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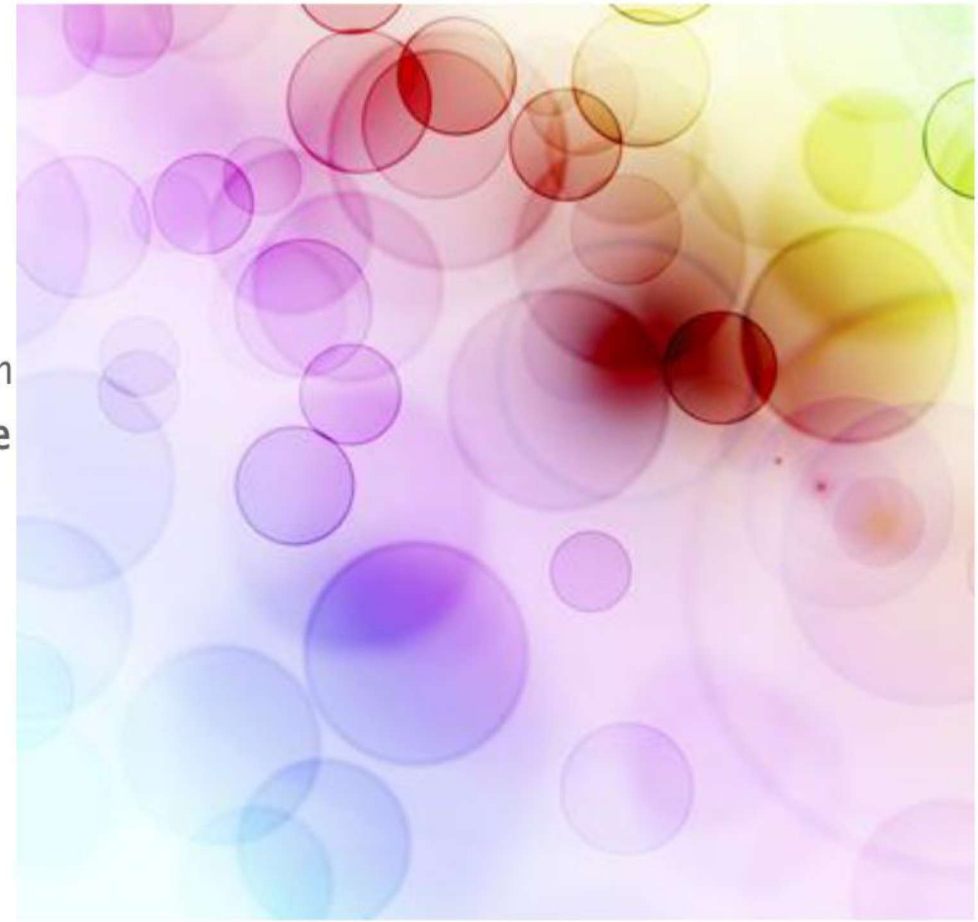
Muscular Dystrophy

Information for patients

Muscular Dystrophy Treatment with Stem Cells from your own **Bone Marrow / Adipose Tissue**



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Muscular Dystrophy

Muscular Dystrophy is a group of congenital disorders involving muscle weakness and loss of muscle tissue which is progressive and can be worsen over time. Majority of the forms of muscular dystrophies are affecting infants as well as children between the age group 2-5 years, with the timely progression of the disorders these children are succumbed to the paralysis leading to death in their twenties.

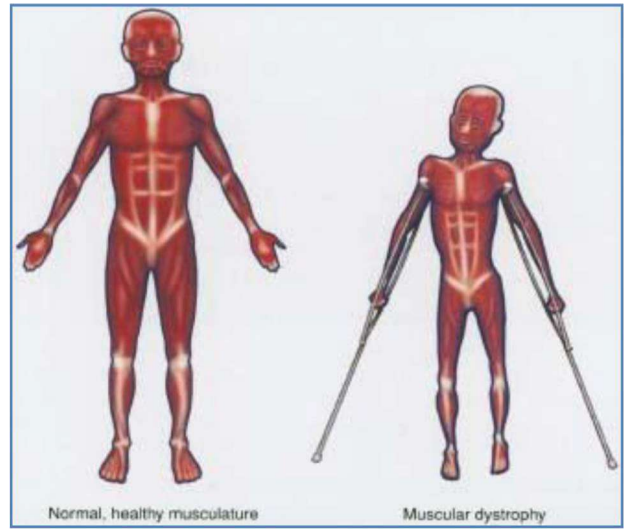
The group is generally categorizing different types in terms of deployment and extent of muscle weakness, age of onset, rate of progression and pattern of the epigenetic and genetic abnormality posed.

