Cell therapy industry: the new direction of biotech

Clever cell culture for the innovation of biologics
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Therapeutic unmet need and an ageing population has produced a compelling argument and drive for innovative treatments that can ease this increasing global burden. The cell therapy industry is set to be a major component of innovative treatments, which truly hold the power of restorative or regenerative medicine. Consequently the industry, with changes in funding and regulation around the world, is pushing back all boundaries and the market is set to reach $1billion in 2011 and $5.1billion by 2014[1]. However, there is much to be done within the healthcare industry in-order to help this industry flourish and reach its full benefit for patients.

Attracting the major players

The increasing vitality of the cell therapy industry has attracted the attention of both large investors and traditional 'big pharma', all seeking market share in this lucrative field. Traditionally slow to take up emerging technologies, even large pharmaceutical companies see cell therapy as having the potential to change the face of medicine by mid 2017[2]. This has resulted in a raft of new strategic partnerships between large pharmaceutical companies, stem cell research-based companies and academic institutions.

The new market will be driven by the need for truly restorative or disease modifying therapies, rather than the standard focus on palliative treatment[3]. The establishment of cell therapy treatments for: heart failure, insulin-dependent diabetes, stroke, Parkinson's disease, spinal cord, Alzheimer's and renal disease will have two effects — provide improved care for patients and reduce the economic burden of these chronic diseases[4].

Growth and expectation

Cell therapy products are currently following a similar pattern of growth to the monoclonal antibodies market[2], which is now producing multi-billion dollar products. This growth is expected to bring a paradigm shift in the way that today's untreatable or poorly treated diseases are dealt with. Support from major companies means that clinical trials can be elevated to well-designed, large scale, Phase III trials and supported right through to commercialisation. This will help reduce time-to-market and elevate cell therapy into the mainstream as the new standard of care.

Cell therapy promises to make a major contribution to healthcare and some of the main drivers of the market include:

- restoring normal function for debilitating diseases;
- enhanced patient outcomes;
- lowering the economic burden of healthcare (potential savings of $250 billion per year);
- increasing productivity; and
- lowering the burden of chronic and degenerative disease due to increased life expectancy.

The need for better characterisation methods

All major indicators show that cell therapy will form an important part of future of healthcare. However, to achieve its full potential it will require regulatory, legislative, and technological support as well as further investment.

One key industry challenge is the characterisation of cell therapy products to ensure consistency and reproducibility - essential across all processes, from R&D to final product. Characterisation provides greater control throughout manufacturing and a better awareness of product quality, safety and efficacy.

Important characterisation parameters for stem cells include: sterility, purity, potency, ID markers, stability, safety and efficacy. These ensure more efficient monitoring of cells at all stages of development, ultimately resulting in increased product performance and less variability.

Undertaking efficient characterisation using current methods is difficult, labour and time-intensive, and can be a major bottle-neck when bringing cell therapy products to market. More efficient, robust characterisation methodology is therefore needed to validate and support the industry — particularly as European and North American stem cell regulations and guidelines evolve[2].

Molecular characterisation solutions

New technologies are needed to fulfil current and future characterisation requirements of the cell therapy industry. One of these is microRNA (miRNA) profiling, which allows characterisation at the molecular level providing a direct link to the biology of the cell. By enhancing the miRNA profiling technique with proprietary algorithms, companies such as Sistemic, can now provide analytical tools that deliver robust and information-rich characterisation and quality control methods to monitor cells easily and effectively.

