

The Usher Challenge – New \$10 Million Goal

Kimberling Usher Research Laboratory

Institute for Vision Research, University of Iowa

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Just 18 months ago, the Institute for Vision Research at the University of Iowa announced a philanthropic matching campaign designed to raise \$4 million in 4 years to develop gene and stem cell treatments for all genetic types of Usher Syndrome. Within two weeks of that announcement, another major donor joined the effort allowing us to increase the 4 year goal to \$6 million. In the past few months, additional major donors have joined the “challenge side” of the campaign which will now allow us to match every gift for Usher Syndrome Research, dollar for dollar, until \$10 million is raised.

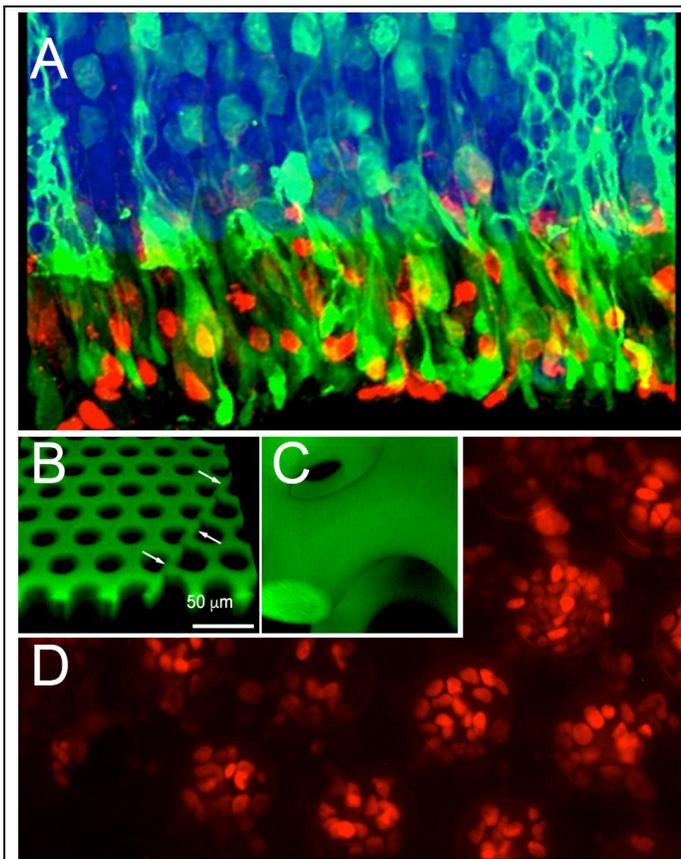
The millions of dollars that have already been donated and matched in the first 18 months of the campaign have helped us:

- Devise a culture system for human donor retina to allow novel viral vectors to be developed for gene therapy of large Usher genes.
- Double the size of our gene therapy manufacturing facilities to allow multiple new treatments per year to be manufactured in a nonprofit setting.
- Develop a modular robot-assisted stem cell reprogramming and differentiation facility to speed our development of patient-derived polymer supported photoreceptor grafts.
- Continue Project Usher, a philanthropically supported program that allows individuals with Usher syndrome who cannot afford commercial genetic testing to obtain a state of the art genetic test. Physicians interested in ordering a test through Project Usher can learn more at www.projectusher.org.
- Embark upon a natural history study of all forms of Usher syndrome. We already have data in hand from more than 250 patients with disease-causing genotypes in one of these genes (130 patients with *USH2A* alone).



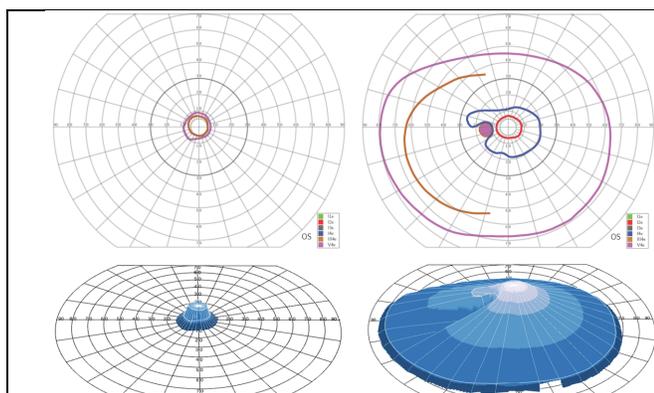
Budd Tucker and Ed Stone stand beside the world’s largest robotically-assisted modular Biospherix tissue culture installation wearing the IVR team jerseys for the upcoming “Great Bike Ride Across Iowa” (<https://ragbrai.com>). The IVR has put together a team of scientists and supporters from across the United States to ride in this summer’s event to raise awareness and support for our grass-roots-funded effort to cure all forms of inherited retinal disease.

You can follow a daily blog of our ride on Facebook beginning on Sunday July 22.



Panel A: human donor retina used to facilitate development of large capacity vectors for Usher Syndrome gene therapy.

Panels B-D: Loading of biodegradable, immunologically compatible, cell delivery scaffolds with OTX2 positive photoreceptor precursor cells (red) for autologous photoreceptor cell replacement and treatment of retinitis pigmentosa.



Goldmann visual fields of the left eyes of two patients from our Usher Syndrome natural history study. The patient on the left has very advanced USH1F while the patient on the right was recently diagnosed with the same disease. The upper panels show the conventional 2D depiction of the visual fields while the lower panels reveal the conversion of these data to 3D volumes, which are more intuitively reflective of the patient's total visual function.

All funds raised through the IVR Usher Challenge go directly to the laboratories and clinics pursuing the cures for Usher Syndrome. Philanthropic support allows us to freely share everything we do. Every aspect of our work from the blueprints of our manufacturing facility to the composition of our tissue culture medium is non-proprietary and freely available to any institution that might be interested in joining the effort to get more therapies for inherited eye disease into clinical trial as quickly and inexpensively as possible.

To make a gift online, please visit: www.givetoioowa.org/usherchallenge.

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