Massachusetts Eye and Ear performs first FDA-approved gene therapy procedure for inherited disease
20 March 2018

Massachusetts Eye and Ear made medical history on Tuesday by performing the first post-FDA approval gene therapy for patients with a form of inherited blindness. The occasion marks the beginning of a new era in medicine, as it is the first time any FDA-approved gene therapy has been given to a patient for any inherited disease. The treatment, commercially identified as Luxturna, was developed by Spark Therapeutics and approved in December by the Food and Drug Administration (FDA) for patients aged 12 months and older. Luxturna has been shown to improve visual function in children and adults with inherited retinal disease caused by mutations in the gene RPE65. It is the first and only FDA-approved gene therapy has been given to a patient for any inherited disease.

"I am proud of our team for earning the distinction of being selected as a Center of Excellence for the administration of Luxturna," said Eric Pierce, M.D., Ph.D., Director of the Inherited Retinal Disorders Service at Mass. Eye and Ear and the Chatlos Professor of Ophthalmology. "It was a natural fit because of our department's ongoing experience with developing gene and genetic therapies here in the Ocular Genomics Institute and with their testing in ongoing clinical trials."

"Today we celebrate the decades of work by many individuals to bring gene therapy from science fiction to clinical reality for patients with inherited
retinal disease. Gene therapy will now join the list of innovative therapies used routinely at Mass. Eye and Ear to treat patients. We are thrilled at Mass Eye and Ear to be one of the first centers to offer this life-changing therapy to patients, and proud of our long, successful history of developing translational retinal therapies," said Joan W. Miller, M.D., Chief of Ophthalmology at Mass. Eye and Ear and Mass General Hospital, and the David Glendenning Cogan Professor and Chair of Ophthalmology at Harvard Medical School. "Our hope is that our ophthalmology community can leverage Luxturna's success to accelerate the development of similar gene therapies for the many blinding retinal diseases that still afflict our patients."

Provided by Massachusetts Eye and Ear Infirmary

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