CHOROIDEREMIA RESEARCH FOUNDATION EXPANDS SCIENTIFIC RESEARCH OF INHERITED RETINAL DISEASE WITH ITS LATEST GRANT AWARDS

Studies will explore new research areas that may provide more opportunities to accelerate progress toward a treatment or cure


Luise de Lemos, PhD, Champalimaud Foundation

SPRINGFIELD, MA, UNITED STATES, November 28, 2023 / EINPresswire.com/ -- The Choroideremia Research Foundation (CRF) is pleased to announce its latest scientific research grants, supporting its mission to find a treat or cure for choroideremia (CHM). Award recipients are as follows:

Tomas S. Aleman, MD, University of Pennsylvania

STUDY: Gene Therapy for Choroideremia: Redefining Cellular Targets and Treatment Windows

AIM: To provide a new background to justify a clinical trial for CHM that will target specific windows in the degeneration process using improved vectors to target both the retinal pigmented epithelium (RPE) as well as photoreceptors.

Specific Aims:
1. To analyze the short-term natural history of the early photoreceptor specific dysfunction (rods vs cones) in CHM. The evaluations will stem from cross-sectional and longitudinal evaluations already performed on hundreds of CHM patients in researcher's database.
2. To establish the short-term relationships between the structural abnormalities determined with spectral domain optical coherence tomography (SD-OCT) and RPE abnormalities as assessed with fundus autofluorescence (FAF) and the colocalized retinal dysfunction.

3. To define new photoreceptor specific outcome measures mostly based on rod vision and in early disease stages to establish new therapeutic windows and eligibility for treatment focused on early stages of rod degeneration.

GRANT: $100,000

Luisa de Lemos, PhD with the Champalimaud Foundation has received the 2023 Randy Wheelock Award to recognize emerging scientists and research professionals working on CHM or related vision loss issues.

STUDY Pathways of Retinal Pigment Epithelium Degeneration in Choroideremia


Toma S. Aleman, MD, University of Pennsylvania

AIM: Gene replacement depends on the cell's survival into which functional genes are delivered. It will not be therapeutic beneficial once extensive degenerative events have occurred in the diseased retina. Therefore, complementary therapies are of extreme importance.

Specific aims:

1. To identify the most relevant pathways or potential biomarkers involved in the degenerative mechanism that occurs in RPE in the context of CHM disease.
2. To validate the relevant key targets and molecular pathways involved in RPE degeneration in a CHM mouse model.

GRANT: $50,000

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 about all research studies the CRF supports, please visit

curechm.org/research

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 $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 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 curechm.org

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