystrophy.

But the most frequently seen complications are muscle tightness, contractures, bony deformities, scoliosis (S- shaped curvature of the spine), heart problems & breathing difficulties.

Complications due to prolonged steroid treatment include-

- Cataracts Fig. 2.1
- Cushingoid features (moon face) Fig. 2.2
- Obesity 2.3
- Short stature Fig. 2.4
- Constipation
- Hypertension
- Delayed puberty
- Behaviour changes (irritability, hyperactivity)
- Occasionally slight increase in body hair.

The most common complications seen in different muscular dystrophies are:

1. Breathing Complications

- Patients with muscular dystrophy are at risk of breathing complications due to spinal muscle weakness & progression of spinal deformities.
- Breathing complications mostly occur only after the person stops walking. But in case of Congenital muscular dystrophy, breathlessness is noticed even when the person is still walking.

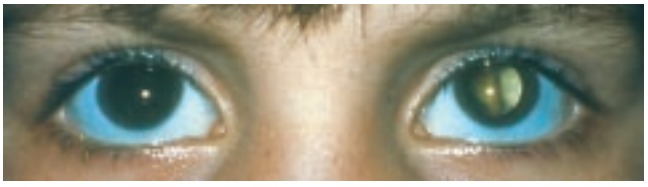


Fig. 2.1: Cataracts

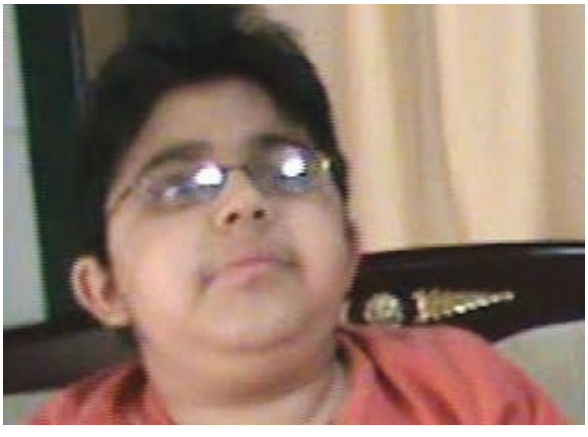


Fig. 2.2: Cushingoid features (moon face)

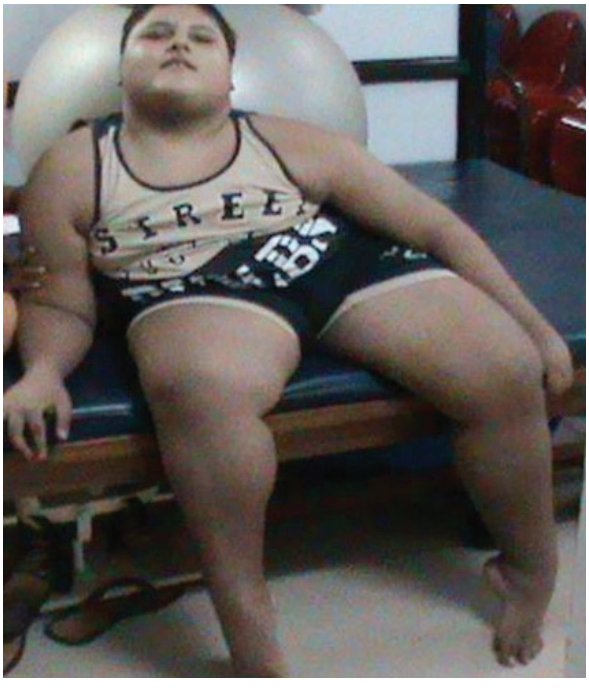


Fig. 2.3: Obesity

Fig. 2.4: Short stature

- Shoulder weakness is the earliest sign indicating the onset of breathing muscle weakness.
- Breathing complications in muscular dystrophy majorly include reduced lung expansion leading to collapse of lung tissue, which in turn causes chest infections. It also causes difficulty in coughing which increases the accumulation of mucus in the lungs.
- In Myotonic dystrophy, there is combination of breathing muscle weakness and dysfunction of the breathing centres in the brain. Persons are at risk of aspiration pneumonia due to failure of swallowing muscle function.
- Difficulty in breathing at night may even cause breathing failure which can be fatal.
- In later stages of muscular dystrophy, the person may have to be put on a ventilator.
- Breathing complications are the major cause of death in 90% of Duchenne muscular dystrophy patients.

2. Heart Complications

- Involvement of the heart is very common in muscular dystrophy patients.
- Weakness of the heart muscle and replacement of muscle tissue with connective tissue or fat, results in complications of the heart.
- These complications are generally progressive leading to ECG abnormalities and to a poor ability of the heart to pump blood, which may be life-threatening.
- Breathing problems & spine deformities may also affect the functioning of the heart.
- Approximately 70% of boys with Beckers muscular dystrophy have cardiac involvement by age 20.
- Heart problems in Beckers muscular dystrophy are worse than in Duchenne muscular dystrophy patients. Myotonic dystrophy type 1 has more than one system affected with prominent heart problems leading to an increased incidence of sudden death.

3. Psychosocial complications:

- People with muscular dystrophy may experience psychological & social difficulties due to their limited ability to participate in many activities when their friends are doing well.
- They may feel helpless as they become dependent on others.
- Social isolation or withdrawal, emotional disturbances like anger, depression, anxiety & reduced self-esteem are some of the psychosocial issues.
- Stopping schooling (due to difficulties in carrying the child and moving in the school building) has a huge impact on the psychosocial functioning of kids.

- Associated conditions such as ADHD (attention deficit hyperactive disorder), learning difficulties or autism spectrum disorders should be identified early to reduce psychosocial issues.
- A fear about the future may always worry them.

4. Obesity: Fig 2.3

- People with muscular dystrophy often are overweight due to lack of physical activity.
- It adds strain to the weak muscles due to which the person can approach non walking stage faster.

5. Sleep disturbances:

- As muscle weakness increases, the person with Muscular dystrophy will not be able to change his position on his own. Therefore the patient's & his caregivers sleep would be disturbed throughout the night.
- Breathing difficulties also may keep the person awake.

6. Osteoprorosis

- Osteoporosis is the thinning of the bones in which they lose calcium and become softand brittle.
- These soft bones are more prone to fractures.
- Risk of osteoporosis increase with age and loss of walking as the bones are not subjected normal weightbearing.
- Fractures in the walking phase lead long periods of bed rest which in turn could result in loss of walking.

7. Scoliosis Fig. 2.5 Fig. 2.6

- Scoliosis is twisting in the spine (S shaped curvature) caused due to weakness in trunk muscles.
- It progresses when the child is unable to stand or walk.
- It results in poor sitting tolerance and balance, tightness of the trunk muscles and compression of lungs & heart.
- Restricted movement of the rib cage may cause difficulty in breathing & heart functions.

8. Contractures Fig. 2.7, Fig. 2.8

- Contractures are shortening and tightening of the muscle tissue due to which there is restriction in joint movements.
- Contractures occur due to the weakening of certain groups of muscles.
- They are most commonly seen at hips, knees, ankles & elbows.
- Stretching of these contractures cause severe pain.

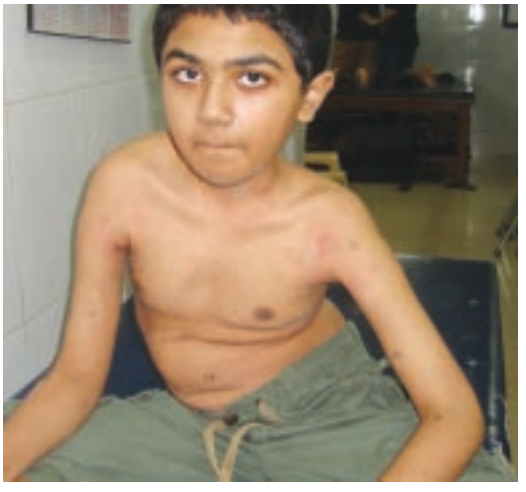


Fig. 2.5

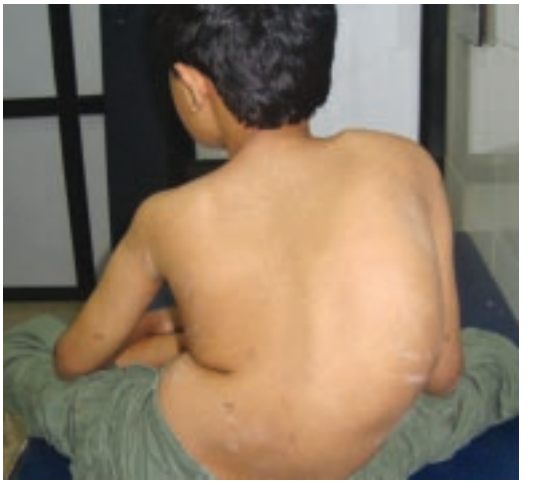


Fig. 2.6

Scoliosis of Lumbar spine

Fig. 2.7: Contractures of hip

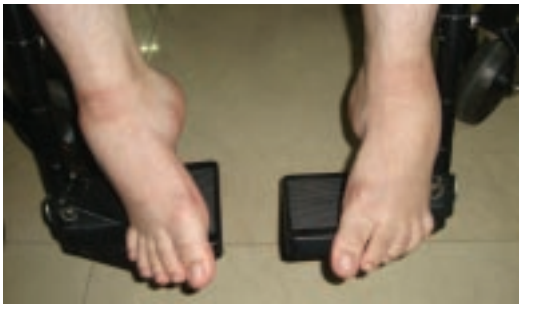


Fig. 2.8: Contractures of foot

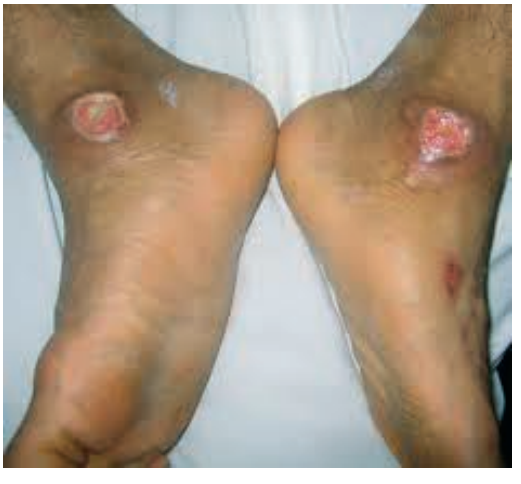


Fig. 2.9: Pressure sores

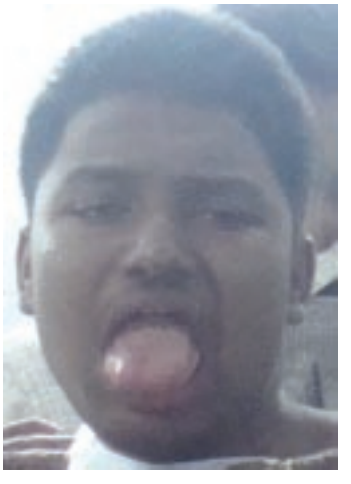


Fig. 2.10: Pseudohypertrophy of tongue

9. Pressure Sores Fig. 2.9

- Staying for a long time in any particular position in muscular dystrophy could result in pressure or bed sores.
- Although sensation is generally not affected, persons with MD are at a risk of developing pressure sores as they are unable to re-position themselves on their own. Also, being overweight can increase the risks considerably.
- Pressure sores develop mainly on bony prominences including the spine, ankles, knees, shoulders and head, depending upon the position of the patient.

10. Swallowing

- Some children with muscular dystrophy have specific problems with swallowing. Food or drink may go down the wrong way (goes to breathing path instead of going to the stomach). This is called aspiration.
- If this happens regularly, the child could be more prone to chest infections and find it hard to put on weight.
- Some may eat very slowly due to the shape of their mouth or enlarged tongue (pseudo hypertrophy) or weakness in their chewing and swallowing muscles. Fig. 2.10
- Also children may have weakness in their arms and upper limbs due to which they are unable to feed themselves.

DESIDERATA

Go placidly, amid the noise and the haste and remember what peace there may be in silence.

As far as possible without surrender, be on good terms with all persons. Speak your truth quietly and clearly and listen to others, even the dull and ignorant, they have their story. Avoid loud and aggressive persons; they are vexatious to the spirit. If you compare yourself to others you may become vain and bitter, for always there will be greater and lesser persons than yourself.

Enjoy your achievements as well as your plans. Keep interested in your career, however humble it is a real possession in the changing fortunes of time. Exercise caution in your business affairs, for the world is full of trickery. But let this not blind you to what virtue there is; many persons strive for high ideals, and everywhere, life is full of heroism.

Be yourself, especially do not feign affection. Neither be cynical about love; for in the face of all aridity and disenchantment, it is as perennial as the grass. Take kindly the counsel of the years, gracefully surrendering the things of youth.

Nurture the strength of spirit to shield you in sudden misfortune. But do not distress yourself with imaginings. Many fears are born of fatigue and loneliness.

Beyond a wholesome discipline, be gentle with yourself. You are a child of the universe, no less than the trees and the stars. You have a right to be here. And, whether or not it is clear to you, no doubt the universe is unfolding as it should.

Therefore be at peace with God, whatever you conceive him to be. And whatever your labors and aspirations in the noisy confusion of life, keep peace with your soul.

With all its shams, drudgery and broken dreams, it is still a beautiful world.

Be cheerful. Strive to be happy!

– Max Ehrmann

SECTION - B :

Treatment and Care

3:

Care Considerations

Caring for a child or a young adult who has functional loss at an early stage of life becomes very difficult and emotionally challenging for the parents or care givers, as we wish the best for our children and wish they get the best out of their lives. For families with a muscular dystrophy patient care giving becomes a full time job. Muscular dystrophy is a genetic disease which causes weakening of the skeletal muscles. As the disease is diagnosed, the symptoms start manifesting and many changes take place within the individual as well as in the environment surrounding the individual. It is then that the parent or care giver plays a very important role in helping to create a safe environment at home and taking proper medical care of the individual affected.

1. Providing strong support:

It is very commonly seen that parents with a child having any sort of debilitating illness, are excessively protective about their child, which limits the child's functional abilities and discourages his (her) chances and extent of an independent life. It is very important to change this sort of mind set. It is very important for a child to be prepared to grow up, grow old and also be independent. They should be encouraged to do their tasks independently. Simple things like eating, wearing clothes, etc. should be made to do independently. The individuals should also be encouraged to participate in a lot of activities like that of school or college or work which would take their focus off the disability.

2. Strong family support:

As the individual is a diagnosed with muscular dystrophy or any other debilitating illness it affects the whole family especially if it is a small child or young adult. Parents or care givers have to be strong both mentally and emotionally. It is very important that the family members help the child accept the diagnosis and help him deal with the condition. The family should be united and open which in turn

gives space to each family to cope with the condition accordingly. It is important to have family outings, picnics, get-togethers or movies etc to create a healthy environment both for the individual and the family members.

3. Addressing the breathing problems/caring for the lungs:

Patients affected with muscular dystrophy such as Becker's and Duchenne muscular dystrophy may often experience breathing problems. The muscles required for breathing are affected and as the child / adult grows, the lung muscles become weaker and this eventually leads to difficulty in breathing i.e hypoventilation. After the individual stops walking, difficulty in coughing starts and a cough assistant may be required. In the night, the individual may face some difficulties in breathing and may require a CPAP machine for deep breathing. And as age progresses, self breathing becomes difficult so an assistive device like ventilator or CPAP may be required for breathing.

If the following are present a physician or pulmonologist should be consulted:

1. Tired feeling all the time.
2. Frequent headaches in morning or throughout the day.
3. Difficulty in concentration.
4. Sleep & drowsiness without any reason.
5. Waking up frequently in the night or difficulty in waking up in the morning or having nightmares.
6. Feeling short of breath after waking up.

What to do?

1. A doctor should be consulted at least once in a year and forced vital capacity test should be done.
2. Flu and pneumonia shots should be taken compulsorily.
3. Regular breathing exercises should be done under the supervision of professional physical therapist.
4. Proper antibiotics should be administered in case of any infection or chest infection.
5. If admitted for any surgeries, doctors should be informed to not give any inhaled anesthesia or succinylcholine. (This is very important and can be lifesaving.)
6. If any breathing machine is used, care should be taken to ensure that the apparatus is properly fitted and also timely sterilized.

4. Endocrine system:

Endocrine system in a human body is made up of glands which make hormones. Hormones are very important for growth, puberty, weight and bone health.

It is often seen that patients of Duchenne muscular dystrophy don't have endocrine problems at birth. But usually since these boys are on steroid medicines for a long time, it changes the way our body's natural hormones are made and work. This can cause changes in growth, weight, puberty and bone health.

 # Excessive weight gain:

It is often seen that people suffering from muscular dystrophy especially in Duchenne muscular dystrophy have a tendency for weight gain. This happens because these patients have reduced ability to walk or they have lost their mobility, so less energy is burned off and due to side effects of medications e.g. steroids there is often increase in appetite.

Why it is important to maintain adequate weight?

1. To reduce the burden on weakened muscles.
2. To reduce the risk of surgery.
3. To reduce strain on respiratory muscles.
4. To decrease the risk of scoliosis.
5. To reduce difficulty of transfers, especially for care takers if the individual is immobile.
6. To minimize the effect on the patient's ability to walk.

Prevention of weight gain:

1. Diet should be healthy and balanced in order to prevent excessive weight gain. This should be done under counseling of a dietician.
2. Physical activity is a must in order to burn off extra calories e.g. swimming, physical exercises.
3. Adequate amount of sleep is required as it is often seen that inadequate sleep may contribute to weight gain.
4. Drinking lots of liquids especially before meals is necessary.

- **Bone health:**

Osteoporosis is often seen as a side effect of steroid medication. Osteoporosis is a condition in which there is thinning of the bones. The bones become brittle and weak and can lead to fractures. People of muscular dystrophy often develop osteoporosis due to lack of exposure to sun, nutrition, hormonal deficit and physical activity as physical activity is very important for strong bones.

How to detect osteoporosis or fracture?

1. Cannot be felt unless there is a fracture.
2. Commonly encountered fracture is of the spine.
3. If persistent pain is present in limp, leg or arm or back, a doctor should be consulted and x-ray should be done to determine fracture or thinning of the bones.

Prevention of osteoporosis:

1. Bone health should be monitored by regular checkups.
2. Diet should be healthy and should contain adequate calcium and vitamin D.
3. Physical activity is a must. Weight bearing exercises and gentle stretches of the tight muscles or contractures needs to be done.
4. If on a wheelchair, proper seatbelt should be used to prevent falls or fractures.

Tests to monitor the bone health:

1. Backache or limb pain, fractures, vitamin D and calcium intake etc should be monitored by regular checkups.
2. X-ray of the spine should be done to rule out scoliosis or fracture.
3. DEXA scan should be used to measure Bone Mineral Density (BMD) to check if there is any thinning of the bone.
4. If young, x-ray of left hand and wrist must be taken, which helps to interpret the bone age and pubertal menstruation.

Important notes:

1. Due to lack of physical activity people with muscular dystrophy often develop weak bones.
2. Any limp or back pain concerns should be shared with a doctor.
3. Calcium and vitamin D are required in adequate amounts.
4. Physical activity, to the maximum possible extent should be carried out.

Treatment:

1. Observe without medications by giving healthy diet.
2. Supplements of vitamin D or calcium can be tried under a doctor's counseling, provided it is within age limit.
3. Bisphosphonates: Helps to preserve the bone mass and reduces the risk of fractures and pain. They can be taken orally as a pill or liquid or intravenously. It is important to know the benefits and side effects of the treatment before taking the treatment. These must only be taken if advised by physician.

- **Delayed puberty:**

  Puberty is the process in which body's hormones causes a person's body to change and make the body ready for reproduction. Signs of puberty or maturity are facial hair, body odor, acne, pubic hair and more importantly change of voice seen in boys. Puberty begins by the age of 11-14 years and is very important for bone health.

Effects of steroids on puberty:

1. Puberty begins late or doesn't start at all.

2. Abnormal development of puberty.
3. Short height.
4. Very less or no facial hair or pubic hair.
5. Hoarse voice or high voice.
6. Smaller penis size.

Important facts:

1. As the boy looks younger than his age, people around may treat him immaturely. This sometimes becomes disturbing for the young boy as he would like to be treated according to his age.
2. The good part of being short is that it is easier for the care givers or parents for transfering or carrying him.
3. Delayed puberty, may be upsetting for the parents or the child, but what is more important is that taking steroids helps maintaining muscle strength.
4. The only concern about delayed puberty can be not getting necessary hormones required for stronger bones.
5. Consult a doctor if there are no signs of puberty around 13-14 years of age.

Consult your doctor:

1. Doctor may change the dose of steroid and carefully monitor for any changes in muscle strength.
2. Doctor may suggest a visit to the endocrinologist.
3. Endocrinologist may consider testosterone treatment which is a male puberty hormone; it may bring about typical teenage behaviors' like mood swings, or cause more interest in sex or may change the body's look. Though these medicines have their own side effects, it is very important to consult the correct doctor as many doctors don't have much experience in using testosterone to help people with Duchenne.

- **Short height**:

  Short height means height below normal age limit. Being short may not affect your child's health but may affect his self esteem and may lead to emotional or behavioral problems. In boys with Duchenne, steroid can be a reason of short height as it slows the growth.

Important facts:

1. Family physician or parents should keep growth records of the child.
2. Parents should not prevent use of steroids with concerns about short height.
3. Short height makes it easier for transfers for the parents.
4. The only available solution could be reducing the dosage of steroids but which in turn can affect his strength.

5. Short height has no adverse effects on the health. Growth hormone should not be taken as it may have its own adverse and unwanted effects.
6. If the child has depression due to his short height, psychological support should be provided.
7. Opinion of endocrinologist can be considered.
8. Emotional support is very essential.

Tests:

1. Blood tests should be done to rule out any other reason for slow growth.
2. X-ray can be taken to measure the bone age.
3. Caring for the heart: It is very important to take proper care of the heart because in many forms of muscular dystrophy, as one grows older, problems with the heart may develop, for e.g. in Duchenne or Beckers muscular dystrophy. The major complication seen is cardiomyopathy and many times symptoms don't appear very early but the heart is already damaged.

Symptoms of cardiomyopathy:

1. Chest pain
2. Vomiting
3. Weight loss
4. Difficulty in doing routine work
5. Inadequate sleep
6. Stomach pain.

Test to rule out:

1. Cardiac MRI/2D echo: This test helps to ascertain whether the heart is pumping blood properly. It gives a detailed picture of the heart and its function.
2. ECG: In this test, stickers are attached to the chest, which measure the rate and rhythm of the heart.

Rhythm problems:

1. Arrhythmia: abnormal rhythm
2. Tachycardia: faster heart rate
3. Bradycardia: slower heart rate

Important facts:

1. A heart check up should be done every 1-2 years, from the time of diagnosis.
2. In Duchenne and Becker's muscular dystrophy, till the age of 10, both the above mentioned tests should be done every alternate year. After the age 10, it should be done every year.

3. If there is abnormality found in any of the test, it should be repeated every 6 months and necessary medications should be started.
4. Before any surgery, both the tests should be compulsorily done.
5. Even if there is no abnormality found, medications should be started before the problem starts.
6. Caring for the muscles: In muscular dystrophy, as the disease progresses, the muscles become weaker day by day. The protein that is required is either absent or present in very small amounts. For e.g. in Duchenne muscular dystrophy, dystrophin is either present in small amounts or completely absent. After normal use, the muscles are unable to heal properly and these damaged muscles are replaced by connective tissue and fat.

Important facts:

1. The muscles of the hip and pelvis start becoming weaker first, so over time, getting up from the floor become difficult and falls become frequent.
2. As age increases or disease progresses, muscles of the back, shoulder, arms, and chest also start becoming weaker. More assistance is required in doing activities of daily living.
3. As the age increases, due to less range of motion and being in constant position for a long time, contractures develop.
4. Muscles of heart, breathing, swallowing start getting weak and need special attention.

What to do/what can be done?

1. Regular checkups with the doctor.
2. Start steroid as it helps to maintain the muscle strength for a longer period and helps delay the disease progression.
3. Start physical therapy under a physical therapist as wrong exercises can damage the muscles also.
4. Use splints which prevent contractures.
5. Do regular stretches to prevent tightness and contractures.
6. Make use of adaptive devises for e.g. Wheelchair or electronic scooter.

4:

Multidisciplinary Approach

Muscular dystrophy is a degenerative disorder of the muscles, characterized by progressive weakness of the skeletal and cardiac muscles. It has a genetic origin and definitive cure would possibly come with gene therapy, gene products or drugs altering the genetic defect. Due to a present lack of a cure or a definitive treatment option, the management has so far revolved around steroids and nutritional supplements, prevention of deformities / contractures, reducing breathing difficulties / chest infections, supportive care and therapy. The more recently evolving treatment options of newer drugs, stem cell therapy and gene therapy are discussed in the next section on recent advances. In this section, we are trying to bring to you in a simple and lucid way, a better understanding of how steroids need to be used, how physiotherapy, occupational therapy help in maintenance of muscle softness, how speech therapy and yoga therapy can help improve respiratory reserves. It also deals with something as basic as importance of diet for your child and patient, what you need to know about the psychosocial implications of this disorder. At the same time, more specific needs in advanced cases with muscle and bony contractures, bent / twisted spines and breathing difficulties have been discussed.

Medical Management

Drugs/Medications:

In the future, our hope is that a wide range of drugs will be available for the treatment of muscular dystrophy. But at present, the only drug option available for the musculoskeletal symptoms of muscular dystrophy are steroids.

Steroids

In muscular dystrophy (especially in DMD), steroids are currently the mainstay of the available medical treatment. Its use in India, however, is not very popular, though the

inclination to use steroids for short term has gained some weight. In other countries, such as the USA, Canada, Australia, etc. it has found an important place in the treatment regime for management Duchenne muscular dystrophy and Becker's muscular dystrophy patients. However, parents abroad are well informed about the choices, the effects and side effects, possible benefits and risks for their child. After understanding everything, they are then allowed to make a decision about whether they want to use it or not.

So, it is very natural, for parents of our DMD / BMD children as well as adult patients with MD, to wonder, whether they should choose to use steroids or not? The decision is more critical in childhood MD rather than adult MDs, since the aim is to slow down the deterioration. We would strongly recommend that parents and patients should make an informed choice about any therapy or treatment offered to them, since information is the best armament. Once you are equipped with the right information, you can ask the right questions to your doctors and then arrive at a proper decision for your child or yourself. Due to the importance and confusion about the use of steroids in many parents and patients minds, we are presently this chapter in a question-answer format.

Are there any studies in use of steroids in muscular dystrophy? What are their results?

Many research studies on use of steroids in DMD have been carried out. These (13 studies analyzed in the Cochrane review) have shown that they have a positive impact on walking and muscle strength. However, none of them have conclusively shown that walking can be prolonged. The other functionally important outcomes such as time taken to rise from the floor (Gower's sign), nine meters walking time and four stair climbing time was seen to have improved by an average of about 2 to 4 seconds by use of corticosteroids over a period of six months. Again, the impact of this in improving the quality of life and reducing associated problems has not be quantified. However, overall it is accepted that corticosteroids improve muscle power and bodily functions over short term (six months to two years). The long-term benefits and harms are not very clear.

Which are the steroids commonly used? How do they have to be taken?

1. The most commonly used steroid is **Prednisolone.** Prednisolone has a beneficial effect on muscle strength and functioning in boys with Duchenne muscular dystrophy and is offered as treatment, especially, in the ambulatory phase, when the child is still walking. Recommended dose for starting prednisone is 0.75mg / kg / day. That means in a child who weighs 20 kg, the dosage would be 15mg in a day.
2. Prednisolone is available as syrup or tablets. The commonest available brands are Wysolone (Wyeth), Deltacortil (Pfizer), etc. They are available in strength of 5mg, 10mg, 20mg. So, a dosage requirement of 15 mg / day can be given as 5mg three times a day. After consuming the medications, certain side effects seen for initial few hours are mood swings, hyperactivity, etc. If side effects require a decrease in prednisone, tapering to dosages as low as 0.3 mg / kg / day could still give significant

but less robust improvement.

Other regimes suggested to reduce the incidence of steroid-associated side-effects include alternate day dosing, lower dose daily regimes and intermittent regimes (eg. 10 days on / 10 days off; high dose on weekends). It is important to note that none of these regimes have been tested against the daily dosing schedules so that their relative efficacy in the long term is not known.

Ambulatory phase: For persons upto 40 kg, dosage increases with age. Maximum dose of prednisone is approximately 30mg / day.

Non ambulatory: For persons usually above 40kgs, long term steroid therapy is maintained. The prednisolone dose is often allowed to come down to 0.3 to 0.6 mg / kg / day.

3. **Deflazacort,** an alternative steroid, has been recommended for the treatment of Duchenne muscular dystrophy in countries where it is available. It is said to have similar, anti inflammatory and immunosuppressive effects, but with lesser severity of side effects. The starting dosage generally prescribed for deflazacort is 0.9 mg / kg / day. That means, in a child of 20kg, the dosage to start with would be 18mg / day (in 2-3 divided doses). In the ambulatory phase, maximum advised dose of deflazacort is 36 mg / day. In India, it is available as Asteride (Dr.Reddys'), Defnalone (Lupin), Cortimax (Zuventus), Deflazen (Torrent), Nestacort (Cipla). They are available in strength of 6mg, 24mg tablets (also available in syrup formulations).

What are the important points which need to be considered before and while taking steroids?

1. The correct time to start with steroid therapy is when the motor functions are at a plateau and when the motor skills have stopped improving but are not worsening either. Normally the right age is 4- 6 years as they gain their motor skills by this time.
2. All the required vaccinations should be taken.
3. Starting steroid treatment for boys or young men who are non ambulatory or have stopped walking should be decided by the physician.
4. Steroids should never be stopped suddenly and regular visits should be made to physician.
5. Before considering for any surgery, the surgeon should be informed about it since the steroid suppresses immunity, so chances of infection increase.

How is the dosage monitored or managed?

The recommended dosage protocol has been discussed earlier.

1. Maintenance dose has to be monitored and balanced based on growth, response and side-effects. This needs to be reviewed periodically.

2. Dose reduction of approximately ¼ to 1/3 is suggested if any unmanageable side effects occur.
3. Steroid therapy should not be abruptly stopped.
4. Tapering of dosage should be done gradually. First half of the regular dose should be taken for one week, followed by further reduction of the dose to 1/4th (in the 2nd week), 1/8th in the 3rd week and so on.
5. Steroids have to be taken after food, since it causes acidity or reflux.
6. It is usually should be accompanied by antacids syrups and proton pump inhibitor drugs such as Omeprazole (Tab Omez (Dr. Reddys) , Tab Ocid (Zydus)) The dosage is 20 mg once a day for adults and 1mg/kg/day for children in a single or twice a day dose). These however should be strictly taken only as advised by your physician.

What are the side effects?

Immediate side effects could be acidity or gastroesophageal reflux. Hence, steroids have to be taken after meals, with antacids or proton pump inhibitors.

Other commonly noted side effects are weight gain ,moon face, growth retardation, delayed puberty, behavioral changes, reduced immunity, high blood pressure, cataracts, osteoporosis, growth retardation, peptic ulcers, etc.

Of these, the most worrisome in children is growth retardation, weight gain and osteoporosis (increased brittleness of bone leading to high risk of fractures). These could be the major considerations for starting as well as continuing steroid therapy.

How and when do we take a decision to start steroids for our child?

Benefits and side effects of corticosteroid therapy need to be monitored. The decision of treatment with corticosteroids requires a balanced discussion of potential risks. Many issues including the ideal age/functional stage for initiation of treatment, the optimal glucocorticoid regime, and the age for discontinuation of glucocorticoid treatment are still being understood. Long term results on walking, respiratory and cardiac functions and quality of life need further investigation and more understanding to be developed. Hence, we would recommend that parents and patients should take the advice of their pediatrician or neurologist for starting steroids. Apart from the dosage, your doctor will also titrate/change the dose as the child grows, help adjust the dose or change the regime in case of side effects and overall monitor any changes.

Summary:

We realize that this section on steroids must have raised more questions in your mind than give you answers therefore we are making the following conclusions:

In summary, corticosteroid therapy in Duchenne muscular dystrophy improves muscle strength and function in the short term (six months to two years) but the long-term benefits are unclear. There are definite side effects of taking steroids.

We recommend that [1] You understand both the potential benefits and possible risks, [2] Discuss these with your pediatrician or neurologist. If your doctor in his / her experience recommends to you that steroids are beneficial then you should consider them. But if your doctor does not believe that in his / her experience steroids are beneficial then we suggest that you do not consider them.

You have to understand that whatever decision you make, there will be benefits and there will be adverse consequences. You will need to weigh both of these and see which fits in with your vision of your childs future and then in conjunction with your doctor choose one course of action.

Apart from steroids, popularly prescribed are additives or nutritional supplements such as vitamin E, coenzyme Q, omega 3 fatty acid, etc.. These moleculas have general protective effects, but their role specifically in muscular dystrophy is still not clear.The utility is not proven, but on the otherhand, neither are they harmful.

Vitamin E : Vitamin E is an antioxidant and anti inflammatory. It has been found to have protective effects in stress induced muscle injuries.Vitamin E deficiency has been understood to lead to a nutritional myopathy like condition, which is associated with increased creatine kinase levels.Rectification of deficiency ,leads to muscle protection and amelioration of muscular dystrophy.Hence, the rationale of using vitamin E in muscular dystrophy.

However, no conclusive evidence has yet been generated in studies carried out on muscular dystrophy subjects. A study in which, high dose alpha tocopherol (2000 IU / day) was administered to 33 patients of proven muscular dystrophy over 3 years. The changes or progression seen in these patients was not significantly different from the placebo group.Another study, in which the administration of selenium and vitamin E was tried in a group of 20 boys with muscular dystrophy. No boy showed any practically usable increase of muscle strength during the year of treatment.

Coenzyme Q : Coenzyme Q, again, is an antioxidant, which acts as a'scavenger' of oxidative free radicals. Free radicals cause damage to the muscle or any tissue at the cellular level. In certain studies, it has been found that Coenzyme Q improves physical performance of patients with muscular dystrophy and it has been recommended the supplementation to be continued indefinitely.It is especially recommended in muscular dystrophies associated with cardiac dysfunction.

Omega 3 fatty acid : Omega 3 fatty acids are long chain fatty acids, which have effect on strengthening the cell wall, reducing inflammation as well as antioxidant effect.Studies in experimental animals has shown that omega 3 fatty acids supports formation of new muscle cells to repair the injured muscle.However, evidence is still not clear on its protective effects in muscular dystrophy.

Orthopedic Management:

The primary goal is to prevent development of contractures / deformities and maintain walking for as long as possible. A regular program of physiotherapy and use of orthotics (calipers and splints) prevent joint contractures / deformities which can help in ambulation as well.

Once muscle contracture develops and walking becomes increasing difficult, soft tissue surgery is suggested to maintain the limb alignment and joint position. Surgery improves the walking balance & prolongs the ability of the child to walk.

Surgeries recommended for DMD, based on stage of the patient:

1. Before contracture begins (Early-Extensive ambulatory approach): Release / opening up of muscles of the hip, thigh and ankle.
2. When difficulty in walking starts due to tightness of muscles: Ankle and knee release. This is called Moderate ambulatory approach.
3. Correction of toe walking (equinnus gait)-Minimum Ambulatory approach
4. When the child ceases to walk: Aim of surgery is to re-establish walking. This is Rehabilitative approach.
5. The Palliative approach is the one in which the child is wheel-chair bound and surgery is only done for relief of pain, comfort, ease of nursing care & for shoe wear.

Different experts have different opinions about the timing of the surgery. But a general rule / advice is to perform the surgery within the window period of 3 - 6 months after the child stops walking, to help the child walk again. Early surgery at the onset of contractures can prolong ambulation / walking by 2-3 years more than patients that didn't undergo surgery.

Delayed operations after the child has stopped walking for more than a year will not establish ambulation. Also, obesity, osteoporosis and poor cardiovascular status are risk factors for surgery.

Ankle and Knee Surgery:

Ankle surgery comprises of percutaneous heel cord tenotomy, "Z" lengthening, and recession or transfer of tibialis posterior to correct dynamic varus. Fig 4.1 and 4.2

Knee surgery consists of hamstring lengthening, or tenotomy. Fig 4.3 and 4.4

Soft tissue surgery is required when knee deformity exceeds 20 degrees of flexion.

Hamstring lengthening and long leg bracing will be enough to correct this degree of deformity. Rarely a supracondylar osteotomy with rigid fixation is required.

Hip flexion release involves recession of the anterior hip muscles including the sartorius, rectus and tensor fasciae latae.

Post-operative rehabilitation should be aggressive. Weight bearing should start on the

Percutaneous release of hamstrings and calf muscles

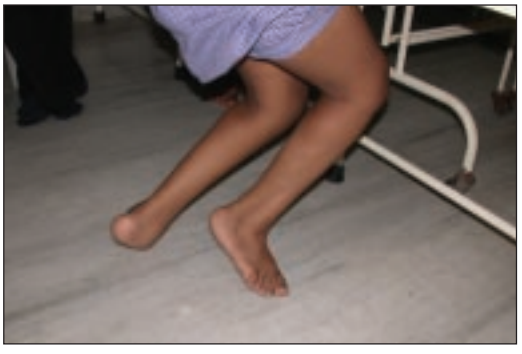


Fig 4.1: Pre-Op picture

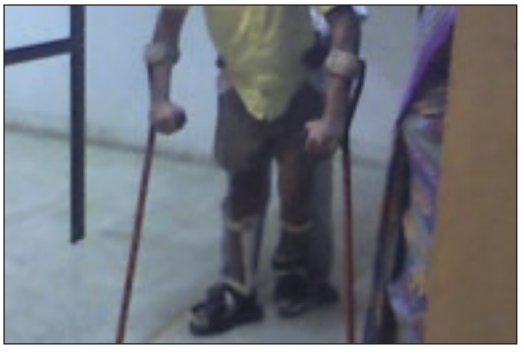


Fig 4.2: Post-Op picture

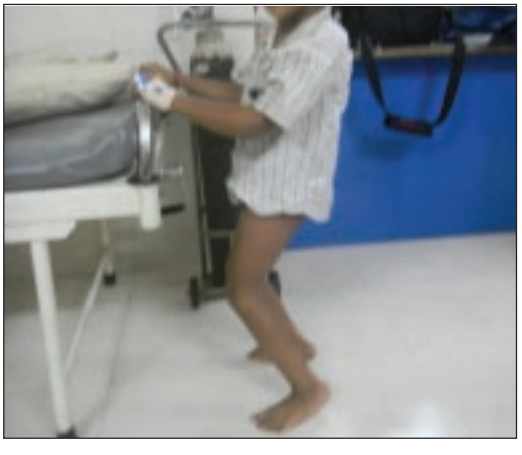


Fig 4.3: Pre-Op picture

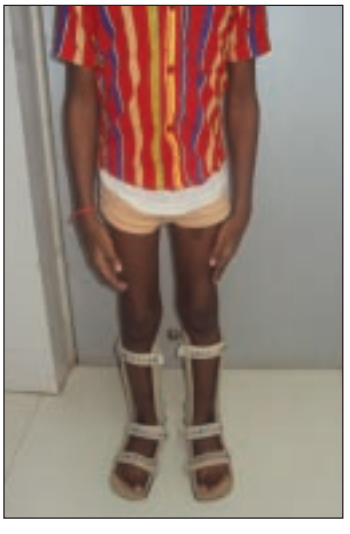


Fig 4.4: Post-Op picture

first post-operative day, gradually progressing to assisted walking as soon as possible. Any bed rest or immobilization enhances the muscle weakness and thus casting should be avoided as far as possible. Immediate fitting of orthosis /calipers/splints helps in early ambulation/walking, thus preserving muscle strength.

Spinal Surgery: (Fig 4.5, 4.6, 4.7, 4.8)

Almost all children with DMD will develop scoliosis once they are non-ambulatory. Thus preservation of walking ability is paramount. Spinal screening for scoliosis is mandatory for all children with DMD.

Spinal deformity is common in teenaged boys with Duchenne muscular dystrophy. Approximately 90% of boys with DMD will develop severe scoliosis. This, many a times, cannot be controlled by nonsurgical means such as bracing or adaptive seating. Surgical stabilization/operation for scoliosis is usually recommended when the curve is more than 20 degrees before pulmonary function worsens. A decreased chest function goes against the possibility of operation.

The typical curve pattern is a long sweeping thoraco-lumbar scoliosis (curve of the lower back), flexible and associated with pelvic obliquity (oblique angle of the hip).

Mild curves < 10 degrees should be watched closely and as the cobb angle exceeds 20-30 degrees, surgery can be considered.

Forced vital capacity/chest and lung capacity decreases by 4`% each year and by 4 % for each 10 degrees of curve. Thus progression of scoliosis and deterioration of pulmonary function go side by side making delayed surgery precarious and unsafe.

Specially designed screws and rods are use to fix the spine. This provides good stability allowing better correction of the spinal curve.

Surgery improves the seating ability of the child, preserves pulmonary function and prevents skin break down due to improper posture.

