Choroideremia Research Foundation Acquires Spark Therapeutics, Inc., CHM Gene Therapy Assets, Advances Data Analysis

CRF acquired Spark Therapeutics, Inc.'s SPK-7001 CHM gene therapy assets and is analyzing long-term data to identify key clinical measures for development.

Illustration representing CRF's analysis of choroideremia gene therapy clinical and natural history data to support future research.

SPRINGFIELD, MA, UNITED STATES, January 23, 2026 /EINPresswire.com/ -- In 2025, Choroideremia Research Foundation (CRF) acquired all Spark Therapeutics, Inc. assets related to its SPK- 7001 gene therapy for treating Choroideremia (AAV2-hCHM-101) following the decision to discontinue the program.

The acquired assets include intellectual property rights, biological materials, orphan drug designations, Phase I/II clinical trial data, and Natural History Study data.

Spark's clinical trial evaluated the safety and preliminary efficacy of subretinal CHM gene therapy in 15 participants affected by choroideremia (CHM), an inherited retinal disease. One eye was treated and the other eye served as a control. The safety goal was met. Some participants have self-reported stabilization in the treated eye prompting CRF to analyze the five-year follow-up data.

CRF and University of Virginia Faculty members, Drs. Tom and Shannon Barker, and CRF Chief Science Officer Dr. Mike McConnell are facilitating a University of Virginia Biomedical Engineering Student Capstone Project titled "Clinical Trial Data Analysis: Finding endpoints that matter to choroideremia patients." The student team is evaluating data from ophthalmic measurements completed during Spark's clinical trial.

Given the slow and uneven progression of CHM, it is difficult to distinguish therapeutic effects of a treatment from the normal variability between treated and untreated eyes over the relatively

short span of a typical clinical trial. This evaluation of clinical data from Spark's trial is being done in tandem with an evaluation of related natural history data of clinical observations for over 125 CHM patients in order to identify ophthalmic endpoints that can establish vision stabilization, or improvement, following therapeutic treatment. CRF aims to publish these findings to inform regulatory action and approval in any potential, future research or clinical trial related to Spark's gene therapy or other treatment.

CRF thanks its volunteer team members and its collaborators at Spark, Roche, University of Pennsylvania, and the Children's Hospital of Philadelphia for their support in this first-of-its-kind asset transfer and data review.

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About Choroideremia
Choroideremia (CHM) is a rare inherited form of blindness affecting approximately 1 in 50,000 people. Due to its X-linked inheritance pattern, males are most severely affected, with females usually experiencing much milder visual impairment. Symptoms begin in early childhood, with night blindness and restriction of visual field being the earliest noticeable effects, eventually progressing to complete blindness. An estimated 6,000 people in the United States and 10,000 in the European Union are impacted by choroideremia. There are currently no approved treatments for choroideremia. For more information, visit curechm.org/#choroideremia

About the Choroideremia Research Foundation Inc.
The Choroideremia Research Foundation was founded in 2000 as an international fundraising and patient advocacy organization to stimulate research on CHM. Since its inception, the CRF has provided approximately $6 million in research awards and is the largest financial supporter of CHM research worldwide. Research funded by the CRF has led to the development of a CHM animal model, the pre-clinical production of gene therapy vectors currently in clinical trials, and the CRF Biobank which stores tissue and stem cell samples donated by CHM patients. For more information, or to make a donation to support research, visit curechm.org

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