Most of them are not simply muscular dystrophies but are multisystem disorder with indications in variety of body systems including heart, gastrointestinal systems, endocrine glands etc.

Different Varieties of Muscular Dystrophies can be listed as

- Duchenne Muscular Dystrophy
- Myotonic Dystrophy
- Baker's Muscular Dystrophy
- Limb-girdle muscular Dystrophy
- Oculopharyngeal Dystrophy
- Distal Muscular Dystrophy
- Mitochondrial Dystrophy

The prevalence of MD is very unlikely e.g. the DMD being the commonest form occur 1 out of every 50000 people.
Each type of MD is distinct with a rare genetic mutation but generally it affects the normal structure and thereby functions of voluntary muscles causing



the onset. It is most commonly the X-linked disorder i.e. many of its affected genes are located on X chromosomes. Thus women will only be carrier of the disorders and can be diagnosed prenatally for the find out the possibility of her male child getting affected with the disorder.

What Causes Muscular Dystrophy?

Our movements like the way we think, the way we walk are basically governed by a strong network of muscles, brain and spinal cord. Muscles are made up of bundles of two types of cells known as "Satellite Cells" and "Fiber Cells". Muscle fiber cells are responsible for shaping up and movement of muscles. These fiber muscle cells are often subjected to normal wear and tear due to overuse. Satellite cells come in the picture to repair damaged cells or to produce new fiber cells to keep on the function.

In case of Muscular Dystrophy due to genetic abnormality such as lack of particular protein, muscles are weak and hence subjected to constant damage; in that case satellite cells are overburdened and thus the process of repairing is slowed down or ceased; fully stopping the muscular function completely. The possible complications associated with the progression of the disorders are

- Cardiomyopathy with heart failure
- Cataract
- Decreased ability to self care
- Depression

- Lung failure
- Mental Impairment
- Scoliosis

What is the prognosis?

Symptoms of muscular dystrophies are the result of deterioration of body's muscles, due to death of the muscular cells. Most of them are cleared by the age 5. Since the progression is very fast many people need a wheel chair by 12, and are subjected to paralysis and death by early twenties.

Some of the general symptoms include problems associated with the coordination and mobility, muscle weakness, twitching, stiffness, fatigue, breathing trouble, intellectual disability etc. The physical examination and medical history will help your doctor determine the signs of muscular dystrophy. Some of them are

- Abnormally curved spine
- Joint contracture
- Low muscle tone

Some other forms of MD's causing cardiomyopathy can be identified with arrhythmias. Other than these genetic tests, nerves tests, blood tests are also performed to confirm the disorder.

Is there any treatment?

Customarily there are no known cures available for Muscular Dystrophy but most of the available treatments are mainly focused controlling the symptoms but these treatments are associated with other complications, such as oral medications of steroids are prescribed to many children to keep them walking.

Orthopaedic support such as braces to mobility and maximize independence. Physiotherapy and yoga may help patients to strengthen the muscles and improve their self care abilities. For prevention, prenatal diagnosis is always advised with as much as 95% accuracy for the detection.

The recent technology advances of stem cells have paved a new avenue for the treatment of Muscular Dystrophies. The recent technology advances of stem cells have paved a new avenue for the treatment of Muscular Dystrophies. These cells having characteristics of becoming any or all cells of the body; can be guided to be lost muscle fiber cells, reducing the pain and thereby slowing down further deterioration.