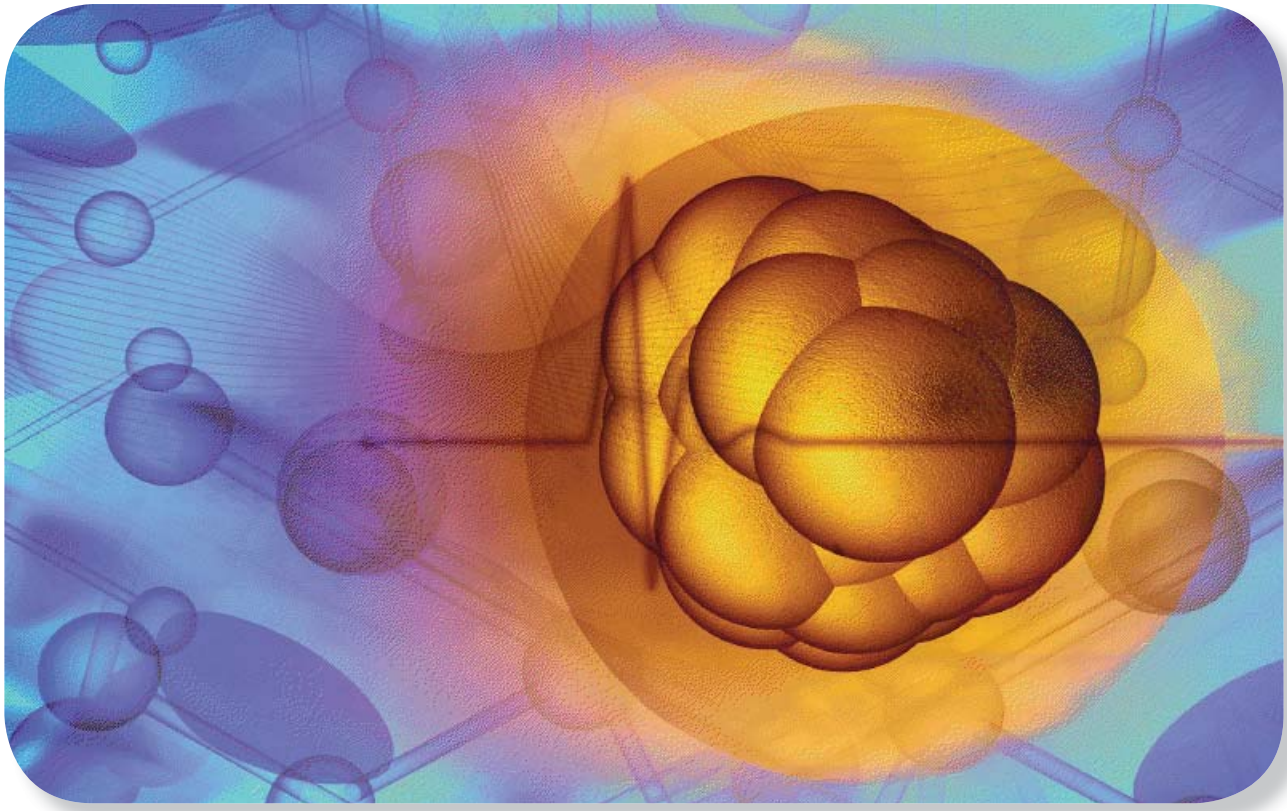
The cell therapeutics industry is rapidly gaining pace and hopes to deliver patient and economic benefits for the whole healthcare sector. In order for the full potential of this field to be realised, a concerted effort is needed to establish vital regulatory, legislative and technology support. Additionally, the appropriate data required to ensure the highest levels of quality and efficacy will be critical to future success. New technologies utilising miRNA to deliver characterisation at the molecular level offer the detail required. However, enhanced analytical approaches, such as that taken by Sistemic, are essential for efficient, cost-effective data collection and analysis.

1. Mason C, Brindley DA, Culme-Seymour SJ, Davie NL, Cell Therapy industry: billion dollar global business with unlimited potential, Regen. Med. 6(3), 265-272, 2011

2. Jyothikumar V, Stem Cell Therapy Market in Europe, Frost and Sullivan Market Insight, August 2011

3. McKernan R, McNeish J and Smith D, Pharma's developing interest in stem cells, Cell Stem Cell 6: pp517-520, 2010

4. Mason C and Dunhill P, The strong financial case for regenerative medicine and regen industry, Regen Med 3(3), pp351-363, 2008



miRNA profiling will form a powerful tool for characterisation of stem cells in the development of new treatments in regenerative medicine