Choroideremia Research Foundation Hosts International Science Symposium

Global Researchers Gather to Discuss Rare Genetic Eye Disease Treatment Options

PHILADELPHIA, PA, UNITED STATES, August 20, 2019 /EINPresswire.com/ -- On June 6-7, the Choroideremia Research Foundation (CRF) hosted its 2019 International Choroideremia Symposium in Philadelphia, Pennsylvania. In conjunction with the Center for Advanced Retinal and Ophthalmic Therapeutics (CAROT Center) at the University of Pennsylvania, CRF developed an agenda geared toward answering key questions raised by ongoing research and developing consensus opinion on paths forward for the organization and its community. The Symposium was attended by 20 international experts in choroideremia (CHM) from five countries, in addition to representatives from biotech, industry and the patient community.

The Symposium was held using an open moderated discussion format, engaging all in attendance to help discuss topics and answer questions using the collective expertise of those in attendance. The first day focused on pre-clinical topics which could provide better understanding of disease mechanisms and, in corollary, additional avenues toward the development of new therapies. Much discussion revolved around the underlying mechanisms of retinal cell dysfunction and death in CHM, which like many retinal diseases requires additional investigations. The group discussed a series of potential therapeutic approaches including the use of stem cells, neuroprotective agents, optogenetics, reversal of the normal aging process in cells, and how research on these approaches could be supported. Day two of the Symposium reviewed ongoing natural history studies and clinical trials, bringing together leaders in these respective areas to identify lessons learned from these trials and potential next steps. Lastly, robust conversation was held to begin developing a classification system to stage the progression of CHM in patients, as well as the ideal outcome measures to be used in future clinical trials.

“The 2019 International Choroideremia Symposium was a tremendous success,” says Christopher Moen, MD, Chief Medical Officer of CRF. “The topics reviewed at the Symposium identified several opportunities for research projects which could drive CHM toward future therapies that could work alongside gene replacement therapy. We’re excited at the future of CHM research and fortunate to have such tremendous support from our medical and scientific community.”

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.

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 over $2 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, visit www.curechm.org

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