CHOROIDEREMIA RESEARCH FOUNDATION ACCELERATES SCIENTIFIC RESEARCH OF RARE INHERITED RETINAL DISEASE AWARDING TWO GRANTS

SPRINGFIELD, MA, UNITED STATES, September 7, 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:


Bhanu P. Telugu, PhD

Mariya Moosajee, MBBS, BsC (Hons), PhD, FRCOphth, consultant ophthalmic surgeon and clinical academic ophthalmologist with Moorefield Eye Hospital, University College, London

STUDY: An investigator-led pilot study for the use of ataluren to treat nonsense-mediated choroideremia

AIM: To support a clinical research fellow for 12 months initially to work directly with the PI and Co-PI to set up the phase 1/2 clinical trial of ataluren for CHM. and acquire the necessary approvals to open the site for recruitment.

- Objective 1. Obtain ethics and regulatory approval for the new site.
- Objective 2. Identify patients with suitable nonsense mutations and establish baseline clinical and molecular prognostic features such as level of CHM transcripts and nonsense-mediated mRND decay (NMD) activity determined from blood samples.

When objectives are achieved, the next phase is to conduct a phase 1/2 clinical trial of ataluren for 10 CHM patients with nonsense mutations over four years.

GRANT: $40,600 (pending objectives achieved, another $40,600 may be award to fund the

clinical trial)

Bhanu P. Telugu, PhD, associate professor in the Division of Animal Sciences at the University of Missouri in Columbia, and the founder, President and Chief Scientific Officer of RenOVAte Biosciences, Inc., a Maryland based start-up company

STUDY: A Novel Murine Pre-Clinical Model for Choroideremia Research

AIM: To generate and characterize fetal specific knockout of CHM.

Mariya Moosajee, MBBS, BsC (Hons), PhD, FRCOphth

CHM is ubiquitously expressed in many tissues (>25 tissues; source: NCBI) including the placenta, and constitutive or global knockout of CHM results in embryonic lethality in mice. Contrary to humans and pigs, maternal specific imprinted x-inactivation takes place in mice, resulting in placental insufficiency and embryonic lethality.

Using proprietary technology, the research team endeavors to generate a fetal specific conditional knockout CHM gene sparing the placenta.

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 and sign-up for our newsletter on the latest research news at curechm.org/get-involved

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About Choroideremia
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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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