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Cellular therapies for rare diseases

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Clinical programs directed at rare diseases present many unique challenges for clinical research. Most druggable targets have been identified and exploited and the sciences of drug (be they small molecules including oligonucleotides or biologics) or device development has dramatically advanced in recent decades. With that advance, the opportunity to develop products for large populations of common diseases has largely disappeared. At the same time the phenotypes for many common diseases have been split into multiple smaller populations and even genotypes focusing more and more programs on smaller populations. Planning to complete increasingly complex studies in smaller but more homogeneous patient groups has become increasingly competitive and costly. Against this clinical research landscape, cellular therapies as the third major branch of clinical research have arrived. Initially working with fetal, embryonic, bone marrow, adipose or cord blood derived stem cells, the field has been less regulated than drugs and devices, leading to a proliferation of clinics and claims that have not all been through a rigorous and appropriate review. Attention is now evolving from the ethically and immunologically challenging programs involving allogeneic stem cells to autologous, organ specific stem cells. This can be best achieved by generating directly reprogrammed precursor cells for that organ. These promise greater safety and easier production, for a very complex product and will allow this third branch of medical research to start to tackle many rare diseases where cellular regeneration may be required for clinical benefit. To capitalize on this timely opportunity, time and cost efficient direct reprogramming to lineage-specific precursor cells is vital. With this advance, cellular therapies will take their rightful place in the physicians'armamentarium against injury, disease and degeneration, just as healthcare costs in advanced countries look set to spiral completely out of control.

Biography

Stephen Shrewsbury is currently an Executive Vice President of Development and Chief Medical Officer at Fortuna Fix, leading their novel cellular technology into multiple clinical programs focused on regenerative medicine, several of which are rare diseases. He moved to lead inhaled antibiotic programs at Chiron Corporation (in Cystic Fibrosis and other rare diseases) before becoming CMO sequentially to MAP Pharmaceuticals, Adamas Pharmaceuticals, AVI BioPharma (now Sarepta Therapeutics; where he opened their first IND for Eteplirsen in Duchenne muscular dystrophy and planned their oligomer programs against Ebola and Marburg Hemorrhagic Fevers) and Aquinox Pharmaceuticals. He is the author of Defy Your DNA and over 70 scientific papers, holds several patents and contributed to many awarded grants, mostly for work on rare diseases.

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