Trefoil Therapeutics Begins First Clinical Trial of Regenerative Treatment for Patients with Corneal Endothelial Dystrophies

Phase 1/2 "INTREPID" trial to evaluate safety and ability of engineered FGF-1 to regenerate corneal endothelial cells

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SAN DIEGO--(BUSINESS WIRE)--Trefoil Therapeutics today announced it has initiated the first clinical trial of its engineered Fibroblast Growth Factor-1 TTHX1114 for the regenerative treatment of people with corneal endothelial dystrophies (CED), including Fuchs Endothelial Corneal Dystrophy. The Phase 1/2 trial ("*INTREPID")* is designed to evaluate TTHX1114's safety and ability to stimulate the regeneration of corneal endothelial cells lost due to CED when administered by intracameral (back of the cornea) injection.

"Corneal endothelial diseases, and specifically Fuchs Dystrophy, are among the leading causes of corneal transplantation," said Francis W. Price, Jr., MD founder and president of Price Vision Group and the Cornea Research Foundation of America. "There is a high unmet need for a pharmacologic option that would allow treatment of these diseases earlier and potentially reduce or eliminate the need for surgical intervention. TTHX1114 has demonstrated in multiple preclinical models the ability to stimulate corneal endothelial cell proliferation and drive the regeneration and healing of the corneal endothelium. This trial provides the first opportunity to evaluate the potential of this innovative new drug in patients."

"This clinical trial marks an important milestone that moves us one step closer to the potential to give patients better vision with fewer complications via a pharmacologic alternative to corneal transplants from donor tissue with," said David Eveleth, Ph.D., Trefoil Therapeutics' CEO. "Intracamerally delivered TTHX1114 is the first of two engineered FGF-1 products Trefoil is developing to treat corneal diseases. We are also advancing a topical product for the treatment of corneal ulcerations that is expected to enter clinical trials next year."

Corneal endothelial cells line the interior of the cornea and are critical to maintaining the cornea in its appropriate hydration state which enables the cornea to function properly. CED, characterized by excessive loss of these cells, results in diminished vision and, in severe cases, can lead to blindness. CED is also a contributor to poor outcomes in eye surgery including cataract surgery.

CED affects an estimated four percent of the U.S. population. Fuchs Endothelial Corneal Dystrophy is the leading cause of corneal transplantation in the U.S. Corneal transplant is currently the only treatment option for many people with CED. Although transplant surgery with human donor corneas may be effective in restoring vision, post-surgical recovery can be challenging, and most patients require long-term immune suppression therapy to minimize the risk of graft rejection.

The rationale for TTHX1114 as a potential therapeutic is based on the well-known ability of the naturally occurring FGF-1 molecule to stimulate cell proliferation and migration as well as protect cells from stress and injury. Unlike naturally occurring FGF-1, TTHX1114 has been engineered to increase its half-life, enabling its use as a pharmaceutical. TTHX1114 was discovered in the laboratory of Trefoil co-founder, Michael Blaber, PhD, at Florida State University, which licensed the compound to the company. Trefoil received significant support for the development of TTHX1114 in preparation for the clinical study through a collaboration with NIH's National Center for Advancing Translational Sciences.

The *INTREPID* trial is a prospective, multi-center randomized, masked, placebo-controlled, study involving up to 71 patients with moderate to severe corneal endothelial dystrophy. Patients will receive four intracameral TTHX1114 injections at 7-day intervals. The study's primary endpoint is the change in corneal endothelial cell count from baseline to Day 56. Secondary endpoints include measures of visual acuity, corneal edema, and other measures of corneal endothelial cell growth. An observational study preceded the trial, in which patients underwent standard ocular assessments.

Trefoil is conducting the INTREPID trial under an IND submitted to the U.S. Food and Drug Administration.

About Trefoil Therapeutics

Trefoil Therapeutics is a private biotechnology company focused on leveraging its engineered fibroblast growth factor-1 protein technology platform to develop first-in-class pharmacologic treatments for serious corneal endothelial diseases and epithelial disorders. Trefoil's lead product candidate is TTHX1114, an engineered form of FGF-1 designed to stimulate corneal endothelial cell proliferation and migration, thereby reversing vision loss caused by CED. The technology underlying Trefoil's platform was developed by co-founder Michael Blaber, Ph.D., and is licensed from Florida State University.

Safe Harbor Statement

The nonclinical research discussed in this press release is preliminary and the outcome of such studies may not be predictive of the outcome of later clinical trials. Future clinical trial results may not demonstrate safety and efficacy sufficient to obtain regulatory approval related to the nonclinical research findings discussed in this press release.

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