SOFIA SEES HOPE
PO Box 705 | Ledyard, CT 06339
860-556-3119 | info@sofiaseeshope.org
www.sofiaseeshope.org

Submitted to: DUR committee, OHCA, OUHSC via email on March 30, 2018

Re: Patient Access to Treatment for Inherited Retinal Dystrophies and Other FDA-Approved Rare Disease Treatments

Dear Members of the Committee:

We write today to strongly urge you to not restrict access to voretigene neparvovecrzyl (brand name Luxturna) for those with biallelic RPE65 mutation-associated retinal dystrophy. Requiring a certain degree of blindness in an individual before treatment will be allowed not only goes against federal health recommendations, but it flies in the face of basic humanity.

Sofia Sees Hope is a nonprofit advocacy organization that works on behalf of those with rare inherited retinal diseases (IRDs). We are dedicated to patient advocacy and education to advance research to cure blindness caused by rare inherited retinal disease. We represent families across the United States. We full support the principle that all FDA-approved treatments should be made available to all those who will benefit from such treatments.

The proposed prior authorization requirements for Luxturna would exclude many individuals who suffer from life-altering vision loss due to RPE65 genetic mutation. As it stands now, Oklahoma requirements state the “member must have best corrected visual acuity of 20/60 or worse in both eyes and/or visual field less than 20 degrees in any meridian in both eyes”. We are asking this VA/VF requirement be stricken as it serves only to reduce cost by restricting access.

As per the FDA, the optimal window for reversing vision loss is during the early phase of the disease. Any withholding of treatment increases the potential for failure and increases the likelihood of recurrent disease activity. Access to a full range of options is essential for people with IRDs to effectively manage their own disease course, and for their physicians to make the most optimal treatment decisions. We urge the DUR committee, OHCA, and OUHSC to continue to allow physicians to have the ability to choose the best product and the timing of prescribing the treatment for their patients.

We would be happy to provide more information for the Committee as it considers these proposals. Please feel free to contact me danielle@sofiaseeshope.org or 860-556-3119.

Sincerely,

Danielle Chiaraluce, COO