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KEYWORDS

Duchenne's muscular dystrophy

Dystrophin gene

Becker's muscular dystrophy

Orphan diseases

Mesenchymal stem cell therapy

Leukemia inhibitory factor

Stem Cells for Muscular Dystrophies

Subadra Dravida C.E.O

An orphan disease is a rare disease (according to US criteria, a disease that affects fewer than 200,000 people) or a common disease that has been ignored. But, the true definition not known by many is that a disease category that has not been taken up by any pharmaceutical industry to research on as it provides little financial incentive for the private sector to make and market medicines to treat or cure. Duchenne Muscular Dystrophy (DMD) is one such category in India that is totally not recognized by any healthcare schemes or research initiatives or NGOs or clinicians communities, worth a consideration for offering solace/treatment options to the suffering society. First visit to the doctor by the worried family is usually directed to a Neuro-physician, who would categorically declare that the affected kid will die soon and so medically advised to go home! Such is the level of compassion or practice or medical knowledge in the society that we are in. Despite the urgent need for research in Orphan diseases, also requires a multidisciplinary personalized approach like community participation of practicing Scientists, Clinicians and Parents of the affected kids in order to find innovative medical solutions gathering the need into expertise and applications.

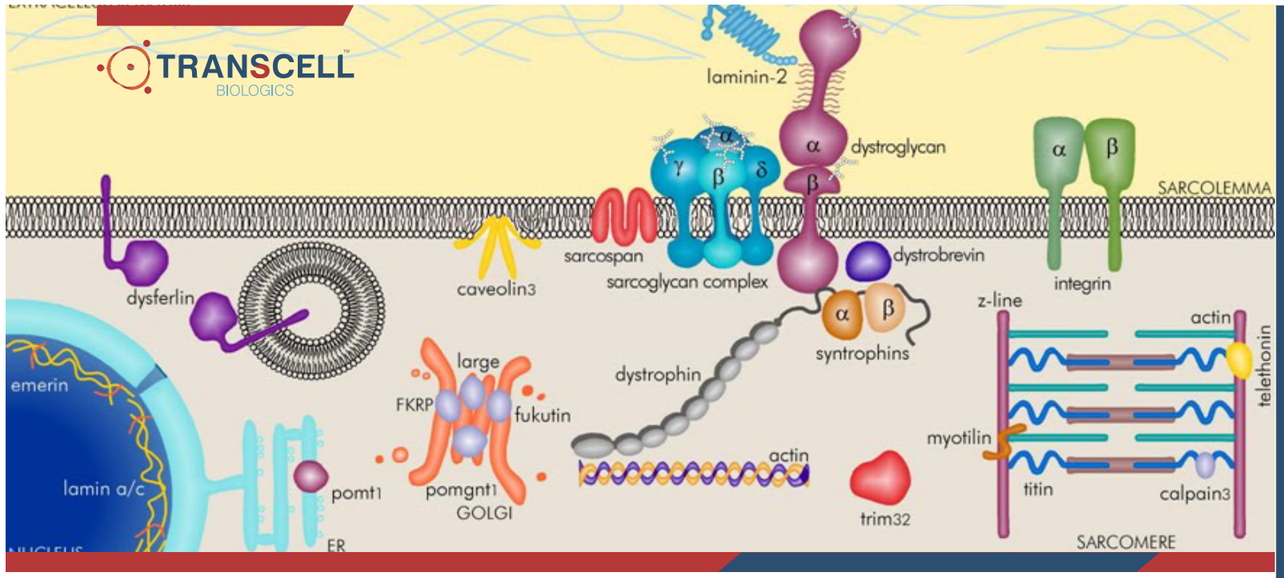
Trying to get the facts, it is probable that Orphan diseases will never benefit from a specific drug or any available therapy, need for alternative research lines like Stem cells repairing the continuously degenerating muscles, inflammation associated is the only hope for this disease to be pursued. But, disruptive research has to happen translating into clinical application as patients can't wait! For once if the investing community believes in scientific breakthroughs alleviating the human suffering as their ROI, it is Paradisso for human kind!

Here, I remain heavy with suffering patients in my thoughts!

January 2017 Transcomm highlights the Orphan disease DMD and possibilities with Stem cells to address the debilitating medical condition. Any application or research with stem cells is possible only if they were captured at the right event and stored in right condition.

So, Dear Readers, Please encourage, educate and evangelize your families, extended families, friends to store stem cells now!

Subadra



A study conducted by the Indian Society for Clinical Research (ICSR), states that 70 million Indians suffer from life threatening rare diseases. Although we have made immense progress in science and medicine, there is little that is known of the rare diseases prevalent in India, and a paucity of affordable therapy available for these diseases. Children form a part of nearly 50% of the population affected by these diseases, and only 30% of these children, live till the age of five.

