RetroSense doses first patient in phase I/II RP clinical trial

March 23 2016

A Wayne State University startup company announced today the first successful dosing of a patient in a clinical trial that is a major step forward for patients with vision challenges.

RetroSense Therapeutics LLC, a privately held biopharmaceutical company, successfully dosed their first patient in the first clinical trial to

evaluate the safety of RST-001. The study is titled "Phase I/IIa, Open-Label, Dose-Escalation Study of Safety and Tolerability of Uniocular Intravitreal RST-001 in Patients with Retinitis Pigmentosa (RP)." RST-001 is designed to restore some vision in patients with RP, a genetic condition that leads to the progressive degeneration of rod and cone photoreceptors (cells found in the retina that sense light), resulting in severe vision loss and blindness.

"Successfully dosing our first patient with RST-001 represents a significant milestone in the development program," said Sean Ainsworth, CEO of RetroSense Therapeutics. "Treating the first human patients should provide key insights into the potential for optogenetics in vision restoration and beyond. We hope to establish the power of using a gene therapy application of optogenetics to improve vision in individuals with currently incurable blindness."

David G. Birch, Ph.D., chief scientist and executive officer of the Retina Foundation of the Southwest and the principal study investigator, added, "Patients enrolling in the trial understand that we are exploring brand-new territory, but are excited about the possibility of restoring some vision."

The initiation of this clinical study results from the culmination of several years of research and collaboration with researchers at leading institutions, including Zhuo-Hua Pan, Ph.D, at the Ligon Research Center of Vision in Wayne State University's Kresge Eye Institute and Richard Masland, Ph.D., at Massachusetts Eye and Ear Infirmary, early pioneers in optogenetics for vision restoration.

The study is composed of two parts. An initial dose-ranging study is proposed whereby three dose levels of RST-001 will be studied in three separate groups of adult patients with advanced disease. This first part of the study is aimed at determining a single dose of the experimental

agent, which is safe and well tolerated, to further evaluate in a fourth group of patients. The second part is aimed at obtaining additional safety data at the highest tolerated dose and providing important additional clinical data to guide the design of future efficacy studies.

"This is an exciting breakthrough for RetroSense," said Joan Dunbar, associate vice president for technology commercialization at Wayne State University. "The extraordinary efforts of Dr. Pan and his collaborators are now one step closer to finding a way to help restore sight because of the important work that RetroSense is doing. It is a long process, but their vigilance and commitment to restoring sight may one day soon give hope to many people."

In August 2015, the company's Investigational New Drug (IND) application for RST-001 received clearance from the United States Food and Drug Administration (FDA). The clinical trial tracker is NCT02556736.

RetroSense Therapeutics is developing RST-001 as a first-in-class gene therapy application of optogenetics. Optogenetics refers broadly to means of conferring light sensitivity to cells that were not previously, or natively, light sensitive. By applying optogenetics to retinas in which rod and cone photoreceptors have degenerated, RetroSense is working to confer new light sensitivity to the retina, with the expectation of some degree of improved or restored vision for affected patients.

In 2014, the FDA granted Orphan Drug designation for RST-001 based on its development as a treatment of RP, a rare disease that affects an estimated 100,000 people in the United States. As a designated Orphan Drug, RST-001 is eligible for various development incentives under the Orphan Drug Act, including a potential waiver from FDA's application user fees, certain tax incentives and Orphan Drug exclusivity.