Short Communication

iMedPub Journals
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Journal of
Stem cell biology and transplantation

2021
Vol. No. S(7)

AAV8 gene therapy for hemophilia

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Abstract

Significant advancements have been made in the field of gene therapy using recombinant adeno-associated viral vector (rAAV) based gene delivery systems that has demonstrated their safety and applicability resulting in commercial approvals of few products: Luxturna for a rare inherited retinal dystrophy, and Zolgensma for spinal muscular atrophy. The main challenge of making a rAAV gene therapy product is the development of a robust and scalable GMP-compatible process for a large scale manufacturing and purification of rAAV virus like particles (VLPs). Our ongoing efforts to optimize a scalable process using HEK 293 suspension cell line cultured in chemically defined media to produce rAAV8 based products at 10 L and 50L Bioreactor scales have resulted in a robust and consistent process for the production of Intas's rAAV8 FVIII and rAAV8 FIX gene therapy candidates for Hemophilia A and B respectively for the preclinical studies. In addition to this a scalable microbial process for GMP grade plasmids used for transfection in the production of these rAAV VLP's have also been established. Analytical release assays and characterizations assays have also been established for these products.The talk will focus on Intas's rAAV8-FIX and rAAV8-FVIII product and process development, analytical characterization and proof of concept studies using FIX knock out animal model for Hemophilia B prior to assessment of the preclinical toxicity and safety.

Biography

Head of The Center for Gene and Cell therapy at Intas Pharmaceuticals. Working on the development of recombinant Adeno-associated viral (AAV) and Lentiviral (LV) like particle (VLP) based gene and cell therapy products.Prior experience in complex generics as Vice President at Biological E limited, in Vaccine as General Manager at Sanofi, as Director of fermentation technology development center at Dr Reddy's laboratories.Damon Runyon Cancer Foundation Fellow at Stanford University School of Medicine working in the field of cell division and cytokinesis. PhD from Tata Institute of Fundamental Research in Biophysics and M.Sc in Chemistry (Gold Medal) from University of Hyderabad.

Publication of speakers

1. Lakshmikanth S Gandikota et al ; The Localization of Inner Centromeric Protein (INCENP) at the Cleavage Furrow Is Dependent on Kif12 and Involves Interactions of the N Terminus of INCENP with the Actin Cytoskeleton, 2007 Sep 18.
2. Lakshmikanth S Gandikota et al ; A mitotic kinesin-like protein required for normal karyokinesis, myosin localization to the furrow, and cytokinesis in Dictyostelium, 2004 Nov 23.

Citation : Lakshmikanth S Gandikota; AAV8 gene therapy for hemophilia ; Stem Cell 2021;February 10;Dubai ,UAE.