LEGACY

SPRING/SUMMER 2024

A Publication for Supporters of the FOUNDATION FIGHTING BLINDNESS

A Vision of Hope and Light: Fai and Patrick's Journey with the Foundation Fighting Blindness

In the heart of Arizona, Fai Mo and Patrick McKelvey have charted a path marked not just by challenges but by immense hope and unwavering commitment. Meeting 24 years ago, they embarked on a journey together, one that would soon include an unexpected companion: vision loss. Fai's diagnosis of retinitis pigmentosa (RP) was a turning point that became the catalyst for a profound connection with the Foundation Fighting Blindness, a beacon of hope for many navigating similar paths.

The moment Fai discovered the Foundation was as serendipitous as it was life-changing. A poster for a VisionWalk caught his eye during a routine visit to his retinal specialist, sparking curiosity and leading him to his first VisionWalk in Arizona. Attending the Arizona VisionWalk was a transformative experience as they discovered the Foundation wasn't just leading the charge in groundbreaking research; it was creating a community, a family united by shared experiences and a collective vision for a future without blindness.

Inspired by the optimism and resilience of those he met, Fai soon found his calling by volunteering with the Arizona chapter as president and organizing events like VisionWalk and Dining in the Dark. His involvement with the chapter became more than an act of support; it became an affirmation of his and Patrick's commitment to the cause. It was here, amidst this vibrant community, that the decision to include the Foundation in their legacy plan crystallized.

The Foundation Fighting Blindness stands at the forefront of over 45 clinical trials, each a step closer to treatments and cures for blinding diseases. Fai and Patrick's legacy gift is a testament to their belief in the Foundation's mission and its capacity to change lives. Their story is a powerful reminder that legacy giving is an opportunity for anyone, at any age, to make a significant impact.

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Above: Patrick (left) and Fai (right) outside with greenery and water behind them.

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LEGACY GIFT

All About Beneficiary Designations – The Easiest Way to Make a Legacy Gift!

Did you know a beneficiary designation is among the simplest of all charitable gifts?

It's true! It's as easy as filling out a form. You specify the individuals and/or charities you want to support and determine the percentage of the assets you want each beneficiary to receive.

You can help find treatments and cures for blinding retinal diseases with your designation to the **Foundation Fighting Blindness** (Tax ID: 23-7135845).

This can be accomplished in three easy steps:

✓ STEP 1: DETERMINE WHICH ASSET

- Retirement Account*
- Brokerage Account
- Bank Account
- Trust
- Life Insurance

**For tax reasons, a retirement account is often the best charitable option.*

✓ STEP 2: REQUEST A FORM FROM YOUR MANAGING INSTITUTION

- Many institutions have online forms available.

✓ STEP 3: DETERMINE A PERCENTAGE OR AMOUNT YOU'D LIKE TO LEAVE TO THE FOUNDATION FIGHTING BLINDNESS

- Any amount is meaningful and appreciated!

Once you've completed this quick and easy process, be sure to let us know at Legacy@FightingBlindness.org or by filling out a form on our website at **www.FightingBlindness.org/LegacyForm** or use the QR code on your mobile device.

Making your wishes known helps us plan ahead and continue to fund the best possible research toward treatments and cures. If you've included the Foundation as a beneficiary, we'd love to recognize your generosity! And if you choose, we will welcome you to the Foundation's Legacy Society.

Cover Story

CONTINUED FROM FRONT COVER

"Legacy giving is about carrying the torch for future generations," Fai shares. "It's about ensuring that one day, we can all celebrate victories over vision loss together."

This sentiment echoes through the Foundation's community, a diverse yet united group, all connected by the shared dream of finding cures.

Fai and Patrick's decision to include the Foundation in their estate planning is a beacon of hope, illuminating the path for others to follow. It's an invitation to join a movement that transcends individual experiences, uniting us in the pursuit of a brighter, clearer future.

For those inspired by Fai and Patrick's story, considering legacy giving as part of your estate planning is a powerful way to contribute to this ongoing journey. Regardless of your age or stage in life, your support can lay the groundwork for breakthroughs that will one day transform the landscape of blinding diseases. Together, we can ensure that future generations will not only dream of a world without blindness but will one day live in it.

VISIONS 2024

FOUNDATION FIGHTING BLINDNESS

VISIONS '24

JOIN US AT VISIONS 2024!

Friday, June 21–Saturday, June 22, 2024

Chicago Marriott Downtown Magnificent Mile
540 N. Michigan Avenue, Chicago, IL 60611

VISIONS 2024, the global conference of the Foundation Fighting Blindness, on June 21–22, 2024, provides an excellent opportunity to connect with others from the blind and low vision community and learn about the latest research advancements, products, and services for members of our community. Register today by visiting: **www.FightingBlindness.org/VISIONS2024**

Beacon Therapeutics Reports Encouraging Interim Results from Phase 2 Clinical Trial for XLRP Gene Therapy

Beacon Therapeutics has reported vision improvements for five of eight patients receiving the high dose of its X-linked retinitis pigmentosa (XLRP) gene therapy in the Phase 2 SKYLINE clinical trial. Known as AGTC-501, the emerging gene therapy is for patients with mutations in RPGR, the gene most frequently associated with XLRP.