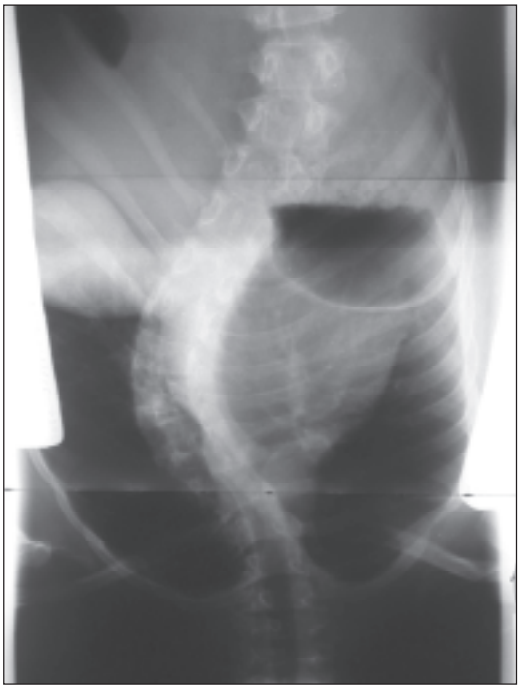


Fig 4.5: Pre-Op picture

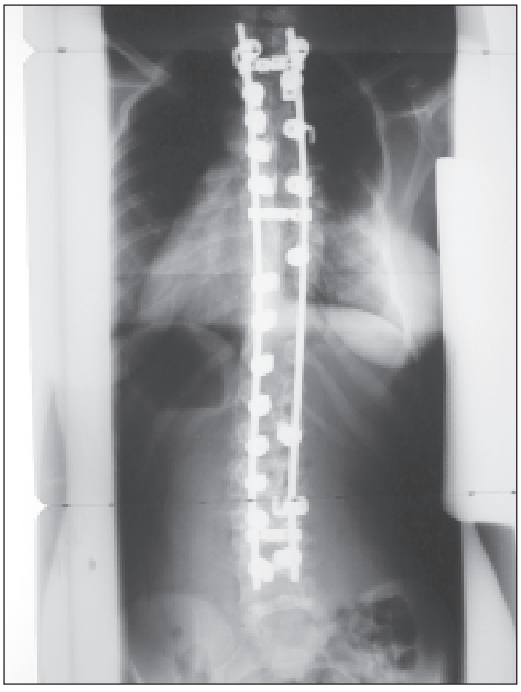


Fig 4.6: Post-Op picture

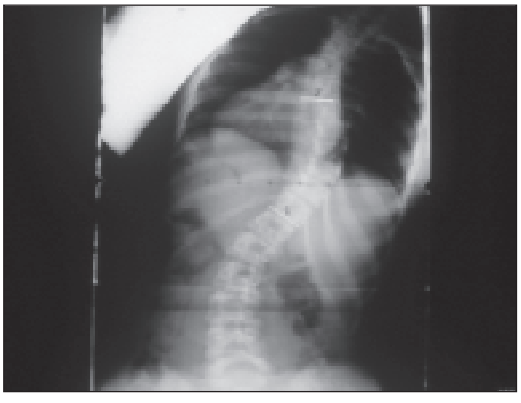


Fig 4.7: Pre-Op picture

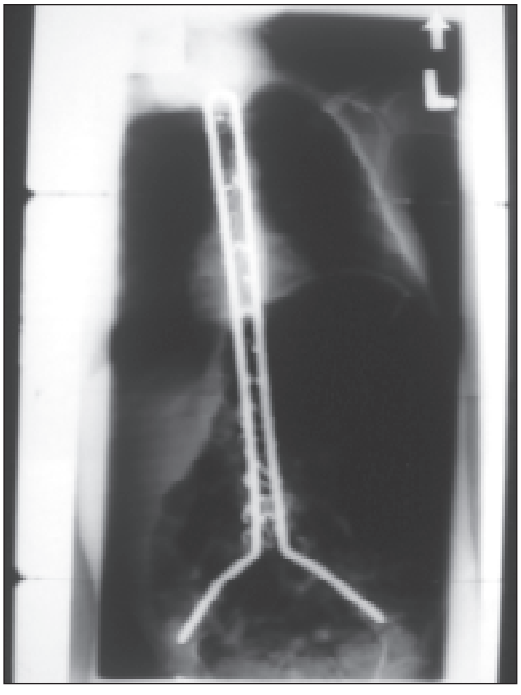


Fig 4.8: Post-Op picture

Respiratory/breathing problem management:

In the previous chapter 3, we have mentioned how to take care of the lungs / chest. But in this part, we have tried to explain how to manage the complications and problems of the chest. Children with Duchenne or Beckers muscular dystrophy usually encounter lung problems as the symptoms begin at a very early age and they become non ambulatory (stop walking) at a young age. In the later stages, they need ventilator support for breathing. In order to keep the respiratory muscles strong and prevent complications e.g. chest infection and respiratory distress, it is necessary to follow certain management regimes.

Management:

Prevention of chest complications can be achieved by the following measures:

- Maintaining good oral and dental hygiene.
- Encouraging proper positioning during sitting and sleeping to prevent spinal deformities like scoliosis and kyphosis.
- Taking regular vaccination by which preventable respiratory illness can be prevented like BCG, DPT, measles, hemophilus, pneumococcal diseases and yearly influenza vaccine.
- Being attentive and aware of other co-existing problems like asthma or any allergies as they target the lungs.
- Avoiding obesity as it becomes an additional strain on weak muscles. Many patients of muscular dystrophy are already at risk of obesity due to lack of movement and physical activity.
- Avoiding lying down especially after meals.
- Administering antibiotics if suffering from any flu or fever as it can lead to chest infection.
- Encouraging more physical activity if the person is able to walk, but without any exertion or fatigue.
- Carrying out deep breathing exercises, Incentive spirometer should be used to strenghthen chest muscles and nebulization if required, moving shoulder and upper trunk muscles, as they can facilitate chest movement.
- Fig of nebulizer: fig 4.9, 4.10
- Fig of Incentive spirometer: 4.11

Fig 4.9

Fig 4.10

Nebulization: A technique of administering medication by spraying it into the respiratory tract. Oxygen may or may not be used to assist in carrying the medication into the lungs. It can be done at home also with proper understanding of the apparatus.

Fig 4.11: Types of Incentive spirometer

Incentive spirometer: it is a small portable device which helps to keep the lungs clear. It helps to strengthen the chest muscles. It is important that wheelchair bound patients use spirometer to maintain their respiratory functions for as long as possible.

Treating chest infections by the following:

- Administering antibiotics under the prescription of physician
- Carrying out nebulization.
- Doing chest physiotherapy and coughing techniques to help keep lungs and airways clean.

Chest physiotherapy management:

In advanced stages of muscular dystrophy, the respiratory muscles becomes weak and breathing becomes laborious. Thus the lung volumes decreases. It needs systematic chest physiotherapy to manage these problems in different stages mentioned below :

- **Stage 1:** Normal respiratory function initially
- **Stage 2:** Weak cough but normal breathing
- **Stage 3:** Inadequate breathing during sleep but normal breathing during day time.
- **Stage 4:** Inadequate breathing during sleep and daytime.

Depending on the stage, the following management techniques can be used:

Stage 1 and stage 2: Normal respiratory function initially and weak cough but normal breathing.

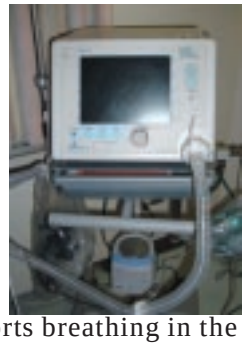
These symptoms can be managed as mentioned in prevention of chest complications.

Stage 3: Inadequate breathing during sleep but normal breathing during day time.

This stage is characterized by fatigue, disturbed sleep and headache in the morning.

The following can be done :

- Pulse-oxmeter can be used by the care-giver at night to monitor oxygen saturation in blood.
- A visit to the chest specialist is suggested.
- **BiPAP** (Bi-level positive airway pressure) Fig 4.12 and Fig 4.13, for breathing assistance can be used if advised.



1. Supports breathing in the night.

2. No surgical procedure is required, only nasal mask or a face mask is used

3. Cannot be used as a 24-hour support as it is uncomfortable.

Stage 4: Inadequate breathing during sleep and daytime :

This stage occurs when the condition becomes more critical, when breathing muscles have become very weak or the patient has been affected with any chest infection like pneumonia. At this stage, respiratory tests show severe abnormalities.

The following measures can be done :

1. Non surgical breathing support:

- Ventilator with mouth piece which can be transferred easily. It can also be attached to the wheelchair for day time support or may be a 24-hour support.
- Among the many ventilators available some special ones worth mentioning are the Pulmonetic -LTV-950 ventilators fig 4.14: It is light-weight and comes with a 1 hour internal battery and a 4-10 hour external battery.
- Ventilator with mouthpiece is ideal for patients who require it during day time and can manage without it for some time. It helps to improve energy levels.

2. Surgical intervention for breathing support:

This is required when the patient cannot breathe adequately or cannot be put on the above mentioned non surgical / non invasive devices.

- During severe complication, patient may require hospitalization in the ICU (Intensive Care Unit).
- When breathing difficulties increase, another surgical option that can help to reduce difficulty is tracheostomy fig 4.15 and fig. 4.16.
- Sometimes, the patient can be weaned off the ventilator (that means ventilator support can be removed) during hospital stay .This means that they can be managed without continuous ventilator support at home or with above mentioned devices or may have to be on 24 hours ventilator fig 4.17 support at home, but they can still lead a normal life.
- Among the many ventilators that can be used at home for long term ventilation, a workhorse effective yet very inexpensive ventilator is the Newmon ventilator (Zephyr industries) that can be used 24/7 in any home for over one year. It had a 3 hours battery life and can be connected to car battery also. It needs hardly any maintenance. fig 4.18, 4.19.

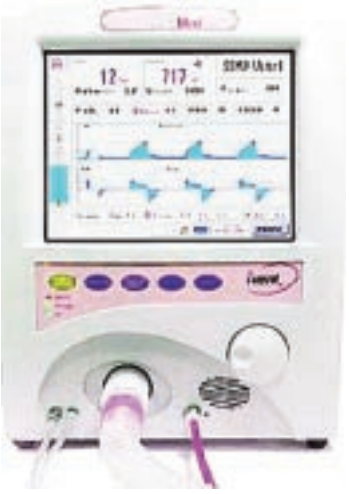


Fig 4.14: Pulmonetic -LTV-950 ventilators

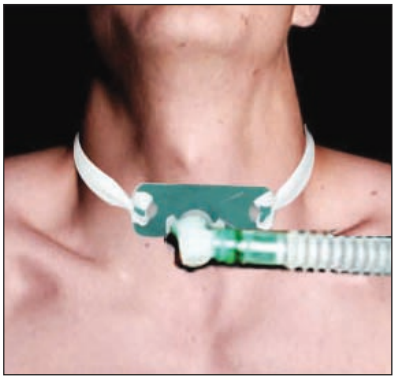


Fig 4.15

Fig 4.16

Tracheostomy: In this surgical procedure, a small hole is made below the neck, which is covered with a mesh and is connected directly to the windpipe.

Before choosing this technique all the possible outcomes should be discussed with the surgeon. It can help in suctioning i.e. removal of cough with the help of tube (done by trained professional or can be done by parents with proper understanding of it).

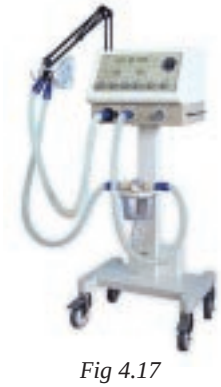


Fig 4.17

Volume ventilator fig 4.17 is a device, which can be used during the night time because it is bigger than IPPB. This device reduces difficulty in breathing when the child is sleeping. Facemask or nasal mask is connected to airway. These devices can be used as and when needed, to improve ventilation.

Example of ventilator management at home

Fig. 4.17

Fig. 4.18

Cardiac / Heart problem management

Cardiomyopathy is a major complication and can be life threatening in muscular dystrophy, especially, Duchenne and Becker's muscular dystrophy as mentioned in the chapter 3.Hence, parents should be alert and aware of possibilities of these problems.

Prevention of cardiomyopathy:

- After the confirmation of the diagnosis, all the cardiac function tests should be done like ECG (electrocardiogram) and echocardiogram (2D echo).
- Cardiac evaluation i.e. check up of the heart should be done every 2 years till the child turns 10. After the age of 10, it should be repeated every year and if any tests show abnormalities, required drugs should be prescribed by a cardiologist.

Management of cardiomyopathy:

- Angiotensin converting enzyme (ACE) inhibitors(eg.Losartan.) could be administered as the first line of treatment even when the cardiac tests are normal.
- Beta adrenergic blocking drugs(Carvedilol) could be used to prevent cardiac failure.
- ACE inhibitors and Beta blockers could be taken together, especially in children with cardiomyopathy.
- Loop diuretics (drugs to cause loss of fluid through urination)could be prescribed when there is retention of fluid.
- Anticoagulation(blood thinners) may be given to prevent systemic thromboembolism(or clotting of blood inside the veins, which may lead to death)

Management of cardiac problems, which includes prevention of or delaying of cardiac complications, has to be done under a cardiologist. But, what parents can do is to have their child / patient checked for cardiac function atleast every year.

Rehabilitation in Muscular Dystrophy:

Physiotherapy and Occupational therapy :

A physiotherapist and occupational therapist play a very important role in management of muscular dystrophy. They are an integral part of the team of health care professionals who take care of you or your child. Infact, they ought to become a defacto member of your family.

What is physiotherapy?

Physiotherapy is a system of treatment which includes preventive measures and restorative measures. Preventive measures include prevention of complications like contractures, pain and sores. Restoration includes functional improvement like ambulation (walking) or wheel chair life by physical modalities such as therapeutic exercises, gait (walking) training with / without appliances such as calipers, walkers, crutches etc.

Who is a physiotherapist?

A physiotherapist is a trained professional with adequate knowledge about the disease, complications and its management in different stages (role discussed in detail later in the chapter).

Who is an occupational therapist?

An occupational therapist is one of the members of the multidisciplinary team involved in caring of the persons of muscular dystrophy. Occupational therapy deals with increasing health and well-being through daily activities. Daily activities are those that people do in their everyday life, such as personal-care tasks, like eating or bathing. It also includes other day to day activities, like housework, play, schoolwork and employment tasks. This helps the individual with muscular dystrophy to engage in his / her activities of daily living (self-feeding, self-care activities, etc.) and leisure activities at the most independent level possible, to improve their quality of life. Occupational therapy may also involve modifying a person's environment, both at home, school or work, to increase the individual's function and accessibility.

The main focus of occupational therapy is on maximizing or maintaining skills and increasing independence by the use of special equipment or use of energy saving techniques.Occupational therapists help in supporting individuals suffering from any type of muscular dystrophy & their families by also looking after their psychological, social and employment needs.

Physiotherapists and occupational therapists together form a big part of your rehabilitation team.

Aims of rehabilitation:

- To educate the patients, parents / caregivers regarding the disease and its outcome. Also, to motivate them to overcome complications and improve their quality of life.

- To plan an appropriate treatment program in co-ordination with other professionals of the rehabilitation team like counselor, orthotist, prosthetist, cardiologist, pulmonologist, speech therapist, neurologist, orthopedic surgeon etc.
- To assess muscle strength, joint mobility, deformities, walking patterns, etc.
- To determine the causes of deformities.
- To assess and correct walking patterns.
- To assess speech ability.
- To conduct cognitive assessment which includes an assessment of intellectual level.

Goals of Treatment program:

- To strengthen all the weak muscles but without fatigue.
- To prevent contractures and deformities.
- To decrease tightness of muscles by stretching them gently.
- To identify the need of adaptive devices like orthosis, calipers, wheelchair, etc. techniques and prescribe to the patient.
- To exercise and improve breathing with breathing exercises, postural drainage and clearing secretions.
- To help maintain good posture.
- To maintain independent walking as long as possible.
- To encourage daily exercises.
- To prevent frequent falls, fractures, pressure sores and stiff joints.

Stage wise rehabilitation management:

Stage 1: Walking is affected and breathing is not affected.

What happens to the affected individual?

- Gets up from the floor using hands on thigh and pushing to get up.
- Climbs stairs very slowly.
- Walks like a duck.
- Is slow in activities as compared to peers.
- Falls often during running.
- Gets tired after long duration of activities.

Goals:

- To prolong independent walking as long as possible.
- To maintain muscle strength.

- To prevent frequent falls and fractures.
- To prevent bed rest for long period of time.
- To continue exercises (shown in chapter 5).

Stage 2: Patient is barely able to walk & has difficulty breathing in the night.

What happens to the affected individual?

- Getting up from floor / low surface is difficult or impossible.
- Climbing stairs is difficult or impossible.
- Falls frequently.
- Walks on toes.
- Overhead activities like combing hair, wearing clothes, washing hair becomes difficult and fatigues (gets tired) easily.
- Breathing becomes difficult at night.
- Gets tired easily.

Goals:

- To prevent any falls or fractures.
- To maintain walking with long leg calipers.
- To maintain muscle length.
- To prevent any chest infection.
- To conserve energy.
- To continue daily activities using special equipments like use of wheelchair for covering long distances for saving energy.

Stage 3: Walking is impossible and breathing difficult throughout the day:

What happens to the affected individual?

- Overhead activities are impossible but some activities like eating, grooming etc. are possible.
- Getting up from lying position is difficult.
- Muscles become tight due to lack of movement.
- Back bone starts twisting (scoliosis).
- Breathing becomes difficult during day time also.

Goals:

- To prevent deterioration of muscle by exercising.

- To prevent contractures and deformities.
- Deep breathing exercises and use of incentive spirometer for improving chest volume.
- To prevent scoliosis and if it develops, prescription of spinal jacket or spinal support.
- To assess requirement of assistive breathing such as BiPap.
- To continue doing daily activities using special equipments like assistive devices mentioned in chapter 6.

Stage 4: Patient is bed-ridden

What happens to the affected individual?

- Contractures or fixed deformities of the foot, elbow, hip, knee, spine and wrist develop.
- Is completely dependent on caregivers.
- Is dependent on assistive devices like BiPap or ventilator for breathing.
- Talking and swallowing with difficulty.
- Chest infections occur frequently.

Goals:

- To continue exercises to maintain muscle strength.
- To prevent worsening of the contractures and deformities.
- Deep breathing exercises and use of incentive spirometer for improving chest volume.
- Prescription of spinal jacket to improve posture and help in breathing.
- To assess requirement of assistive breathing such as BiPap.
- To continue doing daily activities using special equipment.
- To ensure proper to prevent pressure sores.
- To clear the chest of secretions.

Psychological management

Consideration of psychological aspects of the child is important to be taken into perspective, since it has an impact on the overall physical wellbeing of the child too. Cognitive and behavioral changes that happen need to be understood and handled accordingly.

What are the cognitive and behavioural changes or problems associated with muscular dystrophy at different ages?

Childhood:

Cognitive Changes:

During this period, the parents may notice language developmental issues like the child may have difficulty in expressing his / her needs or may have difficulty in remembering a lot of information. This is especially seen in boys with Duchenne's muscular dystrophy because of which they are usually diagnosed as having learning disability especially dyslexia. Children with Duchenne muscular dystrophy may have problems with maintaining attention or concentrating, however these issues may be overlooked by their parents due to their physical condition.

Behavioural Changes:

Usually children at this age display problematic behaviour but children with muscular dystrophy display more impulsive behaviour and poor emotional control. Many of these children are on steroids, which usually have side effects that affect their behaviour. Some side-effects seen are that these children are more rigid, emotionally low and may not be cooperative. Eventually as their condition progresses, boys aged 8 to 10 years start having difficulty in adjusting as they stop walking and start using the wheelchair.

Teenage:

Cognitive Changes:

When young men with muscular dystrophy grow older their cognitive problems increase like difficulty in planning, organizing or completing tasks. As their educational level or work increases, their problems also increase.

Behavioural Changes:

Teenage or adolescence is normally known to be a difficult age, as at this point of time there are many hormonal and emotional changes in the body. Along with these, there could also be a possibility of having a limitation in the physical development. This may be a result of certain medical treatment for example: an individual may have small height or may have delayed puberty which could be an effect of puberty. This could make dealing with the changes of adolescence more difficult. Teenagers could have a difficult time adjusting as they might not get the freedom or independence since their condition will require more care and assistance as compared to others. As muscle weakness progresses, they are at risk of becoming more isolated or socially withdrawn.

Parents should look for signs of chronic sadness, depression or anxiety.

Adults:

Cognitive Changes:

Adults with muscular dystrophy may adjust with their cognitive issues and may find out ways of dealing with them.

Behavioural Changes:

As individuals with muscular dystrophy progress from teenage to adult hood, they may undergo mood changes and emotional changes which would eventually lead to changes in their behaviour. Adults with Duchenne muscular dystrophy would be bed ridden and this would result in loss of independence. This would eventually lead to increased anger, irritability & frustration and may even show signs of depression.

Whereas, adults with limb girdle muscular dystrophy or Becker's muscular dystrophy would develop difficulty in functioning, which could lead to decreased socialization, low self esteem & self image. Also they would have multiple questions as to whether they should get married or if married, whether they should have children.

What is the importance of psychological assessment in muscular dystrophy?

A person with muscular dystrophy is at an increased risk of cognitive and emotional problems; hence early diagnosis and treatment is of great importance.

- Cognitive Assessments: Especially a DMD child with learning disabilities or autism should undergo testing for IQ, memory, attention span, problem solving, etc.
- Emotional and Behavioural Problems: A patient with muscular dystrophy should go to a psychologist for emotional status examination either every 6 months or annually. As many of the patients go into depression or suffer anxiety disorders, which may in turn worsen their physical condition.

Learning and Cognitive Skills in Muscular Dystrophy:

Intelligence is defined as "an individual's ability to adapt and constructively solve problems in the environment" as mentioned by David Wechsler, a well known American psychologist.

Intelligence can be assessed by the means of an intelligence test. There are many intelligence tests available but an accurate IQ can be gained when the IQ test is for the Indian population, for e.g. Malin's Intelligence Scale for Indian Children (MISIC). This IQ test usually has 2 parts i.e. verbal sub-test and performance sub-test.

The verbal subtest includes questions regarding language, for e.g. how are the piano and guitar similar. Whereas performance sub-test assesses visual thinking and motor performance, for e.g. a subtest on block design, where you have to copy a block design. The time taken to conduct an IQ test is 30 minutes to 2 hours.

It is often seen that boys with Duchenne muscular dystrophy usually are at a higher

risk of delays in walking, running and sitting. In a similar way, it is seen that the IQ of these children ranges from above average to below average. However, they are at an increased risk for having low IQ or some learning disability.

What is the cause of cognitive weakness?

Most probably there is a relation to brain functions. It is known that dystrophin is normally present in other tissues as well as muscle tissue, including the central nervous system. The lack of brain dystrophin might therefore play a role in the cognitive functioning of the boys. The dystrophin isoforms Dp427 - C and Dp427 - P would normally be distributed to the hippocampus and purkinje cells in the cerebellum respectively, contributing to greater post-synaptic density. The cerebellum and hippocampus are part of an integrated network that includes connections with the frontal brain regions. This altered postsynaptic plasticity may hinder efficient memory, automation and planning / organization.

Areas of Cognitive Weakness:

- Difficulty in finding words.
- Difficulty in short term memory.
- Difficulty in concentrating.
- Difficulty in switching from one activity to another.
- Difficulty in completing tasks.
- Difficulty in multi-tasking i.e. performing multiple tasks at the same time.

Brain Areas in which Dystrophin has been found:

Brain Area	Function of the Area
Hippocampus	Memory
Cerebellum	Automatization
Frontal Lobe	Planning & Organization

Research is still under process about the role of dystrophin in the brain.

Attention, Listening and Memory:

It is seen that patients with muscular dystrophy have problems remembering many things or assimilating information together, have difficulty in following directions or may not seem to listen. Also, there is a high chance for problems with the ability to focus on a task and with dividing the attention between many activities at a time. Their level of distraction may be high. Individuals with high IQ could also have difficulties dividing their attention.

Strategies to Improve attention, listening and memory problems:

- Sit close to the child and explain to him about the task or the problem to be solved to avoid him getting distracted.
- Break down the instructions and information into simple and specific statements.
- Check if the child has understood what he has been asked to do.
- If the child has difficulty dividing attention between many things at a time then give him one activity to complete at a time this would also help eliminate stress.
- If the child has an attention problem or has reading difficulty, then underline important points so that he does not miss out on important information.
- While teaching the child, use small time durations i.e. instead of a 1 hour long period use short 20 minute periods.
- Either make a to-do list or let your child himself make a list of activities which will help him remember the activities that he is supposed to complete.
- Arrange for extra time to be given to the child for him to complete an examination, as his physical condition might be a barrier or he might have concentration / memory problems.
- If the child is not able to write his examination because of weakness in his hand, arrange for someone else to write the exam for him.

Psychosocial Adjustment to Muscular Dystrophy:

Psychosocial adjustment means the way in which one adjusts to difficult and stressful events associated with muscular dystrophy.

Psychosocial Adjustment has six areas of functioning:

1. Relationship with friends.
2. Dependence on family members / caregivers.
3. Opposition to things or situation around.
4. Productivity.
5. Anxiety or depression.
6. Withdrawal.

Undergoing emotional and behavioral problems is a normal and a healthy way of socio-emotional development. However, research has shown that individuals with physical disability have an increased risk of developing behavioral and emotional problems. This is usually one of the reasons why individuals with muscular dystrophy adapt to the consequences of behavioral and emotional problems. It is usually seen that individuals with muscular dystrophy become more capable of coping with their disability.

Learned Helplessness in Individuals with muscular dystrophy:

Learned Helplessness is when an individual thinks and feels that he or she has no

control over the situation or whatever is happening to him or her. Learned helplessness can lead to many emotional reactions and behavioral problems.

For example:

Negative Chain: An individual with muscular dystrophy who is regularly undergoing physiotherapy, despite which he is deteriorating and is wheelchair bound feels sad and disappointed because he cannot walk independently.

Positive Chain: An individual with muscular dystrophy who can move around in his wheelchair feels that he is self-reliant and independent. He is still happy about the things that he can do by himself.

How to deal with emotional problems?

Everybody undergoes emotional problems but individuals with muscular dystrophy undergo a lot of stress and emotional changes. These changes are related to the different stressful situations arising because of their disease like hospitalization, being wheelchair bound, dependency, etc. Also when individuals take steroids, it could have side effects like mood swings and frequent changes in emotions.

Stages of acceptance:

Phase 1: Denial: "No not me"

Phase 2: Anger: "Why me, it's not fair"

Phase 3: Bargaining: "Yes me, but I still want to"

Phase 4: Depression: "I'm so sad, why bother with anything"

Phase 5: Acceptance: "I am going to have a worthwhile life"

Strategies to handle emotional problems:

- The first step to handling the emotional problems is to understand or give a name to the emotion that he or she is experiencing, for e.g. whether the person is experiencing anger, sadness or fear.
- It is important to make the individual feel that he is being heard. Hence, he should have support in the form of a good listener.
- It is very important to teach the person to express his emotions in a constructive manner.
- The individual could maintain a personal diary where he writes the thoughts & emotions that he is experiencing.
- If an individual with muscular dystrophy is having frequent mood changes which are having negative effects on his health and routine, he should consult a psychologist.

Temper Tantrums:

Temper tantrums are normal issues associated with the process of growing up. The

issue of temper tantrums is usually seen in boys with Duchenne's muscular dystrophy. This is their way of drawing attention to themselves, be it positive or negative attention. Dealing with temper tantrums becomes a tricky situation. If you shout at the child for throwing a tantrum, it may lead to an increase in the tantrums, whereas if you show the child that you are okay with the tantrums then it would lead to encouragement of such a behaviour.

Strategies to handle temper tantrums:

- The best way to deal with this problem is to ignore when the child is throwing temper tantrums. However this technique cannot be always used.
- Time out: This does not mean punishing the child; it means that we are allowing the child to calm down.

For e.g.: we make the child sit in the corner of the room where there is no possible means of entertainment and where the child feels bored. He should be made to sit there for at least 5 to 10 minutes and he should be asked to think why he was given timeout. This would help him avoid being given a timeout in the future.

However, when temper tantrums worsen or may be out of your control then you should consider consulting a child psychologist.

When to consult a Psychologist?

- You feel helpless in dealing with the temper tantrums.
- It leads to stress and negative feelings within the family.
- You are not comfortable with the response after the temper tantrums.
- The child causes some sort of harm to himself.

Coping with Duchenne muscular dystrophy:

Usually it is seen that persons with muscular dystrophy accept their disorder and adapt to it quite well. However, many a times, due to stressful situations or inability to function socially or physically, they may undergo sadness, anger, frustration or guilt.

Strategies to Maximize Coping Skills:

- Be available and open to talk to the affected person.
- Try to identify the problems that the person is undergoing, which are stopping him from functioning up to his maximum potential.
- Allow the individual to be as independent as possible.

Quality of Life:

Quality of life is more important than the quantity of life. This is also true for individuals with muscular dystrophy as they have a whole life with possibilities ahead of them. Hence, it is important to understand that rather than aiming for the quantity of life we should aim to improve the quality of life.

Strategies to improve quality of life:

- Take each day as it comes rather than planning for the future.
- Help the individual find happiness in small things.
- Don't make them feel different or disabled. Treat the individual in the same way as others.
- Safety is very important, but that does not equate to binding him within the house. In fact, he should be given the opportunity to experience situations and activities like other people.
- Help him socialize and keep in touch with other people. Socializing is very important for his well-being.
- Help the individual develop a hobby which keeps him engaged and helps vent out his feelings and emotions.
- Help him develop a positive self-image and self-esteem.
- Let him be as independent as possible.
- Like other people go out and have vacations or small trips, even individuals with muscular dystrophy should be able to take short trips. Make proper arrangements keeping in mind their needs for e.g., a wheelchair accessible hotel.

Strategies for Caregivers:

Ways to manage aggressive & difficult behaviour:

- Develop a weekly routine for the patient, explain it to him and help him stick to it. Incorporate rewards for tasks well achieved, as children are usually noncompliant after exercising every day.
- Incorporate recreational activities in the time table as it is very important to have something to look forward to during the day. This helps to motivate the patient and does not let him get depressed about his condition. For example: Painting or playing board games with siblings or watching television.
- Explain the situation to the patient, if there are any changes made or if he is taken for some therapy that he denies to undergo, for example: If the patient does not want to exercise on a particular day, explain to him the situation, how doing so would have repercussions in the long run and set up a reward system if necessary.
- Try to keep calm when a child is misbehaving. Angry parents and teachers tend to escalate the situation. This would worsen the situation as the child would feel neglected and feel that nobody understands him.
- Focus on the positives: Strategies that only focus on punishment do not promote positive behaviours. Increase motivation, or change attitudes. Rewarding / praising / encouraging good behaviour is more effective in the long run. Look for opportunities to say "yes" instead of "no." ("Yes, you can have a cookie, after you…").

Psychotherapy and drug interventions:

- Well-known techniques exist which help in various areas. These include training for parents to cope with bad behaviour and conflicts, individual or family therapy and behavioural interventions. Applied behaviour analysis may help with certain behavioural issues related to autism.
- Some children & adults may benefit from the use of prescribed medicines which help with emotional or behavioural problems. These medicines can be used under specialized supervision and monitoring for depression, aggression, obsessive compulsive disorder (OCD) or attention deficit hyperactivity disorder (ADHD), when these problems have been specifically diagnosed by a doctor.

Speech Therapy

Parents of DMD/BMD children have noticed that as their age increases and the muscle deterioration progresses, speech also seems to get affected. This in most cases is due to hypertrophy of the tongue muscles. Slowly, swallowing and respiratory problems also ensues. Hence, it is important to identify speech, language, voice, swallowing and respiratory difficulties at the earliest, so that timely intervention can be done.

Attention should be paid to the following points and the level of difficulty should be noted:

- Did the child cry immediately after birth.
- Did neck holding, turning, sitting, crawling, standing with support, walking, saying /ma/, /pa/, /ba/, /a/, /e/, /u/ and uttering meaningful first words occur as per age or was there any delay.
- Does the child have poor attention, thinking, reasoning [inquisitiveness].
- Is there any difficulty in moving the tongue adequately within the oral cavity, rolling on lips, touching palate, flapping to say /r/, lifting posterior to say /k/ or coughing, swallowing. Does the tongue appear thick and protrude out.
- Is there any difficulty in puffing cheeks, blowing, sucking, chewing, and biting?
- Do the lips remain wet always & need to be wiped periodically.
- Is breathing from the nose more laborious? Are there complaints of lack of stamina and breathlessness?
- Is there any difficulty in lifting things?
- Is there a voice change, more towards a nasal tone?
- Does the face lack expressions and appear mask like.
- Does the child complain about reading, writing [errors in spelling & identifying letters] and complex language abilities like creating a detailed story on any topic etc?
- Is the child's speech difficult for a listener to understand?

Here are some simple exercises that can be done at home to reduce above mentioned difficulties in muscular dystrophy patient's adult or child:

Oral motor exercises:

- For poor lip closure:
    1. Hold upper and lower lips together with little pressure and repeat 10 times in a day.
    2. Ask the child to hold an ice-cream stick, whistle or spoon between the lips.
    3. Ask him to close lips together by himself by clenching tongs in lips. [Tongs used for chapatti, papad]

- For inadequate tongue movements:
    1. Protrude tongue and elevate
    2. Move from left to right.
    3. Roll on lips, on teeth, palate, cheeks from inside.
    4. Try to lick candies, lollipops, and ice cream.
    5. Try to remove food particle [dough, chocolate] from the palate with force.
    6. Do back and forth movement of tongue on palate.
    7. Try to cough as an exercise, say /k/ as an exercise.
    8. Say /pa/, /ta/, /ka/ as fast as possible maintaining clarity, speech, loudness.
- For inadequate sucking:
    1. Try to suck liquids from spoon.
    2. Try to suck on lollipops, ice, tangy candies etc.
    3. Suck on gauze piece dipped in honey, chocolate sauce etc.
- For inadequate blowing:
    1. Blow whistles, soap bubbles, thermocol balls, bits of paper, candles etc.
    2. Use respirometer [instrument to reinforce blowing & sucking].
- For breathing problem and unclear speech:
    1. Do deep breathing exercises. Inhale as much as possible from nose & exhale through mouth. This will help to relax neck, shoulder muscles.
    2. Do deep breathing followed by phonation of /a/, /e/, /u/ as much as possible.
    3. Speak slowly by prolonging the initial syllable post deep inhalation.
    4. Exhale after every word. Ex. Count 1 to 10 slowly exhaling after every count.
    5. Practice omkar after deep inhalation. This will help to maintain good coordination between deep breathing, exhaling and speaking and reduce effort while speaking.
    6. In advanced stages of muscular dystrophy where breathing difficulties increase, another surgical option that can help reduce difficulty is tracheostomy. Here a small hole is made below the neck, which is covered with a mesh and is connected directly to the windpipe. Take proper consultation before choosing this option.
    7. Mechanical Aids to help breathing: These devices are simple and portable, which give better ventilation to Duchenne muscular dystrophy children. One of the methods is called intermittent positive pressure breathing [IPPB]. This requires a mouthpiece, which is connected to airway. If facial muscles are weak then facemask can also be used. This improves breathing during the day as it can be easily placed under the wheelchair. But if introduced early

then respiratory functions are preserved better. Volume ventilator is another device, which can be used during nighttime because it is bigger than IPPB. This device reduces the difficulty in breathing when the child is sleeping. Facemask or nasal mask is connected to the airway. These devices can be used as and when needed to improve ventilation.

- For difficulty in swallowing:
  1. Reduce the size of the bolus.
  2. Masticate slowly.
  3. Push behind masticated bolus towards pharynx [throat] slowly.
  4. Drink liquids slowly sipping and then pushing behind. Try chin tucking to collar bone while swallowing. This will close airway passage and reduce aspirations.
  5. If coughing starts, consult a speech language pathologist immediately.
- For delay in speech since childhood:
  1. Talk as much as possible with the child so that the receptive vocabulary increases.
  2. Use lots of pictures & actual objects to teach the child.
  3. Talk slowly using open mouth approach at the level of child's eyes.
  4. Speak loudly and let child observe your lip movements and tongue movements.
  5. Encourage child's every attempt to communicate verbally.
  6. Make it mandatory for child to use sounds to ask for his needs. Let him say /a/, /e/, /u/, pa/, /ma/, /ba/ etc whatever possible.
  7. Consult a speech language pathologist for detailed child oriented goal plan to improve his speech and language.
- For difficulty in hearing:
  1. Consult an audiologist.
- For reduced expressions/ smile:
  1. Massage the cheeks in circular motion and consult a speech language pathologist [speech therapist].
  2. Stretch the lips and hold smile.
  3. Round the lip and hold.
- Other problems experienced, especially in Duchenne muscular dystrophy:
  1. Poor short term verbal memory: Inability to retain new information in brain at a time. For this, try to use lots of pictures to introduce new information. Chunk it into small segments, so that remembering becomes easy. Try to associate it with the known information. eg. For remembering new address: use lots of pictures for landmarks.

2. **Complex language processing might be difficult:** This problem is not noticed easily as the child's day to day communication is not affected. But detailed description of any event or abstract reasoning might be difficult. This can be improved by giving topics to the child to construct 10 sentences. eg. Ask the child to do mind mapping. This increases thinking and reasoning abilities.Fig.4.19

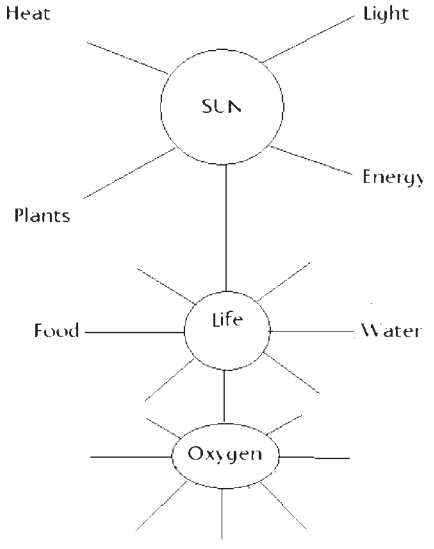


Fig. 4.19: Mind mapping

Mind mapping : Look at the illustration with a flow chart. One word is given to the child, say sun (centre) and it has five arrows where the child has to fill in words associated to sun e.g. solar energy, heat, plants need sunlight, sun is required to survive i.e. life. Again five words related to life; child has to mention words like oxygen, food, water, etc. This will improve association, reasoning and vocabulary as well. This also helps in improving memory and retention.

3. **Difficulties might occur in phonological processing:** The child needs to recognize every letter and needs to associate it to its sound. This is done by the brain. The child may find it confusing to remember letter and sound. This problem again supports the problem with short-term verbal memory. Do not scold the child for making mistakes; rather give more practice with words along with pictures and letters. If not rehearsed regularly, the child would have problem. Practice sight words so that reading becomes easy. Consult a speech language pathologist for further detailed child oriented therapy.

   Multi sensory approach: This involves integration of auditory, visual, tactile and kinesthetic approach. For eg: while teaching the child /p/ and /b/, the major difference between the two sounds is understood by listening because visually both look the same. Similarly for teaching /ka/ child can be explained it's the sound which can be produced by coughing hence child remembers the association with the action of coughing. When asked why is he coughing he hears the sound /ka/ once again and remembers it. Child will also know he has to produce /ka/ by lifting tongue from back and touching it to the roof of mouth. Kinesthetics means actually feeling the movement of tongue while producing the sound, like feeling how the tongue touches the roof of the mouth to produce /ka/ and with how much force tongue is pulled down back while saying /ka/.

4. **Difficulties doing mathematics [Dyscalculia]:** Remembering signs, relative values [greater than, lesser than], abstract or symbolic concepts like ten's place, hundred's

place, money, fraction, etc. is difficult. Memorization of tables, automatic calculations are difficult. For this, give plenty of practical examples for additions, subtractions, greater than lesser than. Multiplications and division exercises need to be understood by the child first.

Ex. 2x2=4 [here explain 2 comes 2 times i.e. twice]

2+2=4 [here 2 are added i.e. the original value is increased by 2]

2x3=6 [here 2 comes thrice 2+2+2, in multiplication the second value is the number of times the first value is added].

2+3=5 [here 3 is added in first number i.e. 2, in addition, the second value itself is added in original value].

2-2=0 [here again second value is decreased from first value. Remember always that the second value should be lesser than first value].

4/2= 2 [Ask the child to make 4 balls and then ask him to make two parts ensuring both the parts have equal balls. This way, explain the concept of division.

Do not go by the regular methods used in school because abstract thinking is difficult for children with dyscalculia. Learning tables will also be easier with the method mentioned above. Even though it is lengthier, it is more practical.

Most of the reading, writing and mathematical errors go unnoticed because of more enlarged problem of muscle weakness.

5. **Motor planning & programming may also be affected:** This is different from muscle weakness. Because of planning and programming errors, the child appears clumsy, uncoordinated with poor eye hand coordination. For eg. Picking up a matchbox, taking out the matchstick and lighting it. This task needs signals from brain to hold the target, which is received by eyes and hands. Then the concerned body parts and muscles carry out the task. But if coordination between the muscles and body parts is inadequate, initiation of right body part is affected which results in uncoordinated eye hand movements. To reduce this difficulty, a verbal clue to carry out tasks and help in reaching the target can improve the difficulty.

6. Social, emotional & behavioral problems are common. Child may fail to express himself adequately; hypo/ hypersensitivity may be seen. These problems occur because of increasing dependency on others. Hence caregivers need to be more empathetic towards their children and also should consider what and how they communicate with their children. Any small thing might hurt them or can frustrate them.

7. Some of the Duchenne muscular dystrophy children might show signs and symptoms of autistic spectrum disorder. These may occur at milder level, but if tackled adequately and early, it can help the child in better language and social development. Use extensive language stimulation using multi sensory approach so that child learns fast. If child tries to remain aloof, don't ignore it regarding it as muscular weakness. Consult a speech language pathologist if any kind of delay or deviance is noticed in child's speech and language abilities.

Yoga Therapy

Bhagwad Gita II - 50

Yoga KarmashuKaushalam: Do the work skillfully. Skill in work leads to perfection in work, which leads to excellence. Excellence in work is Yoga.

