

# PAVING THE WAY TO ACCESSIBLE TREATMENT FOR ALL



Gene therapy offers promise but poses a challenge to current payment models.

BY THOMAS CIULLA, MD, MBA

At a recent meeting of the US Food and Drug Administration's Cellular, Tissue, and Gene Therapies Advisory Committee, clinical trial participants were given the opportunity to describe their experiences before and after receiving the investigational gene therapy voretigene neparvovec-rzyl (Luxturna; Spark Therapeutics). Powerful stories were told, of seeing a loved one's face more clearly, returning to school, trick-or-treating, and holding a job for the first time.

As a retina specialist working for Spark Therapeutics, I felt both great hope and great trepidation at the meeting. The advisory committee members unanimously agreed that the therapy presented a positive benefit-risk profile. This was based on the results of a phase 3 study in which pediatric and adult participants with biallelic *RPE65*-mediated inherited retinal disease (IRD) achieved meaningful improvements in functional vision on the trial's primary endpoint measurement, the multiluminance mobility test. Adverse reactions experienced by trial participants were consistent with the surgical administration procedure and included increased intraocular pressure, resulting in optic atrophy, and loss of foveal function. The most common adverse reactions were conjunctival hyperemia, cataract, increased intraocular pressure, and retinal tear.

With its approval on December 19, 2017, this gene therapy may begin a new chapter in the medical textbook for retinal disorders, requiring proper diagnosis for this specific IRD, and providing a new potential treatment option for these patients with IRDs.

Our health care system is poorly equipped to value this type of therapy. Our system willingly pays for chronic medications, but it is less effective in handling reimbursement for therapies that potentially deliver life-long benefits in one dose or one administration. Unlike other therapies developed to treat chronic, rare diseases, which typically capture the value of the treatment over a patient's lifetime, this proposed gene therapy must capture the value of the benefit it provides coincident with one-time use.

With the average life expectancy in the United States approaching 80 years, the direct and indirect costs of caring for one blind patient can potentially reach millions of dollars. This cost is compounded by other economic factors that extend well beyond treatment regimens, such as loss of productivity, as reflected in the high unemployment rate among blind American adults.

This scenario could begin to change with the adoption of medical breakthroughs such as gene therapy. The costs of developing such innovations are high, and it is more difficult to spread those costs when the affected patient

population is relatively small. A company's ability to continue investing in and developing these potential treatments hinges largely on whether they can find a place in the market.

Success in addressing one form of blindness generates the advanced knowledge and investment of resources necessary to develop potential therapies for other types of vision impairment. This is why the approval of the first gene therapy for biallelic *RPE65*-mediated IRD is so important. If there is not a pathway to move investigational therapies from the laboratory to the clinic, then investors will have little incentive to continue supporting research and development into these types of novel disease treatments.

There has already been some experimentation with health coverage approaches that would tie payment to real-world treatment effectiveness and the ability to reduce long-term use of health services. This progress must continue. New health care financing and coverage systems to make this type of innovation possible are necessary for our nation's long-term health. ■

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