Choroideremia RESEARCH FOUNDATION

For Immediate Release

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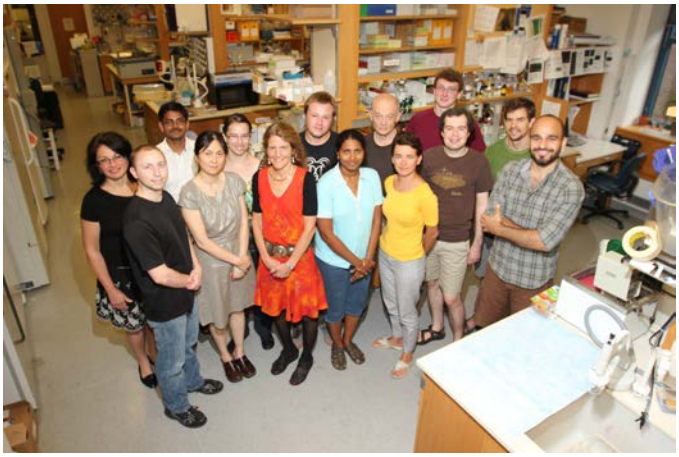
Groundbreaking Gene Therapy Trial Begins in Philadelphia

Philadelphia, PA, January 20, 2015. When doctors told Jeff Benelli he was going blind and would "Never see a treatment" in his lifetime for the rare condition stealing his vision, Jeff refused to give up - and today his stubborn determination is paying off. Today Spark Therapeutics announced the start of the first U.S. Human Clinical Trials to treat Choroideremia ("CHM"), an inherited and currently incurable genetic condition that progressively leads to blindness by damaging the retina of impacted individuals. Were it not for the persistent efforts of a determined few in the patient community, like Jeff, this day might never have come.

Jeff Benelli

"Spark's groundbreaking announcement today brings real hope of a treatment for blindness caused by Choroideremia, and further paves the way for treatments of other retinal diseases impacting people around the world," said Dr. Chris Moen, President of the Choroideremia Research Foundation (curechm.org), the leading advocacy and fundraising organization focused on finding a treatment for CHM. "The Choroideremia Research Foundation is proud to have provided key preclinical funding to Jean Bennett, MD, PhD and her team at the Perelman School of Medicine at the University of Pennsylvania, that has helped bring us to the gene therapy human clinical trials being announced today."


Dr. Jean Bennett and Lab team at Perelman School of Medicine, University of Pennsylvania

Jeff Benelli crossed the finish line of the Philadelphia Marathon less than two months ago as a blind athlete, having now lost all but a sliver of his vision to CHM. It was his fourth marathon in only five weeks as he raised funds and awareness for CHM, and it was a powerful metaphor for Jeff's journey to this day with the marathon finish line being only two miles from where today's announced CHM treatment will begin clinical trials. Joining Jeff in running the full Philly Marathon were a dozen others racing for CHM research, including Danny & Sharyl Boren. On November 16th, just seven days before the Philly Marathon, Danny – a legally blind athlete – had placed 2nd in the Physically Challenged division of the Arizona Ironman Triathlon – a renowned endurance event covering 140.6 miles of swimming, biking, and

running in one grueling day. Together with Jeff and Danny, Team CHM raised over $100,000 to accelerate the pursuit of a cure for Choroideremia at the Philadelphia Marathon and Ironman Arizona.

Danny Boren

"The Choroideremia Research Foundation (CRF) has truly been instrumental in bringing about this study," said Jeffrey D. Marrazzo, co-founder and CEO of Spark Therapeutics. "While the CHM community is small – the disease affects approximately only 1 in 50,000 individuals – they have had a tremendous impact on the pace of research. We continue to be inspired by their efforts to raise awareness of, and develop new treatments for, their disease and consider the CRF and the CHM community critical partners in our success."

