PUBLICCITIZEN

1600 20th Street, NW • Washington, D.C. 20009 • 202/588-1000 • www.citizen.org

December 21, 2017

Jeffrey D. Marrazzo
Co-Founder and Chief Executive Officer
Spark Therapeutics, Inc.
3737 Market Street
Philadelphia, PA 19104

Dear Mr. Marrazzo,

I write on behalf of Public Citizen in Washington, DC. Public Citizen is a consumer advocacy organization with more than 400,000 members and supporters. We have represented the public interest before Congress, the courts and the federal agencies since 1971. Our areas of focus include health and pharmaceuticals, among others. We work to ensure pharmaceutical safety and efficacy and to expand access to affordable medicines in the United States and throughout the world.

The Food and Drug Administration (FDA) has granted Spark Therapeutics, Inc., approval to market the gene therapy Luxturna (voretigene neparvovec-rzyl). Your company's press release states that we should expect an announcement regarding Luxturna's price in early January. Analysts have suggested that the price Spark sets may be exceptionally high – in the hundreds of thousands of dollars or perhaps even more than $1,000,000 to treat a single patient. This sets a harmful new precedent for industry pricing that will strain health budgets, raise premiums and contribute to treatment rationing.

Spark's development of Luxturna has benefited from tax breaks, favorable FDA designations and public research investments. The public deserves to know what return we can expect on our taxpayer support for Luxturna. We also deserve to know whether the price Spark sets for Luxturna has any basis in underlying costs.

We write to request that Spark disclose its research and development and other costs associated with Luxturna, so that analysts, public health programs, private payers and the public may better assess the appropriateness of the list price.

We ask that Spark disclose its Luxturna-related expenditures on clinical trials, reported separately by clinical trial phase and by trial; acquisitions of components and packaging, acquisitions for the purchase of patents and licensing or corporate entities; marketing and marketing research, including educational promotion; federal benefits received including tax credits, grants, patents and exclusivity periods; and the percentage of research and development expenditures that were derived from federal funds.

Public funding supported the development of Luxturna's underlying CRISPR-Cas9 technology. Products related to CRISPR have been patented by scientists from National Institutes of Health-funded labs since

2013.[1] Two of the researchers named on a Luxturna-related patent have affiliations with the University of Pennsylvania.[2] At least one of the inventors received nearly $2 million in federal research grants relating to viral gene therapies from 2005-2009.

In addition, Luxturna's approval was assisted by government programs. It received breakthrough therapy designation from the FDA, giving your company extra consultation with the FDA and expedited review of the application. The product received orphan drug designation and a tax credit for 50% of the associated research and development costs. Luxturna is a biologic therapy, awarded twelve years of exclusivity, and thereby a government-protected product monopoly, regardless of patent terms.

We ask that Spark provide a further detailed disclosure of research and development costs. When Spark asks patients, payers and government agencies to pay an unprecedented and ultimately unaffordable price, Spark at least should help us understand why.

Peter Maybarduk
Access to Medicines Director
pmaybarduk@citizen.org

[1] Letter from Knowledge Ecology International to the Department of Health and Human Services, June 6, 2017, *available at*: https://www.keionline.org/23370/.
[2] See patent for Adeno-associated Virus-Mediated CRISPR-Cas9 Treatment of Ocular Disease *available at*: https://patents.google.com/patent/US20160346359A1/en?assignee=Spark+Therapeutics.