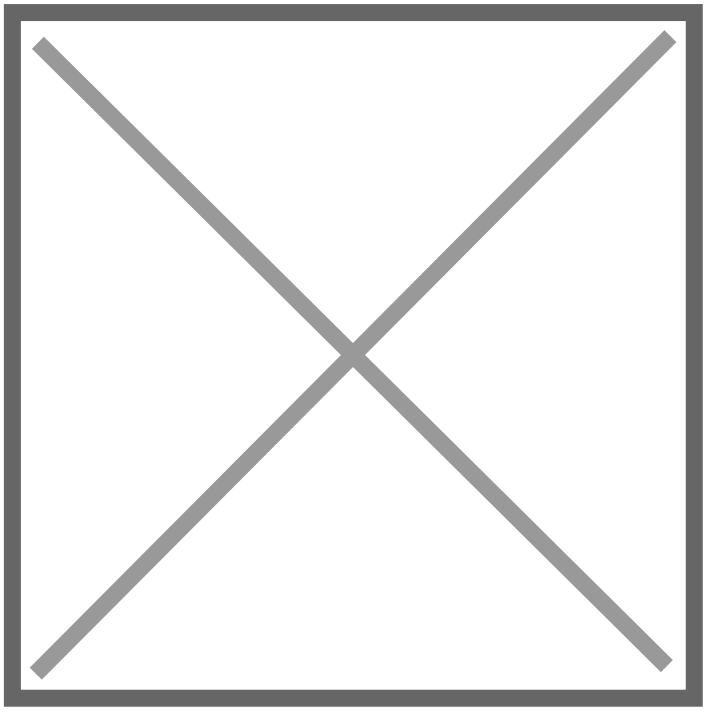
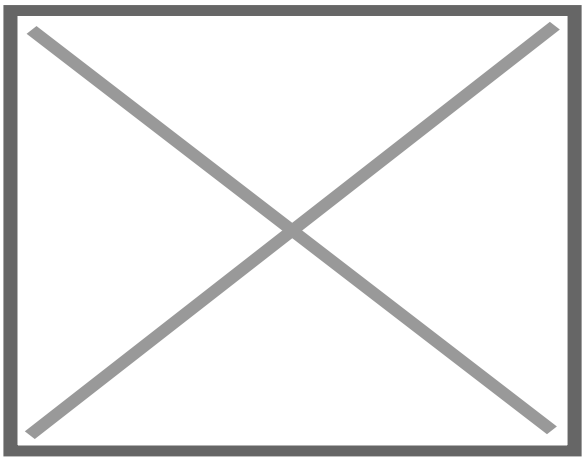
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Track clear for embryonic stem cell research

08 September 2010 | News



The US FDA's first clinical trial approval of human embryonic stem cell (hESC) therapy is a milestone for regenerative medicine. California-based Geron Corporation is all set to conduct the world's first clinical trial of hESC therapy

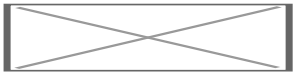


The field of regenerative medicine generates significant public interest, controversy and political debate. And it is no surprise that stem cell therapies are, once again, making headlines. The permission from the US Food and Drug Administration (US FDA) to California-based Geron Corporation, to conduct the world's first clinical trial of a human embryonic stem cell (hESC) therapy, has not only begun what may be the beginning of a new era for medical treatment, but if successful, could potentially open the doors for clinical testing of more human embryonic stem-cell derived cell therapies.

The FDA notification enables Geron to move forward with the world's first clinical trial of a human embryonic stem cell-based therapy in humans. The phase I multi-center trial is designed to establish the safety of Geron's lead hESC-based therapeutic candidate (GRNOPC1) in patients with complete American Spinal Injury Association (ASIA) Impairment Scale grade A sub-acute thoracic spinal cord injuries.

The FDA approval has happened after a year-long hold on phase I clinical trial in September 2009. The clinical hold was placed following results from a single preclinical animal study in which Geron observed a higher frequency of small cysts, within the injury site, in the spinal cord of animals injected with GRNOPC1. In response to those results, Geron developed new markers and assays, as additional release specifications for GRNOPC1, to prove its safety to the FDA. The company completed an additional confirmatory preclinical animal study to test the new markers and assays, and subsequently submitted a request to the FDA, for the clinical hold to be lifted.

Although the primary endpoint of the trial is safety, the protocol includes secondary endpoints to assess efficacy. Once safety in patient population has been established, Geron plans to seek FDA approval to extend the study, to increase the dose and expand the trial, to include patients with severe incomplete injuries, to enable access to the therapy for a broader population. Geron has selected up to seven US medical centers as candidates to participate in this study, and in planned protocol extensions.



FDA nod encourages industry and researchers

The regenerative medicine or stem cell sector makes its global presence with innovative research work happening all across the globe. Technical innovations and regulatory challenges in one country or region has, a profound impact on the regenerative medicine industry worldwide.

