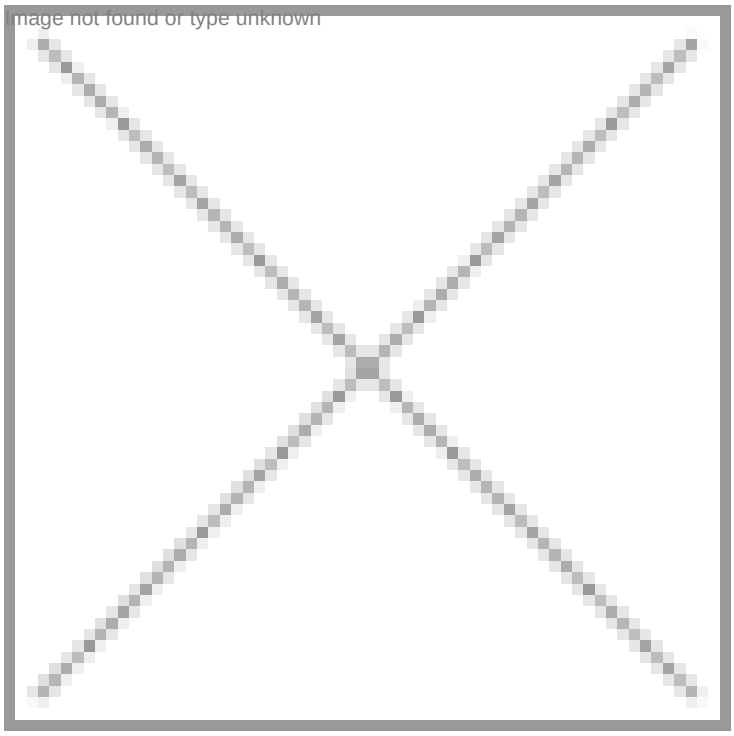
BioSpectrum
the business of Bio & Health Sciences

# Stempeucel trial: A landmark in stem cell therapy

09 December 2011 | News




*Stempeutics starts phase II clinical trial of the first-of-its-kind product, stempeucel, for treating patients suffering from alcoholic liver cirrhosis. The Department of Biotechnology will fund up to 3.5 crore towards this trial, while Stempeutics' contribution is estimated to be approximately 5 crore*



Bangalore-based stem cell company of Manipal Education and Medical Group, Stempeutics Research, announced commencement of phase II clinical trials of its investigational medicinal product, stempeucel, in patients suffering from alcoholic liver cirrhosis. Stempeucel is developed from allogeneic mesenchymal stem cells derived from donated bone marrow using Stempeutics' proprietary technology. Towards this landmark trial, the company has received funding from the Department of Biotechnology (DBT) under the Biotechnology Industry Partnership Program (BIPP). The trial will be funded jointly by Stempeutics and the DBT.

The primary objective of this clinical trial is to assess the safety following a single administration of different doses of stempeucel via the percutaneous puncture of the femoral artery, followed by selective cannulation of the hepatic artery (Seldinger Technique) in liver cirrhosis patients. The secondary objective is to explore the efficacy by assessing the potential of allogeneic mesenchymal stem cells in order to improve the liver function and quality of life in liver cirrhosis patients, and to find the suitable dose of allogeneic mesenchymal stem cells in order to improve their liver function and quality of life.

Success of this project will help India to become one of the leading countries after the US to develop stem cell-based drugs for unmet medical needs like liver cirrhosis.

**So far, so good**

Towards the beginning of 2011, Stempeutics received The Drug Controller General of India's (DCGI) clearance for conducting phase II clinical trials for Stempeucel to treat four debilitating diseases. This would be a multicentric, placebo

controlled, double blind, allogeneic clinical trials addressing osteoarthritis, liver cirrhosis, diabetes mellitus type 2 and chronic obstructive pulmonary disease.

Being the first-of-its-kind stem cell product developed in India, it took almost 15 months to get the DCGI's clearance. The DCGI referred Stempeutics' proposal to the Indian Council for Medical Research (ICMR) for review and recommendation. The ICMR constituted an expert stem cell committee to examine the proposal and the committee accepted the study as phase II clinical trials.

Other companies in India that have made news in the stem cell therapy forefront in the recent years include Reliance Life Sciences (RLS). RLS has established the safety and efficacy of stem cell therapies of autologous bone marrow derived mesenchymal stem cells for cardiac disorders (CardioRel), neurological disorders (NeuroRel), autologous melanocytes grafts for stable vitiligo (ReliDerm M) and allogeneic fibroblast graft for diabetic foot ulcers (ReliDerm DT).

