

# Cell therapy: technologies, companies and markets

Recent ethical controversies surrounding embryonic stem cells have diverted attention from the progress in the broad field of cell therapy

**CELL THERAPY** is defined as administration of cells that have been selected, multiplied and pharmacologically treated or altered outside the body (*ex vivo*). Its aim is to replace, repair or enhance the function of damaged tissues or organs. It is related to and overlaps with several other established technologies, such as gene therapy, tissue engineering and regenerative medicine.

Besides stem cells, several other cell types, such as blood cells, myoblasts, bone marrow cells, and hepatocytes, are used in forms such as encapsulated cells and genetically engineered versions, which can also be referred to as gene therapy. Regulatory and ethical guidelines applicable to any biological therapies were already in place before the controversy surrounding the embryonic stem cells arose.

Important advantages of use of cells include the easy availability, and the efficacy of therapy through localised delivery of substances to the target. The implanted cells can release the therapeutic substance constantly at a rate determined by the cellular feedback mechanism, which prevents undue fluctuations. One example of this is transplantation of pancreatic islet cells for insulin-dependent diabetes mellitus.

## Adult versus foetal sources

Stem cells can transform into other cell types and migrate to the site of injury or disease for repair. Recent reports of success with stem cells from adult sources have prompted the discussion of adult versus foetal sources. Recent advances with adult-derived cells do not mean that adult cells can replace the need for those derived from embryonic or foetal tissue. Embryo-derived cells are more proficient in producing certain specialised type of cells, such as

dopamine-producing neurons and insulin-producing cells. Cells isolated from the adult brain cannot replicate to produce daughter cells. These cells have a limited life in culture, which does not give enough time to coax them to transform into another tissue.

Bone-marrow transplants have been used traditionally for bone-marrow rescue following chemotherapy. Now, peripheral blood is used as a source of these cells. For example, highly purified peripheral blood stem cells are autografted following myeloablative therapy in patients with lymphoma. Some of the striking achievements with cell therapy have been repair of the myocardial infarction in patients with heart attacks. Other applications for cell therapy include degenerative neurological disorders, such as Parkinson's disease, stroke, spinal cord injury, muscular dystrophy, cancer, haematological disorders, myocardial infarction, liver failure, burns, wound healing and osteoarthritis.

**"Embryo-derived cells are more proficient in producing certain specialised type of cells"**

## Over 200 companies involved

The wide range of applications of cell therapy is in keeping with the large number of companies involved – currently exceeding 200. Of these, about 30 are involved in stem-cell technologies, either providing sources for stem cells or developing technologies for stem-cell transplantation. Other companies provide technologies for cell culture, cell lines and cell sorting, or provide encapsulation technologies for cells transplantation. There are also a

few gene-therapy companies with technologies for *ex-vivo* genetic modification of cells. Some vaccine companies are developing cell-based cancer therapy. A number of tissue-engineering companies and companies involved in regenerative medicine are also developing cell therapies. Finally, some companies are providing supportive services for testing and regulatory approval of cell-therapy products.

Haematopoietic stem-cell transplantation and cell/gene therapy involving *ex-vivo* genetic modification of cells are the most advanced technologies, but stem-cell technologies need further development for other applications.

## \$78bn market in 2010

Of 105 companies profiled in the recently released report on cell therapy\*, 40 had collaborations relevant to cell therapy. The total value of the cell-based markets is estimated to be \$18.2bn (£?bn) in 2001, \$32.1bn in 2005, and \$78bn in 2010, according to the report.

The currently anticipated restriction on developing new stem-cell lines in the US may have temporarily set back the share values of stem-cell companies, as has a general slide in biotechnology. But this will not have any negative long-term effect on the world-wide markets, as stem-cell research continues in Europe and other countries such as Australia. ▀

## BY KEWAL JAIN

\* K. K. Jain (2001) Cell therapy - technologies and commercial opportunities. Jain PharmaBiotech, Blaesiring 7, CH-4057 Basel, Switzerland (<http://pharmabiotech.ch>; Tel & Fax: +4161-6924461; E-mail: [jain@pharmabiotech.ch](mailto:jain@pharmabiotech.ch)).