THROSSELL-HILLIER FAMILIES RESEARCH AWARD WINNERS ANNOUNCED

Research will focus on choroideremia nonsense mutations

CRF has funded approximately $5 million in research studies during its 23-year history.

SPRINGFIELD, MA, UNITED STATES, March 23, 2023 /EINPresswire.com/ -- The Choroideremia Research Foundation (CRF), in conjunction with Choroideremia Research Foundation Canada (CRFC), is pleased to announce three recipients of the Throssell-Hillier Families Research Award. The generosity of the families has made it possible for the CRF/CRFC to simultaneously fund several studies to accelerate the pace of research to find a treatment or cure for choroideremia (CHM), specifically in the interest area of nonsense mutations, which affects approximately 1/3 of CHM patients.

> We are very excited to support a diverse range of cutting-edge research proposals. We continue to push the boundaries of our knowledge by supporting this innovative group of researchers."
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> *Neal Bench, CRF board president*

The scientific research to be supported includes the following investigators and studies:

STUDY: Translational read-through as therapeutic approach for CHM-patients with disease-causing nonsense mutations
Kerstin Nagel-Wolfrum, PhD, Institute of Molecular Physiology, Johannes Gutenberg University of Mainz, Germany

STUDY: Development of a modular CHM minigene for testing rescue of the LET variant
Joseph Porter, PhD and John Lueck, PhD, Department of Pharmacology and Physiology, University of Rochester Medical Center, Rochester, NY

STUDY: CRISPR-Based Gene Editing for Choroideremia
Miguel Seabra, MD, PhD and Pedro Antas, PhD, Global Eye Health Initiative, Champalimaud Foundation, Portugal

"We are very excited to support a diverse range of cutting-edge research proposals," said Neal

Bench, CRF board president. “In cooperation with Choroideremia Research Foundation Canada (CRFC), we continue to push the boundaries of our knowledge by supporting this innovative group of researchers.”

CHM is an X-linked inherited retinal degeneration, caused which manifests in progressive nyctalopia and peripheral vision loss. While males are primarily affected, female carriers of CHM may also present with variable retinal characteristics depending on expression of the CHM gene. To date, the CRF has funded approximately $5 million in research studies during its 23-year history.

For more information about all research studies the CRF supports, please visit curechm.org/research/

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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

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 $5 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 mouse 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 curechm.org

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