Discussing ELSI in Novel disease treatment

Overview of the disease:
Hereditary retinal dystrophies are a broad group of genetic retinal disorders that are associated with progressive visual dysfunction and are caused by mutations in any one of more than 220 different genes. The RPE65 gene provides instructions for making an enzyme (a protein that facilitates chemical reactions) that is essential for normal vision.
Mutations in the RPE65 gene lead to reduced or absent levels of RPE65 activity, blocking the visual cycle and resulting in impaired vision. Individuals with biallelic RPE65 mutation-associated retinal dystrophy experience progressive deterioration of vision over time. This loss of vision, often during childhood or adolescence, ultimately progresses to complete blindness.
Novel gene therapy:
voretigene neparvovec-ryzl works by delivering a normal copy of the RPE65 gene directly to retinal cells. These retinal cells then produce the normal protein that converts light to an electrical signal in the retina to restore patient's vision loss. Hence, it should be administered only to patients who have viable retinal cells as determined by the treating physician.
voretigene neparvovec-ryzl (Luxturna®) is a novel, single dose gene therapy for* patients with confirmed biallelic RPE65 mutation-associated retinal dystrophy. Luxturna® is commercialized by Spark therapeutics® in the US, and by Novartis (NVS) in Ex-US countries.

Current regulatory & reimbursement pathway:
Luxturna was approved in the US by the Food and Drug Administration in December 2017. The European Medicine Agency approved in November 2018.
After regulatory approval, it was launched in the United Kingdom and Germany, among other countries in Europe. In the UK the overall cost is GBP 613.410,00 and in Germany: Euro: 690.000,00. Both UK and Germany reimburse Luxturna's treatment, as after reaching a confidential discount-based agreement between NVS and NICE, it resulted in Cost/Effective for the NHS; the G-BA, the German health technology assessment, granted with 'considerable added benefit' based on favorable clinical efficacy: improvement of functional vision/orientation, light sensitivity, and perimetry.
France approved the product only for the hospital chart. (See: https://www.has-sante.fr/upload/docs/application/pdf/2019-08/luxturna_summary_ct17535.pdf & https://www.inahta.org/upload/2019/19043_Luxturna.pdf)
In Latin America, Brazil is the first country to issue regulatory approval during August 2020. (http://antigo.anvisa.gov.br/noticias/-/asset_publisher/FXrpx9qY7FbU/content/aprovado-primeiro-produto-de-terapia-avancada-no-brasil-/219201/pop_up?inheritRedirect=false)

Ethical, Legal and Society considerations:
Hypothesizing further approval in your country, provide comments on the following:

1. What kind of information would you like to know in advance to support a comprehensive decision?
2. What ethical and social question/s come to your mind if you have to make a reimbursement decision?

Please go to the following link: https://tinyurl.com/ELSAHTA, you will find a map where you can post your answers, clicking in the country where you live.