About Stem Cells

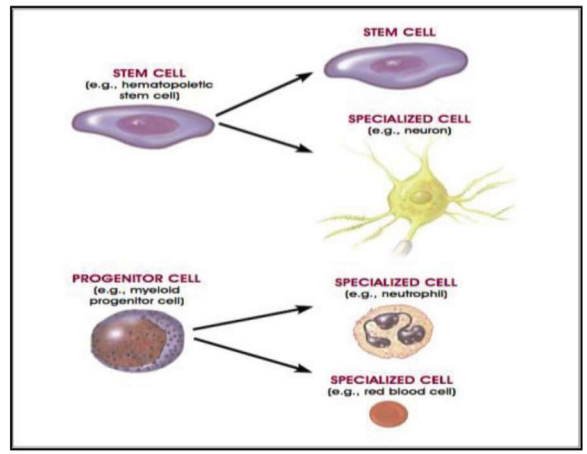
Stem cells are basically the “Master Cells” of the human body. These unspecialized cells lie dormant throughout the life and get activated when signals elicit from the site of injury. Upon enlivening they get navigated to the site of injury with the help of strong immune response stimulated by the body. At the site with the help of a programmed cell division they get differentiated into the specialized tissue specific cells. These newly formed daughter cells will be replaced for the damaged one, thus ceasing further progression of the diseased cells.

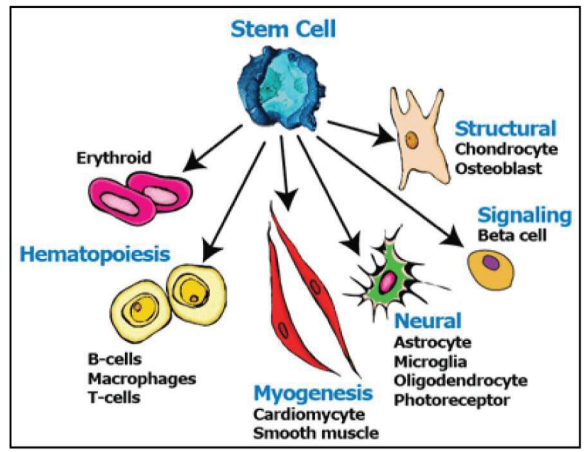
Thus, stem cells are known to be vital to the body for its normal wear and tear due to their exclusive characteristics such as

1. Undifferentiated cells capable of giving rise to any tissue specific cells.

2. Capability of prolonged self renewal having an unlimited life span and thus maintaining their number intact.

3. The ability to trigger the secretion of certain hormones and grow factors at the site of injury to facilitate damage repair.





Types of Stem Cells

With respect to their origin these Stem cells are broadly classified into two main naturally occurring cell types such as Embryonic stem cells and Adult stem cells. The third one is reprogrammed in the lab to satisfy ethical as well as scientific demands; they are named as Induced Pluripotent stem cells

Embryonic Stem Cell

These cells are the most preliminary cells isolated from the inner cell mass of the blastocyst of preimplantation stage embryos. With their unlimited expansion capacity and ability to differentiate into almost all cell types of the body; earlier they were considered as the potential sources of the regenerative medicines. But the increasing burden of ethical speculations and animal studies showing abnormal occurrence of tumor ex vivo rendered them ineffective for the therapeutic application.

Adult Stem Cells

These cells are settled in adult body organs. They are less controversial due to their ease of isolation

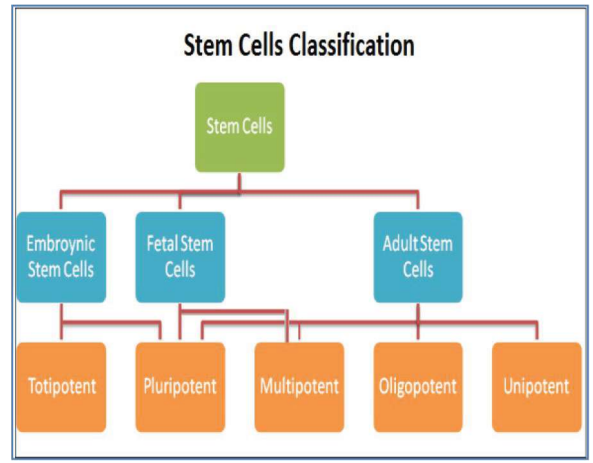
and their production does not require any destruction of embryo. These cells pose a very low or no risk of immune rejection when applied as therapeutic.

