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Allergan acquires gene therapy start-up

September 7, 2016 | News | By BioSpectrum Bureau

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Allergan plc and RetroSense Therapeutics LLC, a private, clinical-stage biotechnology company focused on novel gene therapy approaches to restore vision in patients suffering from blindness, announced that Allergan has acquired substantially all of the assets of RetroSense in an all-cash transaction. Under the terms of the transaction, Allergan has paid RetroSense a $60 million upfront payment, and has agreed to potential regulatory and commercialization milestone payments related to its lead development program, RST-001, a novel gene therapy for the potential treatment of Retinitis Pigmentosa (RP).

"The acquisition of RetroSense and its RST-001 program builds on Allergan's deep commitment to eye care, and our focus on investing in game-changing innovation for retinal conditions, including Retinitis Pigmentosa, where patients desperately need treatment options," said Brent Saunders, CEO and President of Allergan.

Retinitis Pigmentosa (RP) is a group of rare, inherited genetic disorders characterized by progressive peripheral vision loss and night vision difficulties followed by eventual central vision loss and blindness in many cases.

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RST-001 is first-in-class gene therapy application of optogenetics, a therapeutic approach that confers 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, the technology introduces additional light sensitivity to the retina. In 2014, RST-001 received an Orphan

Drug Designation by the US FDA for the treatment of Retinitis Pigmentosa.

The RST-001 optogenetic approach employs a photosensitivity gene, channelrhodopsin-2, to create new photosensors in retinal ganglion cells to potentially restore vision in retinal degenerative conditions. In August 2015, RetroSense's Investigational New Drug (IND) application for RST-001 received clearance from the United States Food and Drug Administration (FDA). In March 2016, RetroSense initiated a Phase I/IIa clinical trial to evaluate the safety of RST-001 in patients being dosed, and in August 2016, the low dose cohort of patients had been safely dosed.

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"The RST-001 program and its optogenetic gene therapy approach could be a real breakthrough in the treatment of unmet needs across a host of retinal conditions, including RP," said David Nicholson, Chief Research and Development Officer, Allergan. "The team at Allergan is excited by the prospect of advancing an entirely new approach in the treatment of retinal diseases, and this technology is highly complementary to our ongoing development programs in this critical treatment area."

"With its deep commitment to eye care, strong eye care professional community network, and its commitment to advancing innovation across retinal conditions, Allergan was the most compelling partner and the best strategic fit to advance the development of RST-001 and maximize the potential for this technology platform," said Sean Ainsworth, former CEO of RetroSense Therapeutics. "The addition of RST-001 to Allergan's world-class eye care development and commercialization organization will position this potentially revolutionary technology to be used by ophthalmology professionals to improve the treatment of patients with retinal diseases around the world."

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