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ASGCT Celebrates FDA Approval of Voretigene Neparvovec (Luxturna) as Treatment for Inherited Retinal Disease

Groundbreaking gene therapy is the first AAV viral vector product to be approved for use in the United States.

December 19, 2017—Today the Food and Drug Administration (FDA) approved Voretigene Neparvovec (Luxturna, Spark Therapeutics), an innovative one-time gene therapy treatment used to improve vision in patients with established genetic vision loss due to Leber congenital amaurosis or retinitis pigmentosa, both inherited retinal diseases (IRD).

"This is a class of diseases for which there have been no pharmacological treatments," Dr. Katherine A. High Co-Founder, President, Head of Research and Development at Spark Therapeutics says. "There are more than 250 genes involved in vision and we hope that this may pave the way for the development of treatment for other inherited retinal diseases/causes of congenital blindness."

As the first AAV viral vector product approved for clinical use in the United States, Luxturna represents a historic landmark, not only in the field of gene therapy, but in the history of medicine.

"Having the first approval of a gene vector delivered directly to a person sets a precedent for other groups to move forward to develop gene therapies to treat other inherited and acquired diseases," Dr. Jean Bennett, F.M. Kirby Professor of Ophthalmology at the University of Pennsylvania's Perelman School of Medicine and a leader in the development of Luxturna, says. "Until now, there has been no path."

Luxturna opens the door as a proven viral vector product for the many AAV products currently under development and in clinical trials throughout the country.

"There are going to be a lot more approaches using AAV coming down the line both in eye gene therapy and in other diseases such as hemophilia," Dr. Helen E. Heslop, ASGCT President and Director of the Center for Cell and Gene Therapy at Baylor College of Medicine, Texas Children's Hospital and Houston Methodist Hospital says.

As the largest professional society representing gene and cell therapy in the world, ASGCT is undeniably proud of the advancements made in gene therapy in efforts to improve the lives of those with IRD and offers a sincere congratulations to all who worked to bring this new therapy to patients.

"Luxturna has the potential to have a major impact for affected patients based on the clinical trials that have shown improved vision," Dr. Heslop says. "We hope this approval will bring the treatment to a larger group of patients who can benefit from the therapy."

Other ASGCT reports on the history of Luxturna and the development process are available on ASGCT.org. The full FDA release regarding the approval of Luxturna is available online.

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About ASGCT

The American Society of Gene & Cell Therapy is the primary professional membership organization for scientists, physicians, patient advocates, and other professionals with interest in gene and cell therapy. Our members work in a wide range of settings including universities, hospitals, government agencies, foundations, biotechnology and pharmaceutical companies. ASGCT advances knowledge, awareness, and education leading to the discovery and clinical application of gene and cell therapies to alleviate human disease to benefit patients and society.