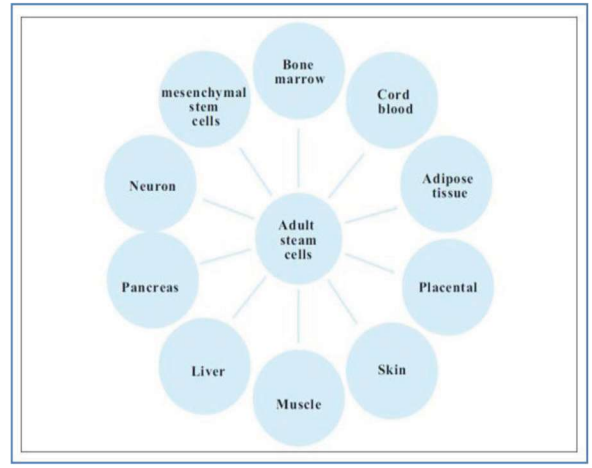
Since they are present in an adult fully grown organ, they are capable of differentiating cell of their lineage only contrary to the embryonic stem cells giving rise to all lineages. Apparently these cells can be directed to become specific cells with the addition of specific growth factors. These properties make them attractive candidates for the therapeutic application in the contemporary field of regenerative medicine.

Induced Pluripotent Stem Cells (iPSCs)

These are adult somatic cells genetically reprogrammed in vitro to an embryonic stem cell like state by being forced to express gene such as OCT-4; important for maintaining Pluripotent property of embryonic stem cells. Although the technology is still in a juvenile state; additional research is needed to use them in transplantation medicine.

Out of the different types mentioned above Adult Autologous Stem cells are known as the most potent and versatile. They are thus the most opted cell sources due to easy isolation, demonstrative plasticity and minimum ethical barricades.





Examples of Autologous stem cell sources are Bone Marrow, Adipose Tissue etc and we have achieved ultimate expertise over it.

The Advancells Muscular Dystrophy Treatment

Advancells uses very comprehensive and individualised treatment pattern, by obtaining stem cell from two sources adipose-derived stem cells (ASCs) and bone marrow-derived stem cells (BMSCs). Through our multidisciplinary team of specialists; we are giving the best treatment possible to improve recovery.

The entire procedure consists of following phases:

Pre Treatment Assessments, Stem cell procedure, Stem cell Implantation and Rehabilitation.

Objectives of the Treatment

Our Treatment objectives are to detect the causative factor and composite a treatment plan to reduce pain, increase mobility and maximise independence in the best possible way.

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Type of Treatment

The procedure exploits the use of Autologous Stem Cells isolated from your own body.

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Being an Autologous they are not rejected by the body and hence are completely safe and risk free. At present we are isolating stem cells from two sources such as **"Bone Marrow"** and **"Adipose Tissue"**. Both these sources are available in abundance from where stem cells can be easily isolated without many manipulations.

Once you are enrolled with us for the treatment, we will intimate you regarding the date and time of the treatment. The entire treatment plan will be divided into three parts;

1. **Inductive Support**: - Complete assistance will be given to the patient in all pre treatment procedures such as consultation, hospitalization (If required), Assessments.

2. **Stem Cell Procedure**: - Generally the entire procedure takes around 7-8 hrs including 1-2 hrs of source aspiration, 2-3 hrs of stem cell isolation and injection of the isolated stem cells back in the body. The processing of sample is done in a state of the art class 10000 clean room facility wherein we strictly adhere to maintain quality of standards. Wherein the extracted sources undergo minimum manipulation such as been spun in a centrifuge to cull out a stem cells.