Prior to the formation of the Choroideremia Research Foundation in 2000 there was very little hope that the progressive vision loss resulting from CHM could be halted, yet now only 14 years after the group began,a treatment for CHM is entering US clinical trials for the first time. The experimental treatment announced today delivers a corrected gene to the retina of affected individuals through the use of a modified virus. The corrected gene is designed to replace the genetic defect that causes CHM, and it is hoped this correction will stop further loss of vision. While CHM is one of the first conditions to be treated using gene therapy, it is believed this form of treatment could eventually be used to treat a wide range of genetic diseases.

For Jeff Benelli it is a moment he was told would never come, but one he never stopped believing in. "This has been the ultimate underdog story," says Jeff; "when we were told no one was working on a cure, and there was nothing that could be done to save our vision, we set out to change that. Thanks to brilliant researchers like Dr. Bennett, and the folks at Spark Therapeutics, we can finally see the finish line in our race to end blindness caused by CHM."

For more information on Choroideremia and the Choroideremia Research Foundation, visit http://www.curechm.org/

About Choroideremia:
Choroideremia (CHM) is a rare inherited disorder that leads to blindness. CHM is caused by a specific genetic defect that causes a degeneration of retinal cells at the back of the eye that are essential to sight. CHM is genetically passed through families by an X–chromosome linked genetic defect. Because of this, except in rare cases, only males have loss of sight. A father will not pass the gene to his sons, but his daughters will be carriers. Mothers can be carriers and have a 50 percent chance of passing the disorder to their sons, who will suffer sight loss, or to their daughters who will be carriers. While CHM's progress varies among individuals, loss of night vision typically occurs in childhood, loss of ability to drive typically occurs in the twenties, progressive vision loss eventually results in total blindness by the fifties or sixties.

About the Choroideremia Research Foundation (CRF):
The Choroideremia Research Foundation is a registered 501(c)3 tax-exempt nonprofit organization created to raise funds in support of scientific research leading to a treatment or cure of Choroideremia. In addition to providing critical funding to leading researchers the CRF provides support through its community of families and individuals who are impacted by CHM, and helps to raise awareness about CHM among medical professionals and the public at large.

About Spark Therapeutics:

Spark is a gene therapy leader seeking to transform the lives of patients suffering from debilitating genetic diseases by developing one-time, life-altering treatments. Spark's initial focus is on treating orphan diseases where no, or only palliative therapies, exist. Spark's most advanced product candidate, *SPK-RPE65*, is in a fully-enrolled pivotal Phase 3 clinical trial for the treatment of rare blinding conditions due to mutations in the RPE65 gene. Spark is leveraging the experience and technology utilized in the development of *SPK-RPE65* to address a broad spectrum of blinding conditions, starting with the development of *SPK-CHM* for the potential treatment of choroideremia, for which it is currently enrolling patients in a Phase 1/2 clinical trial. Spark is also establishing a pipeline of gene therapy candidates to treat hematologic and neurodegenerative disorders, including through a global collaboration with Pfizer Inc. around the development and commercialization of *SPK-FIX* for the treatment of hemophilia B. Spark's integrated gene therapy platform builds on two decades of research, development and manufacturing at The Children's Hospital of Philadelphia, including human trials conducted across diverse therapeutic areas and routes of administration. To learn more, visit www.sparktx.com.

About CRF-Sponsored Research:
Dr. Jean Bennett is a professor at the F.M. Kirby Center for Molecular Ophthalmology at the University of Pennsylvania and is the lead researcher in Spark Therapeutics' CHM gene therapy clinical trial. Her pioneering gene therapy work uses an adeno-associated virus encoded with the healthy CHM gene to introduce the healthy gene into affected patients, thereby stopping the progressionof the disease. In addition to gene therapy research the CRF is also funding Pharmaceutical Therapies and Sight Replacement Therapies targeting Choroideremia, and is creating a CHM BioBank that will provide greatly improved access for researchers from around the Globe to study CHM.