As we have seen in this chapter, that management of debilitating and degenerative diseases like muscular dystrophy involves multifold aspects. It is generally a combination of conventional medical treatment with rehabilitation techniques. The importance of rehabilitation techniques such as physiotherapy, occupational therapy and psychological rehabilitation cannot be over emphasized. All of the above therapies prove to be significant in managing the conditions arising out of the underlying neurodegenerative disease such as muscular dystrophy. But as with all forms of treatment, there is always room for improvement. This therapeutic gap can be filled by an ancient India wisdom for treatment of various conditions of the mind and body, namely Yoga Therapy.

As much as a fancy term Yoga is globally these days, its utility and impact in improving the well being of an individual is an established fact. Yoga is increasingly used by people all over the world to improve their physical fitness, as a stress reliever, to improve concentrations and lastly to enhance the spiritual experience. But the extrapolation of yoga therapy in the treatment of a myriad of diseases has only surfaced in recent times. In order to truly gain the massive benefits from this ancient science, one needs to grasp the complete meaning of Yoga and the manner in which it heals both, the mind and the body.

The philosophy of yoga originated some 2000-3000 years ago, although according to Hindu texts its origin can be linked right to the beginning of mankind. It was Saint Patanjali who organized the science of yoga into a systematic practice and is rightly known as The Grammarian of Yoga. So what is Yoga? It is the union of mind and body, of conscious with the subconscious, of Jivatma with the Parmatma. It is a way of life. It is in effect the science of healthy living wherein one attains physical, intellectual, mental and spiritual harmony.

The practice of the simplest asanas in Yoga can be used to treat and even avoid common ailments and improve the inner harmony & concentration and reduce stress thereby enhancing performance in our day to day activities. In its simplest form, these asanas involve breathing exercises, simple stretching and relaxation of various muscles of the body.

The Science behind Yoga : How does it heal ?

Medical practitioners and yoga experts believe (and studies have shown) that Yoga helps to lower the body's stress response by reducing levels of the hormone cortisol. Cortisol is also called stress-hormone as it is produced under response to stress. Yoga also boosts levels of the feel-good brain chemicals like GABA, serotonin, and dopamine, which are responsible for feelings such as relaxation and contentedness. These neurotransmitters are the targets of various mood medications like antidepressants and anxiolytic (anti-anxiety) drugs. The fact that yoga is linked to improved levels of these neurochemicals is a significant benefit that yoga provides. In addition to suppressing the stress response, yoga is also believed to stimulate the parasympathetic nervous system, which calms us down and restores balance after a major stress event is over. When the parasympathetic nervous system switches on, blood is directed toward endocrine glands, digestive organs and lymphatic circulation, while the heart rate and blood pressure are lowered. Thus our bodies can better absorb nutrients from the food we eat and more effectively eliminate toxins due to the enhanced circulation. The body enters into a state of restoration and healing.

Researchers have also discovered that yoga improves health in part by reducing inflammation. Patients who practice yoga have significantly improved levels of biomarkers like C-reactive protein (CRP) and interleukin-6 (IL-6). Yoga, thus, balances the body, the hormonal system and the stress response. It helps to rebalance and heal the body.

Yoga for Muscular Dystrophy

Inclusion of a therapeutic practice like yoga, in tandem with physiotherapy and occupational therapy can help mitigate the conditions of muscular dystrophy and greatly improve the quality of life. Yoga therapy uses asanas that involve stretching and relaxation of various muscles, in combination with deep breathing techniques to improve muscle tone and reduce pain. Research shows that the benefits of yoga for movement disorders include improved strength, flexibility, balance, overall fitness and quality of life.

Each individual has a different degree to which MD has caused physical and neurological degeneration. Muscle spasms, atrophy and rigidity associated with movement disorders often restrict balance and range of motion. Limits to balance and range of motion restrict the ability of individuals with movement disorders from practicing traditional yoga poses in a way that is beneficial to them. Yoga for movement disorders is marked by a practice that addresses the needs particular to people living with movement disorders. Yoga therapy needs to be customized according to the patient after studying his medical history in detail. Each asana has a specific purpose and helps specific areas of the body.

Thus not every asana can be prescribed to every patient in general. This is one of the main reasons that parents must help the individual suffering from muscular dystrophy in practicing Yoga on a regular basis. Also, it is of absolute importance to consult a yoga expert to evaluate which asanas can be recommended to the individual, in order to facilitate his / her existing physiotherapy

While physiotherapy is important to increase muscle strength, the entire exercise can be tiring and painful. But Yoga helps in reducing the physical and mental stress. Deep breathing techniques help in infusing more oxygen into the blood as opposed to regular shallow breathing. This improves blood circulation, strengthens the weakening muscles and helps in the removal of toxins that accumulate in the body.

Yoga also addresses the mental and emotional damage caused by the illness. It helps to infuse positive energy into the individuals and thus help them to increase their inner strength to fight off depression & anxiety that characterize such illnesses. It inducts a surge of hope into the individuals.

We will illustrate here some of the simple yet powerful asanas that help in muscular dystrophy.

Pranayama

Prana means Life and Ayam means control. Pranayam means control of the inner force of human life. The breath we breathe in and out is regarded as Prana which means bioenergy that endows man with ultimate potential for self-development. It is the vital / life force. But man must suitably control and channelize the prana or use it for right end. Yoga prescribes various practices of Pranayama or control of Prana popularly referred to as breathe control. The yogic breathing itself becomes a prayer, a satisfying spiritual experience in which one is aware of the living presence of God.

Benefits of Pranayama

1) Better blood circulation.
2) More oxygenation.
3) Longeveity i.e.full health life.
4) Mental concentration.
5) Increase in lung elasticity and capacity.
6) Purifies blood.
7) Emotional control.
8) Cheerfulness.
9) Prevention to diseases (many cases cured also).

Life is a series of activities. We conclude one activity only to start another. A preparedness kriya allows a person to be mentally prepared for an activity.

Sukhansana is one of the many asanas which quieten the mind. This asana is named for preparedness or conditioning. But muscular dystrophy patients have to sit in a

comfortable pose. If patients are not able to sit, they can do by laying down as well.

There are 3 types of Pranayama which we describe here:

Pranayama (I): Equalization of inhalation & exhalation;

Technique: Sit firmly and comfortably. *Breathe in out for equal counts. *Breathe 10 times.fig1 yoga

Fig. 4.20

Benefits:

- Augments pleasant feelings throughout whole body.
- Helps to calm the mind.

Pranayam (II): Inter costal Breathing

Technique : Hands on the side of the chest,*Make chest rise up as you breathe in for 3 seconds and fall as you breathe out for 3 seconds. *Repeat 10 times.

Fig. 4.21

Benefits:

- Activates the abdominal organs, provides a gentle massage, releases flatus and reduces fat in the abdominal region (best for muscular dystrophy because fat can be reduced).
- Helps in respiration and relaxation.
- Useful in insomnia.

Pranayama (III)

Technique: Sit in an asana for preparedness. *Use the little finger to block the other nostril. Breathe out from the open nostril. *Do same for the other nostril. *Repeat 10 times.

Benefits:

- Sedative effect on the tone and rhythm of the heart & brain.

Asanas for Muscular Dystrophy

1. Parvatasana scrap Fig. 4.22 and Fig. 4.23

Technique: Sit in comfortable pose,*Both the hand should be on both sides of the body. *Inhale for 3 seconds, raise both the hands simultaneously upward and above the head, palms facing upward. *Keep the elbows straight & join the palms. *Pull the abdomen slightly inside. Hold breath. *Exhale for 3 seconds, bring the arms down.

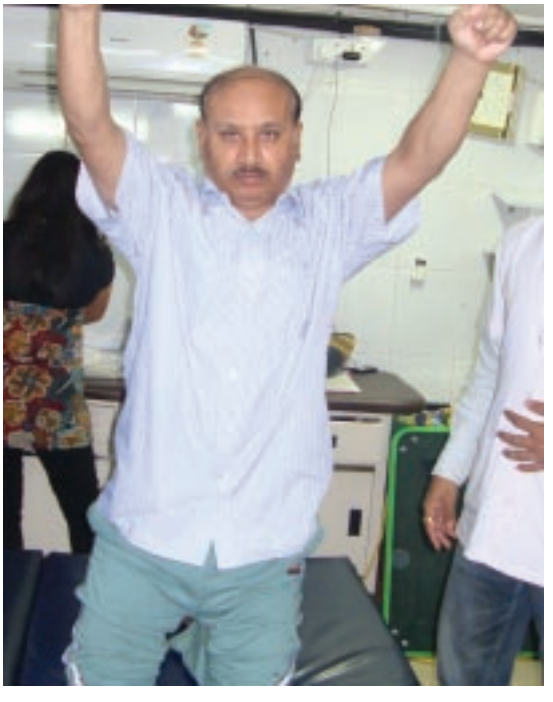


Fig. 4.22

Fig. 4.23

Benefits:

- Stretches all the abdominal and pelvic muscles.
- Loosens the hip joints.
- Helps to reduced the flatty and flabby abdomen.
- Improves the shape of body.(It is very beneficial for MD).

2. Yastikasana

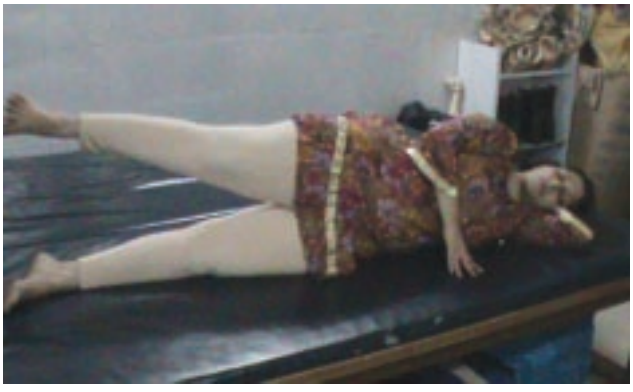


Fig. 4.24

Technique: Lie on back legs fully extended and arms extended at the side. *Be relaxed, inhale and raise arms above the head, rest them on the floor and stretch. *Holding breath, slowly stretch the body at full length, the toes and fingers pointing outward, as if trying to reach out. (Any attempt at maximum stretching of the body should be only during retention of breath). *Repeat 3 to 4 times with in-between pose.

Benefits:

- Corrects faulty posture.
- Tenses the usually relaxed abdominal and pelvic muscles.
- Offers relaxation, when maintaining a non-stretch, passive attitude.
- Removes spasm.

3. Stretching

In muscular dystrophy limbs are always weak, if one cannot to do the asanas properly, then patients have to stretch only to the extent they can. But stretching is compulsory for patients with MD. In Yoga, slow stretching is very beneficial. Patients need to do the stretching regularly.

Fig. 4.25

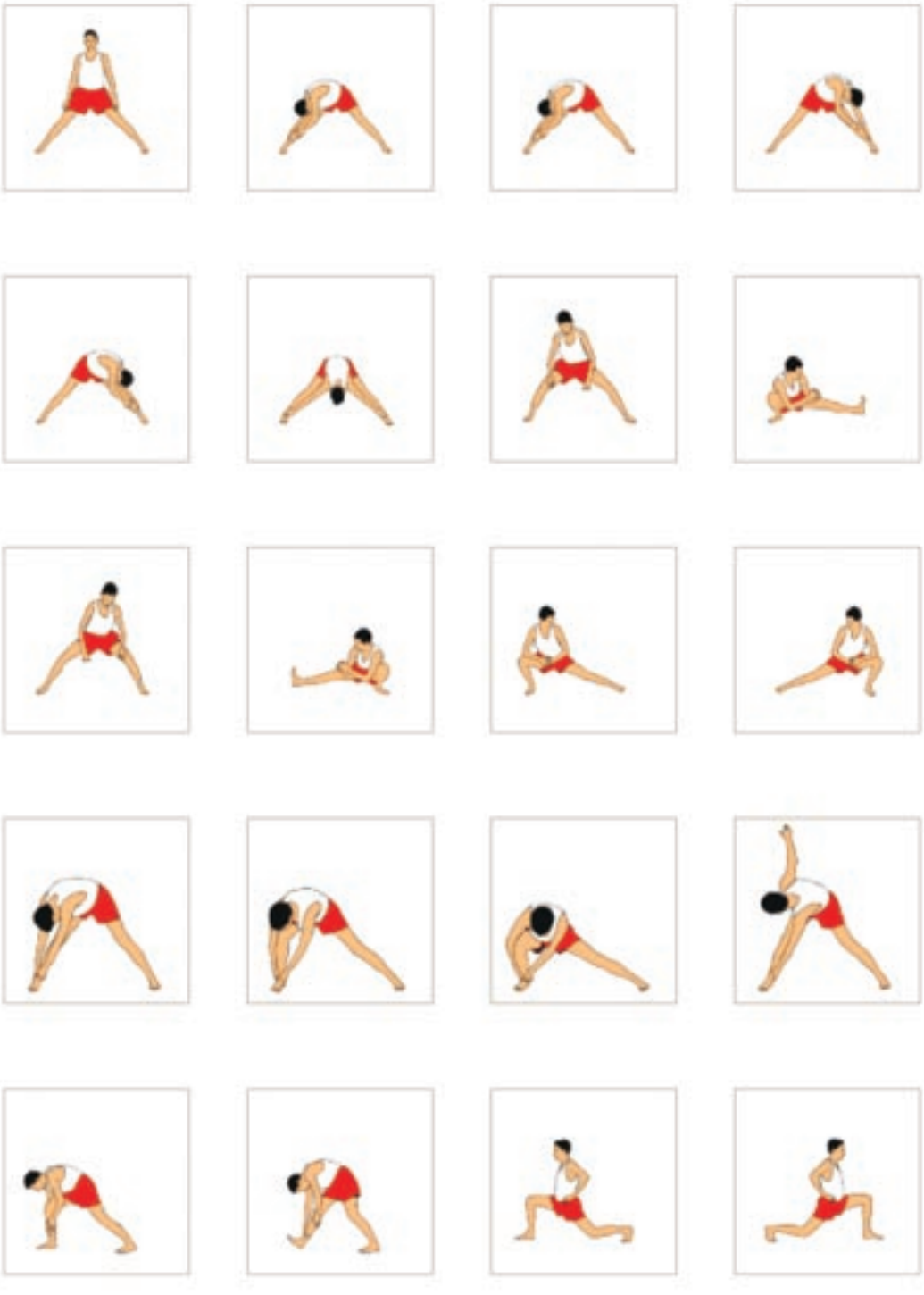


Fig. 4.26

Saint Arbindo : Overall development of personality by gaining knowledge of self, to develop oneself physically, mentally and spiritually

Nutrition In Muscular Dystrophy

Importance of Diet Therapy

Muscular dystrophy is a genetic disorder leading to loss of muscle function and wasting and progressive muscle weakness. Delayed growth, short stature, and increased fat mass are typical characteristics and impact on the nutritional status and energy requirements. However malnutrition is also a commonly seen feature towards the end stage of the condition that can be altered with texture modification and supplemental feeding.

Why is Nutrition Therapy Important???

Nutritional therapy can aim in improving the overall quality of the life of the patient. Nutritional care in the early stages of the condition can prevent or rather delay onset of other osteoporosis and other deficiencies like Calcium and Vitamin D. The body replaces the muscle tissue with fat and connective tissue. Altering the fat intake is therefore essential.

Research has demonstrated inadequate nutrient intake of protein, energy, vitamins (water and fat soluble), and minerals (calcium and magnesium). Significant correlations were found between measures of strength and certain individual nutrients (e.g., copper and water-soluble vitamins).

A complete balanced diet keeping in mind the nutritional requirement and the current nutritional status of the patient is essential in muscular dystrophy.

The most common nutritional hazard in muscular dystrophy is related to weight gain. This is mainly due to lack of physical activity along with increased food intake. It is a myth that by eating fewer meals a day, ones weight can be controlled. Smaller frequent meals are advisable as it not only balances the food intake but also eases digestion. When food is eaten in smaller quantities it enables complete digestion of the food and slow release of the energy which helps in steady utilization by the body.

Although it is difficult for the patient to target the Ideal Body Weight (IBW), an average on plus or minus 5 to 7 kilograms can be permitted.

The following are the requirements per day:

Age / Sex	Ideal Body Weight	Energy (Kcals)	Proteins (G)	Fats(G)
1-3 yrs	12	1240	22	25
4-6 yrs	19	1690	30	25
7-9yrs	27	1950	41	25
10-12 yrs (boys)	35.4	2190	54	22
10-12 yrs (girls)	31.5	1970	57	22
13-15 yrs (boys)	47.8	2450	70	22
13-15 yrs (girls)	46.7	2060	65	22
16-18 yrs (boys)	57.1	2640	78	22
16-18 yrs (girls)	49.9	2060	63	22

The energy requirements per day have to be altered depending on the current weight of the patient and the quotient of physical activity. This will be determined by the dietitian.

The per day meal must be distributed in such a way that post afternoon, the choices of food must be light and easy to digest. The breakfast, midmorning, lunch must be calorie dense so that the energy produced can be utilised throughout the day.

Fig. 4.27

Carbohydrates:

Carbohydrates are the energy givers of our daily diet.

Our Indian diet is very balanced and includes a wide variety of cereals like jowar, bajra, ragi (nachni), poha, rawa or sooji, maize or makkai, wheat, wheat bran (with the outer husk), dalia are a few to name. Cereals are energy dense and also contain loads of fibre and micronutrients which are required by our body in small amounts. Cereals form the bulk of the staple Indian Diet. They also contribute towards the carbohydrate intake hence must be taken in lower amounts during the second half of the day as physical activity is restricted and may lead to weight gain.

Fig. 4.28

Proteins:

Proteins contribute towards the growth and development, muscle development, cell and tissue repairs all of which are essential for Muscular Dystrophy. Proteins also add bulk to or diet and bring a feeling of fullness as the body takes longer time to digest them. All foods except sugars, oil and fats contain protein to varying degree. Foods like animal protein: ie:- fish, chicken , meat and eggs, dals, pulses, sprouts, oilseeds, soyabean and nuts are of high biological value which are utilised very easily by our body. Proteins from vegetable sources are however minimal, but they contribute a larger extent towards the fiber and micronutrients in our diet.

Fats:

The culprits of our diet!!!!

Yes fat intake can become the culprit of our diet if consumed in excess. For that matter, MODERATION IS THE BYLANE TO GOOD HEALTH.

Fats aid in digestion of fat soluble vitamins ie:- Vitamin A, Vitamin D, Vitamin E, Vitamin K. Hence must not be eliminated completely from the diet. Our body requires a minimal of 3tsp of oil which gives about 135 kcals per day. The fats from oils, butter, ghee, dalda, vanaspati are the visible fats. Apart from these we have the non - visible fats coming from coconuts, groundnuts, dry coconuts and a negligible amount from vegetables.

Junk foods contain plenty of visible and invisible fats which can lead to obesity….

Fig. 4.29

Antioxidants:

Antioxidants neutralize the free radicals by giving them the necessary electrons. Some amount of antioxidants is manufactured within the body, but most of the antioxidants are present in fruits and vegetables.

Muscular dystrophy causes free radical overload along with oxidative stress. It is hence essential to consume a diet rich in antioxidants. Research has shown that manipulations in the free radical may have therapeutic promise for the treatment of MD.

Antioxidant rich foods:

Raisins, strawberries, Plums, oranges, cherries, Spinach, capsicum, onion, corn, brinjal, almonds, walnuts, clove cinnamon, oregano, lemon, ginger, beetroot, guava and pineapple to name a few.

Green tea polyphenols, such as EGCG, are known to be powerful antioxidants. Because inflammation is involved in the degradation of muscle tissue in MD, oxidative stress is believed to play a role in this process. Green tea and its active constituents may improve MD prognosis by reducing this oxidative stress. There was also biochemical evidence that green tea extract reduced oxidative stress in muscle cells.

Start with just one cup per day and then increase to 2 to 3 cups per day. A dash of honey and lime can be added to enhance the taste of the tea. Avoid adding sugar.

Coenzyme Q10. Coenzyme Q10 (CoQ10; also called ubiquitin) is a powerful antioxidant and mitochondrial respiratory chain cofactor. It possesses membrane-stabilizing properties and is capable of penetrating cell membranes and mitochondria. Muscle cells expend a great deal of energy and are rich in mitochondria. As an essential cofactor, CoQ10 acts to facilitate a complex series of reactions that occur within the mitochondria. Known as the respiratory chain, these chemical reactions ultimately supply energy, which may be stored for later use or readily expended.

The dosage of CoQ10 id however decided by the neurologist.

Calcium and vitamin D.

By the time they reach 10 years of age, many boys with MD will have lost the ability to walk. Confined to a wheelchair, they inevitably develop bone-weakening osteoporosis, although the process often begins before patients become wheelchair bound. In fact, although bone density in MD has received relatively little attention. This indicator of declining bone health was especially advanced in patients on corticosteroid therapy. The scientists also found that patients had lower-than-normal levels of a form of bioactive vitamin D.

In normal individuals, vitamin D and calcium are known to play a crucial role in the maintenance of healthy bone mineralization and density. Although vitamin D is generated within the body in response to adequate sunlight, exposure to sunlight sufficient to guarantee an adequate supply of vitamin D may be problematic.

Calcium and vitamin D rich foods: Milk and milk products(but must be skimmed), ragi, leafy vegetables, codliver oil, soyabean, eggs, vitamin D fortified foods,sprouts, all condiments and spices.

Glutamine. Glutamine is involved in many metabolic processes. It is an important energy source for many cells.

Some researchers have suggested that glutamine may be "conditionally essential" in DMD because the ability to synthesize glutamine is impaired in MD patients. Research has shown that glutamine supplementation may not be of great help in MD.

Fig. 4.30

Listed below are simple guidelines one must follow:

1. Small frequent meals must be consumed every 2 hourly.
2. A balanced Indian meal includes all the nutrients required for the day.
3. Breakfast is the most important meal of the day.
4. Avoid too much carbohydrate rich foods in the second half of the day. All Indian cereals are rich in carbs - rice wheat, poha, rawa, maida, breads etc.
5. Plenty of protein rich foods to be consumed all throughout the day.All pulses, sprouts, soya bean, panner, nonvegetarian foods such as eggs, fish, chicken, lean meats, milk and milk products are good sources of proteins.
6. Additional supplements for omega 3 fattyacids can help a great deal after stem cell therapy. Some food sources of omega 3 fattyacids are almonds(badam), walnuts (akrod), olive oil, flaxseeds, eggs. These foods must be consumed on regular basis.
7. Including foods rich in znic and selenium also proves useful as patients are seen having a deficiency of the same.
8. Reduction in the total amount of fat ie: oil, butter, ghee, cheese, coconut, groundnuts, selected dryfruits like cashewnuts, kismish is essential.
9. Simple sugars and sweets can add to a lot of calories in the daily diet hence must be avoided as far as possible.
10. Fresh vegetables and fruits not only add fiber to the diet but also lower in calories compared to carbohydrate rich foods or foods high in fat.
11. Whole cereals like jowar, bajra, ragi, oats must be encouraged as they have a higher fiber content.
12. Any fried food if desired must be eaten for breakfast in small amounts as it can be digested throughout the day.

13. Consumption of egg whites increases the protein intake as well as keeping the calories low.

14. Chicken and fish also can be included in the daily diet as long as the preparation does not involve too much of fat content.

15. Cooking methods such as boiling, grilling, steaming can help reduce oil and fat content in the foods.

16. Whole pulses and sprouts must be consumed on daily basis to increase the protein, fiber and micronutrients especially for vegetarian patients.

17. Green tea is found to be very rich in antioxidants that prevent the degeneration of the muscles hence must be consumed daily. It also aids in digestion and helps in weight loss. Atleast 2 to 3 cups of green tea must be consumed per day.

18. Make eating a pleasurable one for the patient.

19. Adding lot of colour to the plate will make eating healthy foods an enjoyable one.

20. Choice of foods that the parents make is very essential as the child looks upon the parent as a guideline for his or her eating habits.

Constipation:

Constipation is one of the major concerns of the parents. Patients do not consume enough fiber and water in the daily diet hence leading to constipation. Excess water leads to frequent urination the fear of which forces them to consume less amounts of water. Drinking small amount of water at equal intervals of half hour to one hour can ease the situation.

Steroids:

MD patients on steroids often have fluid and salt retention causing further puffiness and weight gain. Therefore the fluid and salt intake must be calculated accordingly. They also experience increased hunger pangs which must be controlled strictly.

Sample meal plans: Adults

Early morning: 1 cup warm water

Breakfast: Milk or green tea with poha / upma / idli / dosa / uttapam / khakra / thepla / paratha / wheatflakes / musselli / dhoklas.

Midmorning: fruits and dryfruits with lime juice or green tea or barley water

Lunch: salads and veggies in plenty, chapattis or bhakris with dal and any pulse or protein foods(chicken or fish),small quantities of rice and curd.

Evening: green tea, milk, tea (any one)

Any dry roasted snack - biscuits, kurmura or rice puffs, sandwiches, roasted farsans or chiwda.

Mid evening: soups (homemade) or fruits

Dinner: salads and veggies, chapattis or bhakris with dals or lentils, paneer or tofu veg, or any leafy vegetable.

Bed time: 1 cup milk

Sample meal plans: Children

Early morning: 1 cup warm water

Breakfast: Tea or green tea with poha / upma / idli / dosa / uttapam / khakra / thepla / paratha stuffed with veggies and sprouts / wheatflakes / musselli / dhoklas.

Midmorning: fruits and dryfruits with lime juice or green tea or barley water.

Lunch: salads and veggies in plenty, chapattis or bhakris with dal and any pulse or protein foods(chicken or fish),small quantities of rice and curd. Snack options like pavbhaji, pasta, pizzas can be made with plenty of vegetables or chicken added to it. Use of paneer instead of cheese would be a good option.

Evening: green tea, milk, tea (any one)

Any dry roasted snack - bhel or vegetable or chicken cutlets.

Mid evening: soups or fruits which can be given as fruit salads.

Dinner: salads and veggies, chapattis or bhakris with dals or lentils, paneer or tofu veg, or any leafy vegetable.

Bed time: 1 cup milk

- The meals must look colourful and palatable.
- Include vegetables which add plenty of colour and also contribute to antioxidant content of the meals which is essential in MD.
- Using wheat based products instead of maida is a better choice.
- Dinner must be consumed by * pm to ensure enough time for the food to digest.
- Water must be consumed in between the meals to enhance digestion of the food.
- The meals before the exercise schedule must consist of fruits and dryfruits which can provide instant energy.
- Plenty of water must be consumed during and after exercise.

The overall pattern of the diet remains the same for all patients. However changes in the quantity and alteration in the meal timings must be made as per the lifestyle of the patient.

Medical Checks:

- Regular lipid profile must be done to check the cholesterol levels of the patient.
- A record of the weight of the patient must be maintained.

- Dietary recall can be listed down in diet diaries so that the patient is self motivated to follow a healthy diet.

A monthly visit to the Dietician can prove beneficial so as to monitor the dietary habits closely.

To sum up, a team work, which includes medication, rehabilitation, cardiac and respiratory care, orthopedic intervention, proper nutrition, psychological counseling & yoga therapy can help improve the quality of life in a patient with muscular dystrophy.

Scientific Publications for Medical Management in Muscular Dystrophy

1. Dheeraj Shah. Are Corticosteroids Effective in Duchenne Muscular Dystrophy? Indian Pediatrics. 2008; 45: 401-402

2. G. M. Berneske, A. R. C. Butson, E. N. Gauld, and D. Levy. Clinical Trial of High Dosage Vitamin E in Human Muscular Dystrophy. Can Med Assoc J. 1960 February 20; 82(8): 418-421.

3. 2.Gabriel E., Machlin L. J., Filipski R. & Nelson J. Influence of age on the vitamin E requirement for resolution of necrotizing myopathy. J. Nutr. 1980; 110, 1372-1379.

4. P K Thomas, J M Cooper, R H King, J M Workman, A H Schapira, M A Goss-Sampson, and D P Muller. Myopathy in vitamin E deficient rats: muscle fibre necrosis associated with disturbances of mitochondrial function. J. Anat. 1993; 183, 451-461

5. I. Gamstorp, K.H. Gustavson,O. Hellström,B. Nordgren. A Trial of Selenium and Vitamin E in Boys With Muscular Dystrophy. Child Neurol July 1986 vol. 1 no. 3 211-214

6. Neville H. E., Ringel S. P., Guggenheim M. A., Wehling C. A. & Starcevich J. M. Ultrastructural and histochemical abnormalities of skeletal muscle in patients with chronic vitamin E deficiency. Neurology 1983; 33, 483-488

7. Meydani M. et al. Antioxidant response to exercise-induced oxidative stress and protection by vitamin E. Ann. NY Acad. Sci. 1992; 669, 363-364

8. Urano S. et al. Mobility and molecular orientation of vitamin E in liposomal membranes as determined by 19F NMR and fluorescence polarization techniques. Arch. Biochem. Biophys.1993; 303, 10-14

9. Pizarro M, Lissi E, Reyes J, Holmgren J. Duchenne muscular dystrophy. Effects of vitamin E administration on urinary luminescence. Rev Med Chil. 1998;126(10):1165-72

10. Folkers K, Simonsen R. Two successful double-blind trials with coenzyme Q10 (vitamin Q10) on muscular dystrophies and neurogenic atrophies. Biochim Biophys Acta. 1995; 24;1271(1):281-6.

11. Fiaccavento R, Carotenuto F, Vecchini A. An omega-3 fatty acid-enriched diet prevents skeletal muscle lesions in a hamster model of dystrophy. Am J Pathol. 2010;177(5):2176-84

5:

Exercises and stretches

Before starting any exercise, stretching should be always be done and should be under the supervision of a Physiotherapist or an Occupational therapist. Proper guidance is essential as over-stretching or over-exercising may damage the muscle.

The person with Muscular Dystrophy should start Stretching & exercising before any loss in movement (contracture of joints) occurs i.e. in Stage 1. Both are a must to maintain flexibility and to prevent contractures. It is always much easier to prevent them than to correct them.

Stretches

Regular daily stretches help to maintain muscle length and keep joints mobile to prevent any deformities.

Hold each stretch for 20 seconds and repeat 3-4 times. These stretches should be performed at least 5 times in a day.

If the range is not complete, try to stretch it slowly and gently; just a little more each time.

Do stretching in the opposite direction of the deformity or contracture, so that they help to put the joint into a more normal position.

Do not use too much force and please stop stretching when it starts to hurt.

Stretching is of two types

1. Assisted stretching
2. Self stretching

Assisted - stretching

Below are stretches which should be done by someone else.

Ankle stretching

Place one hand under the knee and hold the heel in your other hand. Gently bend the ankle upward by pushing against the sole of the foot with the forearm.

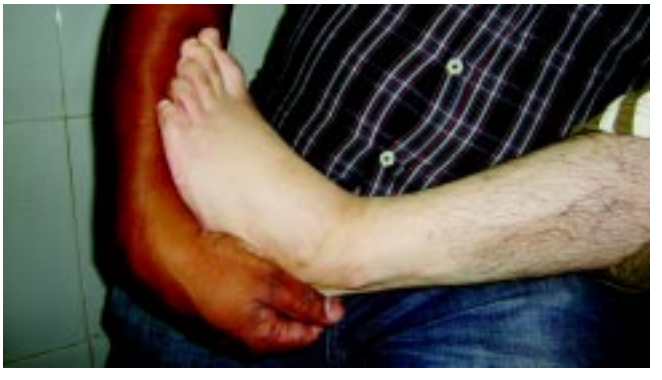


Fig. 5.1

Hip & knee stretching together

Make the person lie on his tummy and place weights on the buttocks and at the ankles as shown in the picture. This stretching should be done for about 20-30 minutes three times in a day.

Fig 5.2: Contractures at hip and knee

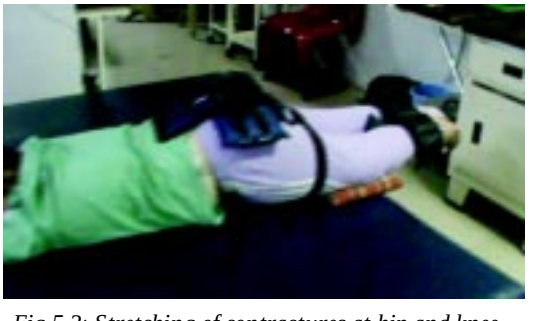


Fig 5.3: Stretching of contractures at hip and knee

Hip stretching

1. Hold the heel of the foot in one hand and place the other hand on the knee. Gently straighten the leg by moving the heel upwards. The knee should not be moved at all.

Fig 5.4

Fig 5.5

2. Place one hand on the hip and support the heel with the other hand. Gently move the hip upwards and inside and then apply downward pressure on the knee.

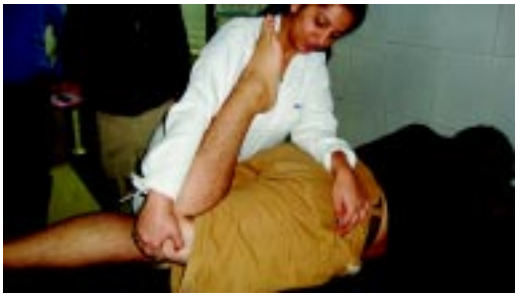


Fig 5.6

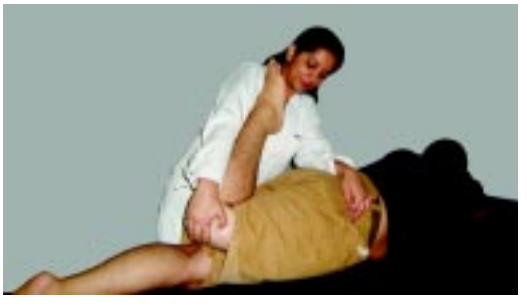


Fig 5.7

3. Place one hand on one knee and with the other hand bend the hip and knee of the other leg as if trying to touch chest. Press down the knee of the other leg.

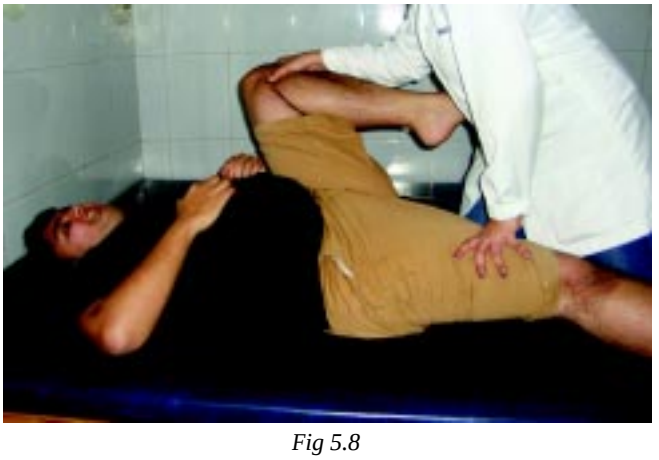


Fig 5.8

Elbow stretching

Keep one hand on the shoulder and hold the wrist with your other hand. Then press the wrist gently downward to straighten the elbow.

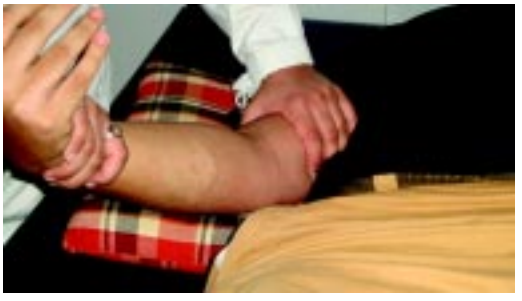


Fig 5.9

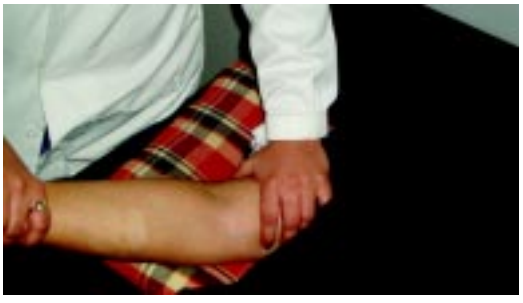


Fig 5.10

Forearm stretching

With one hand cupping the elbow, hold the wrist with the other hand. Slowly turn the wrist outside to make the palm face the ceiling.

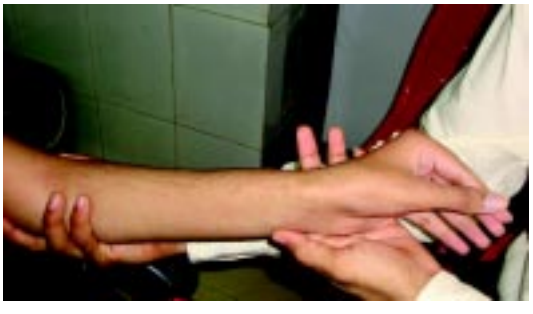


Fig 5.11

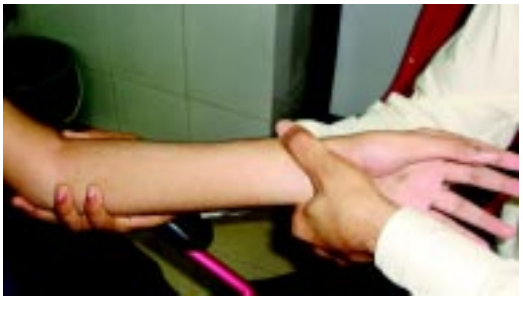


Fig 5.12

Wrist & fingers stretching

Keeping the elbow straight, slowly straighten the fingers. Thumb should be taken out at the side. Then gently bend the wrist and hand back until a stretch is felt in the forearm.

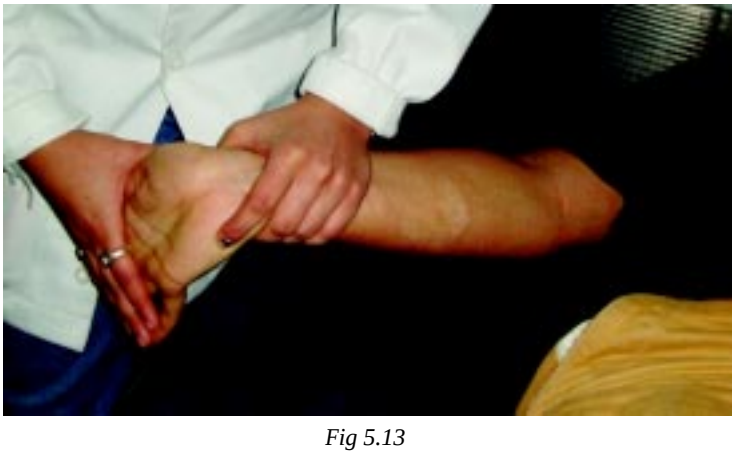


Fig 5.13

Finger stretching

Gently straighten the fingers while holding the wrist.

Fig 5.14

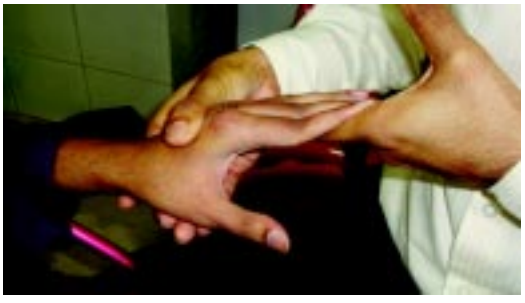


Fig 5.15

Self-stretching

Below are some of the other stretches which could be done by the person himself or herself.

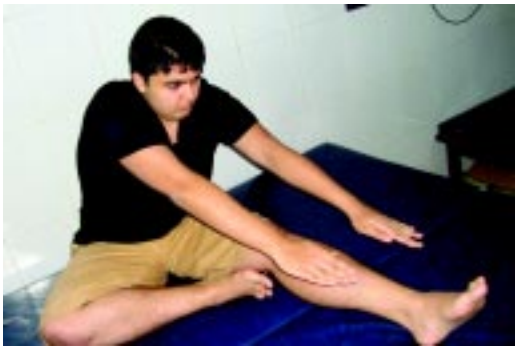


Fig 5.16

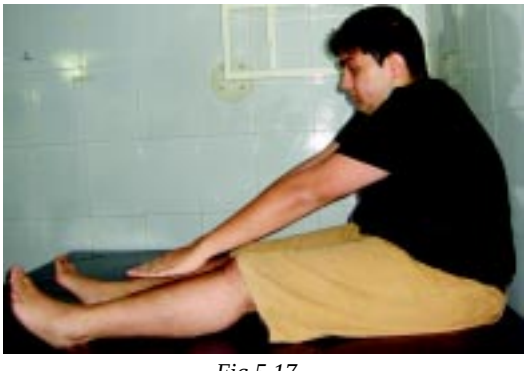


Fig 5.17

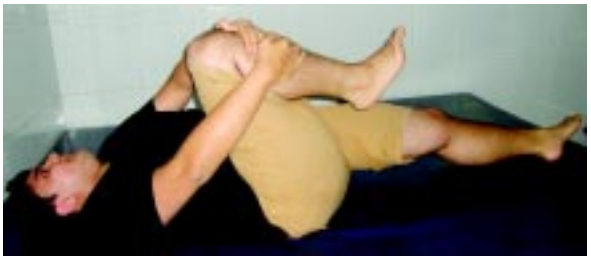


Fig 5.18

Fig 5.19

EXERCISES:

In muscular dystrophy, Range-of-motion (ROM) exercises are usually done. ROM exercises are regularly repeated exercises that straighten or bend one or more joints of the body and move them in all the directions that a joint normally moves.

The main purpose of these exercises is to keep the joints flexible. They can help prevent joint stiffness, contractures, and deformities.

ROM exercises should be done at least 2 times a day.

While exercising, if the person gets tired or if he gets cramps, he should be given rest and decrease the number of repetitions. The person may be able to perform these exercises independently in the earlier phases. But as weakness progresses assistance may be required.