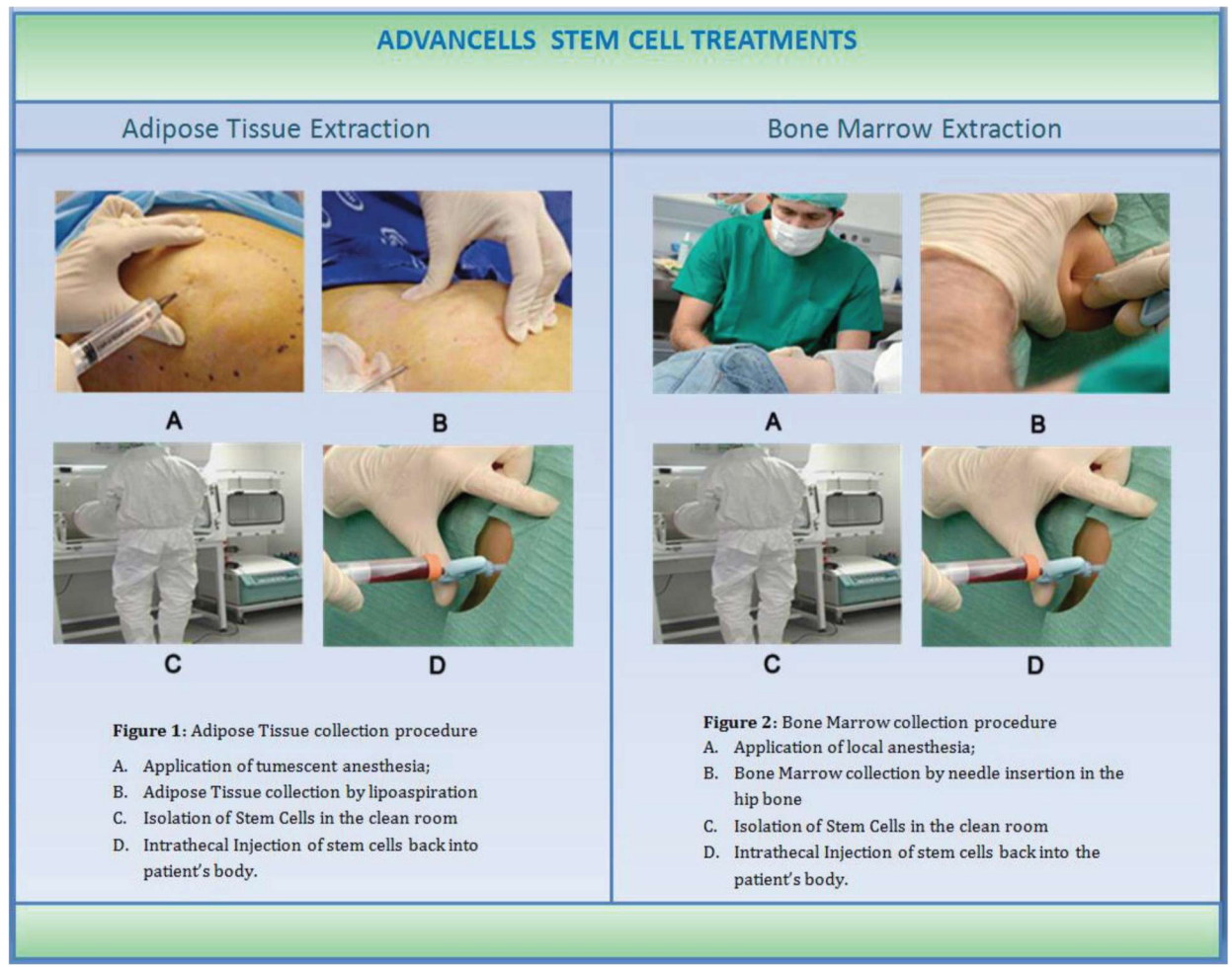


Figure 1: Adipose Tissue collection procedure

A. Application of tumescent anesthesia;
B. Adipose Tissue collection by lipoaspiration
C. Isolation of Stem Cells in the clean room
D. Intrathecal Injection of stem cells back into patient's body.

Figure 2: Bone Marrow collection procedure

A. Application of local anesthesia;
B. Bone Marrow collection by needle insertion in the hip bone
C. Isolation of Stem Cells in the clean room
D. Intrathecal Injection of stem cells back into the patient's body.

3. **Stem cell Implantation**: - Once isolated, intensified and ready for reinstalling back into the body, we work out different mode of implantation; depending upon patient's health condition.

4.

