

## **What Does FFB's Statement of Financial Position Mean?**

First and foremost, it means the Foundation Fighting Blindness (FFB) is in a strong position to continue to fund the most promising research on potential cures for retinal degenerative diseases and to help bring groundbreaking discoveries to clinical trials and the U.S. Food and Drug Administration (FDA) approval process.

Our Research Oversight Committee has evaluated the potential of current retinal degenerative disease research and related science in gene therapy, stem-cell transplantation, and emerging developments in optogenetics and neuroscience. This committee of expert scientists formulated a strategic plan for the allocation of FFB's research funding from its existing assets and future fundraising. That plan calls for FFB's investment of \$20 million per year for basic discovery research, translational research, and proof-of-concept research — the preclinical investigative work that will lead to FDA-approved clinical trials and, ultimately, FDA-approved new treatments.

Translational research is the preclinical work that "translates" laboratory discoveries to establish the potential benefit of a new therapy to patients. This research bridges "bench to bedside" and includes the full gamut of preclinical work that has to be accomplished successfully before testing in humans will be approved by the FDA. If this translational work is successful, human studies must then be done to demonstrate safety and then proof of concept, i.e., treatment efficacy, in humans.

It is estimated that preclinical studies can cost upwards of \$1.5 – \$2 million per project. What's more, the costs associated with clinical testing are much higher than they are to fund the initial discovery phase in the laboratory. For orphan or small market diseases, including retinal diseases, the financial risk of attempting to discover and bring new treatments to market must be reduced before venture capitalists and then biotech and pharmaceutical companies, the only companies that can meet such high costs, are willing to invest in emerging treatments. It is here that the FFB provides a critical bridge between the laboratory and the marketplace by funding the preliminary research and testing on potential therapies.

In summary, a strong financial base is essential to be able to support the laboratory and translational science as well as the preclinical work that will determine the efficacy and safety of new therapies. The FFB will continue to drive the research that leads to clinical trials and must have the funds to do so. Assets held and invested plus additional assets that have resulted from the success of the Gordon and Llura Gund Family Challenge have resulted in over \$128 million being available to support these long-term, milestone-based projects and meet the Research Oversight Committee spending plan targets for the next five years, as described above.

Looking ahead, current and future fundraising is essential to sustain the momentum FFB-funded projects have created. Additional assets provide flexibility to both respond quickly to innovative, large-scale opportunities as they arise and serve as a safety net, i.e., a reserve fund, in the case of philanthropic shortfalls or market downturns.