Exercises are of three types:

1. Active: where the person performs the exercise on his or her own.(stage 1)
2. Active assisted: where the person performs the exercise with some help from someone else (stage 2&3) and
3. Passive: where the exercise is entirely done by someone else (stage 4).

   Shown below are active ROM exercises.

Neck Exercises

1. Slowly turn the head to each side as far as possible without pain, hold and return to the center.

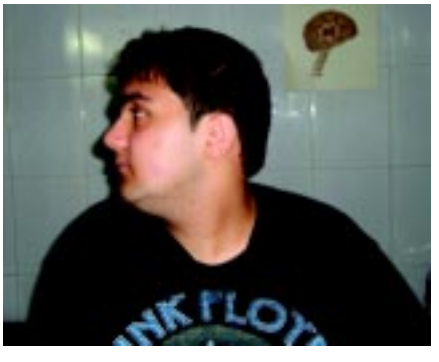


Fig 5.20

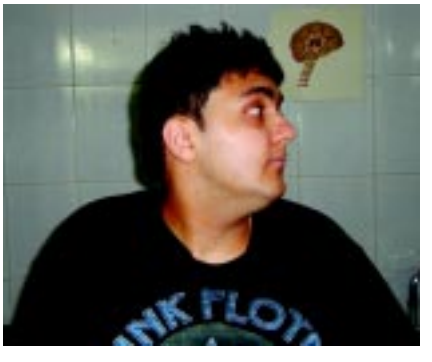


Fig 5.21

2. Tilt the head sideways on each side, so that the ear moves towards the shoulder; hold and return to centre.

Fig 5.22

Fig 5.23

3. Bend the head downwards, slowly taking it up to look at the ceiling.

Fig 5.24

Fig 5.25

Shoulder and Arm Exercises

1. Lift both hands straight in front and to the side, from your waist all the way over your head.

   From front

Fig 5.26

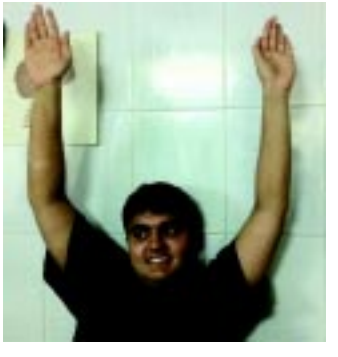


Fig 5.27

   From sides

Fig 5.28

Fig 5.29

o In later stages when the above shoulder movement is not possible, suspension & slings are used to do exercises actively.

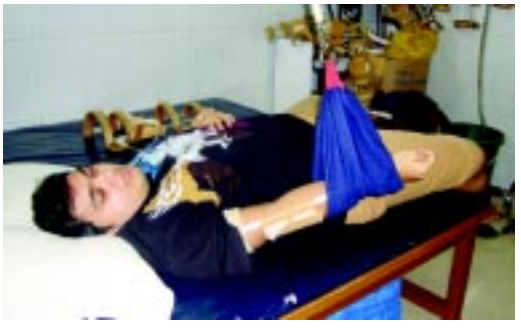


Fig 5.30

Fig 5.31

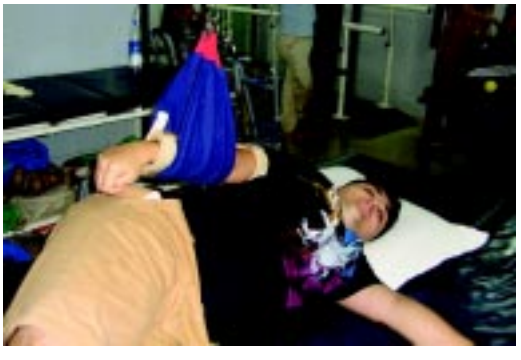


Fig 5.32

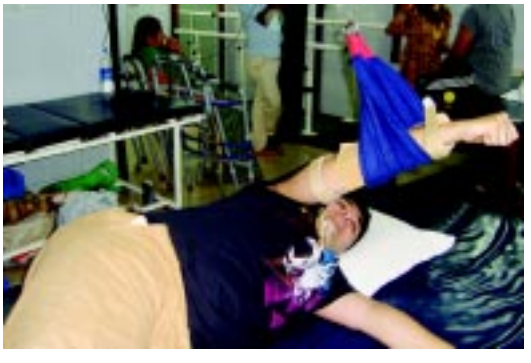


Fig 5.33

2. Hold one arm straight out to the side at shoulder height, and then bring the arm in front of chest, keeping the elbow straight. Go just to the point where your arm starts to bend, then return to starting position.

Fig 5.34

Fig 5.35

3. Squeeze shoulder blades together in the back by taking shoulders back. Then relax and repeat.

Fig 5.36

Fig 5.37

4. Bring both shoulders up towards your ears, then relax and repeat.

Fig 5.38

Fig 5.39

Elbow exercises

1. Biceps: Hold the entire upper limb in a straight position. Then bend the elbow so that fingers touch the shoulder on the same side. Relax & return to starting position.

Fig 5.40

2. Triceps: Hold arm over head with elbow bent. Then straighten the elbow with fingers pointing to the ceiling. Relax & return to starting position.

Fig 5.41

Fig 5.42

3. Forearm: Hold upper limb by side and elbow bent with palm facing the ground. Slowly turn forearm to face ceiling. Relax and return to starting position.

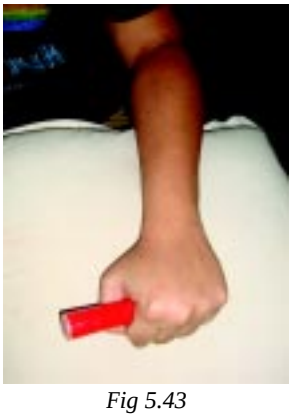


Fig 5.43

Fig 5.44

Repeat the above exercises for the other elbow.

Wrist Exercises:

1. Hold wrist in neutral position with palm facing downwards. Then lift wrist up towards the ceiling. Relax and return to starting position.

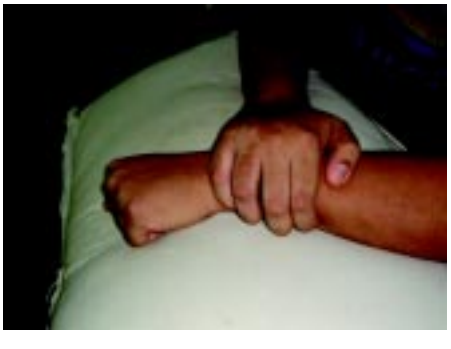


Fig 5.45

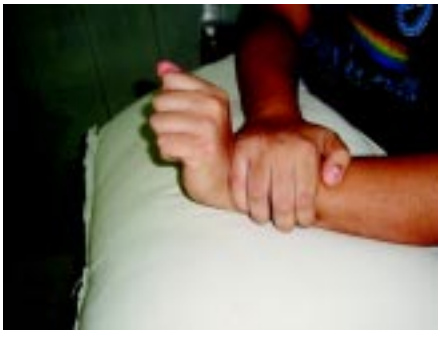


Fig 5.46

2. Hold wrist in neutral position with palm facing the ceiling. Then raise wrist as if trying to touch forearm. Relax and return to starting position.

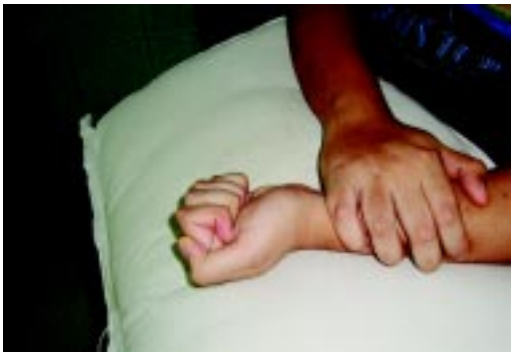


Fig 5.47

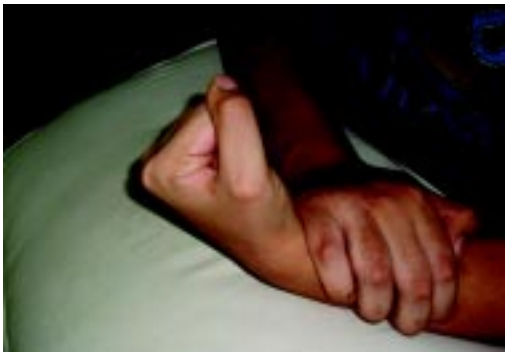


Fig 5.48

Repeat the above exercises for the other wrist.

Hand and Finger exercises:

Sitting with hand out in front on a pillow or table, the following exercises can be performed.

1. Opening and closing the hand. First make a tight fist. Then open and relax hand.

Fig 5.49

Fig 5.50

2. Open hand and stretch the fingers as far apart as possible. Bring fingers together again.

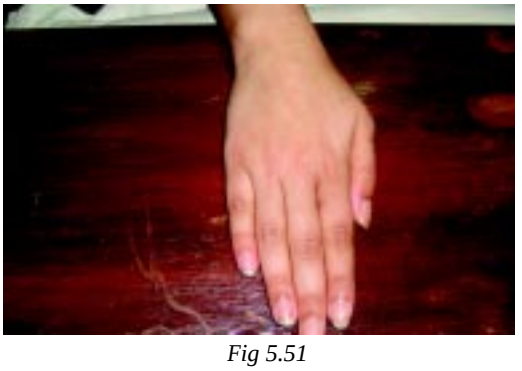


Fig 5.51

Fig 5.52

3. Counting on fingers. One at a time, bring each finger to touch the thumb.

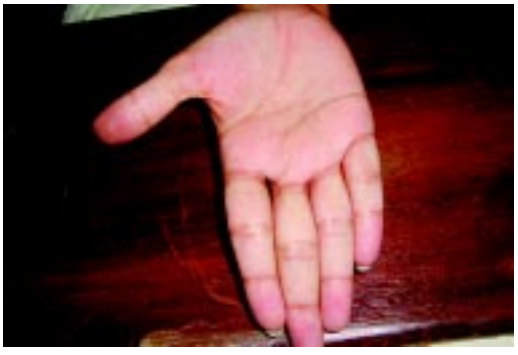


Fig 5.53

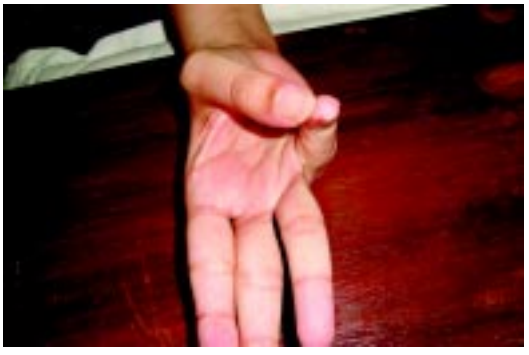


Fig 5.54

4. Thumb exercise. Move your thumb and place it across the palm. Move it out to the side again.

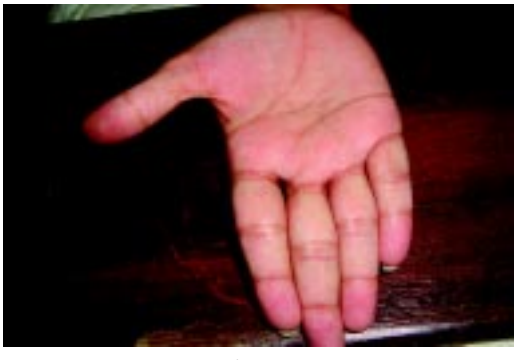


Fig 5.55

Fig 5.56

Repeat the above exercises for the other hand and fingers.

Hip exercises

1. Lie on back with legs in a straight position. Slowly lift one leg without bending knee. Lift leg as much as possible. Then return to starting position.

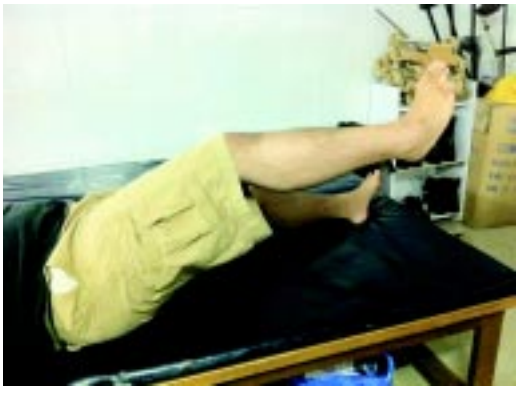


Fig 5.57

2. Lie on one side. Then lift the leg which is on top taking it sideways as much as possible without bending knee. Then return to starting position.

Fig 5.58

3. Lie on stomach. Lift leg backwards as much as possible without bending knee. Then return to starting position.

Fig 5.59

Fig 5.60

Repeat the above exercises for the other leg.

- During later stages when the above hip movements are not possible to do actively, suspension & slings are used.

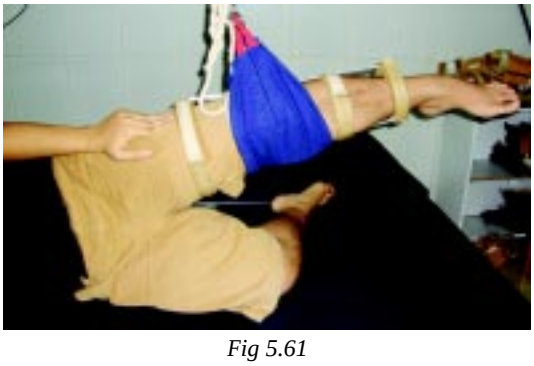


Fig 5.61

Fig 5.62

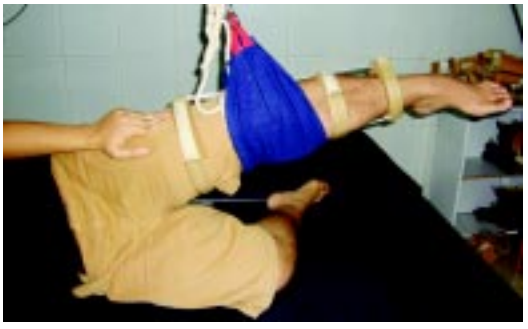


Fig 5.63

Fig 5.64

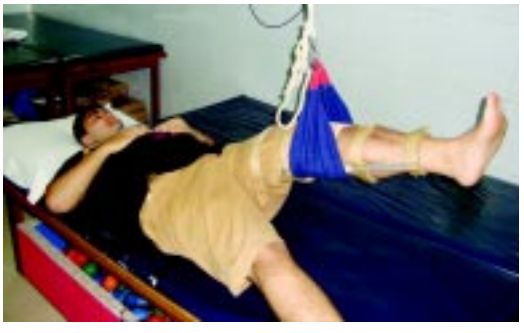


Fig 5.65

Fig 5.66

Knee exercises

1. Sit with hip & knees in 90°. Straighten knee out in front without bending the back. Then return to starting position.

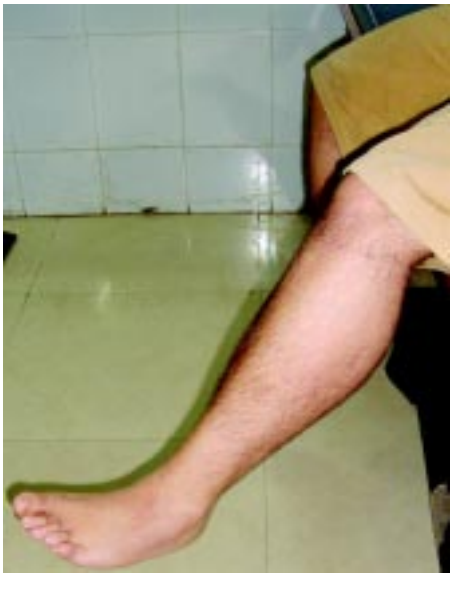


Fig 5.67

2. Lie on stomach. Bend knee without lifting hip. Then return to starting position.

Fig 5.68

3. Lie on back. Place a rolled towel below knee. Press knee down for 5 seconds, then relax.

Fig 5.69

Repeat the above exercises for the other knee.

Foot exercises

1. Keep feet flat on the floor. Then slowly lift front of foot (heel touching floor) as if trying to point toes to the ceiling. Then lower them back. (Don't lift thighs off chair.)

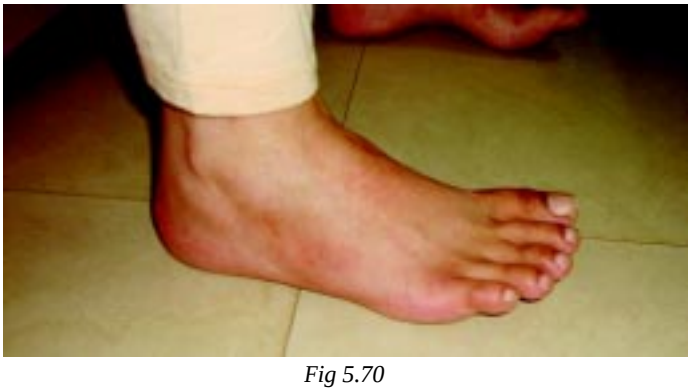


Fig 5.70

2. Keep heels on the floor. Then slowly turn foot inwards as if to check sole. Relax and return to start position.

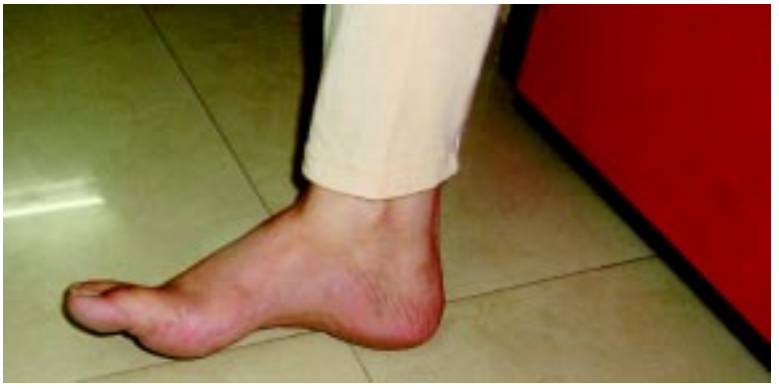


Fig 5.71

3. Keep heels on the floor. Then slowly turn foot outwards. Relax and return to start position.

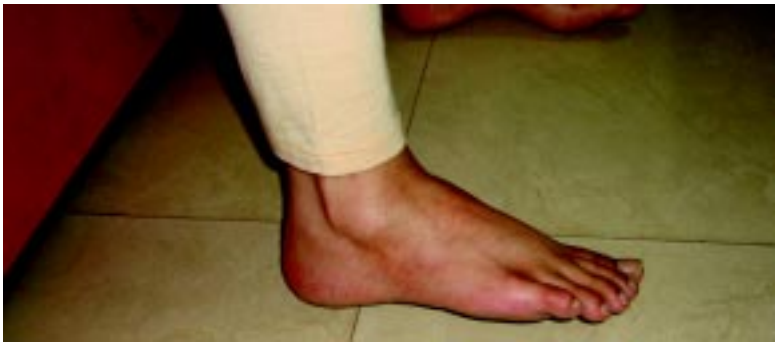


Fig 5.72

Repeat the above exercises for the other foot.

Trunk Exercises

1. Upper Abs: Lie on back with hip & knees bent. Place hands across shoulders or on knee and attempt to come to sitting position. Return to starting position.

Fig 5.73

2. Lower abs: Lie on back with hip & knees bent. Slowly bring knees to touch chest without the help of hands.

Fig 5.74

3. Bridging: Lie on back with hip & knees bent. Lift hips up as much as possible. Then return to starting position.

Fig 5.75

4. Back extensors: Lie on stomach. Raise head, neck & chest off the mat as much as possible. Then return to starting position.

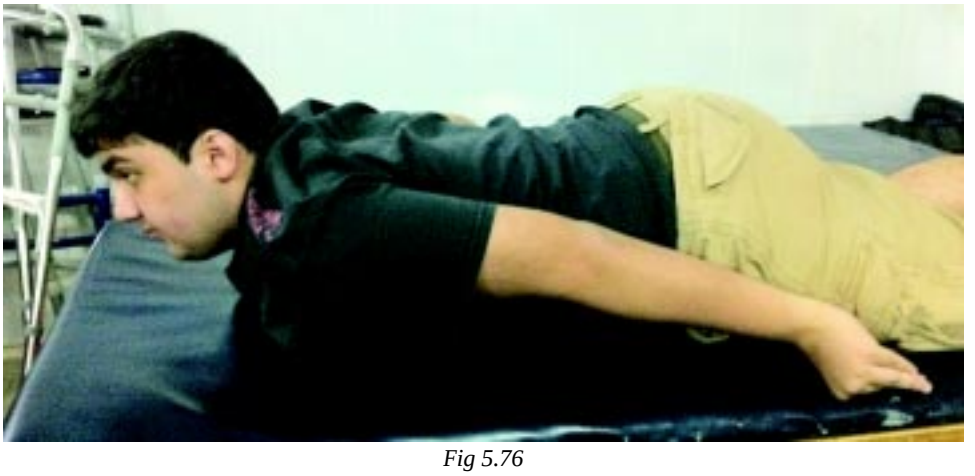


Fig 5.76

Other exercises:

1. Rolling

Fig 5.77

2. Crawling

Fig 5.78

3. Kneeling

Fig 5.79

4. Ball catching & throwing

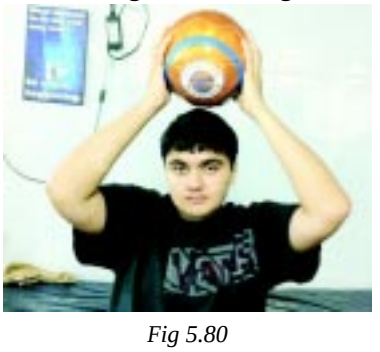


Fig 5.80

5. Ball Kicking

Fig 5.81

Facial exercises

Fig 5.82: Blowing cheeks

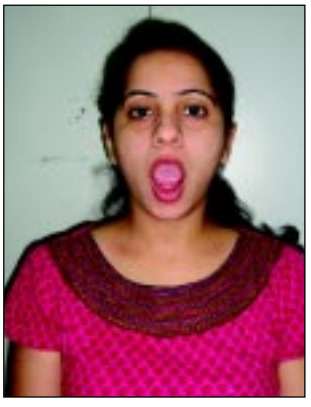


Fig 5.83: Opening mouth

Fig 5.84: Clenching teeth

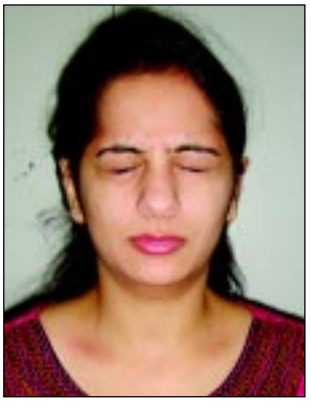


Fig 5.85: Close eyes tightly

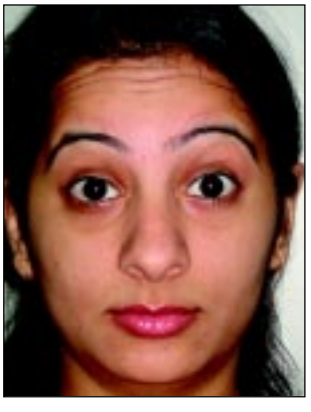


Fig 5.86: Raising eyebrows

Fig 5.87: Smiling

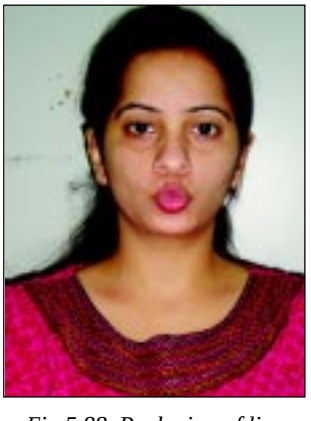


Fig 5.88: Puckering of lips

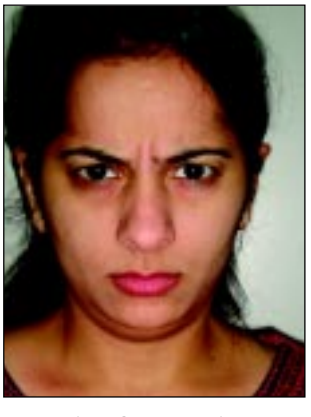


Fig 5.89: Frowning

Fig 5.90: Creasing of nose

Fig 5.91: Twisting face to one side

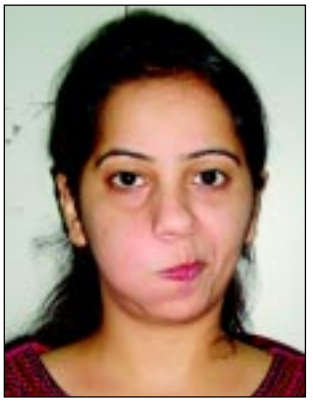


Fig 5.92: One side blowing of chee

Repeat for other side of face

Specific exercises for lips

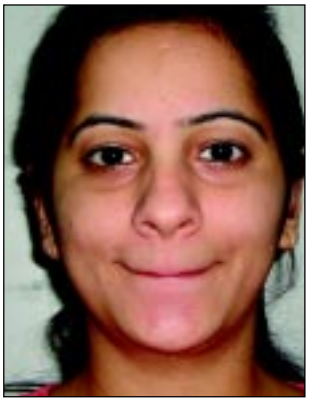


Fig 5.93: Pressing lips together tightly

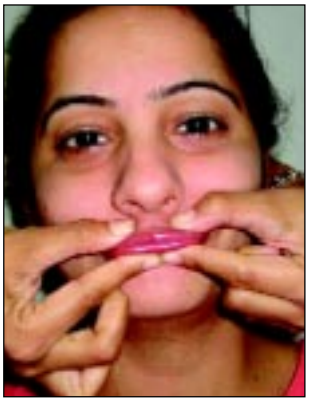


Fig 5.94: Stretching exercise to improve lip seal

Fig 5.95: Stretching lips and holding smile

Fig 5.96: Hold spoon tightly with lips and suck fluids from it

Fig 5.97: Hold object with lips tightly

Fig 5.98: Pressing tongs with lips to improve jaw closure

Specific exercises for tongue

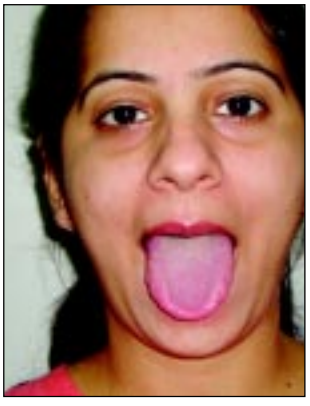


Fig 5.99: Stick out tongue as much as possible

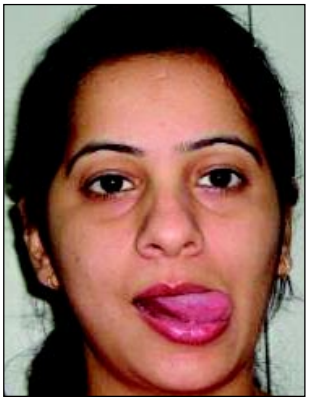


Fig 5.100: Move tongue to left

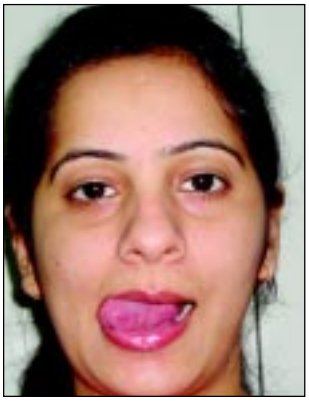


Fig 5.101: Move tongue to right

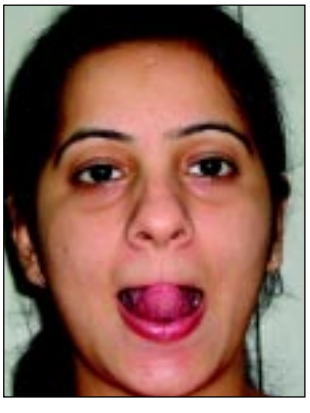


Fig 5.102: Touch tongue to upper lip

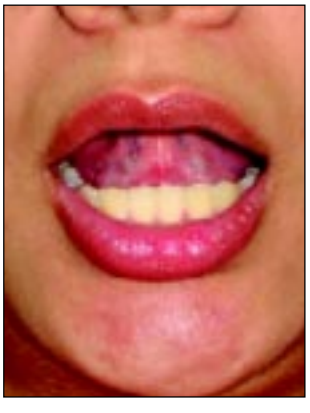


Fig 5.103: Touch roof of mouth with tongue

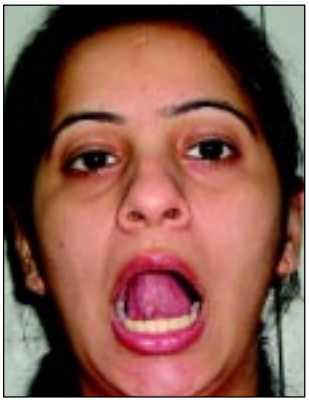


Fig 5.104: Touch roof of mouth just behind teeth with tongue

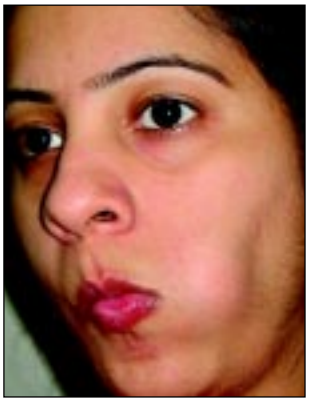


Fig 5.105: Push cheek with tongue

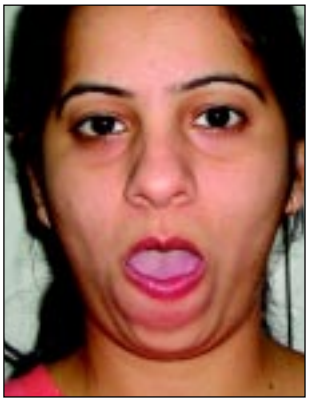


Fig 5.106: Move tongue in front of lower teeth

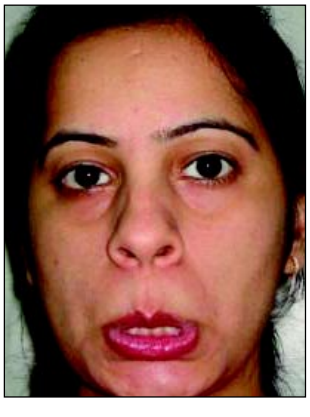


Fig 5.107: Move tongue in front of upper teeth

Breathing exercises

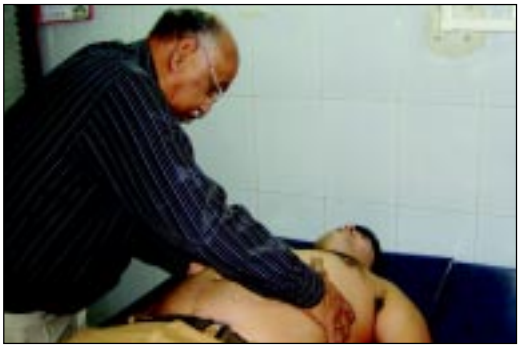


Fig 5.108: Lower costal breathing

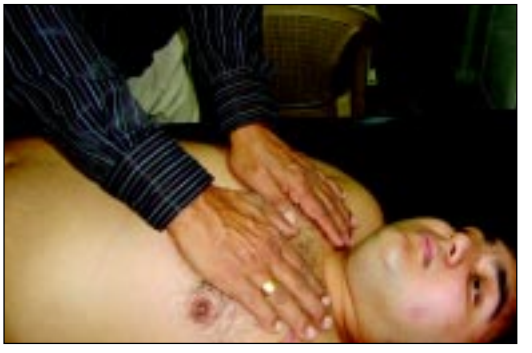


Fig 5.109: Apical breathing

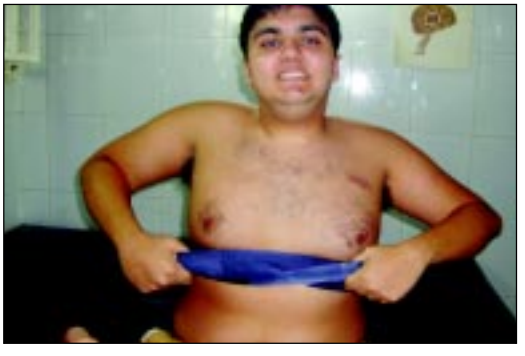


Fig 5.110: Auto assisted deep breathing

Fig 5.111: Diaphragmatic breathing

Activities for persons with muscular dystrophy:

The person should remain active and continue normal activities for as long as possible. Staying active is important, and therefore bed rest should be avoided. Participation in activities like sports gives opportunities to develop a competitive spirit, self-confidence and a way of making new social contacts, which can help to raise self-esteem and reduce social isolation.

Following are a list of activities in which a person with MD could safely participate :-

Cycling

- Cycling is an activity and exercise for movement of legs.
- It can be used for travelling.
- It also helps to maintain a healthy heart.

Fig 5.112: Low geared tricycle

Swimming

- Swimming is an excellent form of exercise for people with MD as it helps maintain muscle strength, movements and good heart health.
- It helps support the body and may be used to exercise limbs which are too weak.
- A warm pool aids in relaxation and soothes sore muscles.

Fig 5.113

Fig 5.114

Computers

- The internet offers a lot of support and information to people with muscular dystrophy
- It is a good way of making new friends and enjoying hobbies, such as downloading music and games.

Caring for a pet

- A pet provides a loving & protective environment.
- The pet extends companionship and can also give hours of enjoyment irrespective of the disability.

Fig 5.115

Horse riding

- In the early stages of muscular dystrophy the person may enjoy sitting on a horse.
- It is a good exercise for helping them to maintain their balance reactions.
- In the later stages of their illness, a ride in the horse-cart may still be enjoyed.

Fig 5.116

Other activities

- Creative writing, painting, graphic art and some crafts, such as model-making, playing cards, etc. help in maintaining fine movements of the hand.
- Singing, blowing whistles or balloons, using musical instruments like mouth organ, flute, etc. help in maintaining breathing capacity.
- Reading the newspaper or books help in gaining knowledge.
- Listening to music helps in relaxing and calming the mind.
- Sedentary activities like watching TV, playing games on mobile are enjoyable activities for most people, but these are more suitable and advised only in the later stages.

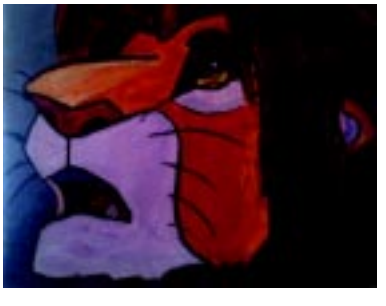


Fig 5.117 *Fig 5.118* *Fig 5.119*

Artwork by people with muscular dystrophy

6:

Assistive devices and alternative techniques

We understand that parents and patients often have reservations in using aids to help mobility or activity. The general concern is that

a) It would make them dependent on these aids,

b) That using mobility aids such as wheelchair makes the child / patient feel more handicapped.

Keeping in mind these concerns, we have put together information regarding use of specific devices for various problems, including hand and leg splints, other assistive devices as well as alternative techniques. This will help you understand better the function of or need for each and how it can help your child / the patient to improve his / her overall function.

We would encourage you to go through this information, so that you will gain a better perspective about how these devices and techniques can make your child's & your life easier and fruitful. A lot of options have been included in this chapter, many of which are available in India. Some of these, which are not available in the markert, can be custom made, taking inputs from an occupational therapist. These would enhance or improve overall efficiency, reduce or delay the development of deformities & scoliosis and hence delay complications. This will lead to conserving of energy, improvement of overall self esteem of the patient and reduction of the stress on the caregiver. However one must remember that if used incorrectly these devices can have harmful effects too. Therefore it is recommended that all assistive devices must be used only under the supervision of your Rehabilitation therapist.

Assistive devices:

1. Adaptive equipment :

Persons with muscular dystrophy may need various pieces of equipment that can help to maintain or improve their quality of life in daily living tasks, school tasks or work tasks. Assistive devices range from very simple & inexpensive (straws) to very complex & costly systems (remote operated door opening system). They are used to compensate for weakness and fatigue. Some of the devices are not easily available in India, but with the help of a local occupational therapist these devices could be purchased or made.

Listed below are some of the assistive devices they may use in different activities:-

- **Equipment to help in eating**
  - Plate guards or high-rimmed plates may help to prevent food from falling off the plate and assist in loading a fork or spoon.

Fig 6.1: Plate guard

Fig 6.2: High rimmed plate

  - Enlarged handles of spoons, forks & knives for better grip.

Fig 6.3

  - Angled head spoons when eating with normal spoons is difficult.

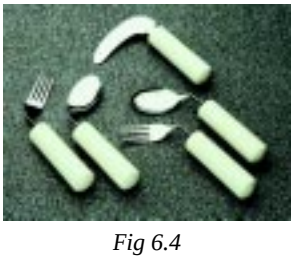


Fig 6.4

– Mobile arm supports when upper arm strength is poor.

Fig 6.5: Mobile arm support used in activities like eating, brushing teeth, etc.

– Use of the 'Arm thing' which is an arm support device, when strength in arms in not enough to bring hand to the mouth.

Fig 6.6: The 'Arm thing'

– Electric feeding tool, which is a system to deliver food in the mouth, for example, a rotating plate and a manually or electrically operated spoon. Some are powered, others are manually operated via hand or foot controls.

Fig 6.7: Electric feeding tool

- **Equipment to help in Drinking:**
  – Lightweight cups and mugs.

– Easy to grip cups having two handles.

Fig 6.8: Light weight, easy to hold cup

– Straws with straw holders when holding the cup is difficult.

Fig 6.9: Straw with specialised straw holder

- **Equipment to help in Grooming:**

– Battery-operated toothbrush.

Fig 6.10

– Battery-operated / electronic shaver.

Fig 6.11

– Enlarged / long handles of toothbrushes / combs.

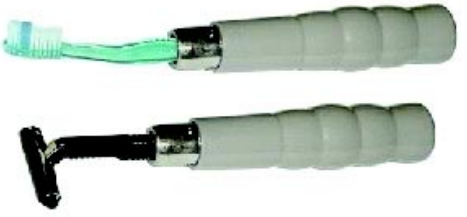


Fig 6.12: Enlarged handle for easy grip

Fig 6.13: Long handle for easy reach

– Toothpaste dispensers, when squeezing of the tube is difficult.

Fig 6.14: Sensor based toothpaste dispenser

– Foam based shaving cream which is easy to apply.

Fig 6.15

– A 'bite support' between the upper and lower teeth may be used to keep the mouth open when opening the mouth is tiring during brushing of teeth.

Fig 6.16

- **Equipment to help in bathing & toileting:**
  - Anti-skid mats & flooring to prevent or reduce falls.

Fig 6.17: Anti-skid mat

Fig 6.18: Anti-skid tile

  - Shower chairs to sit & bathe when standing requires too much balancing.

Fig 6.19: Shower chair with back support and rubber ferrules

  - Bath lifts (The main function of the bath lift is to lower the seated user down in the bath and lift them back up from the bottom of the bath to the height of the bath rim).

Fig 6.20: Bath lifts used mainly in bath tubs

  - Grab bars & railings in bathroom / toilet.

Fig 6.21: Grab bars attached to both sides of commode

– Soap on a string or soap dispensers when holding grip is affected.

Fig 6.22: Soap on a string

– Soap holder worn on hand to apply soap on body.

Fig 6.23: Hand mitt for soap holding and easy application

– Hand sanitizer instead of using soap.

Fig 6.24

– Long handled scrubber / sponges / loofahs to clean feet, back & for hair.

Fig 6.25: Long handled brush for body

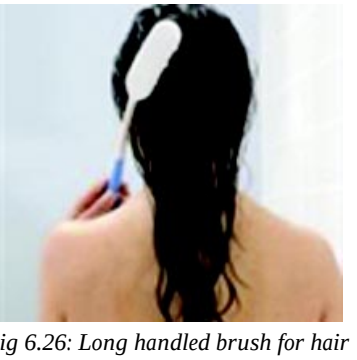


Fig 6.26: Long handled brush for hair

– Overhead / Hand showers when lifting mug of water is difficult.

Fig 7.27: Overhead shower

Fig 6.28: Hand held shower

– Shampoo tray for washing hair at bedside.

Fig 6.29: Shampoo tray

Fig 6.30: Shampoo basin

– Over-toilet chairs (fixed or on wheels) when sitting in Indian toilets is difficult.

Fig 6.31: Commode chair

Fig 6.32: Over-toilet chairs (with wheels)

– Use of toilet paper swipe to clean self.

Fig 6.33

– Raised toilet seats to reduce effort in getting up.

Fig 6.34

Fig 6.35

– Urine pots to reduce the number of visits made to the toilet.

Fig 6.36

– Sensor based taps / built up handled taps when hand grip is poor.

Fig 6.37: Sensor based taps

Fig 6.38: Lever attached to tap

– Bath robe instead of a towel for drying self.

- **Equipment to help in Dressing**
  - T-shirts & elastic waist pants when buttoning or zipping is difficult.
  - Clothes which do not require ironing (anti- wrinkle clothing) to avoid looking untidy.
  - Use of velcro instead of buttons / zippers.

Fig 6.39

– Dressing stick to help when hand function & bending is limited.

Fig 6.40

– Front or side opening undergarments for increasing independence & maintain privacy.

– Zipper pull (a hook connected to an enlarged handle is placed in the eye of the zipper to pull the zipper up or down) for easy in zipping.