Vision improvements were measured using microperimetry, a test that measures light sensitivity at several loci (points) in the central retina. The test also captures retinal images. The five of eight patients responding to the high dose of AGTC-501 had improvements in retinal sensitivity of at least 7 decibels in 5 or more loci. Responses of the six patients receiving the low dose of the therapy were similar to untreated eyes in the high dose group.

AGTC-501 was well-tolerated, and no clinically significant safety events were associated with treatment.

Beacon plans to launch its Phase 2/3 VISTA clinical trial for AGTC-501 during the first half of 2024.

The Foundation Fighting Blindness funded successful canine studies of XLRP gene therapy at the University of Pennsylvania School of Veterinary Medicine that helped make the XLRP gene therapy clinical trial possible.

Opus Reports Vision Improvements for Patients in LCA5 Gene Therapy Clinical Trial

Opus Genetics has announced vision improvements for the first three adult patients in its Phase 1/2 gene therapy clinical trial for Leber congenital amaurosis 5 (LCA5), a rare, severe, inherited retinal disease. Some of the patients, who had been almost totally blind since birth, can now see and identify objects for the first time. The company has also reported positive safety data for the trial thus far.

Opus plans to administer the next highest dose of its LCA5 gene therapy to the next cohort of adult patients in mid-2024. The company also has plans sometime in the future to dose patients as young as 13 years of age.

“We are delighted to see some vision bestowed to LCA5 patients, enabling them to see objects they’ve never seen before,” says Rusty Kelley, PhD, managing director of the RD Fund, the Foundation’s venture philanthropy arm. “While the trial is still at an early stage, the preliminary

data is encouraging for patients and Opus, which is achieving its goal of developing effective gene therapies for inherited retinal diseases which aren't being addressed by other companies."

The LCA5 gene therapy clinical trial is the first launched by Opus, a company founded in 2021 by the RD Fund, as a majority shareholder in a $19M Series Seed financing to support emerging retinal disease therapies in or nearing early-stage clinical trials. The company is led by Ben Yerxa, PhD, former chief executive officer of the Foundation.

Ascidian to Launch Clinical Trial for Stargardt Disease RNA Editing Therapy

Ascidian Therapeutics has received authorization from the U.S. Food & Drug Administration to launch a Phase 1/2 clinical trial for ACDN-01, the company's RNA editing therapy for people with Stargardt disease, an inherited form of macular degeneration caused by mutations in the ABCA4 gene. The company plans to begin enrollment for the clinical trial, known as STELLAR, during the first half of 2024.

Unlike genetic therapies that deliver an entire healthy gene (DNA) to replace the mutated gene, or which edit DNA, ACDN-01 re-writes RNA, the genetic message derived from DNA that cells read to make proteins. Healthy proteins are essential to the survival and function of all cells in the body, including those of the retina. ACDN-01 specifically re-writes RNA exons, the regions where mutations are most likely to occur for the ABCA4 gene. Ascidian estimates that ACDN-01 can address mutations in ABCA4 for a significant percentage of people with Stargardt disease because the editing replaces a large number of exons all at once.

jCyte Announces Plans to Launch Phase 3 Clinical Trial of Cell Therapy for RP

The biotechnology company is preparing to launch a pivotal, Phase 3 clinical trial in the U.S. for its emerging jCell therapy. The company's announcement came after a successful planning meeting with the U.S. Food & Drug Administration. Participant enrollment for the Phase 3 trial is expected to begin during the second half of 2024. More details of the trial are forthcoming.

jCells are similar to stem cells that haven't yet fully developed into mature photoreceptors. The cells are injected into the vitreous, the soft, gel-like substance in the middle of the eye. Intravitreal injections have a good record of safety and are commonly administered for other conditions in a doctor's office. jCells are designed to release proteins known as neurotrophic factors to preserve photoreceptors regardless of the mutated gene causing vision loss.

Previously, the company reported on 76 patients in the Phase 2b trial for jCells. In the Phase 2b trial, 39 percent of patients receiving 6 million cells, the high dose of the treatment, had improvement at 12 months post-treatment in best corrected visual acuity (BCVA) of 10 letters (two lines on an eye chart) or more, with 26 percent improving by at least 15 letters.

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This and previous issues of **Legacy** are available online, where you can get the latest retinal research information, as well as updates on the Foundation's activities, on your PC and mobile devices.

For an online and accessible version of **Legacy**, visit **www.FightingBlindness.org/Legacy-Newletter**

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