CHOROIDEREMIA RESEARCH FOUNDATION EXPANDS SCIENTIFIC RESEARCH OF RARE INHERITED RETINAL DISEASE AWARDING THREE GRANTS

SPRINGFIELD, MA, UNITED STATES, April 16, 2024 /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:

Robert James Casson, MB, MS Hons, Professor of Ophthalmology, University of Adelaide, and Kiora Pharmaceuticals

STUDY: Validation of functional vision testing in a population of patients with retinitis pigmentosa & choroideremia (ABACUS-1)

AIM: To test the hypothesis that intravitreal injection of KIO-301 can be safely administered and may restore light responsive neural transduction to the brain in late-stage CHM patients by activating retinal ganglion cells no longer connected to viable photoreceptors. As per guidance received by the FDA during a Type B (Pre-IND) meeting, to prove clinically meaningful efficacy in a clinical trial, the use of a validated functional vision test(s) will be required.

- Objective 1: To validate the use of four

Bhanu P. Telugu, PhD

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

functional vision tests to enable an accurate and reliable assessment of efficacy in participants with late-stage retinal dystrophies (choroideremia + RP).

• Objective 2: Conduct a Type-D meeting with the US FDA to obtain written feedback regarding several planned functional vision test design, methodology, plan validation and data analysis.

GRANT: $20,000

Mariya Moosajee, MBBS, BsC (Hons), PhD, FRCOphth, Consultant Ophthalmic Surgeon and Clinical Academic Ophthalmologist, Moorfields Eye Hospital, University College, London, United Kingdom

STUDY: Further development of CHM nonsense mutation readthrough compounds and maintenance of the CHM mouse colony

Robert Casson, Robert James Casson, MB, MS Hons

AIM: To support a clinical research fellow for 12 months to maintain, feed, and breed the mice colony to continue to supply CHM researchers and industry partners for their research studies.

GRANT: $25,000

Bhanu P. Telugu, PhD, Associate Professor in the Division of Animal Sciences at the University of Missouri, President and Chief Scientific Officer of RenOVAte Biosciences, Inc.

STUDY A Novel Porcine Pre-Clinical Model for Choroideremia Research (year 3)

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

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: $30,000

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/

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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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