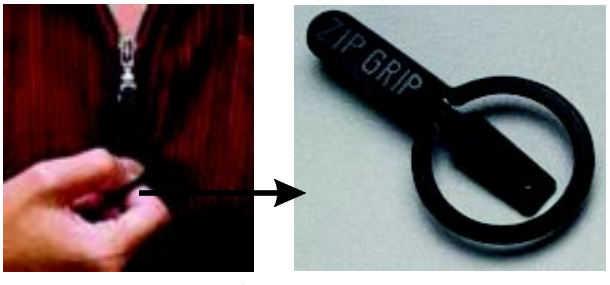


Fig 6.41

– Button hook, oversized buttons with large loops for easy buttoning.

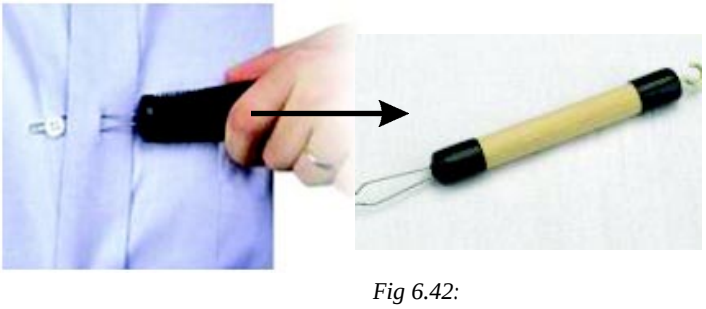


Fig 6.42:

– Reachers for wearing shoes when bending is difficulty.

Fig 6.43

– Sandals with velcro straps instead of shoes with laces.

Fig 6.44

- **Equipment to help in Sleeping**
  – Blocks under the bed to raise the height in order to help the person to get up independently.

Fig 6.45

  – Bed safety rails to provide support for getting up and prevent falls.
  – Hospital-style beds because of the features like side rails, adjustable height and adjustable mattresses for raising or lowering head or feet.

Fig 6.46

  – Firm mattresses for easier shifting in bed.

– Mattress overlays made from foam, rubber, gels or in a honeycomb style designed to be comfortable and encourage good blood circulation to the skin.

Fig 6.47: Mattress overlays

– Alternating pressure / turning mattresses, turning beds. Turning beds where-the entire bed rotates, not just the mattress to prevent pressure sores and to reduce the number of times the person or caregivers have to wake up to change positions.

Fig 6.48: Turning beds

- **Equipment to help in shifting and handling**
  – Transfer boards to shift from one surface to another when lifting self is difficult.

Fig 6.49

– Handling belts to hold the person better during shifting.

Fig 6.50

– Sliding sheets to help turn and shift the person during transferring.

Fig 6.51

– Hoists to lift the person and transfer them where required.

Fig 6.52: A hoist is a piece of equipment used to transfer a person from one place to another in a supportive sling

- **Telephones**

– Use a phone holder instead of holding phone in the hand.

Fig 6.53

– Speed dialing or touch screen phones for easier dialing.

– Hands-free headsets and voice-activated systems to avoid holding phone in the hand.

Fig 6.54: Bluetooth device

- **Adaptive IT equipment:**

– Alternative types of keyboards.

Fig 6.55: Keyboard with touchpad

– Word-recognition software, word-prediction, on-screen keyboard to reduce typing efforts when hand movements are limited.

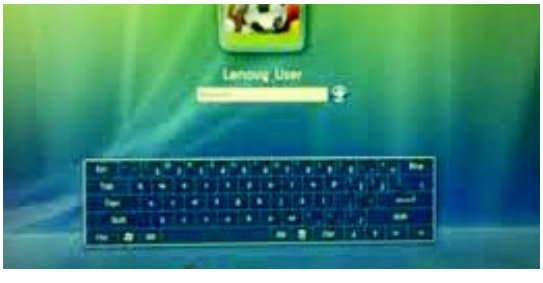


Fig 6.56: On-screen keyboard

– Voice recognition software & voice operated computer systems as an alternative to typing.

– Dictaphones can be used for recording notes.

Fig 6.57: Dictaphone

– Touch screens or touch pads instead of using a mouse.

– Mouse alternatives, such as touch-pad mouse, joystick, trackball or finger operated integral joystick.

Fig 6.58: Touch screen mouse

Fig 6.59: Touch pad joystick

- **School:**

– Slight inclined boards for easy writing.

Fig 6.60: Inclined writing board

– Enlarged pencils, grippers for better holding.

Fig 6.61: Pencil grippers

– Tape recorder to record explanations in class.

– Computers / laptops to write notes / exams.

– Special seating / individual chair to maintain proper posture.
– Special cushion seats for long hours of sitting.

- **Home :**
  – Hand rails & grab bars to use for support.
  – Ramps at the entrance for easy wheelchair entering.

Fig 6.62: Aluminium ramp

  – Double or sliding doors are options for widening.

Fig 6.63: Sliding doors

  – Light switches at a level which can be easily reached from a standing or wheelchair position.
  – Automatic lifts instead of climbing stairs.
  – Stair lift to ascend stairs in later stages when climbing stairs is no longer possible.

Fig 6.64: Indoor stair lift

– Door knob extenders (a long support attached to taps, door knobs, stove handles and lamp knobs for easy opening).

Fig 6.65: Door knob extender

– Remote operated door openers.

Fig 6.66

– Help / Call switch which could be used by a touch or head turn.

Fig 6.67: Call switch

Here is an example of a patient,in Mumbai itself,who has made certain modifications in his house, which has helped him become more independent and less dependent on his caregivers, inspite of his severe muscle weakness.

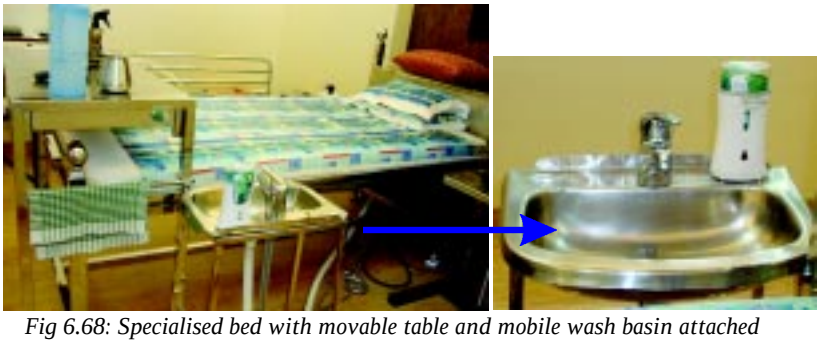


Fig 6.68: Specialised bed with movable table and mobile wash basin attached

Fig 6.69: Chair lift at side of bed

Fig 6.70: Bathroom

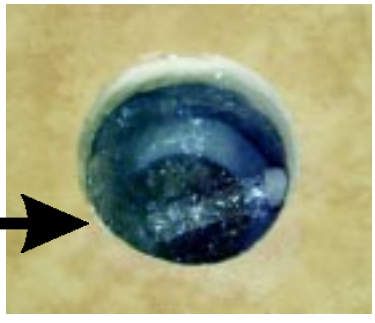


Fig 6.71: Toilet outlet

Fig 6.72

Fig 6.73

Wheel operated surface for moving indoors

Fig 6.74: Mobile bed

2. Orthoses

Orthoses are splints, callipers, braces or anything worn externally to support the limbs or body.

Common orthoses include:

Elbow & Hand splints

- These splints are used to stretch the tight muscles and keep them in a proper position.

Fig 6.75: Resting hand splint

- They are to be worn to maintain a continuous stretch to maximize the effect and also help in daily function. For eg. short cock up splint to help in gripping and a push elbow splint to maintain elbow movement.

Fig 6.76 : Short cock-up splint

Fig 6.77: Push elbow splint

Night Splints

- Night splints are designed to be worn at night and are usually for keeping the feet in a proper position.
- The splint starts at the toes and finishes just below the knee. Hence they are also known as Ankle Foot Orthoses (AFOs).

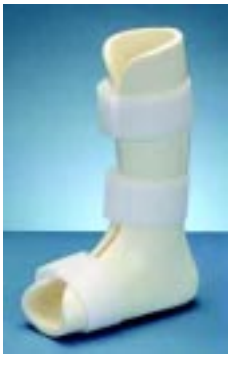


Fig 6.78: Ankle Foot Orthoses

- Along with passive stretching, wearing the night splints is necessary to delay development of contractures.

Callipers (also known as KAFOs - knee, ankle, foot orthoses or push knee splints)

- These splints extend from the toes to the hips and are designed to support the legs to prevent bending of the knee, making it possible to continue walking or standing, despite muscle weakness.
- Usually, it is used only for walking short distances.

Fig 6.79: Knee, ankle, foot orthoses

Spinal jacket/brace

- This is a jacket made to support the back when there is twisting of the spine. It helps the child to be in a straighter position so he can use his arms better and breathe better.
- The role of the jacket is to support the weak muscles to improve body balance and sitting stability.
- Bracing can be used when the surgery is not chosen or when waiting for spine surgery.

Fig 6.80: Spinal brace

Wearing schedule of Splints

- In the beginning the splint or brace needs to be worn for only a short period of time (approx for 10 -15 mins only, at a time) for the person to get used to it.

Gradually they can be worn for 2 hours at a time with breaks after every half an hour. At that time the limb should be moved throughout its range.

- Every time after removing the splint, the skin must be checked for reddened areas or wounds.
- If redness does not go away even after the splint is removed for 10-15 minutes after removing, then check with the therapist. Changes may be needed to be done to the splint.
- To absorb sweat, a thin sock can be worn under the AFO and a vest or T-shirt under the spine brace instead of wearing it directly on skin. An absorbent powder can also be used if required.

3. Standing frames

- When walking is no longer possible and it is difficult to stand without support, a child should still be encouraged to continue weight bearing on legs.
- Standing has health benefits in terms of maintaining the range of movements in the joints and good posture. It is also beneficial for the functioning of all body systems.
- Having the ability to stand also makes transfers much easier. Standing frames, boards and tilt tables are available to support a child in a standing position.
- Some people may make use of a sophisticated wheelchair with a built in standing facility.

Fig 6.81

Fig 6.82

Fig 6.83

Different types of standing equipment

4. Mobility Aids

- Braces, canes and walkers are helpful for the person to continue walking when falls increase due to imbalance & increasing weakness. They also reduce fatigue and give more stability.

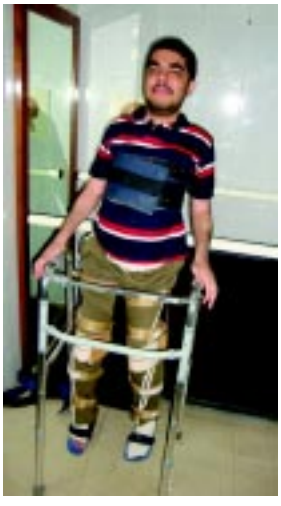


Fig 6.84: Standing with support of walker

Wheelchair

- As the young person's condition worsens and the walking ability decreases, it will become necessary for him to consider using a wheelchair for long distances. While he or she will be encouraged to walk as much as possible, as it maintains muscle strength, prevents deformities and osteoporosis (reduction of calcium from the bones) and helps to control weight gain; there will come a stage when he is unable to walk at all. At this stage, he may have to think of using a wheelchair for indoors & outdoors.
- Initially, only a manual wheelchair may be required for use. Basic requirements of a wheelchair include a firm seat and back, seating to support good posture, removable arm rests and swing-away footrests. Lap boards can be used to do table activities instead of moving to a chair.

Fig 6.85: Manual wheelchair with lap tray

- When the muscles of the arms become weaker, propelling a wheelchair may become difficult, in which case, using a wheelchair which is electronically operated may be a better option. When a person cannot sit for a long time in a straight position, a reclining back rest can also be thought about.

Fig 6.86: Powered wheelchair

- Wheelchairs are also available which move the person from a sitting position to a fully supported standing position just at the press of a button. Gradually with time, a trunk strap and head & neck support will be also required.

Fig 6.87: Powered sit to stand wheelchair

Seating of wheelchairs

- When the person begins to use a wheelchair, the seating will require special attention.
- The seating will have to provide correct amount of postural support and pressure relief to reduce the risk of contractures, deformities & pressure sores and to maximize comfort.
- Good posture support may also help to preserve chest & lung function. The wheelchair should have a back support that helps improve the posture & minimize development of spine deformities and a cushion, which helps relieve pressure.

Scooters

- A motorized scooter is helpful when walking long distances is difficult and tiring. Scooters can be used only by a person who is able to sit straight for a long period of time, has fair arm strength & fine motor control.
- There are several options to choose from when buying a scooter: three- or four-wheeled models for balancing.
- Some models are made of very light materials, designed to be dismantled for transporting, for example, in the boot of a car, boat, airplane or train.

Fig 6.88: Battery operated scooter

Fig 6.89: Indian supportive wheel scooter

Cars

- Hand operated controls enable persons with muscular dystrophy to drive a car long after they stop walking.
- The brakes, accelerator and the clutch are all controlled by hands.
- There are a variety of hand controls on the market but the best one should be decided along with the caregiver, patient & therapist.
- Some cars also can also have ramps which allow the person to enter the car on the wheelchair.

Fig 6.90: Hand operated car controls

Fig 6.91: Car with ramp

Alternative Techniques

Alternative Techniques are compensatory strategies used to carry out daily activities of living when normal movements are difficult, for e.g. supporting elbows on the wash basin while brushing teeth. Below are some very simple adaptations which can be used to achieve the desired result:

In the home

- Dress / bathe / brush teeth by sitting instead of standing.
- When walking has stopped, the person can crawl while moving in the house.
- Bend from waist & neck while combing hair or eating when overhead activities are difficult.
- Bend on high level table / bed while doing upper body dressing / undressing.
- When washing hair, take support by sliding hands up the wall to reach the head.
- For eating, brushing or combing hair, support the elbow with the other hand or on a few books or pillows or a high table at shoulder level, to help in bringing the hand to the face.

Fig 6.92

Fig 6.93

Fig 6.94

Alternative method of brushing teeth, combing hair and eating

- While wearing shoes, fold one leg and put it on the other so that putting shoes on the bent leg is easier.
- When chewing and swallowing muscles become weak, eat soft / mashed foods.

In the classroom

- Ideally, the classroom should be located on ground floor or the school should have the facility of a lift.
- Height of the chair and table should be adjusted to encourage independence for as long as possible and for ease in getting up.

- The child's chair should be near the door on the first bench for him to reach it easily.
- The child should be allowed to leave before others (10 mins before time) when leaving the classroom or remain until the last person leaves, to avoid rush in corridors.
- Photocopies of notes could be provided to the children instead of noting down.
- The child should be allowed to use a laptop computer to enable him to keep up with school work, as writing will become harder with time.
- Additional time should be given to complete class work & writing exam papers.
- As the weakness progresses, when the child becomes unable to write, exams should be taken in the form of orals instead of written or a writer should be provided.
- Sports period should be adapted for the muscular dystrophy kid, for e.g. playing with a ball, cricket and badminton in sitting. Later on, games like chess, carom or scrabble etc. can be given.
- The desk should have a locker facility in which a second copy of all the books can be kept instead of carrying a bag from home.
- The other kids at school can take turns with helping the child by giving support while walking, carrying books / bag for the next class or copying notes, etc.
- Additionally, all kids with Muscular Dystrophy should:

  Be given short breaks as needed.

  Be allowed to play physical games at their own pace.

At work

- Jobs which involve sitting activities like supervising, taking calls, teaching are more suitable vocations for muscular dystrophy affected persons.
- But jobs which require more of standing or travelling (especially using public transport), climbing stairs, bending are not advisable.
- Lifts should be used if available instead of climbing stairs
- Revolving chair could be used when the office is not wheelchair accessible. It helps in moving around independently or is easier when one needs to be pushed by someone else.
- For any other chair, a raised chair could be used with a table of suitable height.
- Computer could be used instead of writing.
- Special keyboard, mouse, etc for helps in energy saving.
- Shelves should be positioned at easily reachable levels e.g. (waist level).
- Grab bars or railings in toilets should be installed.
- Raised toilet seat should be used.

Fig 6.95: Revolving chair

Fig 6.96: Chair raised with help of blocks

Following are the lists websites where assistive devices are available and can be ordered from:

- www.pedderjohnson.com PEDDER JOHNSON for mobility Aids, daily Living, bedroom, bathroom & toilet accessories
- www.assistivetech.net The National Public Website on Assistive Technology for mobility Aids, daily Living, bedroom, bathroom & toilet, recreation and communication accessories
- www.apd-india.org Association of People with Disability for light weight assistive devices like knee ankle foot orthoses, ankle foot orthoses, spinal braces, crutches, footwear, walkers, tricycles, wheelchairs, multipurpose chairs and home assistive devices, toilet commode wheelchair, motorized tricycle and petro model for two wheelers bars and rails, specialized seats, modified western commodes, mobility equipment, transfer assists and ramps.
- www.disabilityproducts.com Disability Products for mobility Aids, daily Living, bedroom, bathroom & toilet accessories.
- www.mtedevices.com MTE Devices, Inc for the Arm thing

Stores and Facilities Dealing with Assistive Devices

- **Artificial Limbs Manufacturing Corporation of India**

  Manufacturers of : Othotics, Spinal braces, Mobility Aids

  Head Office: Artificial Limbs Manufacturing Corporation of India, G. T. Road, Kanpur - 208016. Tel No. 0512-2700051/2770897, Fax No.0512-2770617 Email : alimco_hq@vsnl.net Website : www.artlimbs.com

- **SeniorSSuperStoreS**

  Manufacturers of: Self care Devices, Mobility and Transportation Devices, Devices for House Work, Safety / Security Devices, Leisure Activities Devices, Communication Devices, Dressing Devices

  Contact Address: SeniorSSuperStoreS 4115, Highway 813 Conway, SC 29526, Phone: 843-421-3766, Toll Free Phone 1-888-719-7938, E-mail: mail@seniorssuperstores.com, Web:http: / / www.seniorssuperstores.com /

- **Navchetan Orthopedic Appliances**

  Manufacturers of: Splint, Brace, Finger Splints.

  Contact Address: Opp. ST Depot, Jamnagar 36005, Gujarat. Tel.: + 91 - 288 - 2564198 Fax : + 91 - 288 - 2564198, E-mail: info@navchetan.com, Web:http: / / www.navchetan.com /

- **MP Industrial Corporation**

  Suppliers of specially modified scooters, mopeds and auto rickshaws

  585, Bhagirathpura, Indore 452 003. Phone: (0731) 2423404, 2422260,

- **Surgico-Furn (India) Ltd**

  Manufacturers of: Tricycle, Wheelchair fully reclining, Wheelchair fixed and folding, Commode pot chairs, Walkers

  E 77 / 1 BSR Industrial Area, Ghaziabad. Tel: 0575-702232, Fax: 0575-752627 Email: surgico_furn_ltd@hotmail.com

- **New Generation Ortho-Prosthetic**

  Manufacturers of: Polypropylene functional braces, Walking calipers, Spinal braces, Walkers

  Contact Address: 66-A, Street No, 2, Krishna Nagar, Near Baba Balak Nath Mandir, Safdarjung Enclave, New Delhi 110 029. Phone: 011-26164999 / 26109773 E-mail: rjain@vsnl.net.in Vimal.cpo.@hotmail.com

- **SAGE (Everest Engineers)**

  Manufacturers of: Wheelchairs, Walking aids, Commode chairs

  184 - A, Peera Grahi, Ramlila Park, Delhi 110 041. Tel.: 5587151 Fax: 5673476 Web: http: / / www.sageindia.com

- **Otto Bock India**

  Manufacturers of: high-end wheelchairs, foam seating cushions, gel cushions,

  OTTOBOCK Healthcare India Pvt Ltd., Behind Fairlawn Housing Society, Sion Trombay Road, Chembur, Mumbai 400 071. Tel.: (022) 25201268. Fax: (022) 25201267; E-mail: otto@bom5.vsnl.net.in

  No. 1, Masjid Moth, Opposite Niti Bagh, New Delhi 110 049. Tel.: 011-2625 4739 Fax: (011) 26251876; E-mail: otto@nda.vsnl.net.in; Web: http: / / www.ottobock.in

- **Technical Automotives**

  Manufacturers of: Rajhans auto tribikes, Tricycle, Wheelchairs, Walking aids, Walking frames, Low cost calipers, Commode, Urine pot / bottle

  Contact Address: 8 / 3 Old Ganga Bridge, Unnao 209 001, Uttar Pradesh, Post Box No 460, Kanpur 208 001. Phone: 843-421-3766; Phone: 0512-2825158 / 2825539

- **Vir Engineers**

  Manufacturers of: Kinetic Honda 100 cc adapted motorcycles, Chetak 150 cc adapted motorcycles

  Contact Address: 145, Bharat Apartments, Sector 13, Rohini, Delhi 110 085 Phone: 011-27860991

- **Sammons Preston,** a Division of Patterson Medical (formerly Sammons Preston Rolyan)

  Manufacturers of: Mobility Devices, orthosis, daily living, bathing & toileting accessories

  Contact Address: Sammons Preston, a Division of Patterson Medical (formerly Sammons Preston Rolyan) P.O. Box 5071, Bolingbrook, Illinois 60440-5071 United States. Phone: 843-421-3766; 800-323-5547 or 630-378-6000; Fax: 800-547-4333 or 630-378-6010; E-mail: customersupport@patterson-medical.com; Web: http://www.sammonspreston.com.

- **Scooterville Discount Mobility**

  Manufacturers of: Mobility Devices

  Contact Address: 1536 Myrtle Avenue, Monrovia, California 91016, United States Phone: 800-994-0454 or 800-689-0030; Fax: 626-628-3956; Web: http://www.scooterville.com

- **Carex Health Brands** (formerly Apex Medical Corporation and Carex Health Care Products / Carex Rubbermaid)

  Manufacturers of: Dressing Devices

  Contact Address: PO Box 2526, Sioux Falls, South Dakota 57101-2526, United States Phone: 800-328-2935 or 800-526-8051; Fax: 888-616-4297; E-mail: customerservice@carex.com; Web: http://www.carex.com

- **Intercodev Inc. / GripAdvantage**

  Manufacturers of: Dressing Devices

  Contact Address: Division of Intercodev, Inc., 1178 N. Spend A Buck Drive, Hernando, Florida 34442, United States. Phone: 800-272-4919 or 352-344-2771; Fax: 352-344-3327; E-mail: info@gripadvantage.com; Web:http://www.gripadvantage.com

- **Simple Comforts**

  Manufacturers of: Recreational Aids

  Contact Address: 410 Belle Air Lane, Warrenton, Virginia 20816, United States. Phone: 800-361-1440 or 540-341-7135; E-mail: sales@simplecomforts.com Web: http://www.SimpleComforts.com

- **The Wright Stuff, Inc.**

  Manufacturers of: Self care devices

  Contact Address: 111 Harris Street, Crystal Springs, Mississippi 39059. United States Phone: 877-750-0376 or 601-892-3115; E-mail: info@thewright-stuff.com; Web: http://www.thewright-stuff.com.

- **The Caregiver Partnership**

  Manufacturers of: Self care devices

  Contact Address: 333 North Commercial Street, Suite 350 Neenah, Wisconsin 54956, United States. Phone: 800-985-1353 or 920-729-7091; Fax: 920-729-6457; E-mail: tomw@caregiverpartnership.com;
  Web: http://www.caregiverpartnership.com.

7:

Care for caregivers

Most handbooks or instruction guides are focused on the patient only. With the patient, we believe, due consideration needs to be given to the caregivers health, emotional as well as physical. Along with the patient, a lot of changes and adjustments happen in the caregiver's life. So, it important for them too, to take care of their health. This chapter is solely focused on suggestions for caregivers, which could help them pick up signs of stress and its effects earlier, so that they can rectify or minimize them by taking steps at appropriate time. To be able to help your child or family member recover from their problem in the best possible manner, you have to be healthy and strong yourself. An unconnected but interesting example to highlight this is the flight safety instruction that is given to all passengers before the start of any flight. Among the many instructions given is one on the use of Gas masks if the cabin pressure falls during the flight. The instruction given to parents with children is always like this " those with children, first put on the mask yourself and then put on the mask for your child". What this means is that you have to be well first before you can help your child.

Caring for caregivers

Caregivers of persons with muscular dystrophy are people who take care of them, often parents or spouses. Some caregivers are family members; some others are paid help. They help the individual with:

- Activities of daily living like going to the toilet, bathing and dressing, eating etc.
- Exercising.
- Moving from one place to another.
- Positioning.
- Wearing and taking off splints or braces.
- Taking medicine.

Caregiving is not an easy job as it involves 24 hours a day of caring for the individual by the time the person reaches the last stage of muscular dystrophy.

During the period of their role as a caregiver, some of the problems faced by parents & spouses are: added physical stress to present work, increased financial stress of medications, assistive technology etc, lack of sleep, change in life pattern, social isolation from friends & family due to lack of time and emotional stress due to worry & concern about the future.

Signs that the caregiver is suffering from stress:

- The person feels tired throughout the day.
- The person experiences behavioral changes like feeling overwhelmed and irritable.
- The person tends to sleep for a long time or very little.
- The person gains or loses weight too quickly.
- The person loses interest in activities which were earlier enjoyable.

Tips to help caregivers:

Caregivers should always remember that if they don't take care of themselves then they won't be able to look after the one they care for.

Below are some tips suggested for caregivers:-

1. Don't be over-protective. Encourage the person with muscular dystrophy to do daily activities by himself as much as possible.
2. Use adaptive devices or technology to make him more independent (e.g. use of a long handled scrubber for bathing) and reduce physical stress of the caregiver (e.g. use of hoists or lifts instead of lifting). (Refer to Chp 6)
3. Talk to family or friends & take help, to get some relief from duties.
4. Exercise regularly to promote better sleep; reduce tension & depression and increase energy and alertness. (Refer to a physiotherapist or doctor first)
5. Call & keep in touch with family & friends even though it may be for few minutes in a day through telephone or social sites like Facebook.
6. Do things which are enjoyable like watching comedy movies / serials as laughter and joy can help reduce stress.
7. Indulge in hobbies / fun activities with the affected individual to strengthen emotional bonding.
8. Join a social or support group to meet people going through the same phase and share thoughts & ideas.
9. Use energy saving techniques (mentioned below) to reduce tiredness.
10. Refer to a physiotherapist or occupational therapist regarding proper handling

and moving techniques, especially to protect ones back.

11. Seek professional help regarding the concerns felt & appropriate medical care whenever required without delay.

Energy Saving Techniques

The main aim behind energy saving is to reduce unnecessary energy expenditure in the body. The five major principles which can be used into daily activities are:-

1. Organize the day, activities & environment

- Plan the daily activity schedule, alternating with heavy and light tasks. Also eliminate unnecessary steps of a task when possible.
- Gather and arrange supplies or tools for daily activities before start.
- Plan to do the heaviest work when feeling most energetic.
- Organize environment to reduce excessive carrying, bending & reaching.
- Avoid or reduce tasks that aren't very important or seek assistance to utilize energy for more liked tasks.

2. Use energy saving tools/equipment

- Use of electric appliances to save energy

  E.g. electric shaver, washing machines, microwave oven etc.

Fig 7.1: Automatic washing machine

Fig 7.2: Microwave oven

- Use assistive devices such as long handled reaches, to minimize the need to bend over to pick up objects from the floor. (Refer to Chp 6)
- Use equipment which is easy to hold e.g. spoons, brushes / combs with built up / enlarged handles. (Refer to Chp 6)
- Replace existing heavy items with lighter ones; for example, use plastic plates & cups rather than china & glass.

- Use wheeled trolleys to assist pushing and carrying heavy objects.

Fig 7.3

3. Work with adequate rest breaks

- Take enough rest on completing a task and before moving onto the next one.
- Always rest before getting exhausted.

4. Avoid tiring and faulty postures

- Sit down for doing activities whenever possible. Avoid tasks that require prolonged standing, squatting or stooping.
- Avoid raising your arms too high above shoulder level.
- Slide objects instead of lifting them.

5. Use of proper body mechanics

- Keep your body straight while performing a task, poor posture consumes more energy.
- Avoid rotating the trunk while bending.
- While lifting / placing heavy objects from / on lower surfaces, bend from knees instead of the back.

Fig 7.4: Wrong way of bending to pickup

Fig 7.5: Right way of bending to pickup

- Keep your arms straight and close to your body while carrying objects, thus dividing the load equally between both arms at the same time.

- Support your elbows on a table or a firm surface while performing a task to avoid positions that make you tired, e.g. during brushing, reading etc.
- Push instead of pulling objects.

A small real life learning incident from Dr. Alok Sharma:

In January 2001 there was a devastating earthquake in the Kutch area of India. I was leader of one of the medical teams that was flown in by the Air force and we set up a medical camp in a village which was severely affected. Almost all the houses in this village had collapsed and half the people were dead. We used to do surgery there on a bullock cart. But before we and the army reached the villagers there without any help from the outside had managed a significant amount of relief work already. Once in the night as we sat by the fireside (there was no electricity), I asked the villagers how come they had organized their own relief work so effectively before we arrived. They told us something that changed the way we looked at life. They told us that immediately after the earthquake the village elders got all the people who were still alive together and gave them just one advice and suggestion. They told the villagers. "Lets not look at or think about what we have already lost. Let's focus on what is still left and try to make the best of that". These simple words were so inspirational. There was no family in the village who had not lost someone. But instead of grieving about their loss each and everyone of the people there started working to salvage what and who was still left. Each one was given specific duties irrespective of what and whom they had lost. Instead of grieving about their losses these people were now working to helping those who were still alive.

There is a big lesson we all, especially the family caregivers, have to learn from this incident. In our patients instead of being upset about what the patients cannot do we need to focus on what they still can do and work towards enhancing that aspect therefore improving the qualities of their lives to the best possible manner despite the disease and disability that they have.

The present is the only time when we can work and achieve, gain and gather. In the past we can now do nothing. In the future again we can accomplish nothing. In the 'dead' moments of the past and in the 'unborn' moments of the future we can never act. These 'living' moments are the only fields to be hammered at and wherein are all the glories of life, all the gains in existence.

Time never stops. It is fleeting. The now is the only auspicious occasion to initiate our new plans. Delays are dangerous, useless and barren. Today is the only day to attempt any great and worthy purpose. Opportunities come to all of us. The diligent catch hold of it. The foolish let it pass. Therefore us be smart and awake to recognize our opportunity to serve and while it is within our reach let us seize it and make it yield to us the results we demand.

What we have is a gift from God. But what we do with that is our own gift to God. Make your life a total gift to God.

- Swami Chinmayananda

SECTION - C :

Recent Advances

8:

New Drug Therapies

The last decade has seen a significant increase in the number of clinical trials being conducted to explore the possibility of various newer drugs & molecules as well as modifications in the indications for use of older drugs to improving various aspects of muscular dystrophy. Most of these drugs are still in Trial phase and will take a few years to be available for regular use depending on the results of these studies. The status of these as listed below is as of the date of publication of this book which is September 2012. The status will change in the coming years as results come in from many of these studies. (Some of the newer research drugs are mentioned here initials and a number. This is the way under trial drugs are referred to by researchers. These are not the actual names of the drugs)

Some of the drugs being studied are listed here:-

[1] TadalaFil (Cialis) and Sildenafil (Viagra) (Status - actively recruiting)

Tadalafil and Sildenafil are PDE inhibitors. Studies have already being published showing the benefeicail effects of these in mice. A acute dosing study is also being conducted at the Cedars -Sinai Medical center on whether Sildenafil and Tadalafil can improve the muscle blood flow during exercise in boys with Duchenne. More information of this can be obtained at www.ClinicalTrials.gov. This is an actively recruiting clinical trial.

Another study on effect of Sildenafil in muscular dystrophy is being carried out by Nicholas Whitehead, University of Washington.

[2] Sildenafil (Viagra) and Prednisolone: (Status - preclinical)

A combinatorial use of Sildenafil and Prednisone to treat Dystrophin-Deficient cardiac and skeletal muscle is being considered for slowing the progression of skeletal muscle degeneration and heart disease .This could in turn improve quality of life, extend

longevity and allow genetic correction at later stages of the disease. Studies of Viagra, an FDA drug that amplifies the nNOS (nitrous oxide)signaling pathway, demonstrated a marked improvement in heart and diaphragm function in the mdx mouse. Currently, it is being determined whether sildenafil and prednisone can be used in combination. This will later pave the way for a clinical trial on young DMD boys.

This is being carried out by Stan Froehner, Ph.D..Data on mice has been completed and compiled.

[3] CAT1000: (present status - preclinical)

CAT1000 chemical group has the action of reducing inflammation (swelling of muscle tissue) and hence, decreases / reduces the speed of degeneration of muscle. Initial testing is being initiated in mice. If successful clinical trials in human patients will start in 1-2 years. Could benefit all boys with duchenne and Becker. Will be a oral medication if the trials show benefits. More information on this is available at www.catabasispharma.com

[4] HT-100: (present status - preclinical)

This drug can decrease fibrosis(conversion of muscle into non elastic fibre like strands / strings / tissue) and allows normal muscle to be formed again. This may help patients to improve heart muscle, muscle of breathing also. Clinical trial / study in patients is expected to start in 2012. More information on this can be obtained from www.halotherapeutics.com.

[5] Spironolactone: (status - preclinical)

The use of this drug in muscular dystrophy is still in very early stage of research. It has been observed that a profound improvement in a mouse model of Duchenne muscular dystrophy resulting from treatment with the FDA approved drugs lisinopril and spironolactone. Muscle strength in skeletal muscles in limbs and those used in respiration was doubled in treated mice compared to untreated mice and function of the heart was also significantly improved. That means improvement in breathing muscles, limb muscles and heart muscles seen in mice with muscular dystrophy. Ongoing muscle damage in skeletal muscles and heart was almost completely prevented. Defining the parameters required for optimal treatment of dystrophic mice is ongoing. This will help in designing clinical trials for the DMD patient population. Study being carried out by Jill Rafael-Fortney, Ph.D. More information can be obtained at www.biomed.osu.edu.

[6] Tamoxifen: (status - preclinical)

This is drug used for breast cancer. Has been found to improve muscle strength, reduces fibrosis and reduces CPK levels in mice, which do not have dystrophin (mdx mice). Research is being conducted in Dr. Urs Ruegg's laboratory. Still in the early research phase. Will be potentially beneficial to all boys with Duchenne and Becker. When the clinical trials start information will be available on www.Clinical Trials.gov.

[6] VB15 : (status - preclinical)

Glucocorticoids (steroids) remain the only drug treatment clinically recognized to improve muscle function in DMD. Their use is, however, often limited by significant side effects such as weight gain, bone weakness, impaired growth, and unfavorable changes in metabolism.

VB15 is a new molecule which is similar to glucocorticoid / regular steroids. It may work better than regular steroids with less side effects. It effects has been studied in muscular dystrophy (mdx) mice. This Resaerch is being conducted by ReveraGen Biopharma Inc> More information van be obtained at www.reveragen.com.

9:

Gene Therapy and Gene Products

The final solution to muscular dystrophy is likely to come from Gene Therapy. Not only parents and patients, but also the medical community too is curious about happenings in the field of gene therapy and its potential for muscular dystrophy. Expectations from these research areas are very high. A summary of the current ongoings and progress in gene therapy and use of gene products is cited here.

[1] Biglycan:

A new method to provide substitute / stand in protein, "utrophin"(instead of dystrophin) to muscles. This could help reduce muscle damage and improve muscle function.

Still in animal experimental stage.

Tivorsan Pharmaceuticals is developing and will be commercializing a recombinant, humanized form of biglycan (rhBGN) suitable for all genetic forms of DMD.

Biglycan is a good candidate for chronic DMD treatment for several reasons: (i) Due to its similarity to the natural human protein expressed in developing muscle, rhBGN is expected to be safe, well tolerated and carry low risk for immunogenicity in humans; (ii) Since biglycan binds striated muscle-specific sarcoglycans, rhBGN could have preferential / selective activity for the muscles responsible for DMD; (iii) rhBGN works through a unique mechanism of action and is effective when administered systemically-administered. In dystrophin-deficient (mdx) mice, rhBGN increased utrophin expression at muscle cell membranes with a corresponding improvement in muscle structure and function.

Human clinical trial will be funded by PPMD(Parent Project Muscular Dystrophy). More information on this can be obtained from www.tivorsan.com

[2] Follistatin gene transfer:

Injection of this gene into muscle can help increase size of muscle and strength of

muscle.It has to be carried by a virus(adeno associated virus). The proposed project consisted of two aims::a)vector production for the clinical trial and conducting the clinical trial. At the present time the toxicology-biodistribution study using AAV1.CMV.FS344 is underway at Charles River Laboratory, INC.

The first aim has been achieved.The second aim,i.e., conducting a clinical trial is starting for Becker MD patients;1st phase of study in patients by Dr Jerry Mendell,Columbus, Ohio. More information on this can be obtained at www.nationwidechildrens.org/ center-for-gene-therapy.

[3] Laminin:

A chemical / protein, which increases / raises level of 2 more proteins / chemicals (integrin and protein),which brings back muscle strength. Safety in mouse is still to be established. Still in preliminary or very early stage of research.

This project by Bradley Hodges, Prothelia has the objective is to evaluate Lam III, Integrin as a potential therapy for DMD and develop a protein which can be delivered intravenously. More information on this can be obtained at www.Prothelia.com

[4] Utrophin:

Research to find small sized chemical / compounds, which will increase Utrophin (cousin of dystrophin) in muscle.

Currently, successful in mouse. Research ongoing to understand availability / use when given orally, as well as safety considerations. More information on this can be obtained at www.ptcbio.com.

[5] Project: SMTC1100, as a candidate utrophin upregulator for Duchenne Muscular Dystrophy

Summit plc (Jon Tinsley, Ph.D) have taken this alternative pharmacological approach to DMD. This strategy could be appropriate for all patients irrespective of their dystrophin mutation .It would also have the potential to target skeletal and cardiac muscle. We have been developing novel small molecules which can transcriptionally upregulate the utrophin gene. SMT C1100 was the final product of an exhaustive chemical screening and optimisation campaign.

In the recent Phase I clinical trial sponsored by BioMarin, SMT C1100 (BMN195) achieved plasma exposure marginally below the predicted efficacy levels and was deemed safe with no reported a serious adverse events. Fast track assessment of a second readily available formulation of SMT C1100 in a new Phase I healthy volunteer clinical trial is being tried . More information on this can be obtained at www.summitplc.com

[6] Eteplirsen (AVI-4658)

This is an ongoing clinical trial. One part of the study is over(phase1 / 2 & 2b).This trial is being carried out by AVI BioPharma.

AVI-4658 consists of short pieces of DNA called "antisense oligonucleotides" or "AONs"

that are being tested for their ability to convert deletions near Exon 51 in the dystrophin gene from non-functional "out-of-frame" deletions to more functional "in-frame" deletions, such as those typically seen in boys and men with Becker muscular dystrophy. The strategy is commonly called "exon-skipping". Through this technology , an attempt is being made to try and form functional dystrophin (active protein), even if it is a short version. This will inturn help to improve, stabilize or slow down disease process.

Results till date are encouraging and show "dystrophin" protein can be reformed, when(E) is given over 24 weeks. Patients did not show any serious side / adverse effects. More information on this can be obtained at wwwavibio.com

[7] PRO044

This clinical trial is active but not recruiting any participants.1st phase / stage of study going on. This drug is being targeted for exon 44 absence. The purpose of the current study is only to know whether PRO044 is safer and what dosage can be used. Study results expected by 2013. Other potential exons which can later be targets- 45,53,52,55. More information on this can be obtained at www.Prosensa.com

[8] Ataluren:

This study is in phase / stage 2.It is aimed for patients with "nonsense mutations" in DMD / BMD. Nonsense mutation is a defect in the gene / coding, which causes protein production to stop before it is formed completely / fully. If made, this medication will be for oral use / can be taken by mouth. More information on this can be obtained at www.ptcbio.com.

[9] ACE-031(hgt-4510)

A drug was being tested and studied for DMD. Currently, study terminated or stopped by US FDA.Is currently being reviewed for adverse events noticed, such as nose bleeds, gum bleeds, dilated blood vessels within the skin, which were noticed in some children.

Goal was to find out whether this drug is safe to be used and what effect it has on muscle strength, mass and function. Before the study was terminated, positive results were observed. The role of this molecule at the cellular level was to prevent other proteins, like Myostatin(which stop muscle growth) from acting on ActRIIB.

The company conducting this trial, Acceleron and Shire, have issued a statement reinforcing their commitment to the global DMD clinical program and the development of ACE-031. To that end, their intention is to start new studies of ACE-031 in DMD with appropriate safety monitoring following discussions with regulatory agencies. More information on this can be obtained at www.AcceleronPharma.com

[10] Gene transfer of human-alpha -sarconoglycan and gamma - sarconoglycan (with a viral vector) is also ongoing. This aims to evaluate safety and effectiveness of gene therapy in treating children and adults with LGMD2D and LGMD2C

10:

Stem Cell Therapy in Muscular Dystrophy

Discussion ({A} The Dilemma, {B}The Debate, {C} Suggestions)

{A} The Dilemma:-

A major dilemma being faced nowadays by parents and patients of Muscular dystrophy is as to whether they should consider Stem Cell therapy as a treatment option. On one hand they are flooded with information on stem cells through the press, media, internet etc and on the other hand they are told by their primary doctors that this treatment is still unproven or that it does not work etc. These diametrically opposite views create a lot of confusion and conflict in the minds of the patients and their families.

In this chapter we shall give some basic information on what stem cells are and how it is done and shall share some of the clinical results obtained with this therapy. Greater details on the methods and the results are available in our previous book "Stem Cell Therapy and other recent advances in Muscular Dystrophy" which can be freely download from the website **www.neurogen.in**. The information here is meant to empower you to make informed choices. However we wish to state at the outset that since the authors of the book have treated almost 350 cases of Muscular dystrophy with Stem cell therapy and have observed and reported significant improvements with no major side effects or complications, there will be a bias in this chapter towards Stem cell therapy. Readers should however understand all aspects of this therapy and then make informed choices themselves.

