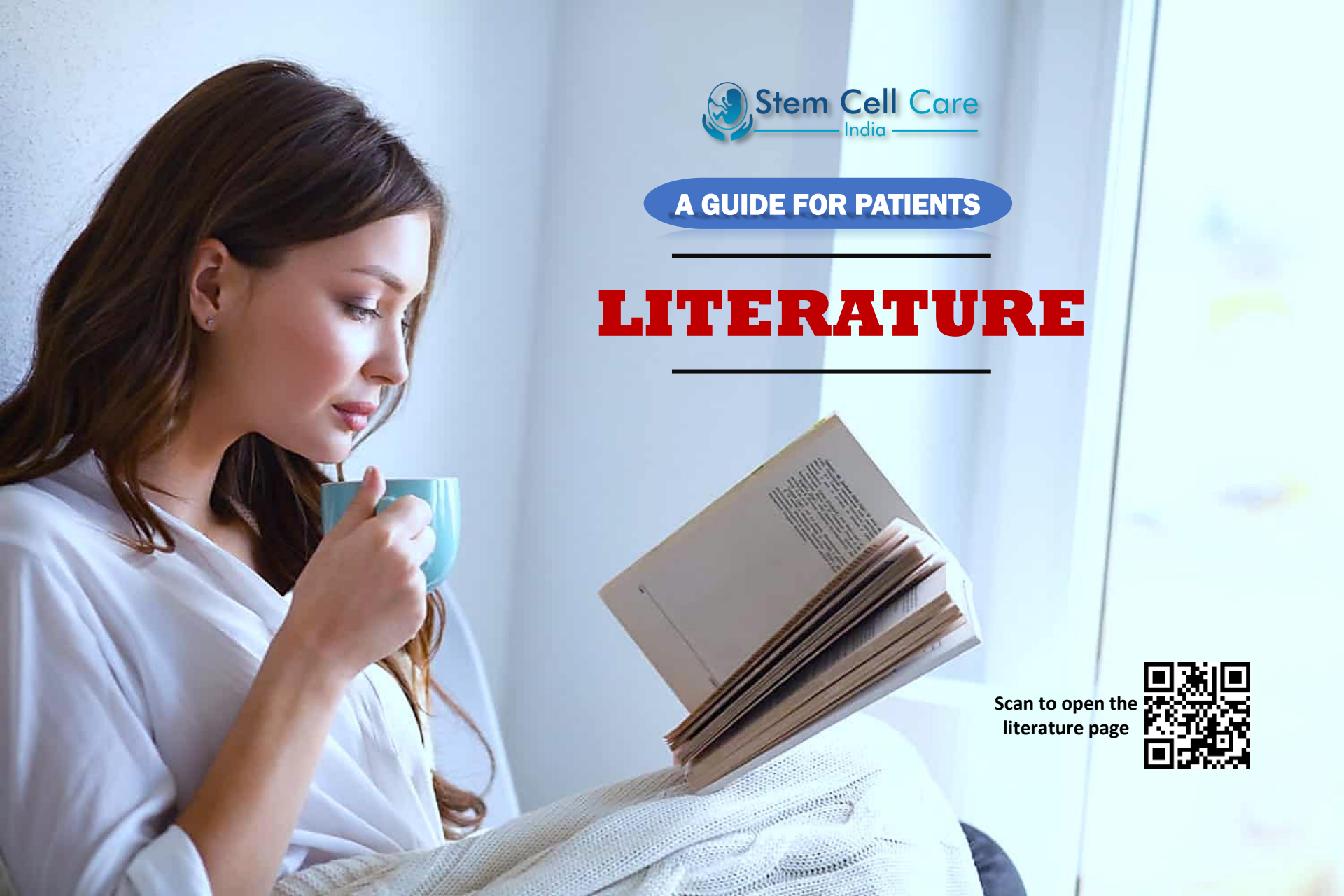

Stem Cell Care India

A GUIDE FOR PATIENTS

LITERATURE

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❖ Muscular Dystrophy

➢ Duchenne muscular dystrophy: current cell therapies

Duchenne muscular dystrophy is a genetically determined X-linked disease and the most common, progressive pediatric muscle disorder. For decades, research has been conducted to find an effective therapy. This review presents current therapeutic methods for Duchenne muscular dystrophy, based on scientific articles in English published mainly in the period 2000 to 2014. We used the PubMed database to identify and review the most important studies. An analysis of contemporary studies of stem cell therapy and the use of granulocyte colony-stimulating factor (G-CSF) in muscular dystrophy was performed.

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➢ Induced Pluripotent Stem Cells for Duchenne Muscular Dystrophy Modeling and Therapy

Duchenne muscular dystrophy (DMD) is an X-linked recessive disorder, caused by mutation of the DMD gene which encodes the protein dystrophin. This dystrophin defect leads to the progressive degeneration of skeletal and cardiac muscles. Currently, there is no effective therapy for this disorder. However, the technology of cell reprogramming, with subsequent controlled differentiation to skeletal muscle cells or cardiomyocytes, may provide a unique tool for the study, modeling, and treatment of Duchenne muscular dystrophy.

In the present review, we describe current methods of induced pluripotent stem cell generation and discuss their implications for the study, modeling, and development of cell-based therapies for Duchenne muscular dystrophy,

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➢ Stem cell therapy for muscular dystrophy

Muscular dystrophy is a heterogeneous group of neuromuscular disorders that manifests as progressive muscle weakness, muscle wasting and, in many cases, death. Although there has been enormous progress in the molecular understanding of muscular dystrophy, there is still no cure. There are, however, several different therapeutic options under investigation, including adult-derived stem cell transplantation. Encouraging and pioneering experiments in mouse models for Duchenne's muscular dystrophy (DMD) demonstrated that myoblasts could be transplanted into dystrophic muscle; these myoblasts repaired a small proportion of damaged myofibres. Subsequent work has been devoted to optimisation of this technique. In doing so, a number of adult-derived stem cells have been isolated, characterised and used in promising animal transplantation experiments. Further research is ongoing, and is clearly necessary to make this therapy a viable treatment option for patients with muscular dystrophy..

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➢ Cell therapy strategies and improvements for muscular dystrophy

Understanding stem cell commitment and differentiation is a critical step towards clinical translation of cell therapies. In past few years, several cell types have been characterized and transplanted in animal models for different diseased tissues, eligible for a cell-mediated regeneration. Skeletal muscle damage is a challenge for cell- and gene-based therapeutical approaches, given the unique architecture of the tissue and the clinical relevance of acute damages or dystrophies. In this review, we will consider the regenerative potential of embryonic and somatic stem cells and the outcomes achieved on their transplantation into animal models for muscular dystrophy or acute muscle impairment.

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➢ The role of stem cells in muscular dystrophies

Muscular dystrophies are heterogeneous neuromuscular disorders of inherited origin, including Duchenne muscular dystrophy (DMD). Cell-based therapies were used to promote muscle regeneration with the hope that the host cells repopulated the muscle and improved muscle function and pathology. Stem cells were preferable for therapeutic applications, due to their capacity of self-renewal and differentiative potential.

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➢ Stem cell transplantation for treating Duchenne muscular dystrophy

To identify global research trends in stem cell transplantation for treating Duchenne muscular dystrophy using a bibliometric analysis of Web of Science

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