Geron's spinal cord injury repair being the most advanced hESC therapy being tested in humans, the approval has triggered a wave of ebullience from scientists, investors and patient advocates globally. Dr Samuel JK Abraham, director of Nichi-In Center for Regenerative Medicine (NCRM), an Indo-Japan joint venture institute, conducting research and clinical applications-protocol development in regenerative medicine, in Chennai, says, "As long as the safety, purity and potency are ensured, science has to move forward. The study using embryonic stem cell-derived product should go forward, particularly in conditions like spinal cord injury, where there are no definitive treatments."

Dr Abraham, however, suggests that equal importance should be given to autologous adult stem cells, and comparative studies on the antigenicity and efficacy of the presently used embryonic stem cell-derived oligodentrocyte precursor cells versus that of autologous adult (bone marrow, peripheral blood, and olfactory neural ensheathing cells) derived stem cells/precursor cells should be undertaken.

Dr Satish Totey, president of Bangalore-based Advanced Neuro-Science Allies (ANSA), a R&D company focusing on different areas of neuroscience, providing a comprehensive scientific approach to stem cell therapy, including basic and clinical research programs, for the development of new therapies; says, "This FDA approval is an excellent development and was long overdue. Geron has an excellent safety and preclinical data on spinal cord injury. This is one area where all other adult stem cells have miserably failed, without any significant improvement."

Commenting on the significance of Geron's study, Dr Totey says, "Demyelination is central to the pathology of injury, and its reversal by injecting embryonic stem cells-derived oligodendrocytes would be revolutionized. There are thousands of patients who suffer from spinal cord injury each year. If this method is effective, then it is a big blockbuster."

The approval, which is world's first clinical trial of a human embryonic stem cell therapy, is likely to break the logjam for other trials of drugs and therapies derived from human embryonic stem cells, believe industry observers. Some of the therapies currently on the horizon target diabetes, Parkinson's disease, cardiac disease, eye disorders and even production of blood.

Dr Totey says, "The announcement is the biggest boost to all other human embryonic stem cell companies currently operating in various countries. However, it will not lead to sudden expansion of funding, as the recent ruling by US district court overruling Obama's stem cell policy, may considerably jeopardize several stem cell projects around the globe." There are not many clinical trials using human embryonic stem cells, waiting FDA nod. Very few companies are working on human embryonic stem cells, he adds.

Dr Abraham believes that if the present trial continues with safety, at least with some efficacy in human patients, definitely it will have many followers.

The issue of embryonic stem (ES) cells depends on the religious and socio-cultural beliefs of the society, and therefore, varies with religions and territories. Dr Abraham says, "Personally, I would say, we have to move forward. In case of IVF, the redundant embryos are destroyed, which I think, should be made available for research and clinical studies, with appropriate consent from all people involved in it."

NCRM presently is not working in the area of embryonic stem cell. But in the long term, plans to start work on iPS cells, because it is free from controversies, like the ES cells.

There are a few groups working on human embryonic stem cells in India, but none of them are in clinical trial or therapy. They are all working on the very preliminary and primitive basic research. Dr Totey says, "We do not have ES cell lines in India, which is clinically eligible. I do not see any near future of human embryonic stem cells in India, especially for clinical application for a variety of reasons."

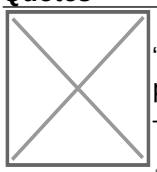
He points out that India does not have the technology of differentiation of ES cells into specific lineage, or proper ES cell lines that are clinically eligible, and can be used in humans. All the cell lines available are for laboratory use.

The other point to note here is that, the country does not have large scale expansion methods for industries. No industry or institute is currently working in this area. The other concern is, the product may be very expensive and out of reach of the common man, as the entire procedure and protocol is very lengthy and time consuming. Adult stem cell therapy may be cheaper. Dr Totey suggests, "The only way to get such a product is to buy readymade technology, or wait for other companies to open their shop, or have industry collaborations or joint venture."

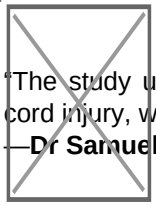
There are five to seven biotechnology companies currently developing stem cell therapies in India including Reliance Life Sciences and Stempeutics. Other prominent institutions such as the National Center for Cell Science and Ruby Hall Medical Research Center both in Pune and the Indian Institute of Science, Bangalore, besides Asian Heart Institute and Research Center and the Maulana Azad Medical College both in New Delhi are among 15 organizations working on stem cell projects. They use both adult stem cells, embryonic stem cells and also more readily available sources such as bone marrow, peripheral blood and umbilical cord blood cells, to find possible cures for many incurable diseases.

The extent to which hESC will actually be able to treat a disease is still unknown. It may take a few years to know the full-efficacy of this therapy. There is still a long way ahead. At least one hurdle, and the major one, is now over with the FDA approval.

Quotes



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