The fundamental questions that arise in the minds of parents and patients are :-

[1] Does Stem cell therapy work for Muscular dystrophy ?

[2] Are there any dangers or risks of doing this therapy ?

[3] What improvements are likely to be seen in the patients with this therapy ?

[4] How do I chose a good center for stem cell therapy and how do I know for sure whether the center I have chosen for stem cell therapy is working to high professional, scientific and ethical standards ?

{B} The Debate :

There are two sides to this debate and we shall address both.

{I} **Point of view - 1 (reasonable view) :-** That Stem cell therapy is safe and works in muscular dystrophy patients in terms of functional improvements and halting / slowing down the disease progression

{II} **Point of View 2 (reasonable view) :-** That Stem cell therapy is not a proven treatment and we are not sure that it works.

In an extreme form these two above points of view sometimes get expressed as :

Point of View 1 (extreme view) :- Stem cell therapy is a definitive cure for muscular dystrophy

Point of view 2 (extreme view) :- Stem cell therapy is a dangerous, banned and unethical form of treatment.

We believe that it is important to listen to, read about and discuss all the points of view before we finally subject our children or ourselves to any treatment that is new.

Lets first look at the :-

{I} **Reasonable Point of view -1(For Stem Cell Therapy):-** which is that Stem cell therapy is safe and works in muscular dystrophy patients in terms of functional improvements and slowing down / halting the disease progression.

For the above:-

Is Stem Cell Therapy Safe ?

To understand this we must first realize that stem cells are not one single entity. There are broadly speaking three different types of stem cells. These are embryonic stem cells, umbilical cord derived stem cells and adult stem cells. Details of these are given later in this chapter. Whereas it is true that embryonic stem cells are potentially dangerous (due to the possibility of their forming tumors called teratomas) and have various ethical issues associated with them , umbilical cord and adult stem cells are not dangerous in any way (there is no risk of tumor formation with them) and are not associated with any major ethical issues. It is the lack of understanding the fact that there are different types of stem cells and that the risks associated with one are not applicable to the other is what creates a lot of confusion.

There are several scientific publications to show that umbilical cord and adult stem cells are safe. In fact a review of all the publications based on these show that there are virtually no major adverse events reported that are connected to these types of stem cells. Based on all the scientific literature and our own clinical experience we can say with a reasonable surety that Adult Stem cell therapy is safe and without any major or significant risk factors.

Is Stem Cell Therapy Effective for Muscular dystrophy ?

Regarding the effectiveness of stem cell therapy in muscular dystrophy we would like to speak primarily of our own experience which is based on over 350 patients treated to date. We have used bone marrow derived autologous adult stem cells which were injected intrathecaly and intramuscularly. Later in this chapter some details of these results are given of 235 patients having a minimum of one year follow-up. Some of this work has been published in scientific peer reviewed international and national journals as well as in a book. 93 % of our patients showed some form of improvement by the end of one year and in all of them the disease progression had halted. (18% showed a significant improvement, 31% showed a moderate improvement and 44 showed a mild improvement). Only 7 % did not show any improvement. The improvements seen were in trunk strength, upper and lower limb strength , standing and gait. Apart from the clinical improvements, MRI scan done 6 months after the treatment showed radiographical evidence of muscle regeneration and EMG studies showed improvements too. Based on this we can state with a reasonable and reliable confidence that Stem Cell therapy is effective in slowing down / halting the progression of muscular dystrophy and that it produces functional changes that improve the quality of life of our patients.

Also from a Ethical point of view there is a basis for offering this form of therapy. As per the Helsinki declaration "The ethical basis of offering stem cell therapy as a treatment option is based on the World Medical Association Declaration of Helsinki-Ethical Principles for Medical Research Involving Human Subjects which states that :-

"In the treatment of a patient, where proven interventions do not exist or have been ineffective, the physician, after seeking expert advice, with informed consent from the patient or a legally authorized representative, may use an unproven intervention if in the physician's judgment it offers hope of saving life, re-establishing health or alleviating suffering. Where possible, this intervention should be made the object of research, designed to evaluate its safety and efficacy. In all cases, new information should be recorded and, where appropriate, made publicly available."

Muscular dystrophy definitely fits into this definition since "proven interventions" do not exist. Therefore from a ethical point of view, as per the Helsinki declaration, for muscular dystrophy patients it appropriate to use stem cell therapy as a treatment intervention.

With our own clinical experience of over 350 patients of muscular dystrophy treated we can say that it definitely helps in "saving life, re-establishing health or alleviating suffering". So despite the fact that as per the principle of evidence based medicine, stem cell therapy is still an unproven treatment but on the basis of the Helsinki declaration it may be used since there are no other proven interventions. If instead of looking at this through the lens of evidence based medicine we look at it from the lens of practice based medicine then we cannot say that it is an unproven therapy since there is enough clinical and published evidence to show that stem cell therapy definitely helps.

Counterview: One swan does not make a summer. Just because at one or a few centers there are good results does not make it a standard of care. It will take many more

centers to show the same results (preferably with a comparison with controls) before we can accept it as a standard of care.

Now let us look at the other point of view which is:-

Reasonable Point of View 2 (Against Stem Cell therapy) :- That Stem cell therapy is not a proven treatment and we are not sure that it works.

For the above :- There is substance to this point of view. Today the practice of Modern medicine is based on what is called "evidence based medicine". For a treatment to become a standard of care it should have been evaluated by multiple centers through what are called prospective, randomized, double blind, placebo controlled studies. This type of evidence is called class one evidence. At present we do not have class I evidence for the role of stem cell therapy in muscular dystrophy. So when your doctor says that this is not yet a proven treatment then based on the principles of evidence based medicine that statement has a basis. By these standards it is also not incorrect for any doctor to say that "we are not sure that it works".

Counter argument: It will take several years (anywhere between 3-7 years) before class one evidence in the form as mentioned above is generated. But till then patients are deteriorating and even dying. What about them? Will we let patients continue to suffer and deteriorate till enough evidence is generated to suit our thinking when a completely safe and reasonably effective treatment is available now that has in a large number of patients shown to halt / slowdown the progression of the disease and improve the quality of life of the patients. Why are we denying children with muscular dystrophy the opportunity to improve the quality of their lives and halt / slow down the progression of the disease. What is more important ? Our principles and standards of evidence based medicine or the quality of lives and disease control of our patients?

Regarding the two extreme views that [1] Stem cell therapy is a definitive cure for muscular dystrophy and the opposite extreme view that [2] Stem cell therapy is a dangerous, banned and unethical form of treatment we wish to state that neither is true. Stem cell therapy does not cure muscular dystrophy. That view are statement is completely incorrect. What is does is that it halts / slows down its progression and produces functional improvements in the patients and improves the quality of their lives. The other extreme view is also incorrect. Stem cell Therapy (specially with adult stem cells and umbilical cord stem cells) is not dangerous in any way whatsoever. It is also not banned treatment. The confusion of its being banned comes from the fact that in the year 2001, President George Bush of America imposed a ban of the federal governmental funding of embryonic stem cell research. (This ban has since then subsequently been lifted by President Obama). It should be noted that [1] the ban was for embryonic stem cells and [2] That ban has subsequently been lifted. Different countries have different regulations and guidelines for use of stem cells. In the US the body to approve this is the US FDA. In India the regulatory body for Stem Cell research is the Indian Council of Medical Research. According to its guidelines it has put embryonic stem cells in the restrictive category but adult stem cells and umbilical cord cell are in the permissive category. It is also not unethical to treat Muscular dystrophy with adult stem cells since it falls under the category of diseases for which there is no

proven intervention and so as per the Helsinki Declaration an unproven therapy can ethically be used.

So in summary whilst Stem cell therapy is not a definitive cure for muscular dystrophy, it is neither a dangerous, banned or unethical form of treatment.

{C} Suggestions to help you make decisions ?

Now here are some suggestions on what you should do as parents :-

There are two steps to this

Step one: *To decide whether to undergo stem cell therapy ?*

Step two : *[a] If yes then where to undergo the Therapy [b] which type of stem cell therapy to undergo ?*

Step one: This is a decision that has to be taken by you yourself after understanding all aspects (the pros and cons) of the treatment. We must realize that for every choice we make there are consequences of two types. Good outcomes and not good outcomes. This is true for whether we make a choice to do something and even when we make a choice to not do something. So if we do stem cell therapy there is the possibility of good and not good consequences. A not good consequence could be a lack of improvement or some adverse event. But if we choose to not do the treatment then too there are good and not good consequences. For example a not good consequence of not undergoing the treatment is a progression and worsening of the disease. We hope that this book and this chapter in particle equips you with the knowledge to be able to make an informed choice. But the final call will still have to be taken by you.

Step two: [a] If yes then where to undergo the Therapy [b] which type of stem cell therapy to undergo ?

Regarding which Stem cell center to take the treatment from our advice would be that you get answers to the following questions when visiting or consulting with a stem cell therapy :-

Question 1: *Does this center have an Institutional Ethics committee??*

In India it is mandatory to have a separate stem cell ethics committee which are referred to as ICSCRT (Institutional Committee for stem cell research and therapy). This is important since a ethics committee evaluates and monitors the work being done at the center.

Question 2 : *Has this center published their results in peer reviewed scientific journals??*

This again differentiates genuine centers working with scientific and academic principles and values from those just set up for commercial purposes. The acceptance of papers for publication entails a process where other doctors and scientists review the data submitted and decide its merits and suitability for publication. This is called peer review and though not a guarantee, it does to some extent ensure that basic scientific and medical principles are being followed for the work that is being published.

Question 3:- *Is special informed consent being taken ??*

If yes you should ask for a copy of the consent and understand it before accepting to undergoing the treatment. As per a Supreme court of India ruling, an informed consent should have the following information [1] Diagnosis, [2] Nature of treatment , [3] Risks involved, [4] Prospects of success , [5] Prognosis if treatment not given, [6] Alternative treatments. In any case these are questions you have the right to ask these from the treating doctors. If the doctors openly and authentically answer all these questions then it is worth considering this center. If the doctors do not give you this information or get upset and angry if you ask questions or are not open and honest about what they do then we recommend that you do not undergo therapy at this center. All this is information that you have a right to and no doctor is doing you a favour by giving it to you.

Question 4 :- *What are the past clinical results of this center with reference to safety and efficacy? What improvements have been noticed in the previous patients treated ?*

You should understand the improvements reported or published by the center and compare these to your own child and determine whether the symptoms that have been shown to improve are the ones in your own child that you want to see an improvement too. You should specifically enquire about any adverse events both minor and major as well as both short term and long term in the patients that have already been treated.

Question 5 :- *What type of stem cells are being used at the center?*

Which type of stem cell therapy to undergo is a very major question.

[a] With our present state of knowledge we would advice extreme caution in considering embryonic stem cell therapy due to the risk of teratoma formation. It will take a few more years before the safety of embryonic stem cells is completely established.

[b] Umbilical cord derived cells are definitely safer than embryonic but one should know which company is manufacturing these cells and should obtain some more information on this company. Is it a reliable company and has good manufacturing facilities and practices then it may be alright to consider them.

[c] However, Adult stem cell taken from the patient's body (autologous) and which have not been manipulated outside the body are the safest of all types of stem cells. Unless there are other compelling reasons, these are the stem cells to be considered first.

So in summary our answers to the fundamental questions we started out with at the beginning of this chapter can be answered as follows:

[1] *Does stem cell therapy work for muscular dystrophy ?*

Answer:- Yes, it does to halt/slowdown the progression of the disease and to cause functional improvements.

[2] *Are there any dangers or risks of doing this therapy ?*

Answer : Almost none with the use of adult stem cell therapy.

[3] *What improvements are likely to be seen in the patients with this therapy ?*

Answer: - Improvements in strength in the trunk, upper and lower limbs as well as in standing and gait.

[4] *How do I chose a good center for stem cell therapy and how do I know for sure whether the center I have chosen for stem cell therapy is working to high professional, scientific and ethical standards ?*

Answer : Select a center that has an institutional ethics committee, that has published its results in scientific journals, where special informed consent is taken and all your queries are satisfactorily answered, that can show you documented improvements in its earlier treated patients as well as safety data and that is preferably working either with adult stem cells derived from the patient or umbilical cord stem cells obtained from a reliable company..

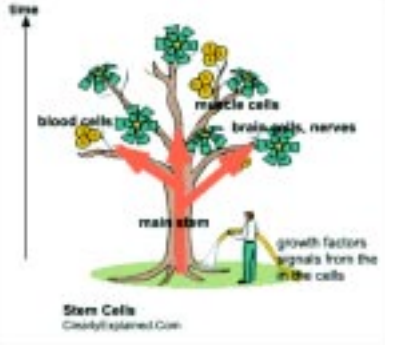
Some Facts about Stem Cells and about the role of Stem Cell Therapy in Muscular dystrophy:

Section I :- About Stem Cells

What follows is a brief description about what are stem cells? For more details you may refer to our earlier published book. "Stem Cell therapy in Neurological Disorders" (which is freely downloadable from our website www.neurogen.in) where there is a detailed and referenced description of the same.

What are stem cells?

Stem cells are specialized cells, which have the ability to multiply and develop into many different cell types in the body during early life and growth. They also help in the repair of the body by dividing and replenishing the damaged cells. When a stem cell divides, each new cell has the potential either to remain as a stem cell or to become another type of cell with a more specialized function, such as a nerve cell, a skin cell, or a red blood cell.



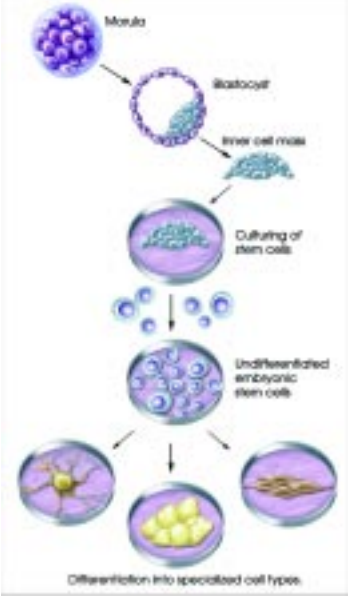
What are the types of stem cells?

Stem cells are of different types, depending on the source from where they are obtained as well as their ability to form different types of cells. The accompanying table show all the different types of stem cells however the 3 main types of stem cells are embryonic, umbilical cord and adult stem cells.

Sr.	TYPES	DESCRIPTION
1	*Embryonic Stem cells*	These are pluripotent cells obtained from the inner cell mass of blastocysts from the IVF clinics.
2	*Fetal stem cells*	The primitive stem cells located in the organs of fetuses are referred to as fetal stem cells
3	*Umbilical cord blood cells*	They are obtained from the umbilical cord immediately after birth, which contains a rich source of hematopoietic stem and progenitor cells.
4	*Mesenchymal Stem Cells*	They are multipotent cells that have the potential to differentiate into multiple lineages including bone, cartilage, muscle, tendon, ligament fat and a variety of other connective tissues.
5	*Multipotent Adult Progenitor Cells (MAPC)*	MAPC are pluripotent cells isolated from bone marrow as well from various adult organs. They contribute to all three germ layers after injection into a developing blastocyst
6	*Marrow-isolated adult multilineage inducible (MIAMI) cells*	They are obtained from human adult bone marrow by culturing BM MNC in low oxygen tension conditions on fibronectin
7	*Multipotent Adult Stem Cells (MACS)*	Pluripotent cells obtained from human liver, heart and BM-isolated mononuclear cells.
8	*Very Small Embryonic Like (VSEL) Stem Cells*	They are pluripotent stem cells obtained from bone marrow which display similar properties of embryonic stem cells.
9	*Gut stem cells*	Multipotent stem cells located in the crypts of Lieberhahn
10	*Bone and cartilage stem cells*	They are stem cells inherent in bone or cartilage to participate in the repair process.
11	*Epidermal stem cells (skin and hair)*	The epidermis contain stem cells at the base of the hair follicle and their self-renewing properties allow for the re-growth of hair and skin cells that occurs continuously
12	*Neural stem c*ells	These cells are isolated from various areas such as the adult CNS including the spinal cord which have the potential to treat various incurable neurological disorders
13	*Olfactory ensheathed cells (OECs)/Olfactory mucosa cells*	They are obtained from the nasal olfactory mucosa and are a rich source of stem cells
14	*Adipose tissue stem cell*	These stem cells are obtained from the body fat and are available abundantly.
15	*Schwann Cells*	These cells are the supporting cells of the peripheral nervous system. They help majorly in axonal regeneration

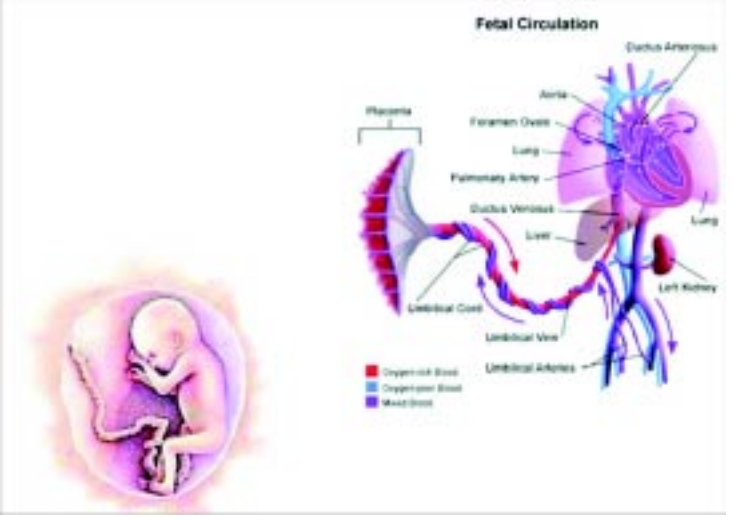
Embryonic stem cells:

Stem cells can be obtained from 3-4 days embryos. This stage in the development of a human is known as blastocyst. Infact, all of us are born from or are a product of stem cells. When we were first conceived (when our mother's egg fertilizes with our father's sperm), we were in one cell stage. Then, we divide and form a clump of cells (16-32 cells), and enter the "blastocyst" stage. The cells obtained from this stage, when grown in a lab, has the ability to form any cells of our body (known as having totipotency).We can imagine that a whole child can be formed from these cells in 9 months (in the womb of a mother).So, theoretically, these are the most potent stem cells. However, they have certain ethical and medical issues surrounding them. In some religions, derivation of stem cells from blastocyst (which are extra after invitro fertilization), is equivalent to taking a life. Apart from that, these cells have potential to form a type of tumor called 'teratoma' (as found in experimental animals).



Umbilical cord stem cells:

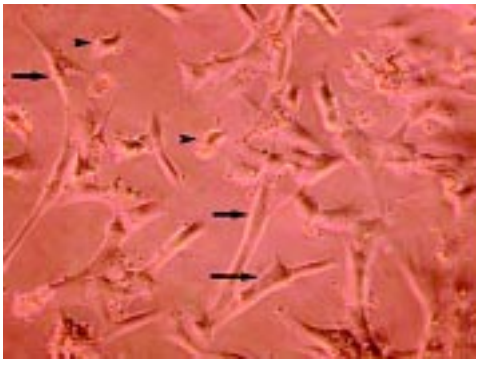
The other source is the umbilical cord blood and placental tissue (Wharton's jelly).This is a rich source of stem cells. The cord tissue is discarded as a waste during the birth of a child. The option of storing this for "potential" future use for the same child can be considered. It is found to be a good source of stem cells for children with blood disorders, such as sickle cell anemia and thallasemia. Sibling cord blood can be used for the patient. The advantages of using cord blood as a source of stem cells are: 1) It is a non-invasive source and can be obtained from the umbilical cord immediately after birth. 2) Available in vast abundance; thousands of babies are born each day and the umbilical cord and placenta are discarded as waste. 3) Despite its high content of immune cells, it has minimal risk of rejection.



Adult stem cells:

Adult stem cells, currently, form the most attractive and safest types of stem cells for clinical use. Stem cells taken from bone marrow, adipose tissue and dental pulp of the patient themselves (autologous) can be used with no risk of side effects. Bone marrow derived stem cells, especially, are found to be safe, feasible and effective option for use in treatment of various disorders, such as cardiac problems, diabetes, joint problems and disorders of the brain, spinal cord and muscles.

Bone marrow, as a source of stem cells, is a practical, easily available as well as replenishable. These have no ethical controversies surrounding them and are termed as 'permissible" by monitoring authorities.



What diseases can be treated with stem cell therapy?

Several diseases and injuries of the nervous and musculoskeletal system that have no other treatment options and were broadly described under the heading of Incurable neurological diseases can potentially be treated with Stem cell therapy. These include muscular dystrophy, autism, cerebral palsy, sequelae of neurological damage following traumatic , infective and genetic conditions of the nervous system, long term paralysis occurring from spinal cord injury, stroke etc.

What are the routes of administration of stem cells?

Stem cells can be administered through various routes such as intravenous, intra-arterial, intramuscular and intrathecal.

[1] Intravenous route: Is by a simple injection of stem cells into the veins like any other drug injection.

[2] Intraarterial route: This is an injection of stem cells through a catheter into the artery which is introduced through a artery in the thigh called the femoral artery. This is also called the interventional route.

[3] Intramuscular route: Injections of stem cells directly into the affected muscles.

[4] Intrathecal route: Injection of stem cells into the spinal fluid through a lumbar puncture injection.

How do stem cells work?

Following stem cell therapy, a patient's first experiences an overall feeling of well being .This is attributed to the release of "good chemical signals", referred to as cytokines and factors known as "nerve growth factors" and "brain derived neurotrophic factors". Over a period of time, the process of "angiogenesis" or formation of new blood vessels is initiated over a few weeks and months. Over 2-3 months, neurogenesis or forming neural cells and/or muscle cells also happens.

In muscular dystrophy, it is understood that stem cells which are implanted stimulate the resident "satellite cell" population in the muscles, thereby stimulating optimum action.

In Muscular dystrophy there often arises the question as to how autologous stem cells work when they are likely to have the same genetic defect at the muscles. The answer to this is that the stem cells don't cure the disease. They by the process of neurotrophic growth factors, angiogenesis and neurogenesis help in repair and regeneration of the already damaged muscles and so produce functional improvements. The advantage is that by virtue of the fact that they are from the same patient they are likely to be accepted more easily at the recipient site and stimulate the satellite cell population.

A scientific study from the Stanford from Stanford institute of stem cell biology and regenerative medicine (Helen Blau, Donald E, Delia Baxter,etal) , published in the journal Cell, has in fact, established that DMD is a disease or disorder of "stem cells". This means that the muscles in children with DMD are defective in normal repair mechanism, which is required to repair daily wear and tear. Hence, stem cell therapy provides this deficit raw material for the muscle to use. In the words of the corresponding author of the paper Jason Pomerantz " If the treatment does not replenish the stem cell compartment , it will likely fail; it would like pushing the gas pedal to the floor when there is no fuel reserve "

Section II : Stem Cell Therapy for Muscular Dystrophy as done at the NeuroGen Brain and Spine Institute (NGBSI)

How is the stem cell transplantation done?

The procedure for stem cell transplantation at NGBSI is minimally invasive, with simple steps or processes. There is no major surgery or incisions required.

The procedure is carried out in 3 steps:

a) **Bone marrow aspiration:** Bone marrow is the place where blood is formed. In simple terms, it can be called a "factory" of blood. As is common knowledge, blood is formed in the hollow space of the bones. The easiest place to take out the bone marrow is the hip bone. Using a thin bone marrow aspiration needle, 80-120 ml bone marrow is aspirated and collected in heparinized tubes.

Fig. 10.5 Bone marrow aspiration

b) **Purification of stem cells:** On the same day, within 3-5 hours, stem cells are separated and purified in the laboratory.

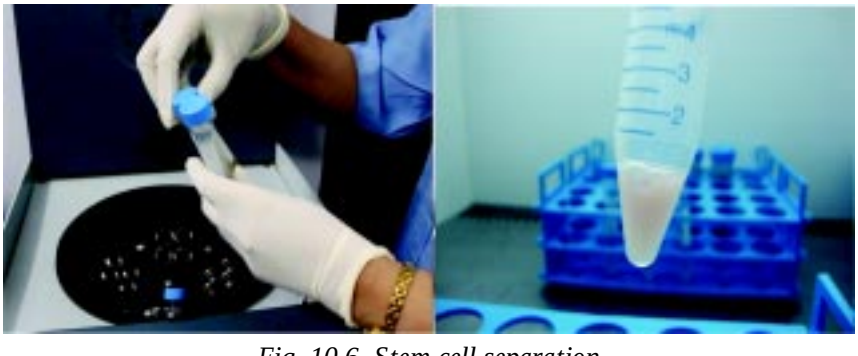


Fig. 10.6 Stem cell separation

c) **Injection:** Stem cells are then injected into L3-4 or L4-5 space. An 18G Touhy needle is inserted into the sub-arachnoid space. After establishing a free flow of CSF, an epidural catheter is inserted into the space and a part of the stem cells are flushed into the cerebrospinal fluid (fluid which flows around the brain and spine) and the remaining cells are injected intramuscularly into specific muscles at specific points called motor points.

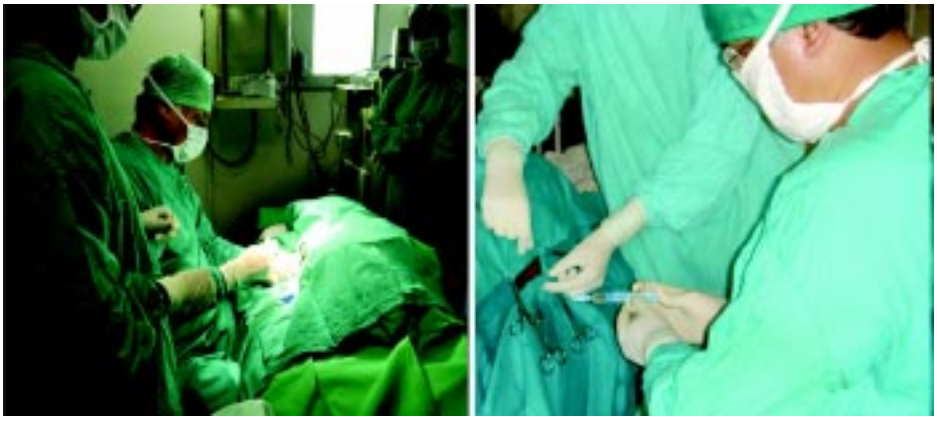


Fig. 10.7 Stem cell injection into the spinal fluid(intrathecal)

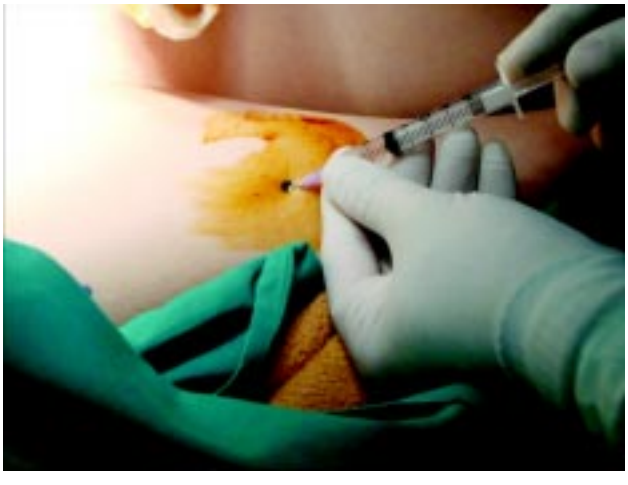


Fig. 10.8 Stem cells injected into the muscles at specific motor points

What has to be done after stem cell therapy?

Following the stem cell transplantation or injection, from the very next day, patient undergoes an intensive rehabilitation process, consisting of physiotherapy, occupational therapy, psychological therapy, positive reinforcement processes, yoga therapy, etc.

It is important that stem cell therapy be followed by a proper exercise regime to gain proper response. Stem cells are considered to be "blank slates", which means they are programmable.

At NeuroGen BSI, our experience has reaffirmed that the cells can be programmed and guided to help regenerate tissues by doing exercise. Scientific evidence supporting this theory is available too. We believe that the stem cell therapy works along with an extended and aggressive Neurorehabilitation process. In fact we call our entire treatment of stem cell therapy and Neurorehabilitation as Neuro-Regenerative-Rehabiltataion Therapy (NRRT). Our long term follow-up reveals that patients who participate in a regular rehabilitation program do overall better than those that don't. The availability of the transplanted stem cells make the rehabilitation process more effective and efficient.

Types of Muscular Dystrophy treated at NeuroGen BSI and their presenting symptoms

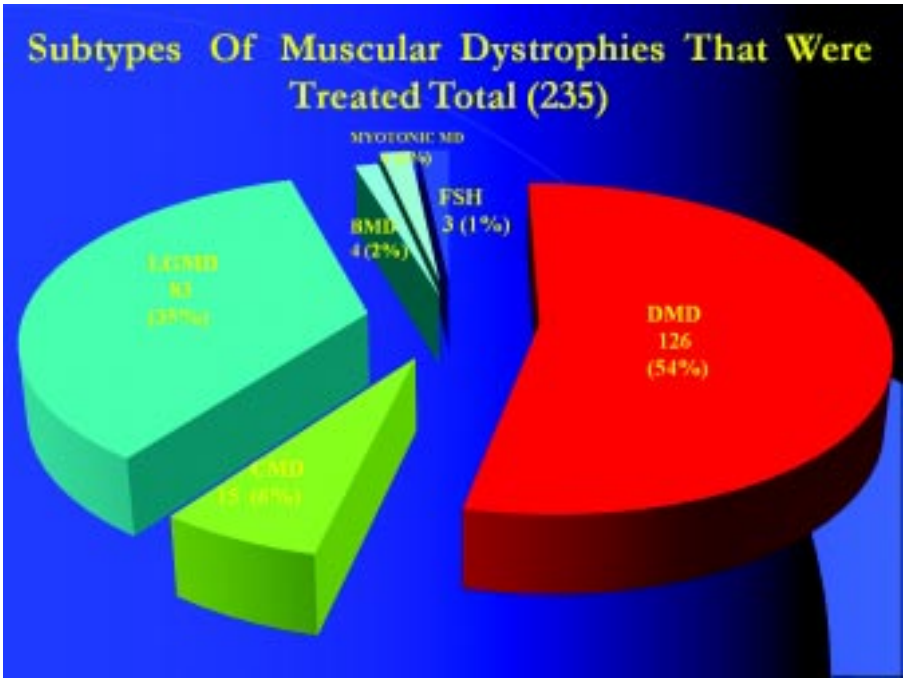


Fig. 10.9

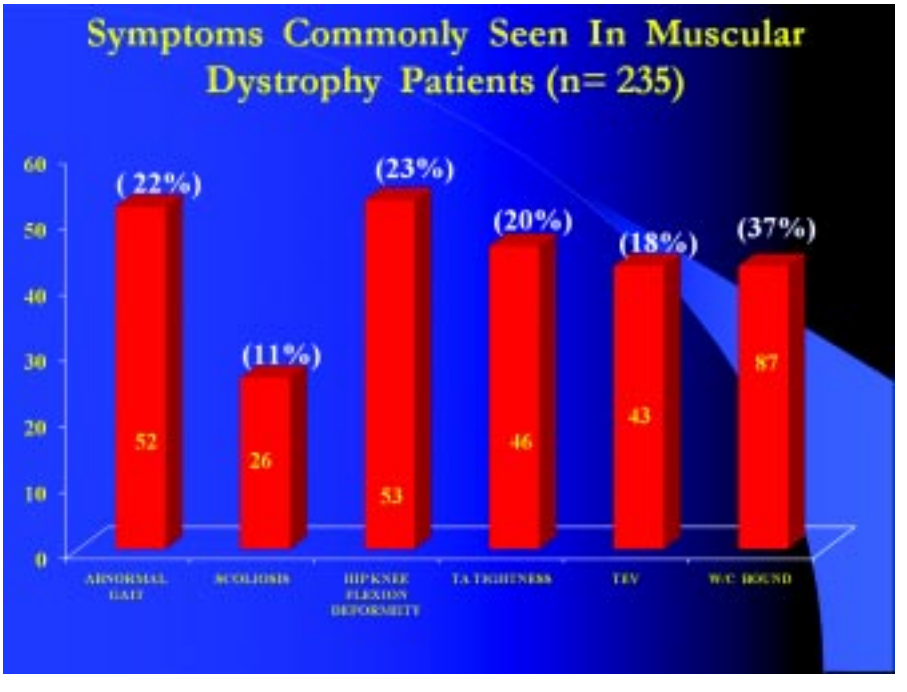


Fig. 10.10

What are the results of stem cell therapy at NeuroGen BSI?

In an experience of having treated more than 1000 cases of various neurological disorders, we have found that about 70% of the patients have benefitted.

In 235 patients of muscular dystrophy, with a follow up of atleast 6 months, 93% patients have sown either improvement or stalling of deterioration. The major areas showing improvements were increase in trunk strength (43%), lower extremity strength (45%), upper extremity strength (36%), ability to stand [with or without support] (64%) and improved gait pattern (78%). Many of these patients showed improved muscle strength on manual muscle testing along with reduction in muscle tightness. Clinical improvements were supported by improvement on electrophysiology (EMG) as well as musculoskeletal imaging (MRI) in certain patients, in the form of muscle regeneration.

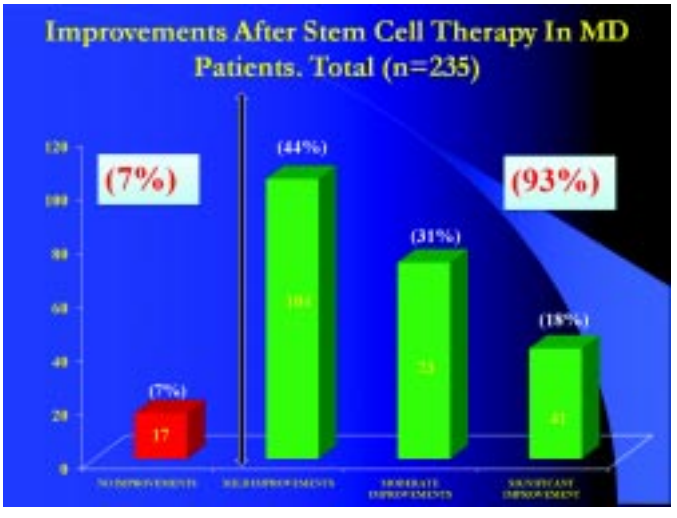


Fig. 10.11 Clinical improvements seen after Stem Cell Therapy in MD patients

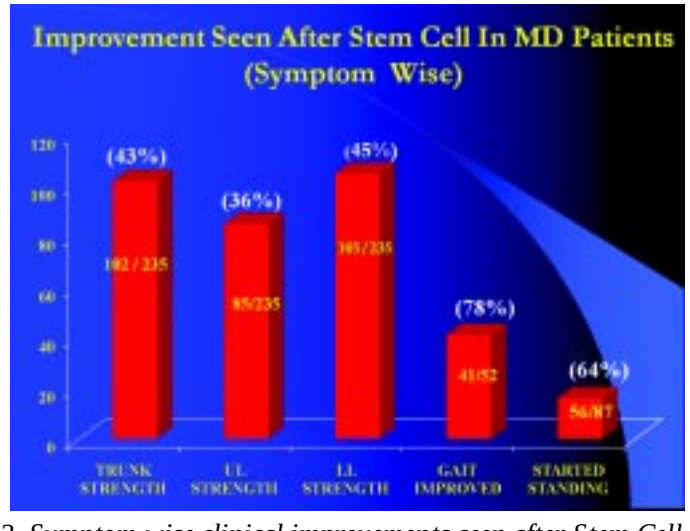


Fig. 10.12 Symptom wise clinical improvements seen after Stem Cell Therapy

Radiological improvements seen after Stem Cell Therapy

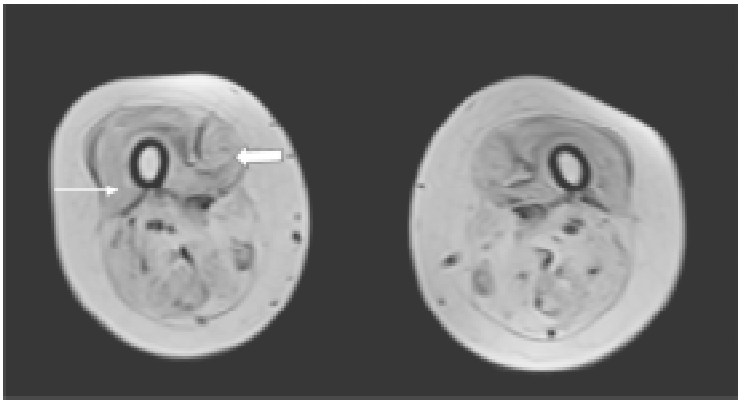


Fig. 10.13 Arrows showing fatty infiltration in the muscles before stem cell therapy. Very few dark or black areas (denoting muscle) are seen.

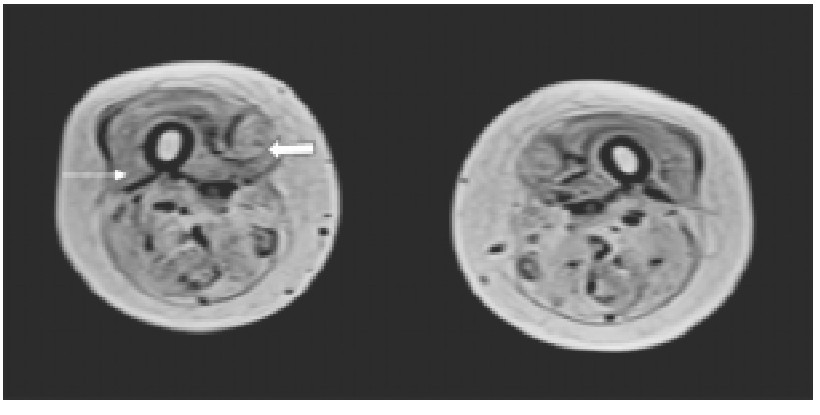


Fig. 10.14 Arrows showing muscle regeneration in muscular dystrophy after stem cell therapy(more dark or black areas are seen- which indicate muscle fibre)

EMG Improvements seen after Stem Cell Therapy

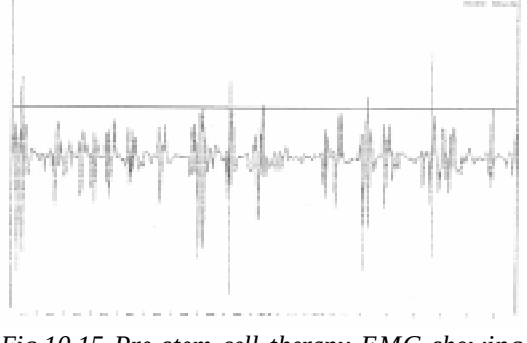


Fig.10.15 Pre stem cell therapy EMG showing reduced interference pattern in Vastus Medialis Muscle, with myopathic potentials.

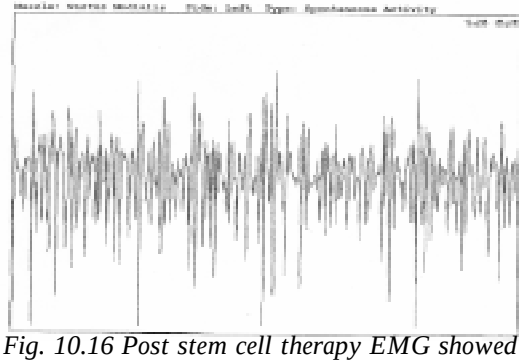


Fig. 10.16 Post stem cell therapy EMG showed complete interference pattern in Vastus Medialis Muscle with motor unit potentials.

Section III: Review of worldwide research for the role of Regenerative Medicine in Muscular dystrophy.

All over the world, the scientists have been exploring the possibility to replace the missing proteins such as dystrophin, sarcoglycans etc in muscular dystrophy, by introducing muscle stem cells. These cells will help in formation of new muscle cells. Such new cells would be protected from the progressive degeneration and further restore the muscle function. Studies have also shown that stem cells can be used to deliver a functional dystrophin gene to skeletal muscles of dystrophic mice. Currently, a lot of emphasis has been put on the use of satellite cells. They have the ability to regenerate large parts of musculature. Other cells such as muscle derived stem cells, mesoangioblasts, pericytes, adipocytes etc have also shown promising usage for muscular dystrophy.

A review of the literature about cell based therapies and research is simplified below for parents who are keenly following these new area of research.

Cell therapy has evolved as one of the promising treatments for muscular dystrophy. The main goal of cell therapy is to directly regenerate wasted, adult muscle fibres through intravenous administration or targeted injection of stem cells, which function to block muscle loss and restore, at least partially, the normal muscular activity. The way these stem cells function and reverse the effects of cell death includes differentiation (changing into muscle cells), cell fusion(fusing with muscle cells), and secretion of cytokines or paracrine effects (release of chemicals which help repair).

Stem cells show high plasticity, i.e. the ability to change into other types of cells. The plasticity can be explained by transdifferentiation (direct or indirect) and fusion. So far, bone marrow-derived stem cells and their paracrine factors(chemicals released by them) have shown all the necessary attributes for tissue regeneration, such as angiogenesis(formation of new blood vbessels), inhibition of apoptosis(or self death of cells), anti-inflammation(reduce swelling in the cells), immunosuppression, stimulation of endogenous cells(cells within the muscle mass). Thus, research into these factors and mechanisms has shown that stem cell therapy greatly enhances the potential and variety of therapeutic applications for muscular dystrophy.

Homing signals are extremely important for the efficacy of cell therapy because it is via these effects that the precise localization of transplanted cells is possible and can be improved. Damaged tissue releases factors that induce homing. These chemical factors or signals are finally responsible for the stem cells to reach the specific area where repair has to be done.