Mekhla Singhania
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Muscular dystrophy is one such rare genetic disorder that affects several in India. An X linked recessive disorder, this disease affects more males than females. While in most developed nations, MD patients survive till the age of 20-23, lack of awareness of this disorder in India has led to most MD children dying at the age of 13-17.

A neuromuscular disorder, which causes degeneration of a set of muscle cells, Muscular Dystrophy starts by destroying the skeletal muscle first, and then progressively deteriorates the internal organs as well. Of the several types of MD, Duchenne MD is the most common. It is caused by a mutation in the DMD gene. Several stem cell therapies have been proposed for DMD.

A loss-of-function mutation in the dystrophin gene, leads to DMD. In a study conducted by Dumont et al (2015), it was found that dystrophin association with a regulator of cell polarity, specifically serine-threonine kinase Mark2 protein, leads to its increased expression in satellite cells (activated muscle stem cells). Thus, absence of dystrophin leads to several intrinsic defects, such as inability to localize cell polarity regulator Pard3. These intrinsic defects, eventually impair muscle regeneration process, because if reduced generation of myogenic progenitors. Hence, it was concluded that the reasons for muscle wasting in DMD can be attributed to impaired regeneration of intrinsic satellite cell dysfunction and myofiber fragility.
In a study conducted by N. Ito et al (2016), it was found that treatment with leukocyte inhibitory factor (LIF) helped to maintain the undifferentiated state of satellite stem cells and also helped to maintain their transplantation efficiency. It was stated that since LIF has been reported to be functionally involved in muscle regeneration, and has alleviated the pathology of a mouse model of DMD, its role `in transplantation efficiency must be analysed. Research also stated that to improve the migration of the transplanted cells, a combination of LIF with other cytokines such as bFGF, would be required.

Ray of hope for Muscular dystrophy patients

Anand Soorneedi
Process Scientist

In a recent clinical trial, doctors at AIIMS have found a significant improvement in 20 patients who received stem cell therapy for treating MD.The report of the trial has also been published in the Journal of Stem Cells in its recent issue. Patients suffering from the disease die between ages of 13 to 21 years. According to Dr. B.S. Rajpoot, who led the clinical trial, umbilical cord derived stem cells and IGF1 (a protein that in humans is encoded by the IGF1 gene) was used for stabilization as well as reversal of muscle damage.Initial observations over a period of three months have shown that stability in muscle function has improved and that there was a progressive decline in calf size of the DMD patients up to three years after stem cell transplantation, indicative of increased muscle strength. The added advantage no deleterious effects when using umbilical cord tissue derived stem cells is indicative of the safety of this treatment modality.

Stem cell therapy new hope to cure Muscular Dystrophy: Dr Nandini

Somashekhar, a medical store owner from Mumbai ,who was unable to stand on his feet and walk since past 15 years due to Becker's Muscular Dystrophy disorder could now carry on his daily routine without any problem, thanks to Mumbai based NeuroGen, Brain and Spine Institute. Somashekhar is the recipient of a stem cell based therapy, which helped him recover within a span of six months. Dr NandiniGokulchandran, deputy director and head of medical services of NeuroGen said stem cell therapy is new hope for patients suffering from so far incurable debilitating disease called Muscular Dystrophy, disorders connected to brain and muscles.

Forst Duchenne MD Patient to be Approved for Adult Stem Cell Treatment in US Turns 30

Ryan Benton, a MD patient recently celebrated his 30th birthday thanks to an allogenic adult stem cell transplantation he has undergone. This was the first ever documented case of a MD patient who has lived beyond the normal life expectancy for patients with MD. Due to the restrictions in the US, Ryan had to take several trips to panama to undergo the experimental stem cell therapy. The FDA finally realized the importance of using stem cells for treating MD and granted approval for Ryan to undergo the treatment in the US on a regular basis which led to a more effective reversion of the disease's progression.

Chances for Muscle Regeneration Improved with Pre-Transplant Cell Treatment
Dr. Shin'ichi Takeda's research group at the Department of Molecular Therapy, National Institute of Neuroscience, National Center of Neurology and Psychiatry in Kodaira, Japan has successfully shown that muscle stem cells, treated with leukemia inhibitory factor (LIF) are better at forming new muscle fibers when transplanted into the body. Satellite stem cells, found in the muscle have been studied as a therapeutic approach for many years to induce the production of new muscle fibers in people with muscle disease such as Duchenne muscular dystrophy. But studies on the transplantation efficiency of these satellite stem cells in vitro have not yielded positive results. But now, pre-treatment of the satellite cells with LIF, a cytokine could help maintain the undifferentiated state of these cells. According to Dr. Shin'ichi Takeda,"This research enables us to get one step closer to the optimal culture conditions for muscle stem cells".

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