

Is the first commercial gene therapy product within sight?

October 15 2015

Highly anticipated phase III clinical trial results of Spark Therapeutics's gene therapy to treat visual impairment are due by year-end and could have profound implications for the broader gene therapy field, leading to the first approved product in the U.S. A detailed analysis of the design and use of SPK-RPE65 to treat Leber congenital amaurosis type 2 (LCA2), the possibility of expanding its use to other patient populations, and the anticipated impact of a first commercial gene therapy product is explored in *Human Gene Therapy Clinical Development*.

Joshua Schimmer and Steven Breazzano, Piper Jaffrey & Co., New York, NY, predict the likelihood that the SPK-RPE65 phase III data will meet the primary goal of improving patients' ability to see and function at night or in dimmer light levels. Secondary endpoints include enhanced visual acuity and peripheral field light sensitivity or an effect on the underlying degenerative process.

In the article "[Investor Outlook: Focus on Upcoming LCA2 Gene Therapy Phase III Results](#)," the authors examine the science supporting a gene therapy approach to treating LCA2 in which an adeno-associated viral vector delivers the RPE65 gene (which is mutated in LCA2) to the eye via subretinal injection. At least 10 other biotechnology companies have [gene therapy](#) products in development targeting retinal diseases.

"Josh and Steve have done a wonderful job summarizing this potential milestone," says Human Gene Therapy Clinical Development Editor James M. Wilson, MD, PhD, Director of the Gene Therapy Program,

Department of Pathology and Laboratory Medicine, University of Pennsylvania Perelman School of Medicine, Philadelphia, PA.

More information: The article is available free on the *Human Gene Therapy Clinical Development* website until November 15, 2015.

Provided by Mary Ann Liebert, Inc

Citation: Is the first commercial gene therapy product within sight? (2015, October 15) retrieved 8 May 2024 from

<https://medicalxpress.com/news/2015-10-commercial-gene-therapy-product-sight.html>

This document is subject to copyright. Apart from any fair dealing for the purpose of private study or research, no part may be reproduced without the written permission. The content is provided for information purposes only.