Encouraging and pioneering experiments are being carried out in animal models for various muscular dystrophies. Diseased tissue may be regenerated in side (in vivo) the animals by transplantation of healthy cells that can extensively multiply. Stem cells have this intrinsic ability, and are used in certain clinical settings to enhance or restore damaged tissue.

In attempts to regenerate muscle cells replete with dystrophin in the muscles of patients with dystrophin deficiency, several types of muscle-derived cell transplantation

strategies have been tested in animals and in a few DMD patients. A muscle precursor cell, known as the myoblast, was one of the first cell types explored in DMD studies. In 1990, the first muscle stem cell transplantation was carried out in a 9 year old DMD patient, showing dystrophin production. Soon after, many clinical trials in DMD patients were conducted. Although, it was found to be a safe procedure, clinical benefits were recorded in none.

Since, myoblast transplantation has not shown effective results mainly due to rapid death of most injected myoblasts and the failure of injected myoblasts to migrate more than 0.5 mm away from the injection site, satellite stem cells have been explored as an alternative. These cells which are specialized muscle stem cells, have the ability to both replicate(form cells like themselves) and change into various types of muscle cells.

A study was carried out to track the pathway of bone marrow derived stem cells (BMSC) to satellite cell to myofiber, a green fluorescent protein-positive (a fluorescing dye which can track cells) BMSCs were transplanted into irradiated recipients(mice in which immunity is destroyed by radiation, so that they can accept a non-self tissue). Irradiation served to both ablate the bone marrow compartment and decrease satellite stem cell numbers in muscle tissue. These green cells were identified in muscle tissue of bone marrow transplanted (BMT) recipients by their structure .These cells also were found to have the ability to self-renew and differentiate into myotubes (which are the immature of muscle) , when grown outside the body in the laboratory. The number of these stem cells in the BMT-recipient mice was greatly increased when the animals underwent physical activity for 6 months. This study is important because it provides evidence that BMSC can change to muscle cells. This is found to occur via repopulation of the muscle stem cell compartment. Another study in 2008, showed that adult muscle mononuclear cells (AMMCs) in -sarcoglycan-deficient dystrophic mice were 35 times more efficient at restoring sarcoglycan compared to cultured myoblasts. The single injections of AMMCs provided long term benefit for muscular dystrophy and found persistent regeneration after 6 months.

A few other myogenic precursor(immature forms of muscle cells) cell types distinct from satellite cells have also been explored. One of them is the muscle-resident side population (SP) cells. A study has shown that these cells could serve as a vehicle for delivering the dystrophin gene contained in a viral vector into mdx(animal model of muscular dystrophy) mice. Muscle derived stem cells (MDSCs) or multipotent adult progenitor cells (MAPCs) have also reported to have a high capacity for muscle regeneration. However, compared to conventional satellite cells, many of these SP cells do not display a promising myogenic potential.

Little progress has also been made towards the use of embryonic stem cells (ESC) to study its potential in muscle regeneration. However, due to high rate of rejection and ethical issues related to ESCs, not many studies have been carried out on humans, which are necessary to demonstrate the therapeutic benefits of ESCs in muscular dystrophy patients.

Blood and muscle derived CD 133+ cells have shown to give rise to dystrophin-positive

fibers when transplanted into mdx (animal model of muscular dystrophy) mice. (37) These cells have been demonstrated as safe, following their intramuscular transplantation.

Experimental studies have shown that sub-populations of human umbilical cord blood (HUCB) cells have myogenic potential(potential to convert into muscle cells) and can differentiate into muscle cells. Animal studies (in mice) have that when human umbilical cord cells were injected in dystrophin deficient and dysferlin deficient mice, muscle cells were formed. A study was carried out on 82 progressive muscular dystrophy (PMD) patients, which were treated by using double transplantations of bone marrow and cord blood stem cells. No adverse reactions / side effects were reported. It was found that 37.8% obtained a remarkable efficacy, 45.1% were effective and 17.1% had no change. Hence, it was found to be safe, convenient and effective therapy for PMD.

Mesenchymal stem cells (MSC) are also attractive candidates for the treatment of muscular dystrophy. Bone marrow derived mesenchymal stem cell transplantation in mouse models have shown that the cells reach the damaged muscle and result in repair and regeneration.This has been confirmed by transplanting bone marrow derived MSCs and tracking them to the damaged muscle along with formation of the deficient protein the muscle (eg. Dystrophin in mdx mice) after a month.

A few years back, human fetal MSCs were injected into muscle, veins and into the abdomen (intraperitoneal) mouse embryo .On analysis after a period of time, cells injected into the abdominal fluid and into the blood reached all organs.

In mdx mice, stem cells derived from adult adipose (fat) tissue have homed to or reached and changed into muscle cells as well as repaired injured muscle tissue.

Scientific publications regarding stem cell therapy by NeuroGen

A. Stem cell therapy in muscular dystrophy

1. Alok Sharma, Nandini Gokulchandran, Guneet Chopra, Pooja Kulkarni, Mamta Lohia, Prerna Badhe, V.C.Jacob. Administration of autologous bone marrow derived mononuclear cells in children with incurable neurological disorders and injury is safe and improves their quality of life. Cell Transplantation, 2012; 21(1):S1-S12
2. A. Sharma, P. Kulkarni, G. Chopra, N. Gokulchandran, M. Lohia P. Badhe. Autologous Bone Marrow Derived Mononuclear Cell Transplantation In Duchenne Muscular Dystrophy-A Case Report. Indian Journal of Clinical Practice. 2012; 23(3):169-72
3. Dr. Suvarna Badhe, Ms. Pooja Kulkarni, Dr Guneet Chopra, Dr Nandini Gokulchandran, Dr Alok Sharma. Dystrophin Deletion mutation pattern and Cardiac involvement in 46 cases of Dystrophinopathies. Asian Journal of Clinical Cardiology. August 2012 (in Press)

B. Stem cell therapy in other disorders

1. Alok Sharma, Guneet Chopra, Nandini Gokulchandran, Mamta Lohia, Pooja Kulkarni. Autologous Bone Derived Mononuclear Transplantation in Rett Syndrome. Asian Journal of Paediatric Practice. 2011;15(1):22-24
2. Dr. Alok Sharma, Ms. Pooja Kulkarni, Dr. Hemangi Sane, Dr. Nandini Gokulchandran, Dr. Prerna Badhe, Dr. Mamta Lohia, Dr. Priti Mishra. Positron Emission Tomography- Computed Tomography scan captures the effects of cellular therapy in a case of cerebral palsy. Journal Of Clinical Case Reports. 2012 (in press)
3. Sharma A, Gokulchandran N, Kulkarni P, Chopra G. Application of autologous bone marrow stem cells in giant axonal neuropathy. Indian J Med Sci 2010; 64:41-4
4. A. Sharma, P. Badhe, N. Gokulchandran, P. Kulkarni, V.C Jacob, M. Lohia, J. George Joseph, H. Biju, G. Chopra. Administration of Autologous bone marrow stem cells intrathecally in Multiple Sclerosis patients is safe and improves their quality of life. Indian Journal of Clinical Practice. 2011:21(11):622-625
5. Alok Sharma, Prerna Badhe, Pooja Kulkarni, Nandini Gokulchandran, Guneet Chopra, Mamta Lohia, V.C.Jacob. Autologous Bone marrow Derived mononuclear cells for the treatment of Spinal Cord Injury. The Journal of Orthopaedics. 2011; 1(1): 33-36
6. Alok Sharma, Prerna Badhe, Omshree Shetty, Pooja Vijaygopal, Nandini Gokulchandran, V.C. Jacob, Mamta Lohia, Hema Biju, Guneet Chopra. Autologous bone marrow derived stem cells for motor neuron disease with anterior horn cell involvement. Bombay Hospital Journal. 2011; 53(1):71- 75.
7. Dr. Alok Sharma, Dr. Hemangi Sane, Dr. Prerna Badhe, Ms. Pooja Kulkarni, Dr. Guneet Chopra, Dr. Mamta Lohia, Dr. Nandini Gokulchandran. Autologous Bone Marrow Stem Cell Therapy shows functional improvement in hemorrhagic stroke- a case study. Indian Journal of Clinical Practice. October 2012 (in Press)

Research Publications Regarding Stem Cell Therapy for Muscular Dystrophy World Wide

1. Dezawa M, Ishikawa H, Itokazu Y et al. Bone marrow stromal cells generate muscle cells and repair muscle degeneration. Science 2005; 309: 314-317
2. Dellavalle A, Sampaolesi M, Tonlorenzi R et al. Pericytes of human post-natal skeletal muscle are committed myogenic progenitors, distinct from satellite cells, and efficiently repair dystrophic muscle. Nat. Cell Biol. 2007; 9:255-267
3. Xiaoyun Wu, Shili Wang, Baoli Chen, Xinling An. Muscle-derived stem cells: isolation, characterization, differentiation, and application in cell and gene therapy. Cell Tissue Res. 2010; 340:549-567
4. Orlic D, Kajstura J, Chimenti S, et al. Bone marrow stem cells regenerate infarcted myocardium. Pediatric Transplantation 2003; 7(3): 86-88
5. Cselenyák A, Pankotai E, Horváth EM, et al. Mesenchymal stem cells rescue cardiomyoblasts from cell death in an in vitro ischemia model via direct cell-to-cell connections. BMC Cell Biology 2010; 11(29).
6. Nassiri S M, Khaki Z, Soleimani M et al. The similar effect of transplantation of marrow-derived mesenchymal stem cells with or without prior differentiation induction in experimental myocardial infarction. Journal of Biomedical Science. 2007; 14(6):745-755.
7. Orlic D, Kajstura J, Chimenti S et al. Mobilized bone marrow cells repair the infarcted heart, improving function and survival. PNAS 2001; 98(18): 10344-10349.
8. Shabbir A, Zisa D, Leiker M et al. Muscular dystrophy therapy by non-autologous mesenchymal stem cells: muscle regeneration without immunosuppression and inflammation. Transplantation. 2009; 87(9):1275-1282.
9. Vandervelde S, van Luyn MJ, Tio RA, Harmsen MC. Signaling factors in stem cell mediated repair of infarcted myocardium. J Mol Cell Cardiol. 2005; 39(2):363-376.
10. Deasy BM, Feduska JM, Payne TR, et al. Effect of VEGF on the regenerative capacity of muscle stem cells in dystrophic skeletal muscle. Mol Ther. 2009 Oct;17(10):1788-98
11. Law PK, Bertorini TE, Goodwin TG et al. Dystrophin production induced by myoblast transfer therapy in Duchenne muscular dystrophy. The Lancet.1990; 336(8707):114 - 115.
12. Huard J, Bouchard JP, Roy R, Malouin F et al. Human myoblast transplantation: preliminary results of 4 cases. Muscle Nerve. 1992; 15(5):550-60.
13. Gussoni E, Pavlath GK, Lanctot AM et al. Normal dystrophin transcripts detected in Duchenne muscular dystrophy patients after myoblast transplantation. Nature. 1992; 356(6368):435-8.

14. Tremblay JP, Malouin F, Roy R et al. Results of a triple blind clinical study of myoblast transplantations without immunosuppressive treatment in young boys with Duchenne muscular dystrophy. Cell Transplant. 1993;2(2):99-112

15. Neumeyer AM, Cros D, McKenna-Yasek D, et al. Pilot study of myoblast transfer in the treatment of Becker muscular dystrophy. Neurology. 1998 Aug;51(2): 589-92

16. Mendell JR, Kissel JT, Amato AA et al. Myoblast transfer in the treatment of Duchenne's muscular dystrophy. N Engl J Med. 1995; 333(13):832-8.

17. Karpati G, Ajdukovic D, Arnold D. et al Myoblast transfer in Duchenne muscular dystrophy. Ann Neurol. 1993 Jul;34(1):8-17

18. Daniel Skuk, Brigitte Roy, Marlyne Goulet et al. Dystrophin Expression in Myofibers of Duchenne Muscular Dystrophy Patients Following Intramuscular Injections of Normal Myogenic Cells. Molecular Therapy. 2004; 9, 475-482

19. Miller RG, Sharma KR, Pavlath GK, et al. Myoblast implantation in Duchenne muscular dystrophy: the San Francisco study. Muscle Nerve. 1997;20(4):469-78

20. Wagers AJ, Conboy IM. Cellular and molecular signatures of muscle regeneration: current concepts and controversies in adult myogenesis. Cell 2005; 122:659-667.

21. Anna Polesskaya, Patrick Seale and Michael A Rudnicki. Wnt Signaling Induces the Myogenic Specification of Resident CD45+ Adult Stem Cells during Muscle Regeneration. Cell. 2003;113(7): 841-852

22. LeBarge MA, Blau HM. Biological progression from adult bone marrow to mononucleate muscle stem cell to multinucleate muscle fiber in response to injury. Cell 2002;111:589-601

23. Wallace GQ, Lapidos KA, Kenik JS, McNally EM. Long-term survival of transplanted stem cells in immunocompetent mice with muscular dystrophy. Am J Pathol. 2008;173(3):792-802

24. Bachrach E, Li S, Perez AL et al. Systemic delivery of human microdystrophin to regenerating mouse dystrophic muscle by muscle progenitor cells. Proc Natl Acad Sci U S A. 2004;101(10):3581-6

25. Collins CA, Olsen I, Zammit PS et al. Stem cell function, self-renewal, and behavioral heterogeneity of cells from the adult muscle satellite cell niche. Cell. 2005;122(2):289-301

26. Darabi R, Gehlbach K, Bachoo RM, et al. Functional skeletal muscle regeneration from differentiating embryonic stem cells. Nat Med. 2008; 14(2):134-43.

27. Darabi R, Baik J, Clee M et al. Engraftment of embryonic stem cell-derived myogenic progenitors in a dominant model of muscular dystrophy. Exp Neurol. 2009; 220(1):212-6

28. Stillwell E, Vitale J, Zhao Q, et al. Blastocyst injection of wild type embryonic stem cells induces global corrections in mdx mice. PLoS One. 2009; 4(3):e4759.

29. Gussoni E, Soneoka Y, Strickland CD et al. Dystrophin expression in the mdx mouse restored by stem cell transplantation. Nature. 1999, 401, 390-394

30. Torrente Y, Belicchi M, Marchesi C, et al. Autologous transplantation of muscle-derived CD133+ stem cells in Duchenne muscle patients. Cell Transplantation. 2007;16:(6):563-577

31. Torrente, Y, Belicchi, M, Sampaolesi et al. Human circulating AC133+ stem cells restore dystrophin expression and ameliorate function in dystrophic skeletal muscle. J Clin Invest. 2004, 114: 182-195.

32. Erices A, Conget P, Minguell JJ. Mesenchymal progenitor cells in human umbilical cord blood. Br J Haematol. 2000; 109(1):235-42.

33. Zhang C, Chen W, Xiao LL et al. Allogeneic umbilical cord blood stem cell transplantation in Duchenne muscular dystrophy. Zhonghua Yi Xue Za Zhi. 2005; 85(8):522-5.

34. Kong KY, Ren J, Kraus M et al. Human umbilical cord blood cells differentiate into muscle in sjl muscular dystrophy mice. Stem Cells. 2004; 22(6):981-93.

35. Jazedje T, Secco M, Vieira NM et al. Stem cells from umbilical cord blood do have myogenic potential, with and without differentiation induction in vitro. J Transl Med. 2009;7:6

36. Yang XF, Xu YF, Zhang YB, et al. Functional improvement of patients with progressive muscular dystrophy by bone marrow and umbilical cord blood mesenchymal stem cell transplantations. Zhonghua Yi Xue Za Zhi. 2009; 89(36):2552-6. Chinese.

37. Li Z, Zhang C, Chen GJ, Liu XR. Ultrastructural changes in muscular tissues of dystrophin/utrophin double-knockout mice after bone marrow-derived mesenchymal stem cell transplantation. Di Yi Jun Yi Da Xue Xue Bao. 2004; 24(5):501-4. Chinese.

38. Feng SW, Lu XL, Liu ZS, et al. Dynamic distribution of bone marrow-derived mesenchymal stromal cells and change of pathology after infusing into mdx mice. Cytotherapy. 2008; 10(3):254-64.

39. Feng SW, Zhang C, Lu XL, et al. Mesenchymal stem cells transplanted in mdx mice differentiate into myocytes and express dystrophin/utrophin. Nan Fang Yi Ke Da Xue Xue Bao. 2009; 29(5):974-8. Chinese.

40. Feng SW, Zhang C, Yao XL, et al. Dystrophin expression in mdx mice after bone marrow stem cells transplantation. Zhongguo Yi Xue Ke Xue Yuan Xue Bao. 2006; 28(2):178-81. Chinese.

41. Li Z, Liu HY, Lei QF, et al. Improved motor function in dko mice by intravenous transplantation of bone marrow-derived mesenchymal stromal cells. Cytotherapy. 2011;13(1):69-77

42. De la Garza-Rodea AS, van der Velde I et al. Long-term contribution of human bone marrow mesenchymal stromal cells to skeletal muscle regeneration in mice. Cell Transplant. 2011; 20(2):217-31.

43. Liu TY, Li JL, Yao XL, et al. Transplantation of 3H-thymidine-labeled human bone marrow-derived mesenchymal stem cells in mdx mice. Di Yi Jun Yi Da Xue Xue Bao. 2005; 25(5):498-502. Chinese.

44. Chan J, Waddington SN, O'Donoghue K, et al. Widespread distribution and muscle differentiation of human fetal mesenchymal stem cells after intrauterine transplantation in dystrophic mdx mouse. Stem Cells. 2007; 25(4):875-84.

45. Liu Y, Yan X, Sun Z. Flk-1+ adipose-derived mesenchymal stem cells differentiate into skeletal muscle satellite cells and ameliorate muscular dystrophy in mdx mice. Stem Cells Dev. 2007;16(5):695-706

46. Rodriguez AM, Pisani D, Dechesne CA, et al. Transplantation of a multipotent cell population from human adipose tissue induces dystrophin expression in the immunocompetent mdx mouse. J Exp Med. 2005; 201(9):1397-405.

47. Darabi R, Pan W, Bosnakovski D et al. Functional Myogenic Engraftment from Mouse iPS Cells. Stem Cell Rev. 2011;7(4):948-57.

48. Beck AJ, Vitale JM, Zhao Q, et al. Differential requirement for utrophin in the induced pluripotent stem cell correction of muscle versus fat in muscular dystrophy mice. PLoS One. 2011; 6(5):e20065

Case Reports

Case 1:

This boy began to notice weakness in his lower limbs at the tender age of 5 years. A case of Duchenne Muscular Dystrophy, his weakness gradually increased, making it difficult for him to walk, get up from the floor, climb stairs and perform other mobile activities. By the time he turned 11, his lower limbs had lost all power. He was unable to stand and became wheelchair bound. As a result, he became dependent on external help for his daily activities. Although he continued to attend regular school, his debilitating impairment prevented him from leading a normal life.

He then came to NeuroGen Brain and Spine Institute where he underwent stem cell therapy, followed by intensive rehabilitation. Over a period of time, his muscle strength began improving. His stamina increased. He was able to perform overhead activities with greater ease. But most of all, through hard work and dedication, he became strong enough to stand up and walk a few steps by himself.

Medically, his cpk levels were brought under control and the test scores became quantitatively better.

He worked on his own self very diligently, without once losing the motivation and drive to better his condition.

Case 2:

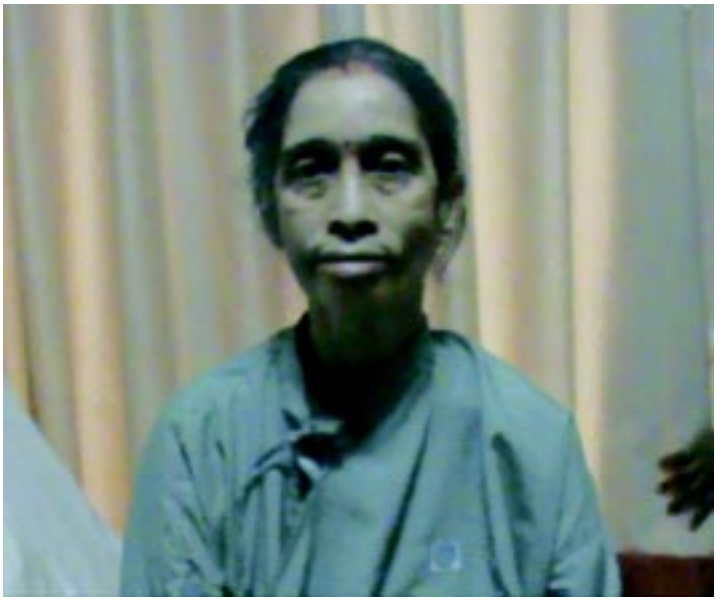
A 36 year old male, running a successful business, started experiencing weakness in his lower limbs at the age of 24 years. As his weakness progressed, his balance began to falter. He would fall frequently while walking, his stamina reduced drastically. He found it difficult to get up from squatting position or low seats. When investigations were done, he was diagnosed with Limb Girdle Muscular Dystrophy.

Although he could perform all the daily activities by himself, his mobility became severely affected.

He underwent stem cell therapy along with intensive rehabilitation therapies. As a result of which, His stability and balance also improved. The frequency of falls went down drastically. He could easily stand up from low positions.

Stem cell therapy has helped stall the progression of his disease. With continued rehabilitation, he aims at enhancing his strength & ability and lead as normal a life as possible.

Case 3:



A 64 year old female reported weakness in upper limb muscles since 1986. This weakness progressed to both the lower limbs. She found difficulty in performing overhead activities, she was unable to lift her child up properly, she would lose balance and fall frequently. She fractured her femur due to a fall and underwent steel plate fixation surgery. The weakness also spread to her facial muscles because of which she could not close her mouth completely. Her stamina had suffered greatly.

Just 3 months after stem cell therapy, she noticed that her stamina had improved significantly. She could perform daily activities with greater ease. Her insomnia problem had been resolved since she could sleep for 7 hours peacefully. Fine motor functions such as using fingers to break food into smaller pieces had improved, strength in arms had increased. She could get up from the bed independently and stand against a board for an hour. Clarity of speech improved with the ability to close her lips.

With continued rehabilitation, her condition has further improved drastically. She had started walking with the help of a walker and slowly she was able to walk without any assistive device. She can perform all her daily activities such as bushing, bathing, dressing, walking around the house with ease. She is able to cut vegetables and even stand for 45 minutes. As a result, she is able to prepare meals for the entire family and is able to clean the kitchen by herself.

Gradually, her ability to use finger movements finely has also returned. She is able to do stitching, hemming and embroidery. With unwavering family support and undaunting courage, her quality of life has improved significantly.

Case 4:

It started when this young boy experienced difficulty in climbing stairs and getting up from the floor. He was diagnosed with Duchenne Muscular Dystrophy at the age of just 4 years. His weakness progressed and by the age of 7 years he stopped walking entirely. Upper limbs too became weak, performing overhead activities became difficult.

He underwent stem cell therapy and after 4 months of intensive rehabilitation, he reported a feeling of well being and stamina. His muscles had become stronger and tighter. His trunk muscles strength also increased. Slowly he was able to stand with the help of splints and boots. His bone density and muscle toning increased. He was able to erect his knees in the 90 degree position while sitting at the edge of the bed.

With constant rehabilitation and a positive mind, he is slowly overcoming the weakness and trying to bring his life back to normal as much as possible.

Case 5:

A 30 year old basketball coach from Nasik, started experiencing weakness in his calf & thigh muscles at 19years of age in the year 2000. His weakness progressed and he consulted doctors. After a series of medical tests, he was diagnosed with limb girdle muscular dystrophy. Gradually, he started encountering difficulty in getting up from low seat as well as walking over rough, uneven surfaces leading to loss of balance while walking. Ability to do overhead activities was also hampered. Overall, his stamina dropped drastically, leading to easy fatigability while carrying out his official duties.

He could still perform all his daily activities by himself. But the emotional and physical effects of this disease hugely hampered his work life, his confidence level and his zeal for life.

He then heard of stem cell therapy. Even though the effectiveness of this therapy was uncertain, he was convinced about its safety and hence decided to give it a shot. Stem cell therapy was followed up by intense rehabilitation such as physiotherapy, occupational therapy, psychological counseling, etc. It began to have a positive effect on his life. His weakness improved slowly. His stamina increased and the effort required to perform daily activities went down. His problem of muscle cramps too, was resolved. He began lifting 5-6 kgs of weight. His muscles started becoming toned and his posture became more erect. He was able to stand and coach on the ground for 10 hours at a stretch without feeling excessive exhaustion. He has been able to continue coaching basketball. He coaches a team of 14 people, one of whom has reached the national level. He was able to coach his team so well that they have won local and district level competitions.

Stem cell therapy helped him to retain the profession that he had chosen for himself & one that he was so passionate about. Being the sole bread earner in the family, he was able to continue supporting his family. This has led to a boost in his confidence and has given him a renewed hope for life.

Case 6:

This child was a healthy boy of 9 and a half pounds at birth. He lived a perfectly normal life of a small boy till the age of 6. His life changed when one day, his teacher called his mother to complain about him being late to the class which was held on the first floor. From then on, a series of investigations followed which led to the conclusion that Shreyas had Duchenne muscular dystrophy. He began faltering in his walk and started falling frequently. He could not climb steps comfortably. Slowly, he lost independence in his daily activities and had to rely on his mother's assistance. He lost all power in his legs and became wheelchair bound.

His life took another twist for the better when his parents decided to try stem cell therapy as a treatment. With rigourous rehabilitation after stem cell therapy, his condition began to improve. Bed mobilities such as like getting up from lying position to sitting, rolling, side lying, which were earlier not possible, became easier & he became able to do them independently. He could maintain his balance on uneven surfaces such as the Swiss ball. He could perform daily living like eating, bathing, dressing, washing up after toilet activities; opening tap, etc. independently. But most of all, he is now able to stand independently and can walk with the help of a walker.

Appropriateness

To be appropriate, to act appropriately. This is to follow the Tao.

Appropriateness is a readiness for the situation as it really is, and not as one might wish it to be. Appropriateness thus has to do with creation and is indeed always creative. It is creative even when it creates nothing-for it is sometimes appropriate to create nothing. And to refrain from creating precisely when one is in a position to create itself is creative. This is true control.

Appropriateness cannot be gauged or measured in terms of necessary and sufficient conditions, for the later only exist in the physical universe- in the realm of doing and having- while the former exists in the realm of being.

To do what is appropriate is to do what is fitting or suitable to a situation. The situation is, however, in flux or change from moment to moment. To carry over "standards of appropriateness" from one moment to another is to fail to complete one moment and to set up a barrier to experiencing the next moment. This is to become stuck. What is appropriate is to have completed and always to be beginning anew-from and as cure. Any "standard of appropriateness" is thus a recipe for a lie.

Appropriateness, the Tao, the way, is revealed as unconsciousness is removed. As one begins to experience life, one's behavior effortlessly becomes more and more appropriate just in the process of life itself.

Appropriateness in a situation and control of a situation without force are thus identical. For appropriate action is not doing anything: it is neither submitting nor resisting; it is just being there.

The definition of the verb form, to appropriate, can now be understood. To appropriate is to take over something as one's own, is to be at cause.

-Werner Erhad

SECTION - D :

Miscellaneous

11:

Frequently Asked Questions

Patient, parents and caregivers have lots of questions in their mind, about the disorder, the progress,the treatment options, transmission, how it will affect the other members of the family,etc.The questions are endless,but in the previous sections, we have tried to answer most of them.

Still, for ease of understanding(even if repetitive) some of the most common and frequently asked questions have been addressed here.This list is not exhaustive by any means.Any questions not included here, would be addressed in specific chapters, as far as possible.

Q1 Is muscular dystrophy a contagious or an infectious disease?

A: No, it is not a contagious or an infectious disease.It is a genetic disorder and can be hereditary.

Q2 Is muscular dystrophy always hereditary?

A: Not always. Many investigations state that about 1/3 of all boys suffering from Duchenne or Beckers muscular dystrophy have no family history.

Q3 Which are the most common muscular dystrophies?

A: The most common is Duchenne and second most common is Beckers, followed by Limb Girdle muscular dystrophy which is the third most common form.

Q4: What is the first sign I will notice if my child has Duchene muscular dystrophy?

A: He may start talking and walking late. But these features occur in many other different conditions. Overall, movements are slower, the child may not be able to run like other kids, could have difficulty in getting up from the floor.The best option is to do a serum creatinine phosphokinase level (blood test), which would give a raised result(almost to the degree of thousands, eg.20,000 IU/ML, etc.).

Q5: Is muscular dystrophy a disease of the childhood only?

A: Not necessary.Some types, such as facioscapulohumeral, myotonic and limb girdle muscular dystrophy,manifest or appear or begin late, either in teenage or early youth .They are slow in progression and less severe than the childhood forms.

Q6: What are the few early signs of adult muscular dystrophy?

A: Facioscapulohumeral muscular dystrophy: weak smile or inability to whistle.

Limb girdle muscular dystrophy: weakness of shoulders and hip muscles, leading to difficulty in getting up from lower surfaces and raising hands above head.

In adult or teenage onset muscular dystrophy, symptoms or difficulties atrt a bit later in life, as opposed to DMD or congenital muscular dystrophy where problems a re noticed, many a times, in infancy itself.

Q7: How is muscular dystrophy transmitted?

A: It is a genetic disorder. It could be passed on from either of the parent via an affected gene.It could also be inherited, partly from mother and partly from father.

- In Duchene muscular dystrophy, the mother is a carrier of affected gene and the disease is transmitted to the male child. Each male born to a carrier mother has a 50% risk / chance of being affected and each female child born is at 50% risk of being a carrier.
- In myotonic dystrophy and facioscapulohumeral dystrophy, one parent could be the carrier (autosomal dominant).
- In limb girdle muscular dystrophy, either both the parents could be a carrier, which means that a combination of defect carried by both parents causes the disease in the child. Both male child and female child can be affected with the disease.

Q8: My son had / has Duchenne muscular dystrophy, can my daughter or me be a carrier?

A: If the genetic defect has been inherited from the mother, there are 50% chances of the daughter being a carrier too. Blood tests, such as genetic testing for carrier can be done to identify it.

Q9: Can this disease be detected during pregnancy?

A: Yes, if a mother is a known carrier, then amniocentesis or chorionic villi sampling (amniotic fluid from the womb) can be subjected to genetic testing for Duchenne or Becker's muscular dystrophy.If positive, it indicates the child is a sufferer. However,if the test is negative, it does not completely rule out the disease.

Q10: Why are muscles enlarged in muscular dystrophy children / patients?

A: The muscles, especially in the calf, are slowly replaced by fat which is known as pseudohypertrophy,i.e. muscles look large, but are falsely big.

Q11: What are the treatment options to help a child with muscular dystrophy to prolong his walking?

A: Surgical release of tight or contracted muscle can lead to immediately standing next day. Second option would be serial casting. These options can help children of muscular dystrophy to prolong independent walking. Regular rehabilitation and stretching also help in improving quality of life. Some people are of the opinion that steroids may help keep the child walking for longer periods.These are conventional methods.

New treatment option, such as stem cell therapy has also been found help improve muscle strength and prolong ambulation in a child.

Q12: In a child confined to the wheelchair,can surgery help him to regain his walking?

A: Surgery may help, but it depends on the status of the child's muscle power. Surgery becomes the best option when the muscles become very tight and are not stretchable at all.

Q13: How does physiotherapy and occupational therapy help muscular dystrophy patients?

A: It helps prevent contractures, keep muscles strong, improve efficiency of functional activities like standing, walking , learning proper methods for transfers (chair to bed, etc,)

Q14: Why should muscular dystrophy patient's exercise? Can exercises be harmful?

A: It is important to exercise, as it enhances physical, social and emotional development.Also, as mentioned earlier, it helps to maintain the child's condition, prevent contractures and deformities.However, if done beyond the capacity or in excess and not under proper supervision, it may lead to deterioration or worsening of condition.

Q15: Can swimming be beneficial?

A: Swimming could be beneficial for the patient.It is advised to start this activity from a very early age as it helps to increase endurance, increase muscle strength and is a good work up for the respiratory and cardiac muscles.

Q16: How do stretches help? : Can stretching be harmful?

A: Stretches prevent development of contractures. A regular stretching program, under proper supervision of a therapist,is a must as it keeps muscles and tendons supple. Yes, it can be harmful, if not done properly.Hence,it should preferably be done by the therapist. Alternatively, parents should learn the correct method under the supervision of a therapist.

Q17: Can weights be used to strengthen muscles in patients with muscular dystrophy?

A: No, using weights won't make the muscles strong because these muscles are different from other people.Lifting heavy weights can ,infact,damage the muscles. There are other exercises which help to strengthen the muscles. Please talk to your therapist about them.

Q18: What are contractures?

A: Contractures are the tightening of the muscles which occurs because not all muscles loose strength at the same time and patients develop irregular postures to compensate for the weak muscles. These irregular postures lead to more tightness in some muscles leading into development of contractures.

Q19: Is wearing night splints compulsory?

A: Wearing of night splints is often recommended as soon as the diagnosis is made. It should be well tolerated and should be accepted by the patient. Ankle foot orthosis holds up the feet ,which is the most comfortable and desired position for the foot.

Q20: Is massage recommended in patients with muscular dystrophy?

A: We generally do not recommend massage for muscular dystrophy patients due to the following reasons:

a) It can fasten muscle damage or degeneration

b) Pressure during massage can inadvertently cause fracture of already brittle or fragile bones of the patients.

Q21: Are alternative therapies like Acupuncture, Acupressure or Ayurvedic recommended?

A: Since, we are not experts in this field, we would not be able to comment on its utility or otherwise for muscular dystrophy. We would recommend you to consult your physician regarding the same.

12:

Genetic Counselling

Parents, especially the mother of a boy afflicted with DMD , often wonder whether they should have another child. When a mother is told that she could be a carrier and her daughter could be a carrier too, she is not only concerned about her son's present , but also her daughter's future. In our day to day practice, we come across parents, some of who are well informed, but many who are clueless as to where they should seek advise regarding genetic testing, what are its implications on the current family and future generations.

For DMD, with genetic testing being more widely and easily available, most of these questions can be addressed reliably.

How is muscular dystrophy transmitted?

Muscular dystrophy is primarily a genetically inherited disorder. Genes linked together on the chromosomes, code for the production of proteins, and they are the material of inheritance. Parents pass these genes to their children, providing them with a complete set of instructions for making their own proteins. Since, both parents contribute genetic material to their offspring, each child carries two copies of almost every gene, one from each parent.

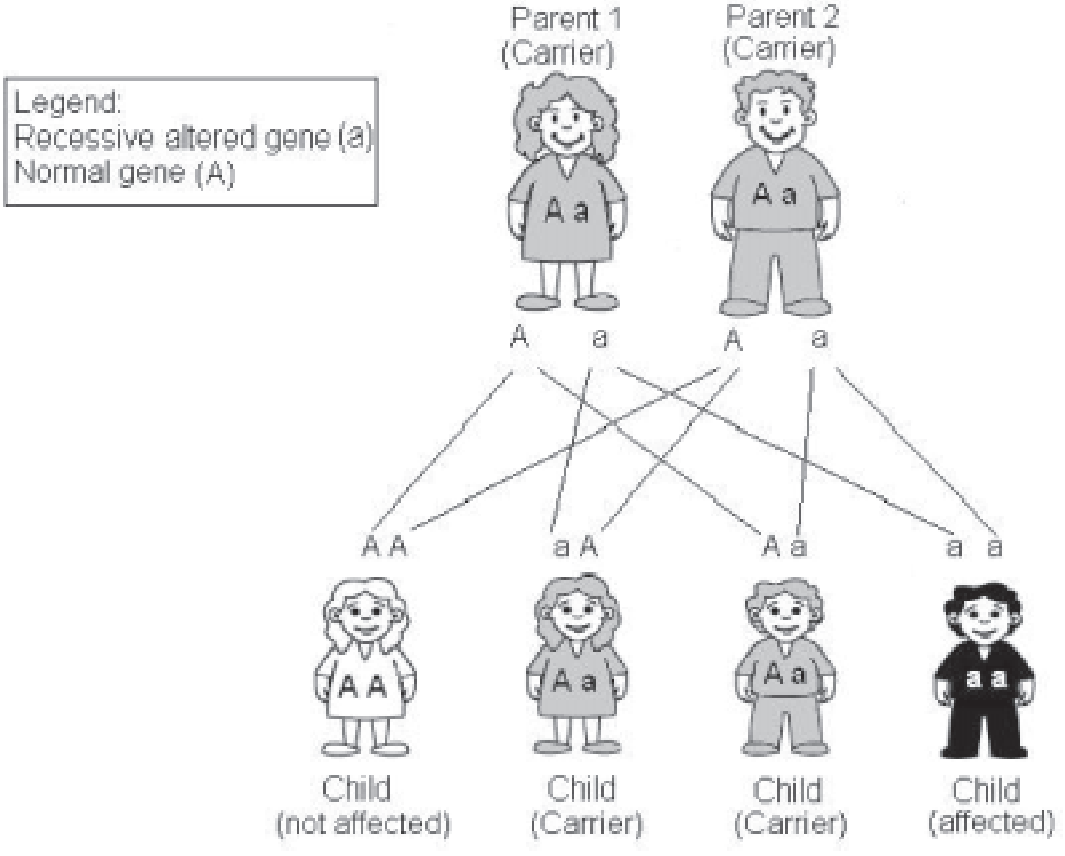
For some diseases to occur, both copies must be flawed. Such diseases are called autosomal recessive diseases. Some forms of Limb Girdle Muscular Dystrophy, Distal Dystrophy and Congenital Muscular Dystrophy exhibit this pattern of inheritance. A person with only one flawed copy, called a carrier, will not have the disease, but may pass the flawed gene on to his children. When two carriers have children, the chance of having a child with the disease is one in four (25%) for each pregnancy. This means that, in certain types of limb girdle muscular dystrophy, either both the parents could be a carrier, which means that a combination of defect carried by both parents causes the disease in the child. Both male child and female child can be affected with the disease.

Some other type of muscular dystrophy occurs when only one flawed gene copy is present. Such diseases are called autosomal dominant diseases. For eg., in myotonic dystrophy and facioscapulohumeral dystrophy,which are autosomal dominant diseases, one parent could be the carrier (autosomal dominant).With only one defective gene from one parent, a child can be a sufferer.

Other forms of LGMD exhibit this pattern of inheritance, as do Myotonic Dystrophy, Fascioscapulohumeral Dystrophy, Oculoperoneal muscular dystrophy, and some forms of Distal Dystrophy. When a person affected by the disease has a child with someone not affected, the chance of having an affected child is one in two.

DMD, BMD, and Emery Dreifuss Muscular Dystrophy ,on the other hand, are X-linked disorders, where in the genes on the X chromosome are defective. This means that males being born to such a mother have 50% chance of having this disorder. That means,in Duchene muscular dystrophy, the mother is a carrier of affected gene and the disease is transmitted to the male child. Each male born to a carrier mother has a 50% risk / chance of being affected and each female child born is at 50% risk of being a carrier.

However, it is also important to be aware that,not all genetic flaws are inherited. As many as one third of the DMD cases are due to new mutations that arise during egg formation in the mother. New mutations / flaws are less common in other forms of muscular dystrophy.



In a child of DMD then, what are the implications for the sister or mother?

The mother of such a child, does have the possibility of being a carrier. If she tests positive for a carrier test, there are 50% chances of the daughter being a carrier too. Blood tests, such as genetic testing for carrier can be done to identify it.

Can this disease be detected during pregnancy?

If a mother is a known carrier, then amniocentesis or chorionic villi sampling (amniotic fluid from the womb) can be subjected to genetic testing for Duchenne or Becker's muscular dystrophy.If positive, it indicates the child is a sufferer.However,if the test is negative, it does not completely rule out the disease.

Though the above few are very simplistic answers to a grave subject, to explain the complexities of the inheritance(how defect/flaw is transmitted from parent to child) and the prognosis of the genetic disorder ,genetic counselling is required.

Genetic counselling provides information to the parents or relatives, about the risk of inherited disorders, the nature of the disorder, ways by which it is transmitted, the risks to other family members, the risk associated with having another pregnancy, and the options for management and family planning.

Genetic counselling is provided by a team of health professionals who work together to provide an individual or family with all the current information and supportive counseling.

While seeking genetic counseling, family members are concerned about (1) risk of disability in their offspring, (2) own risk of disability, (3) appropriateness of exercise or other treatments, (4) prevalence of the disorder, (5) possibilities for predictive and prenatal testing.

During the consultation, family history is collected. Genetic condition may be diagnosed or confirmed in a pregnancy, after birth, in childhood or later in life. The diagnosis may be made on the basis of clinical features, biochemical tests or genetic tests.

Where there is a genetic condition in a family, the counselling team may estimate the risks of other blood relatives, or future children, getting affected by the condition. Following the counselling, the person can take measures to avoid the recurrence of the disorder.

They discuss and arrange appropriate genetic testing, including diagnostic, carrier, predictive and presymptomatic testing, where available

The various tests include:

- Diagnostic testing (to determining if the patient undergoing the test has the disorder)
- IVF and pre implantation genetic diagnosis or PGD (testing the embryo for the disorder before it is used in the IVF procedure)
- Prenatal testing (testing the fetus while in the womb of the mother)
- Predictive testing (tests that can diagnose a disorder that can develop in adulthood)

- Carrier testing (a carrier is someone that carries the disorder and can pass it onto their children but is not affected themselves.).

