Orphan Drug Designation Granted to Bietti's Crystalline Dystrophy Gene Therapy

Krista Rossi

This morning, August 28, 2018, Reflection Biotechnologies Limited’s AAV-based gene therapy, the RBIO-101 program (AAV.CYP4V2), was granted an orphan drug designation by the US Food and Drug Administration (FDA) for the treatment of Bietti's crystalline dystrophy (BCD), a rare retinal degeneration.

"Receiving orphan drug designation from the FDA is a milestone," said Richard R. Yang, founder and CEO of ReflectionBio, in a recent statement. “This brings hope to BCD patients and their families because BCD is a devastating blinding disease for which, currently, there is no approved treatment. BCD is estimated to affect more than 100,000 patients worldwide. As the next step, we plan to advance BCD gene therapy into human clinical trial.”

A progressive atrophy and degeneration of the retinal pigment epithelium (RPE), BCD is estimated to affect over 100,000 global patients. The rare, genetic eye disease is characterized by yellow-white crystals in the retina that may vanish in advanced stages of the disease. Onset typically occurs between early teenage years and the third decade of life. Legal blindness is often reached by the 3rd to 6th decade of life.

“As a rare disease patient, I am proud to be driving research and development efforts for BCD gene therapy together with our collaborator, Dr. Stephen H. Tsang of Columbia University,” added Yang. “The RBIO-101 preclinical data from Dr. Tsang’s lab support the clinical translation for patients with BCD.”