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Perils of Stem Cell Translation: A Commentary

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During the last two decades we have seen the revolution in small molecules and biologics as frontiers in human medicine and now stem cells including other types of therapeutic cellsare sitting on the cuspof an another revolution, the third pillar of human medicine. However, the complexities of living cells and their interactions with host tissue pose a significant new challenge not encountered before in human medicine. But nevertheless stem cells can offer cure rather treatment for various ailments particularly those involving loss or degeneration of specific cells like in Diabetes, Spinal Cord Injury, Alzheimer's and Parkinson's diseases.

Stem cell research is relatively a new area but has assumed nearly a mythical status in attracting tremendous enthusiasm amongst scientific community and general public alike. A tremendous input in terms of human resources, funding and infrastructure development in this field is seen all over the world.The interest in this field is high partly because the way it has been put across to all of us with intense media coverage and promise of therapeutic values and other uses. No doubts the potential applications could be tremendous but there are number of issues/hurdles that have to be addressed before stem cell translation becomes reality.

There seems to be a significant gap between public expectations and the scientific reality to accomplish clinical translation. But nevertheless there are unprecedented developments seenrecently in this field with proof of principle emerging for number of diseases that currently have no cure but only management, like Alzheimer's, Parkinson's spinal cord injury,to name a few where stem cell-based therapies (regenerative medicine) can offer respite. While public attention has focused primarily on their potential in regenerative medicine, stem cells have quietly gained a stronghold in drug development, toxicological appraisal, and biomarker discovery - a move that may bring in a paradigm change in human medicine and therapeutics.

Despite huge progress and potentials, there remain scientific, technical, logistic, financial and administrative/regulatory obligations that are required to be met to move thisfield forward. However, overtly these may be considered playing impediments in the way of stem cells translation. This transition also depends also on what type of stem cells are being considered – adult or embryonic including patient-derived induced pluripotent stem cells (iPSC) for such studies. Adult stem cell (hematopoietic and bone marrow) transplantation has a long history, morally and ethically more acceptable, although not necessarily gone through the rigour of traditional drug discovery pathway but have become a part of the main stream treatments for many malignancies and some genetic diseases. Similarly stem cell-derived skin graft transplantation is following the same route. The Pluripotent stem cells, however, are still battling despite some significant advances and building hopes for diseases like macular degeneration, spinal cord injury, skin grafts and perhaps for some neurodegenerative diseases. There are some moral ethical and safety issues associated with these cells. It may be mentioned here that unlike small molecules and biologics that work on the principle of turning on/off of biological systems, stem cell-based therapies may be more predictable provided integrated properly in circuits such that the milieu/niche of the tissue brings about homoeostasis and thus regulate their functions. This remains the most active area of research in this field.

Because the regenerative medicine (therapeutics) is a new concept and despite a good science behind it, yet there is no single harmonised guiding principlethat can be used to describe the preclinical development path or model for this therapeutics. Several specific technical issues like safety, efficacy, viability and tracing the transplanted cells are the inherent challenges in stem cell-based therapeutics. The existing regulatory framework for clinical trials with stem cell products is not clear or explicit and this gray area is a significant roadblock for transition to clinics. Perhaps the route followed by the blood and bone marrow transplantation initiatives may offer respite i.e. the need-based control human trials and case studies. However, sprouting of spurious stem cells clinics worldwide and the associated patient tourism is a big concern that needs addressing perhaps by proper educational initiatives in this area.

The landscape of stem cell translation is complex and requires liberal investments. An average cost of a pharmaceutical drug to deliver to the market is around 1.4 billion US $ and it is likely to be more for stem cells-based therapy and that cannot be supported with public funding. Thus a typical drug delivery model won't work, and an alternative logistic and financial model such as Alpha Clinics proposed byCalifornia Institute of Regenerative Medicine to achieve widespread clinical application requires due diligence. Given the newness of this area, there remain uncertainties and risks involved that are hampering private investments in this field. Big pharmas and corporate investors are shying away because of these risks. However, similar scepticism for investments was there for DNA recombinant technology and human genome projects; both are now providing dividend far excess than investments made.In time, examples of profitable cell therapeutic applications may evolve through new start-up companies that will attract the interest of major investors or mergers and acquisitions. In the absence of significant venture capital and major pharmaceutical company interest, an alternative system is needed for academic stem cell scientists to partner with industry biotechnology expertise.

The role of biotechnology sector in regenerative medicine is likely to be broader, providing materials, services, and cell manufacturing, suggesting much greater commercial opportunities in the clinical application of cell therapies. In addition, special government initiatives like the 2004 proposition 71 (public bond to fund scientific research) in California that lead to the establishment of the California Institute of Regenerative Medicine for 3 billion US$ and now Japan's 21.4 billion ¥ stimulus package for stem cells and iPSC work (Nature 28th Feb 2013)

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are steps in the right direction. Government special tax rebates/reforms in this business is another way of encouraging biotech companies and entrepreneurs investing in this area. Some stimulus packages in R&D in stem cells through government agencies like NHMRC, ARC, and CSIRO in Australia and likewise in other countries by creating special research groups focussing exclusive in this area will be helpful. Non-to-profit organisations, philanthropists, and foundation funds all can make a difference.

At the moment transition of stem cells towards clinic is a cliff hanger and will continue to pose a series of scientific, clinical, technical and operational challenges over the coming decade. In order to be successful we need to develop an efficient, high-quality 'strategic hub' linking scientific institutions, clinical centers and biotech companies so that transition is steady and efficient. A novel and harmonized pre-clinical model system to ensure the safety and efficacy measures will be mandatory to advance this field, and efforts to ensure more predictable regulatory and recovery pathways will help infuse industry interests in stem cell technologies and their transition to clinic.