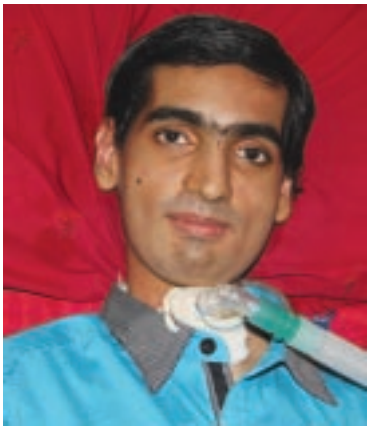
Before undergoing the genetic testing for muscular dystrophy it is very important to have a discussion with both the counselor and the family. There are various factors which need to be considered. These include; the implications of a positive, a negative and an uninformative test result on all members of the family and the effect of these results on the relationships. It is important to remember that the genetic test itself is specific for the disorder being tested; therefore it is unlikely that the genetic testing will uncover any other underlying disorders.

Genetic counseling thus helps people understand and adapt to the medical, psychological and familial implications of genetic contributions to the disorder.

13:

A word from parents/patients

Mr. Chandrashekhar Kant- father of Ankur Kant 26 years old Duchenne Muscular Dystrophy



"We have learned to live powerfully and enjoy it fully in every moment of life, but it was not so 22 years back. What happened was I took my son Ankur for a normal checkup to a child specialist Dr. Athavle. In his routine checkup everything was normal.

As we were about to get out of his cabin I shared my normal observation about my son's activity. I told him my son is like all other kids, the only difference is that when he gets up from floor he takes the support from floor and then of his knees by his palms and stands up straight. He also can run but stops abruptly and falls down.

After I told this to doctor, he examined him by rubber hammer and by making him sit down and get up again and again. I was observing doctor and he looked confused and stressed. He referred us to another doctor at Hinduja Hospital.

Dr. Bernard D'Soza examined him and said your son is a patient of Duchenne muscular dystrophy but let us do the muscle biopsy to confirm it. After the reports, it was confirmed and we had meeting with doctor. They told the same story which parents of every Duchenne muscular dystrophy patient has to listen, like no cure, no possibility, you have to live with it and the ultimate eventuality that you have max 18 to 20 years.

I was absolutely blank, did not know how to react or respond and what to think and the most important was how to accept what is so. When our relatives and friends came to know about it, they came to visit us to express how bad they feel because we were the most unlucky father and mother.

My life was on hold, I did not know what to do, how to do, how to proceed in life and most important was why of all the people it is me. There were only questions without any answer. This life was fired at me from point blank range and there was no way it could miss.

I was left with no choice than to accept what is unacceptable, undesirable and absolute unjust to me. I was not a bad person so that God will enforce such a punishment for me. When I have not hurt anybody emotionally or grabbed somebody's happiness then why it is me. All the time I was occupied by these thoughts. This made me more upset, helpless and totally isolated.

As I had no answer to any of the questions even I was scared to look at my son. My wife was crying all the time, she kept herself away from son. I did not know how to support both of them. I thought best is to go away from this situation and I did it. I went to Goa. Even in the company of beer the thought about my son never vanished, on the contrary it became more torturous.

That is where the first insight came that I cannot run away from what is happening to my family. I had no guts to face the situation and standing in this uncertain position in life made me go crazy.

Even when I was sitting alone my mind did not allow me to get out of this vicious circle. Suddenly I remembered one of the things which doctor said, that the possibility of having such a son is one in 3500. One such boy is sacrificed by the GOD to complete the cycle of mutation and to ensure that other 3499 kids live normal healthy life. So my son happened to be the unique outstanding sole and because of him I have become a special father of a special son. This has happened only to me among 3500 families because God has full faith in me that I can take care of him and support him completely, what he is supposed to complete in his life time. He is a special gift from God to us. Out of this awareness, my being upset completely disappeared.

My thoughts became clear and now I could think about what is workable in alignment with how life works. Every creation of God has definite intentions for things to be the way they are. My son is born with some intentionality and the intention of me being born as his father is to support him, to complete his intentions he is born for. It is important to fulfill the intentions of the creator otherwise we will be discarded as complete human being.

The bottom line of my interpretation was that he is my son because God has faith in me

to take care of him and support him completely, what he is supposed to complete in his available life span. This shift in my paradigm has transformed my approach to living life powerfully.

My son was still the same, his sickness was the same but it stopped diffusing me and making me upset. On the contrary it gave me additional strength to live life powerfully. I HAD THE DEFINITE INTENTION TO LIVE MY LIFE.

This person who was coming back from Goa was a totally different person willing to live for the purpose intentionally. I entered my home with full of vitality and bulldozed everyone. All the complains from everyone's thoughts had disappeared. This is how we have learned to distinguish distinction in life.

We gave up all the conversation which the doctor had given to us about my son's disorder and learned to live up every moment made available to us to generate joy and happiness. In response to attitude towards life, my son has never complained till date that why it is me. He answered this question by saying that it is me because I am capable enough to handle it and I will make a difference in the attitude of other kids so that they could create a new dimension of living a powerful life.

He is my Guru, I have learned all the values of life, I am the way I am because Ankur was gifted to me by God. In support of calling him my Guru, I will share the most important insight he gave to me.

From my young age I had one question "what is the difference between life of a human being and life of animal, say life of a man and life of a dog?. That man is born from mother and even the dog. Man's first feed is mother's milk, same for a dog. Man gets educated in how to earn food and comfort &even dog gets his education. Man finds a match and produces kids, even it is true for dog. Man becomes old and dies, even this happens with dog. Then what is the difference between life of a man and dog?" I have asked this question to some transformed people. Either they have dodged my question or they have given me some irrelevant answer.

I asked the same question to Ankur and he said he knows the difference between life of a man and life of a dog. The difference is that there is no difference in the life. There is a difference in the way they die. I said I did not understand, so he said dog is born as dog and he dies a death of a dog, but the man is born as a man and he dies a death sometimes worst than a dog, like a dog, like a bad man, good man, like a saint or even like a god. All these possibilities exist for a man and not for a dog. The man who chooses today how he is going to die and lives in accordance with it, that man is born like a man lives like a man and dies according to what he chooses to accomplish in life.

So many changes kept on happening in Ankur's life. He stopped walking then he was unable to stand. We used to lift him and take him to school. Then CO2 retention, hospitalization occurred again and again. Ankur came from hospital with tracheotomy and ventilator which has not left him till today. He has taken 4 stem cell treatments and has shown definite improvement at the age of 26 years. This has made a difference in his life and promises to do so in the lives of many young kids. In spite of all these happenings, he is still cool, calm, charming, and joyful and lives a fulfilled life every moment."

Kadambari Karalkar -Shreyas's mother

My son Shreyas,12 years old, is known case of DMD since the age of 6 yrs. I could not believe that I had given birth to a very healthy child whose weight was nine and half a pounds. It was only later that we found out that he is suffering from an incurable disease called Duchenne muscular dystrophy.

At the age of 6 yrs, Shreyas was attending Christ Church School, Byculla and was traveling by school bus every day. One day, his teacher called me and said that whenever he has computer period which was on first floor, "he always comes last so please check if there is any problem and we will help you". I was surprised to hear that. I remember it was in August 2006. I took Shreyas to his regular doctor-Child specialist Dr. Satoskar on Aug 12 2006.I told him about Shreyas .Dr. have asked me check up for CPK. I went for it on next day morning. I went to pathologist Dr. Beke for blood test. Next morning he called me again for blood test when we went there. I had no doubt that Shreyas was healthy. However, CPK level was too high 18,000.

Next morning I went to Dr. Satoskar and showed him the reports. He was very calm and cool because he had prepared himself to talk to both the parents. He told us about DMD. Until that day we were not aware of this DMD. Muscular Dystrophy is an inherited disease.

Muscular Dystrophy Society was founded in 1973 to give cheer to the afflicted, as there was no treatment available for this disease. The society has concentrated on improving

their life style, increasing their ability to stand and walk for more number of years. It is characterized by progressive weakness of the muscle which control movement.

That day it was very difficult for me and Shreyas' father to accept this TRUTH. We cried a lot. We didn't know what to do. On Sept 04, 2006 DMD reports confirmed that Shreyas was DMD positive. I cried for half an hour in the Bombay Hospital, telling nothing to anyone about this. We kept asking a question to GOD-why this to my child? We kept asking help from God. It was HEART breaking news for us, as his mother and father.

Hence, Shreyas new life journey started with Wadia Hospital for physiotherapy. We had to meet neurologist and physiotherapists as well. It was very hard time for me to accept the fact because there is no hope for a life for DMD patient. Doctor suggested steroids. But we decided not to give him any steroid. There was a big question mark about what to do?

Until age of 8 years he was riding the cycle. In between age 6 to 8 years, DMD started showing symptoms. His school was changed when he was in 2nd standard. We shifted him to nearby school. Going on scooter sitting in front of me. He used to climb up and down the first floor with me. For two years, he used to walk himself to school. When he went to 4th std, he stopped climbing up. Then I used to carry him on the first floor. 5th and 6th std was on ground floor so it was easy to take him to the school. Slowly slowly, he stopped climbing down and then walking. We could see Shreyas walking on toes and finding it very difficult to get up from floor, taking support of wall & then knee and then straight up. As his mother, it was very hard for me to see his condition day by day, becoming dependent on me. It felt like someone was hammering on my heart.

At last God listened to my prayers. As per the news in Times of India, dated 2nd January 2010 about Ankur Kant, a miracle of STEM CELL inspired me a lot. We ran to meet Dr.Alok Sharmaji. Finally we met and Shreyas new journey started towards LIFE. We could see a RAY OF HOPE. First Stem Cell shot was taken on 10th may 2010. In the follow up visit, after 3 months, Shreyas reported feeling of well-being and improved stamina. His upper limb and grip strength had increased with independence in activities of daily living like eating, bathing, dressing, washing up after toilet activities. He could sustain standing for 30 mins initially but gradually progressed to 50 mins. At the end of 8 months post therapy, he was able to stand independently with a walker.

One thing about Shreyas was that he NEVER EVER complained about PAIN. He is a very co-operative and obedient child. I am very proud of my son Shreyas that he wants to WALK and he will. During these years he has worked hard to achieve his GOAL.

Payal Jain, 25 years old Duchenne Muscular Dystrophy

The birth of every child marks the beginning of a new life and makes us marvel at the wonders of the creations of the Almighty. I am sure, when my parents held me for the first time; they must have gone through these wonderful emotions. Being born in a joint family, my happiest memories are those of my childhood when I, like every normal child, was an active, healthy & enthusiastic child and I enjoyed a lot with my same-aged siblings & cousins.

But when I turned seven, things began to change. My calves had become quite bulky & as hard as a stone, wherein I started having difficulty walking. I felt uncomfortable standing even on my heels & I began walking on my forefeet. There came a point where everyone started scolding me which now I can understand as the insecurities & fears of my elders and also because I WAS A GIRL.

Now began the battle against those unknown fears. At the age of 9, I under-went muscular biopsy from which it was diagnosed that I have D.M.D. (Duchenne Muscular Dystrophy), which was not only shocking for my parents but also for the doctor because in his 25 years of experience, it was his first case.

Unable to apprehend the severity of the situation, I felt scared, lost & totally confused as frequent falls during walking, difficulty with jumping and other motor skills became prominent. Each night, I would weep in my mother's lap and she would console me saying that everything would be alright soon.

Every morning became a torture because going to school became an uphill task. My classroom being on the first floor, I could only go there with the help of others. My

sports activities were brought to an abrupt halt. It was as if everything had come to a standstill. I stopped going to school regularly and only attempted exams.

Not only were my legs affected, but my overall physical well-being was hampered to the extent that since 2005, I became totally dependent on others. From dressing to combing my hair, from having a bath & even going to the loo, I NEEDED HELP. And that's where the word 'BURDEN' engulfed me into a more lonely despair.

I started losing contact with my friends may be because I discontinued my studies. I was feeling alone, tormented & would keep asking myself why was I not like others and the only thought that kept creeping forth & back was 'WHY ME?'

Staying in a small town, many people would keep coming home and stare at me, making me feel like a butt of ridicule & advised my parents in different ways. On their advice, my parents even took me to tantrics, but deep down, we all knew, it was to no avail.

By this time, I had actually come to terms with the fact that this is what DESTINY had in store for me. I had to accept myself as I was but there was this constant gnawing lingering within me which kept asking, "IS THIS IT?" "HAS EVERYTHING ENDED FOR ME?"

The head would tell me, "accept yourself & try to think positive," but the heart would say, "THERE HAS TO BE A WAY." The heart prevailed & began yet another battle, this time it was FOR ME, WITHIN ME & against my OWN DOUBTS.

I started making myself more aware of the disease under the able guidance of Dr. Bharucha. He made things more clear for me and assured me things were much better for me than others who suffered the same fate.

The first thing I did was I accepted myself as I was. Then came the formidable task of doing what I believed in. I started to concentrate on what I had rather than what I didn't have.

With the permission and support of my family, I decided to share the talents I had. I was always good at painting, drawing, sketching, handwriting & mehendi. My arms were weak but I continued my interest. I started taking classes at home itself and I got a beautiful response. I had small children keeping me occupied & busy the entire mornings & evenings. The best thing I felt, I could relive my childhood memories because they would share all their secrets and happenings at school with me & last but not the least, I felt WANTED.

They say that one step leads to another. My classes gave me such a huge popularity and entirely different outlook. My first solo painting exhibition was held at Jodhpur and this was yet another beginning. Today, when I look back, I hold no grudges and my zeal to try to do something new & creative keeps me in that positive frame of mind.

A special thanks to the Almighty because he gave me the strength to cope with this where many would have given up without a fight and to my entire family which never made me feel less fortunate.

If given a chance, I would love to do something for the children with special needs & one message to all - We don't need your PITY but your ACCEPTANCE and SUPPORT.

Shaan Sood, 19 years old male of Duchenne Muscular Dystrophy

"My name is Shaan Sood and I am from the United States. I am nineteen years old and I am suffering from Duchenne Muscular Dystrophy (DMD), a disease which causes the progressive deterioration and wasting of the ambulatory muscles, lungs, and heart.

I was diagnosed with DMD at the age of three years, when my mother referred me to a physician upon noticing that I had difficulty climbing up curbs and raised areas. After a muscle biopsy was conducted, it was discovered that I indeed was suffering from DMD. Genetic testing revealed that the disease had no previous history in my family line - the incurable disease was the result of a gene mutation. The disease progressed rapidly and simple activities like walking, running and climbing continued to become increasingly difficult.

My mother tried various "treatment" options, including Ayurvedic treatment in India and visits to the muscular dystrophy research center in America, but to no avail and the disease continued to progress. At the age of ten years, my neurologist placed me upon the steroid Deflazacort. Although this rendered side effects such as weight gain, insomnia, and mood swings, the risks were outweighed the by benefits, as we hoped that the steroids would keep me on my feet and walking as long as possible.

When I entered my seventh year of schooling, my falls had at this point heavily increased, and the school administration, fearing a lawsuit, attempted to force me to utilize a motorized wheelchair upon the campus. However, my mother heavily contested this decision, as resorting to a wheelchair would surely speed up the muscle wasting

and leave me bid-ridden sooner than necessary. It is a good thing that she did so, or else I surely would be wheelchair bound at this point in time. My orthopedist's instructions to keep me on my feet for as long as possible proved pivotal in the maintenance of my ability to walk.

At the age of about thirteen, my mother sat me down and explained what exactly my disease entailed, and although I already noticed that I was indeed slower than the other children, this true reasoning made things much clearer. I understood what I was facing now, yet the battle was far from over.

I continued through my education, and of course the deterioration of my walking and climbing abilities continued as well. At my eleventh year of schooling it was noticed that my heart was not pumping well, and 24-hour heart monitor testing revealed that there was evidence of arrhythmia in my heartbeat. At a cardiac specialist's instruction, an implantable cardioverter-defibrillator (ICD) was inserted in my chest as a precaution to correct any further cases of arrhythmia. The ICD delivers a jolt of electricity to the heart if arrhythmia occurs in order to correct the irregularity in the heartbeat. Although the device has never "shocked" me, it is a necessary precaution to prevent cardiac failure.

During my twelfth and final year of grade school, my aunt, living in Mumbai, read about Neurogen Brain and Spine Institute in a local newspaper, and encouraged my mother to bring me to Mumbai for stem cell treatment. I took a month off from school and made the long journey to India.

As most parents are, my mother was apprehensive about the treatment, but Dr. Alok Sharma assured her that there was no risk in the procedure, and I soon received my first round of stem cell therapy. Two days after the procedure, I noticed that my toe-walking had decreased, and my left heel was lying flat on the floor. I returned home to the United States and continued my schooling, coupled with aggressive physiotherapy, and over the next couple months, I was walking, getting up from bed, and climbing stairs with more ease.

In June of 2011, I graduated from grade school with honors, and returned to Neurogen in September of that year for a second round of stem cell therapy. I spent three months in India after the stem cell round to focus on physiotherapy, and continued to maintain my improvements from the first shot, as well as build upon them. I returned home in December and began my university studies. However, I unfortunately neglected my therapy in my attempts to focus on schooling, and whatever exercise I was doing there was either improperly done due to lack of a good physiotherapist, or was not enough, as I began to experience some fatigue and walking difficulties again. I returned to Neurogen in July of that year with the hopes that a third shot would reverse this unfortunate turn of events. Shortly after another month of physiotherapy, I began to regain my former stamina, and plan to spend another four months in India in order to focus on aggressive physiotherapy. My university studies are temporarily on hold, but I plan to finish my undergraduate degree within the next two years.

I am confident that it is not religion, but science that will discover a cure to this horrendous disease, as a cure would not even be necessary if God did not put this curse upon innocent children. The advancements in gene therapy that are occurring

throughout the world at this point in time are breaking the boundaries of modern medicine, but until the point when a permanent cure is found, the stem cell therapy offered by Neurogen is a surefire way to slow down or even stop the rapid deterioration that DMD causes. Even halting the progression of the disease is good enough in this condition, as it allows the victims to live a somewhat improved quality of life.

It is important to remember that this stem cell therapy is only effective if coupled by constant physiotherapy, and children should bear through the pain associated with DMD and its treatment, be it emotional or physical. For those DMD children bound to wheelchairs, wearing splints and standing may be tremendously painful, but this pain should be faced head-on, as this is the only way for these children to improve their quality of life and have a chance at standing or walking with assistance, and eventually on their own. Though I was walking before the stem cell therapy, the manner in which I walk has improved as a result of the treatment, so even those patients that can still walk should still pursue the therapy equally aggressively, if not more so.

Hobbies like music and videogames are important for children with DMD, as much as physiotherapy, as it makes the reality of the condition easier to bear, and provides an excellent way to funnel emotional turmoil. For me, exercise proves to be a good outlet for anger or sadness, normal emotions for children with these conditions. Parents should encourage their DMD children to pursue hobbies and also to partake in physiotherapy, and should keep strong and continue to support and nurture these children."

14:

Association and Support Groups of Muscular Dystrophy

Associations, societies and groups supporting Muscular Dystrophy

The world over, parents, patients, care-givers and other professionals have come together to form unions dedicated to muscular dystrophy. From funding research to providing education & advice to sharing emotional support, the objectives of these organizations span across all aspects related to the disorder. Millions have benefited from their activities.

This section details some of the bigger associations and lists down other popular associations across different parts of the world.

India:

1. The Association for Muscular Dystrophy:

About

The Association for Muscular Dystrophy is a Mumbai-based organization founded by patients & parents, with the support of medical & other professionals who are committed to caring for such affected persons. It was started with a view to bring such people together on a common platform. AMD is a new organization which aims at forming a community of muscular dystrophy affected persons and providing them with help in all possible ways.

Objectives:

- o To create awareness about Muscular Dystrophy.
- o To provide support, emotionally & practically, to one another.
- o To share experiences & exchange ideas for improving quality of life.
- o To provide assistance, monetarily & in kind, for the betterment of such people.
- o To find & evaluate treatment options especially stem cell therapy.
- o To encourage & promote research & education in this field.

Contact:

Plot No.5, Rockland, IC colony,
Borivali (West), Mumbai 400103.

www.musculardystrophyindia.com
help.amd@gmail.com
Tel: Mr Jeegar Mota - 9821151851
Tel: Mr. Chandrashekhar Kant - 9870105515

2. Indian Association of Muscular Dystrophy:

About :

The Indian Association of Muscular Dystrophy (IAMD) was founded in the year 1995 with a mission to assist and rehabilitate the patients of Muscular Dystrophy, India.

Objectives:

- To look after the interests of Muscular Dystrophy patients and to see that they lead a comfortable life
- To have IAMD chapters in all the major cities of the country.
- To integrate MD patients into the main stream of society.
- To make a disabled friendly health resort.
- To encourage implementation of the 1995 Disability Act.
- To bridge the gap between able bodied and physically challenged people.

Contact:

Indian Association Of Muscular Dystrophy (IAMD)
C/o. M/s Stich-n-Style, Hospital Road, Solan,
Distt. Solan, Himachal Pradesh, India
Pin-173212

www.iamd.in / info@iamd.org
Tel: (+91)1792-223183/1792-220212

Other organizations in India:

3. **Muscular Dystrophy Society,**
   4th Floor, J.J. Hospital,
   Byculla, Mumbai 400 027, Maharashtra.

   Tel.: 022 - 2375 3875

4. **Muscular Dystrophy Association India**
   C/o. V.J.Clinic, New no: 6, (Old 21),
   Fourth Cross Street,
   Sastri Nagar, Adyar, Chennai 600 020, Tamilnadu.

   Website : www.mdaindia.org
   mdaindia.org@gmail.com
   Tel.: 91-90032 95482

5. **Sundaram Medical Foundation**
   A Network Center of the Co-operative
   International Neuromuscular Research Group
   Shanthi Colony, 4th Avenue,
   Anna Nagar, Chennai 600 040, Tamilnadu.

   Website : www.smfhospital.org
   Tel.: 91-44-26268844

6. **Muscular Dystrophy Foundation India**
   C/o. People's Watch, No.6, Vallabhai Road,
   Chokkikulam, Madurai-625 002, Tamilnadu

   Website : www.mdfindia.org
   louis@mdfindia.org
   Tel.: 91-9994368550

7. **Jeevan Foundation**
   Head Office : 12/5, Sandilya Apartments,
   Jagadambal colony, 2nd Street, Royapettah,
   Chennai 600 014, Tamil Nadu.

   Website : www.jeevanfoundation.com
   E-mail: help@jeevanfoundation.com
   Tel.: +91 44 28474400 Fax: +91 44 43526647

8. **All India Muscular Dystrophy Association**
Anaj Mandi, Nabha Gate, Patiala 147 001, Punjab

Website : www.mdaindia.com
contact@mdaindia.com
Tel: 91-175-2215786

9. **Indian Muscular Dystrophy Society**
16, Tolaknagar, Paldi, Ahmedabad 380 007, Gujarat.

Website : imdsindia.org

10. **Indian Muscular Dystrophy Association**
M.I.G.72, APHB Colony, Machilipatnam 521 001, Andhra Pradesh

Tel.: 91-086-722817

11. **All India Muscular Dystrophy Association**

Anaj Mandi, Nabha Gate, PATIALA 147 001

aditi98172@yahoo.com
Tel.: 91-175 215 786 • Fax: 91-175 200 786

12. **Muscular Dystrophy India**

Website : www.mdaindia.com
Tel. : +91-0715-2215786
E-mail : contact@mdaindia.com / amardeephira@yahoo.com

13. **Muscular Dystrophy Support Group, South India**
Muscular Dystrophy Society
'Ratnakar' 2nd Floor, Block 5, Narayan Dabholkar Road,
Malabar Hill, Walkeshwar, Mumbai 400 006.

E-mail : ranil@vsnl.com

United States of America:

1. **Parent Project Muscular Dystrophy (PPMD) :**

About :

Parent Project Muscular Dystrophy is a non-profit organization, based in USA. It was founded in 1994 by Pat Furlong, a mother of 2 DMD boys. As the name suggests, it comprises of parents, grandparents & other care-givers of young men suffering from this debilitating disorder. Over the years, PPMD has grown from a small group of people to the largest American organization dedicated to the cause of Duchenne muscular dystrophy, with one aim in mind-to end Duchenne. It focuses strongly on research, to determine the causes, treatment & cure for DMD. Many families have received support in all forms through this organization.

The strength of this organization lies in the unwavering care & concern of parents & grandparents of such individuals and their constant efforts towards improving the lives of their loved ones who have been afflicted with DMD.

Objectives:

- To fund, promote & encourage research & development.
- To create awareness and extend care & support.
- To work with government bodies to ensure that concern for DMD is accounted for during decision-making.
- To integrate the efforts of all medical & paramedical professionals across the globe for the betterment of DMD individuals.
- To build a DMD friendly environment by providing information, care, community support, legislative support, medical help, financial aid & in all other ways possible.
- To connect such similar communities the world over, facilitate an exchange of ideas and extend care & support.

Contact:

401 Hackensack Avenue, 9th Floor,
Hackensack, NJ 07601

info@parentprojectmd.org
http: / / www.parentprojectmd.org
Tel: 800-714-KIDS (5437)
Fax: 201-944-9987

2. **Muscular Dystrophy Association**

About:

For more than 60 years, MDA has provided help and hope for families living with neuromuscular diseases. MDA today is one of the world's leading voluntary health agencies, fostering research and medical care. It is a nonprofit health agency in the United States of America dedicated to curing muscular dystrophy, ALS and related diseases by funding worldwide research. It helps assist with the cost of repairs and help locate durable medical equipment for those it serves. It provides thousands of free flu vaccines. It funds around 200 neuromuscular specialty clinics across the United States of America. It is dedicated to finding treatments and cures for more than 40 neuromuscular diseases which cause progressive muscular weakness. It funds around 300 research projects, spearheaded by scientists worldwide.

Objectives:

- To conduct ongoing public health education programs through webinars, educational speakers, seminars, videos and newsletters.
- To provide comprehensive health care and support services, advocacy and education
- To accelerate therapy development by sponsoring national and international scientific meetings about disease research (such as 2012 MDA Clinical Conference), and through collaborative efforts with federal agencies and other organizations in the U.S. and around the world.
- To both accelerate therapy development and expand resources for families affected by muscle disease.

Contact :

3300 East Sunrise Drive
Tucson, AZ 85718-3208

Website: http://www.mda.org
Email: mda@mdausa.org
Tel: 520-529-2000 800-572-1717
Fax: 520-529-5300

3. **Jain Foundation**

About :

The Jain Foundation is a non-profit foundation whose mission is to cure muscular dystrophies caused by dysferlin protein deficiency, which includes the clinical presentations Limb-girdle muscular dystrophy type 2B (LGMD2B) and Miyoshi muscular dystrophy 1 (MMD1).

Objectives :

- To fund and actively monitor the progress of scientific research projects in key pathways towards a cure.
- To provide financial and logistical support to promising drug candidates to accelerate them to clinical trials.
- To fund clinical trials and studies.
- To encourage collaboration among scientists.
- To educate LGMD2B / Miyoshi patients about their disease and help them with their diagnosis (e.g., funding dysferlin protein and gene mutational analysis).

Contact:

2310 130th Ave NE, Suite B101, Bellevue, WA 98005

Website: https: / / www.jain-foundation.org /
Email: ehwang@jain-foundation.org
Tel: 425-882-1440
Fax: 425-658-1703

Other organizations in USA:

4. **Coalition to Cure Calpain 3 (C3)**

   15 Compo Parkway, Westport, CT 06880

   http: / / www.curecalpain3.org /
   info@curecalpain3.org
   Tel: 203-221-1611
   Fax: 734-668-4755

5. **Facioscapulohumeral Muscular Dystrophy (FSH) Society**

   64 Grove Street, Watertown, MA 02472

   http://www.fshsociety.org
   info@fshsociety.org
   Tel: 617-658-7877
   Fax: 617-658-7879

6. **International Myotonic Dystrophy Organization**

   P.O. Box 1121, Sunland, CA 91041-1121

   http://www.myotonicdystrophy.org
   info@myotonicdystrophy.org
   Tel: 818-951-2311/866-679-7954

7. **National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS)**

   National Institutes of Health, DHHS
   31 Center Dr., Rm. 4C02 MSC 2350, Bethesda, MD 20892-2350

   http://www.niams.nih.gov
   NIAMSinfo@mail.nih.gov
   Tel: 301-496-8190 877-22-NIAMS (226-4267)

8. **Centers for Disease Control and Prevention (CDC)**

   U.S. Department of Health and Human Services
   1600 Clifton Road, N.E., Atlanta, GA 30333

   http://www.cdc.gov
   inquiry@cdc.gov
   Tel: 800-311-3435/404-639-3311/404-639-3543

9. **National Institute of Child Health and Human Development (NICHD)**

   National Institutes of Health, DHHS,
   31 Center Drive, Rm. 2A32 MSC 2425, Bethesda, MD 20892-2425

   http://www.nichd.nih.gov
   Tel: 301-496-5133
   Fax: 301-496-7101

10. **Cure CMD**

    P.O. Box 701, Olathe, KS 66051

    http://www.curecmd.org
    info@curecmd.com
    Tel: 1-866-400-3626

11. **Muscular Dystrophy Family Fund**

    1033 Third Avenue SW, Suite 108, Carmel, IN 46032

    http://www.mdff.org
    info@mdff.org
    Tel: 317-249-8488
    Fax: 317-615-9140

12. **Angel's Muscles**

    Avalon, NJ, Melissa Barone

    www.angelsmuscles.org
    chrbaro@aol.com

13. **Charley's Fund**

    36 Mian Street, PO Box 83 Stockbridge, MA
    Tracy & Benjamin Seckler

    Tel: 413- 289-4300

14. **CIMRG, Centre for Genetic Medicine**

    Research Centre 3, Children's Research Institute,
    111 Michigan Avenue, NW, Washington, DC 20010

    www.cinrgresearch.org
    info@cinrgresearch.org
    Tel: 202-476-5241

15. **Coalition Duchenne**

    Newport Beach, CA, Cath Jayasuriya,

    www.coalitionduchenne.org
    catherine@coalitionduchenne.org

16. **Cooper's Cure**

    P.O.Box # 604, Hermosa Beach, CA 90254,
    Cathy and Scott Jones.

    www.cooperscure.org

17. **Cure Dale's Duchenne**

    The Gainesville Community Foundation
    5214 SW 91 Drive, Suite A Gainesville Fl 32608

    www.curedalesduchenne.com
    ginders@curedalesduchenne.com
    Tel: 352- 367-0060

18. **Cure Duchenne**

    3334 East Coast Highway # 157, Corona Del Mar, CA 92625

    www.cureduchenne.org
    debra@cureduchenne.org
    Tel: 949 - 872-2568

19. **Darius Goes West**

    135 Pine Tops Court, Athens, GA 30606

    dariusgoesweat@gmail.com
    Tel: 706-613-7337

20. **Disabled Children's Relief Fund**

    PO Box 89, Freeport, NY 11520

    Tel: 516-377-1605

21. **DMD Fund**

    PO Box 17371, Encino, CA 91416

    www.dmdfund.org
    kyle@dmdfund.org
    Tel:818-692-5500

22. **Duchenne San Diego**

    12630 MonteVista Road, Suite 202, Poway, CA 92064,

    www.duchennesandiego.org
    info@duchennesandiego.org
    Tel:858-775-7057

23. **Dylan's Footprint**

    98 Taylor Avenue,Greenlawn, NY 11740-1497

    www. dylanfootprint.org
    info@dylanfootprint.org
    Tel: 631-754-5360

24. **Foundation for a Future**

    PO Box 270036, Louisville , CO 80027

    www.foundationforacure.org
    pamela@foundationforacure.org
    Tel:303-257-7287

25. **Foundation for their SAKE**

    5 Cherry Hills Farm Ct,Englewood, CO 80113

    www.perkyjerky.com

26. **The Foundation to Eradicate Duchenne**

    P.O.Box 2371, Alexandria, VA 22301

    www.duchennemd.org
    jwood@duchennemd.org
    Tel: 703-683-7500

27. **Gals for Cal**

    Boston, MA,

    www.galsforcal.com

28. **Hope for Gabe**

    Birmingham, AL

    www.hopeforgabe.org

29. **Hope for Gus**

    Francestown, NH

    www.hopeforgus.com
    hopeforgus@yahoo.com

30. **Hope for Javier**

    P.O.Box251, East Setauket, NY

    www.hopeforjavier.org
    infor@hopeforjavier.org

31. **JBS Keys to DMD**

    67 Forrest Ave, Norwood, MA 02062

    Jbskeys.org
    Tel: 781-269-1175

32. **Jett Foundation**

    42 Elm Street, Kingston, MA

    www.jettfoundation.org
    Tel:781-585-5566

33. **John Owen's Adventure, Inc.**

    5715 Bunker Road, North Royalton, OH 44133

    www.Joainc.org
    Tel: 440-230-1555

34. **Lian's LEEP**

    Norwood, MA

    www.Wix.com/kdm114/liams-leep

35. **Liam Hiatt Foundation**

    P.O.Box 832, Downers Grove, IL 60515

    www.liamhiattfoundation.org

36. **MDA**

    National Headquarters : 3300 E. Sunrise Dr, Tucson, AZ 85718

    www.mdausa.org
    Tel:800-572-1717

37. **MDFF**

    7220 US 31 South, Indianapolis, IN 46227

    www.Mdff.org
    Tel: 800-544-1213

38. **National Center on Birth Defects & Developmental Disabilities**

    1600 Clifton Road, MS E - 87, Atlanta, GA 30333

    cdcinfo@cdc.gov
    Tel: 800-232-4636

39. **Nash Avery Foundation**

    2427 East Lakes of the Isles Parkway Minneapolis, MN 55405

    www.nashaveryfoundation.org

40. **NJCatastrophic Illness in Children Relief Fund Comission**

    P.O.Box 728, Trenton, NJ 08625 - 0728

    Tel: 609-292-0600

41. **Noah's Feat**

    www.noahsfeat.org

42. **Race MD**

    1501 SW Taylor St. Suite 200 Portland, OR 97205,

    www.Racemd.org
    infor@racemd.com
    Tel: 503-278-3273

43. **Rally for Ryan**

    2623 Evercrest Ct., Naperville, IL 60564

    www.rallyforryan.org
    contact@rallyforryan.org
    Tel: 630-922-6049

44. **Romito Foundation**

Firestone, CO,

Romitofoundation.org
Tel: 303-718-2538

45. **Ryan's Hope for a Cure Charitable Foundation, Inc.**

6 Maple Terrace, Plaineville, MA 02762

www.hopeforryan.com
info@hoperforryan.com
Tel: 508-699-3888

46. **Ryan's Quest**

P.O. Box 2544, Hamilton, NJ 08690

www.Ryansquest.org
infor@ryansquest.org
Tel: 609-947-3611

47. **Save Our Boy Foundation**

26 Mariscal Place, The Woodlands, TX 77389

www.saveourboy.org
Tel: 713-392-6001

48. **Suneel's Light**

5140 Main St, Unit 303- 152 Williamsville, NY 14221

www.suneelslight.org
suneelslight@gmail.com

49. **Team Joseph**

Detroit , MI

www.Wix.com / danted / josephpenrod-com2

50. **Teens for Duchenne**

Newport Beach, CA

www.teensforduchenne.com

51. **Two Smiles one HOPE**

P.O.Box 435, Fayetteville, NY 13066

www.twosmilesonehope.com
Tel: 315-415-4414

52. **Zack Heger Foundation**

285 Prospect St.,Norwell, MA 02061

www.Zackhegerfoundation.org
Tel: 617-529 - 9612

United Kingdom:

1. **Muscular Dystrophy Campaign**

About :

The Muscular Dystrophy Campaign is among the oldest and most established non profitable organizations in the UK dedicated to muscular dystrophy. It was founded in 1959 with the primary aim to help the affected children & their families.

Its main focus is on research towards finding a cure and treatment for MD.

Over the years, the campaign has enlisted the support of many distinguished personalities. Lord Richard Attenborough, an acclaimed actor, director and producer served as President for over 30 years & is now an Honorary Lifetime President. His-Highness, Prince Philip- Duke of Edinburgh, has been a patron for over 40 years, since 1966, providing funds and help-in-kind to the campaign for its activities.

Objectives:

- To fund research for developing the cure or treatment for muscular dystrophy.
- To spread awareness about MD and educate families on how to cope with it.
- To provide help to families by way of education, assistive devices, finance, etc.
- To lobby for the cause of MD in the British government.
- To provide information & advice to families.
- To create a community for mutual support of MD.

Contact:

Muscular Dystrophy Campaign
61 Southwark Street, London SE1 0HL

www.muscular-dystrophy.org
info@muscular-dystrophy.org
Tel: 020 7803 4800

2. **Duchenne Family Support Group (DFSG)**

About :

The DFSG was started in 1987 by a small group of parents who had children diagnosed with Duchenne Muscular Dystrophy (DMD). Since then, the number of families has increased dramatically and contacts have been established all over the country, as well as abroad, creating a wealth of information.

Objectives:

- To provide a positive national support network of parents, their families and professionals.
- To bring families together for mutual support, sharing of information and experience, and social activities.
- To provide workshops in such areas as adaptations, physiotherapy, complementary medicine, equipment, lifting and education with the help of relevant professionals.
- To conduct workshops with guest speakers and the opportunity to socialize at our annual conference.

Contact:

78 York Street, London W1H 1DP

http: / / www.dfsg.org.uk
Tel: 0870 241 1857
Helpline number - 0800 121 4518

Other organizations in United Kingdom:

3. **Action Duchenne**

   Headquarters: Epicenter, 41 West Street
   London, UK E114lj

   www.actionduchenne.org
   nick@actionduchenne.org
   Tel: 020 8556 9955

4. **Treat-NMD**

   United Kingdom

   www.treat-nmd.eu
   info@treat-nmd.eu
   Tel: +44(0)191 241 8605

Australia:

1. **Save Our Sons: Australia**

About :

Save Our Sons was incorporated in February 2008 by the Eid family as a charity, seeking to raise public awareness and much needed funding for research on Duchenne Muscular Dystrophy (DMD). Save Our Sons was founded with one goal in mind, to find a cure for Duchenne Muscular Dystrophy.

Objectives:

- To find a cure for Duchenne Muscular Dystrophy
- To support clinical trials for potential cures and treatment.

Contact:

PO box 3 Roseland NSW 2196, Australia

info@saveoursons.org.au
Tel: 0424 669 340

Other organization in Australia:

2. **Duchenne Foundation :Australia**

www.Duchennefoundation.org.au

Few in the other parts of the world:

1. **United Parent Project Muscular Dystrophy**

   Postbus 821, 3900AV Veenendaal, The Netherlands

   www.uppmd.org
   Tel: +31 164 230270

2. **Cura UPA! Duchenne**

   Mexico

   www.Curaupaduchenne.org

3. **Misko Foundation**

   1092 Budapest, Raday u. 8. 1/5, Hungary

   www.misko.hu
   info@misko.hu

4. **Duchenne Heroes**

   Post box 11220, 1001 GE Amsterdam

   www.duchenneheroes.nl
   info@duchenneheroes.nl
   Tel: 088 366 54 60

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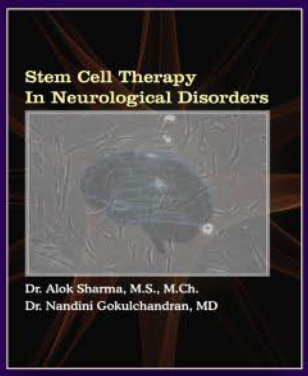
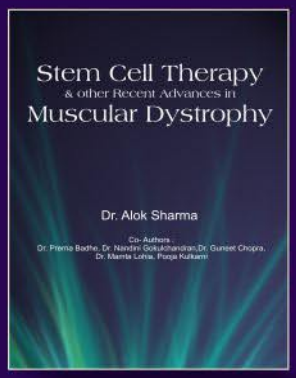
Other books published by NeuroGen Brain and Spine Institute.



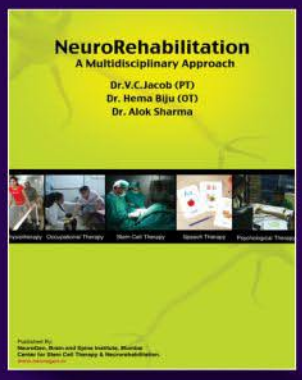
Stem Cell Therapy in Neurological Disorders (271 pages)

This is the first book published in India on Stem Cell Therapy giving insight into the exciting field of neuroregeneration. It is a comprehensive book which covers the basic concepts of stem cells and stem cell therapy in neurological disorders and the future of this novel treatment.



Stem Cell Therapy & Other Recent Advances in Muscular Dystrophy (407 pages)

The book covers three broad sections wherein section 1 deals with information on muscular dystrophy, section 2 discusses stem cell therapy with neurorehabilitation and section 3 details out 100 case reports of muscular dystrophy patients who have undergone stem cell therapy, highlighting their improvements.



NeuroRehabilitation - A Multidisciplinary Approach (527 pages)

This book talks about the importance of rehabilitation therapies i.e. physiotherapy, occupational therapy, speech therapy, psychological therapy along with stem cell therapy in improving the quality of life of patients with neurological disorders.

NeuroGen Brain and Spine Institute

NeuroGen Brain and Spine Institute is a centre for stem cell therapy and neurorehabilitation. It offers a comprehensive NeuroRegenerative Rehabilitation programme for patients suffering from incurable neurological disorders. The aim is to provide these patients relief from their symptoms & physical disabilities, using the safest & most effective available treatments & technologies from the field of neurosciences and regenerative medicine in a professional, scientific as well as holistic & caring manner.

StemCare Foundation

StemCare Foundation is a non-profit organization whose primary purpose is to make available the latest technological medical advances to all patients, irrespective of their socio-economic status. It also aims at creating awareness and supporting basic & clinical scientific research in the field of regenerative medicine.

NeuroGen Brain and Spine Institute
Surana Sethia Hospital, Suman Nagar, Sion-Trombay Road, Chembur, Mumbai-400071, India.
Mob:- (+91) 9920200400, Tel:- (+91 22) 25283706/65220066.
Email: contact@neurogen.in web: www.neurogen.in