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Novel viral-free and oncogene-free induced pluripotent stem cell for orphan disease cell therapies

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Pluripotent stem cells represent a potential regenerative medicine for several orphan diseases because the cells exhibit broad plasticity. Induced pluripotent stem cells (iPSC) have the potential to serve as an autologous as well as an allogeneic cell therapy. However, iPSC therapy has not yet been fully realized because the iPSC reprogramming methods have historically required viral gene delivery and oncogenes in order to create a final iPSC product. Non-integrating iPSC reprogramming approaches like self-replicating ribonucleic acid and Sendai virus have been developed to reduce the tumorigenicity risk. However, these reprogramming methods still pose significant costs and oncogenic risk because they utilized the oncogenes, c-Myc and Lin28. Episomal reprogramming is a safe reprogramming approach to produce clinical-grade iPSC therapies. However, the reprogramming efficiency of episomal vectors has been inefficient and has required c-Myc and Lin28 to compensate for the low efficiency. We have developed a combinatorial reprogramming approach of small molecules and a novel episomal construct that is free of c-Myc and Lin28. The combinatorial approach significantly increased the reprogramming efficiency. Further, the reprogramming method also utilized a well-defined tissue cultured media that is feeder-free, xeno-free and matrigel-free. This combinatorial reprogramming approach is now poised to transition into GMP operations, which would satisfy regulatory requirements. The opportunity now exists to develop clinical-grade and safe iPSC for a variety of orphan diseases.

Biography

Alan B Moy has established a successful career in academia, non-profits and industry. He has received his MD from Creighton University, completed his Internal Medicine Residency at St. Louis University and Pulmonary Fellowship at the University of Iowa. He has served on Faculty at the University of Iowa College of Medicine and College of Engineering with a research expertise in cellular and tissue engineering. He is the Founder and Scientific Director of the John Paul II Medical Research Institute, a 501 (C)(3) and is the CEO and Co-Founder of Cellular Engineering Technologies, a leading stem cell manufacturing company. He is listed in the Leading Physicians of the World by the International Association of Healthcare Professionals. His area of expertise includes pulmonary medicine, cytoskeletal biology, vascular biology, tissue engineering and industrial stem cell manufacturing.

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