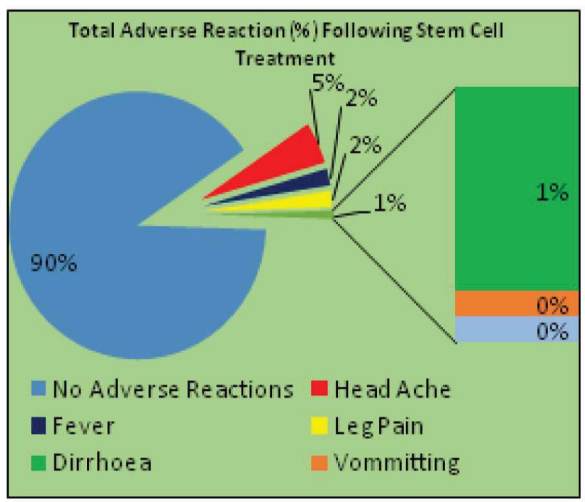
ADVANCELLS STEM CELL TREATMENT		
ELIGIBILITY CRITERIA	STEM CELL SOURCES	IMPLANTATION
Pre Treatment Assessments • Routine Blood Tests • Routine Urine Analysis • Infectious disease testing • Physical Examination • Aldolase, AST, LDH • Creatinine, Myoglobin • X Ray **Pre-op Procedures** • Electrocardiography • Electromyography • MRI • CT Scan • Medical History MRI **Counselling**	The source of stem cells can either be Bone Marrow or Adipose Tissue or Both depending upon the assessment. • **Bone Marrow:-** 100-120 ml of bone marrow is collected from iliac crest with the application of general anaesthesia. • **Adipose Tissue (Fat):-** 100 cc adipose tissue is collected from the belly area with application of local anaesthesia.	Specialists exercise various input options for implanting cells back into the body depending upon physical condition and treatment demand. •**Intravenous Injections (IV):-** Infusion through vein •**Intra-thecal Injections:-** Infusion through spinal cord popularly known as lumbar puncture

4. **Rehabilitation**: - Post treatment care involves reclamation therapies such as Physiotherapy, Occupational Therapy, Speech Therapy, patient's counselling etc for accelerated recovery. The follow up schedule will be provided at the time of discharge.

ADVANCELLS STEM CELL TREATMENTS	
Quality Control Parameters	Post Treatment Care
Cell counting & Viability Assessments • Stem cell isolation and separation from unwanted cells • Number of cells recovered through Trypan Blue Viability Assessments • Percentage of Live cells • Documentation **Flow Analysis /characterisation of Bone Marrow Mononuclear Cells (BMMSCs)** • Total percentage of CD 34+ and CD45+ cells recovered **Flow Analysis /characterisation of Adipose Tissue (SVF Cells)** • Total percentage of CD 73+and CD90+ and CD 105+ cells recovered	**Rehabilitation** • Behaviour and emotional development • Communication & Social interaction • Self regulation, cognitive skills, academic skills and adaptive skills. • Psychological counselling • Evaluation such as ECG, EMG, • Physical Check up • Follow up
Our client will get a third party certificate from an internationally accreditated lab for the cell count and viability.	

Possible Adverse events from the treatments

Since stem cell therapy in minimally invasive and reasonable safe procedure none of our patients treated so far have observed any major offshoot from the transplant, but complaints are consistent with the expected reaction to routine IV/LP injections such as fever, headache, pain, diarrhoea, vomiting and allergic reactions. Less than 5% of our patients have experienced any of these symptoms.



Follow Up

Once you have returned home, a member of our medical team will monitor your progress in given intervals via telephone and email. For your convenience, a telephone 'hotline' is always at your disposal.

General

No additional charges will be incurred unless you are required to extend your stay at the medical center as a result of complications. Costs do not include additional stem cell treatments. If another treatment is necessary, we will discuss potential options with you. You will receive an invoice one week prior to treatment. This invoice must be paid in-full before treatment can begin.

Note: If your bone marrow/adipose tissue sample is negative or the stem cells cannot be administered due to unforeseen medical circumstances, you will only be required to pay charges incurred to that point. In the case of a negative sample, it might be possible to schedule another collection.

Contact us

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