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CLINICAL TRIAL SUCCESS BRINGS RESEARCHERS CLOSER TO A CURE FOR BLINDNESS

Philadelphia, PA (November 2011)--Researchers at Hadassah Hospital in Israel, led by Dr. Eyal Banin, have completed a clinical trial that tested the use of gene therapy to restore sight to patients suffering from Leber's Congenital Amaurosis (LCA). Dana and Yossi, two participants in this study, suffer from Leber's Congenital Amaurosis (LCA), the most severe form of all inherited retinal dystrophies causing congenital blindness. Like others affected, they have experienced severe visual impairment since birth. LCA sufferers experience poor night vision, low visual acuity and a constricted visual field. This low vision continues to deteriorate, leading to total blindness. Other symptoms may include crossed eyes, roving eye movements, unusual sensitivity to light, and/or cataracts. LCA is usually inherited as an autosomal recessive genetic condition. Those with LCA suffer in darkness, without sight and without hope. Until now.

Dr. Eyal Banin, MD, PhD at the Center for Retinal and Macular Degeneration at Hadassah University Medical Center, in collaboration with leading researchers in the United States and Great Britain, performed a clinical trial that has successfully demonstrated the efficacy of gene therapy in the treatment of LCA. LCA is caused by a mutation in the RPE65 gene. In this clinical trial, a normal RPE65 gene was injected into the retina to replace the damaged gene and renew protein production. Participants Dana and Yossi were treated with this gene therapy in just part of the retina of one eye, with dramatic results. Shortly after treatment, both participants noted a substantial improvement in their vision.

When asked about the results of this treatment, Yossi said, "I felt the real change, the real revolution, after 21 days. It was amazing because today I see things that I have never seen before. I'm very proud to be a part of this research." Dana said, "Learning of new treatment was a life-changing event. I'm experiencing a real change. I was surprised to see real improvement in my vision."

Yossi and Dana's self-reporting of visual improvement is corroborated by objective, quantitative measurements of the treated area that also show significant improvement. With the continuation of this research, these scientists will be able to develop gene therapy to treat additional retinal degeneration diseases and make it possible to treat many more patients. To watch a just-released video with more information about this clinical trial, its researchers and study participants, please visit <http://www.mvrf.org/news.php>.

Dr. Banin says: "You cannot imagine what an effect this has had not only on the treated patients, their families and on us, but also on the wider population of patients with retinal and macular degenerations here in Israel, who suddenly feel some glimmer of hope. Without funding from Macula Vision Research Foundation (MVRF), we could not have conducted this clinical trial. We feel very fortunate to have MVRF as our partners in this very exciting journey."

Keith A. Lampman, Executive Director of MVRF, says "We are extremely excited about the results of this study and feel confident that, in close collaboration with our partners across the globe, we are closer than ever to a cure for retinal diseases. MVRF is proud to be supporting the important work of Dr. Banin and others, and look forward to continuing success in the future."

MVRF provides funding to leading researchers around the world who are working to find a cure for all retinal diseases, including LCA, Macular Degeneration, Stargardt's Disease, and others. This Hadassah study was made possible solely through grants from Macula Vision Research Foundation (www.MVRF.org).

Since the inception of MVRF more than a decade ago, the foundation has provided \$15.4 million in research grants to leading international scientists, \$2.3 million of which was granted in 2011 alone. Lampman says, "What makes MVRF especially unique is that 100% of every donation goes directly to research for a cure. All administrative and fund raising costs are paid by a private family foundation, so every cent of every dollar goes directly to the physicians, scientists, and researchers working to find a cure."