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Foundation Fighting Blindness Clinical Research Institute to Launch ProgStar Natural History Study for Stargardt Disease to Help Prepare for Future Clinical Trials

Nine Sites in the U.S. and Abroad Will Evaluate Patients

Columbia, MD (March 13, 2013) – The Foundation Fighting Blindness Clinical Research Institute is launching a natural history study of people affected by Stargardt disease, a genetic retinal condition robbing the central vision of approximately 30,000 people in the United States. Known as ProgStar (The Natural History of the Progression of Atrophy Secondary to Stargardt Disease: A Longitudinal Observational Study), the study has three primary goals: 1) determine the best outcome measures to accelerate evaluation of emerging treatments; 2) better understand disease progression for selecting future clinical trial participants; and 3) identify potential participants for forthcoming clinical trials.

The study, taking place in multiple locations worldwide, will combine both prospective and retrospective analyses. The prospective portion, for which physicians will track the progression of disease in participants, will last two years. Physicians will also retrospectively analyze disease progression by reviewing participants' past medical records.

"Stargardt disease has a complex effect on the retina," says Dr. Hendrik Scholl, of the Wilmer Eye Institute, Johns Hopkins Hospital, ProgStar's protocol principal investigator and study director. "And the rate of disease progression can vary between people and is difficult to measure. These issues can make selection of clinical trial outcome measures and participants challenging. But ProgStar will help us design better human studies of potential treatments and increase our chances of success."

"We are pleased there are gene therapy and cell-based clinical trials currently underway for Stargardt disease, but an existing obstacle for advancing promising treatments to market relies on accepted clinical endpoints for human studies," says Patricia Zilliox, Ph.D., chief drug development officer of the Foundation Fighting Blindness. "By collecting and analyzing this natural history study data from hundreds of participants, we are putting ourselves in a better position to move laboratory research into the clinic and out to Stargardt disease patients hoping for treatments and cures."

During the study, researchers will evaluate a number of outcome measures, including retinal images, visual fields and visual acuity. ProgStar is preparing to enroll its first participants in the spring or summer of 2013, pending regulatory approval to proceed. The study's nine clinician-scientists will recruit as many as 250 total participants from their existing patient bases and no additional recruitment will be conducted. And, the study will not involve administration of potential therapies.

Principal investigators for the study are:

- Dr. Paul Bernstein, University of Utah (Salt Lake City, Utah)
- Dr. David Birch, Retina Foundation of the Southwest (Dallas, Texas)
- Dr. Artur Cideciyan, University of Pennsylvania (Philadelphia, Pennsylvania)
- Dr. Michel Michaelides, Moorfields Eye Hospital (London, United Kingdom)
- Dr. José Sahel, Institut de la Vision (Paris, France)
- Dr. Hendrik Scholl, Johns Hopkins Hospital (Baltimore, Maryland)
- Dr. Janet Sunness, Greater Baltimore Medical Center (Baltimore, Maryland)
- Dr. Elias Traboulsi, Cole Eye Institute at Cleveland Clinic (Cleveland, Ohio)
- Dr. Eberhart Zrenner, University of Tübingen (Tübingen, Germany)

(MORE)

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Participating in study design are Dr. Gerald Fishman, of the Chicago Lighthouse for People Who are Blind or Visually Impaired, Drs. Frederick Ferris and Brian Brooks, of the National Eye Institute, Dr. Johanna Seddon, of Tufts Medical Center, and Dr. Richard Weleber, of Oregon Health & Science University. Dr. Srinivas Sadda, of the Doheny Eye Institute, University of Southern California, will be analyzing imaging and visual field data collected from the study centers and Dr. Sheila West, of the Dana Center, Wilmer Eye Institute, Johns Hopkins University, will coordinate statistical analyses and data monitoring.

About the Foundation Fighting Blindness Clinical Research Institute

The Foundation Fighting Blindness Clinical Research Institute works to accelerate the translation of laboratory-based research into clinical trials for treatments and cures of retinal degenerative diseases. As a support organization and the translational arm of the Foundation Fighting Blindness, it aims to develop a bridge between scientific, clinical, governmental, pharmaceutical and financial communities to advance clinical trials of sight-saving treatments and therapies. The FFB Clinical Research Institute invests funds to support Phase I and Phase II clinical trials to expedite the commercialization of treatments for conditions such as retinitis pigmentosa, macular degeneration, Usher syndrome and the entire spectrum of retinal diseases, and also provides assistance to the management of clinical trials and subsequent commercialization.

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