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February 27, 2019
The Honorable Joe Courtney
2332 Rayburn HOB
Washington, DC 20515

Dear Representative Courtney,

We write today to voice our support for The Faster Treatments and Cures for Eye Diseases Act, H.R. 6421. We join Foundation Fighting Blindness (FFB), the Association for Research in Vision and Ophthalmology, Choroideremia Research Foundation, Usher Syndrome Coalition, Blinded Veterans Association and the National Alliance for Eye and Vision Research.

Sofia Sees Hope has worked for more than five years to advocate for the rare inherited retinal disease (IRD) community, specifically those with Leber congenital amaurosis (LCA). In addition to advocacy and education, we fund research into treatments for blindness caused by IRDs, and provide free access to genetic testing for patient families. To date we have donated more than \$275,000 in total.

This is a time of great hope and excitement in our community, as 2018 saw the first genetic therapy being given to LCA patients with the RPE65 genetic mutation. Spark Therapeutics received FDA approval for its drug, LUXTURN[™], in 2017. Sofia Sees Hope President Laura Manfre testified at the FDA committee review hearing for the drug.

Currently, 23 gene-based clinical trials targeting 13 genes are underway. More than 20 retinal cell therapy trials are in progress, and another 100 genes are under investigation in the preclinical pipeline. But research and development is costly, and the patient pool is small.

The Faster Treatments and Cures for Eye Diseases Act would create a pilot program to facilitate funding by private investors for translational research and advance treatments and cures for blindness and severe vision impairment. Eye Bonds, a key part of the legislation, would finance packages of loans to projects at small labs, universities, and other centers that right now can't

secure needed funding. Repayment under the program goes first to reduce the taxpayers' guarantee before any funds are paid out to private investors.

America lags other countries in funding translational research. The U.S. is currently devoting less than \$750 million a year to translational research efforts, and the majority of this funding does not go toward translational research for specific diseases but instead toward research addressing commonalities underlying all areas of medicine.

Seventeen of your colleagues in the House have signed on as co-sponsors along with the original three congressmen who introduced the bill last July. There appears to be strong bipartisan support, a welcome sign in these current troubled times.

Sofia Sees Hope would ask that you add your name to the list in support of this bill. We welcome any questions you or your staff may have.

Sincerely,

Laura Manfre
Board Chairman and Co-founder
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