For stempeucel, while the DBT will be funding up to 3.5 crore towards this trial, officials from Stempeutics mentioned that it would look at an estimated contribution of 5 crore. Financial backing by the DBT for this initiative, spells good news for the stem cell industry in India, which has been battling paucity of funds to bolster research initiatives.

Dr B N Manohar, CEO, Stempeutics Research, says, â€œOnce phase II trial is successfully completed, the company will be approaching the DBT for phase III clinical trial funding under Biotechnology Industry Partnership Program (BIPP). This phase of the trial shall be completed by December 2012. Overall, 60 patients shall be recruited in five hospitals all across India.â€?

In addition to this, a core team of five members will work on this product development from Stempeutics. â€œCore team consists of scientists, medical doctors, pharmacist and production technologist. Apart from the Stempeutics team, principal investigators from five hospitals will be involved in conducting the clinical trial,â€? added Dr Manohar. The investigational medicinal product for clinical trial is being manufactured at the Manipal facility as per Good Manufacturing Practices (GMP) guidelines. The approximate size of the facility is 7,000 sq ft and it has been audited by a DCGI team for GMP compliance. Once the product is ready, it will be marketed by Cipla.

As far as commercialization is concerned, company officials claim that it is too early to put definite timelines on these points. â€œWe will be in a better position to address this after the phase II trial is completed. The product will be marketed first in India and subsequently in neighbouring countries,â€? adds Manohar.

**Market potential of product**

According to market figures brought out by Frost and Sullivan, the estimated market size for stem cell therapy in India for various indications is approximately 2,700 crore. Out of this total addressable market, the market size for liver cirrhosis is approximately 400 crore. The size of the alcoholic population in India has also increased over the years. Prevalence of alcoholics in India is found to be 21 percent in adult males. This ranges from as low as seven percent in Gujarat to 75 percent in north eastern part of India. Alcoholic cirrhosis develops in 15 percent of individuals who drink heavily for more than a decade.

Generally, liver damage from cirrhosis cannot be reversed and one can only delay further progression and reduce complications by conservative management. When complications cannot be controlled or when the liver becomes extremely damaged from scarring or fibrosis, then it completely stops functioning, orthotopic liver transplantation (OLT) becomes necessary and is considered the standard treatment for advanced decompensated liver cirrhosis. However, the OLT cost is very expensive and moreover it is very difficult to get HLA-matched donors.

It is envisaged that stem cells can help to repair and regenerate the damaged liver, thus decreasing the requirement of OLT and provide therapeutic benefit to patients. Says Dr Manohar, â€œGlobally, until date, no stem cell product has been marketed for liver cirrhosis, but few global clinical trials have been conducted using both mononuclear stem cells and mesenchymal stem cells. It has shown that these cells are safe and that they improve the liver function in these groups of patients.â€?

Whilst the promise of regenerative medicine has been given thumbs up by the industry, experts like Dr Samuel J K Abraham, director, Nichi-In Center for Regenerative Medicine (NCRM), puts in a word of caution with respect to allogenic and mesenchymal stem cells. â€œIn my personal opinion, autologous cells for primary organ failures are acceptable, as several studies confirm the safety exist.When we go for allogeneic, we need to be very careful. Moreover, if one source of allogeneic donor stem cells is administered, and if the patient needs subsequent injection, it should be from the same donor. For this, the donor must be willing to donate or his earlier stem cells must be available for the subsequent administration to the patient. Otherwise by injecting another allogeneic source of cells, what will be the outcome is a question that remains to be answered,â€? he adds.

With regards to mesenchymal stem cells, says Dr Satyen Sanghvi, chief scientific officer, Regenerative Medical Services (RMS), â€œBone Marrow MSC transplantation has been the gold standard treatment option for bone conditions such as avascular necrosis and non-uniting or malunion fractures. Other research using bone marrow MSCs involves the application of cultured or uncultured (directly from bone marrow) cells in cardiac conditions. Several clinical trials are being carried out in many countries where doctors are performing these procedures with patient consent since the regulatory framework is non-existent. MSC application must be targeted to conditions where enough in vitro (lab), and in vivo (in animal & human) data is present and better understanding of the indication-cell type needs to be fostered.â€?

**-Nayantara Som** in Mumbai