

PIPC Patient Blog: Seeing the Promise in Gene Therapy

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By: Janni Lehrer-Stein

A 10-year-old girl [sees](#) her mother's face for the first time. An 11-year-old boy [races](#) through the aisles of Target, marveling at toys he never knew existed. Both children had mere glimmers of vision, and were destined to lose even that because of an inherited eye disease with no treatment or cure. But thanks to the miracles of modern-day medicine, what was once only possible in science fiction novels is now becoming reality.

In December, the FDA approved the first gene therapy for any inherited disease — this one being for eyesight, which is historic news for medical science. It was particularly encouraging for people with inherited rare diseases. For people with vision loss caused by a mutation to the RPE65 gene — which the new gene therapy corrects — this one-time treatment will be forever life-changing. As a member of the Foundation Fighting Blindness, a non-profit organization that has strived for almost fifty years to drive research to treat and cure inherited retinal disease, I have eagerly awaited this moment.

Spark Therapeutics — the company that conducted the clinical trials that showed the treatment's efficacy and that now has the approval to market it — and the many researchers who made this breakthrough possible, are to be congratulated. But, the next question inevitably looms: how do we ensure patient access to the treatment?

Adjustments to traditional insurance coverage will be needed considering the unique feature of this treatment; it is a one-time application in each eye that results in significant positive outcomes. To reach patients and change lives for the better will require government, industry, patients and consumers to work together to develop a new framework for covering this kind of drug therapy.

In that spirit, I was relieved to hear last week that the manufacturer had announced a series of

innovative strategies to facilitate patient access to the treatment, thereby seeking to reduce the risk and financial burden to treatment centers, insurance companies, and patients. Harvard Pilgrim and Express Scripts have already agreed to partner on these new model payment programs. Additionally, the Center for Medicaid and Medicare Services (CMS) is exploring strategies to offer payers the option to spread payments for certain treatments across multiple years.

Alternative payment models such as outcomes-based rebate models are aligned with a pay-for-performance approach, requiring the manufacturer to stand behind the efficacy and durability of the treatment — in this case, a one-dose treatment. And by embracing the principles of paying for “value,” both public and private payers are laying the necessary foundation for patient access in a world of 21st century cures.

The health science industry is making great strides toward developing cures for once incurable diseases. That includes preventions and cures for inherited retinal diseases that for me and millions of people like me, robbed us of our sight. An organization I’m proud to be a part of, the Foundation Fighting Blindness, has helped drive such research in the inherited blindness space. But any new treatment is only as good as a patient’s access to it.

Spark Therapeutics, by thinking outside the box, has created an exciting new model of cost and risk sharing between the drug developer, treatment centers, and insurance companies. If embraced by additional industry leaders, it will propel current research and will greatly enhance patient access to breakthrough treatments.

So bravo to the Foundation Fighting Blindness and to the scores of scientists who have worked so hard — not only to develop the first gene therapy treatment for inherited blinding disease, but also for the potential for this research to reach beyond treatments for blindness, with translational impacts on other inherited diseases. Further, kudos to Spark Therapeutics and its payer partners for understanding the need to both develop innovative treatment and to break down barriers to access for patients. And of course, my thanks to the community of physicians, scientists and our government stakeholders who are needed to work alongside manufacturers, advocates, payers, and others to ensure development of and access to life-enhancing treatment, in this case a ground-breaking treatment to restore vision!

About the Author

Janni Lehrer-Stein is a disability rights advocate and former attorney, a graduate of Yale University and the University of Toronto Law School. She was appointed by President Barack Obama and confirmed by the United States Senate to serve on the National Council on Disability, for which she served two terms from 2011-2016. Currently, Janni is a member of the board of Directors of the Foundation Fighting Blindness, Disability Rights Advocates, and the National Academies of