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1. Russell S, Bennett J, Wellman JA, et al. Efficacy and safety of voretigene neparvovec (AAV2-hRPE65v2) in patients with RPE65-mediated inherited retinal dystrophy: a randomized, controlled, open-label, phase 3 trial. *Lancet*. 2017;390:849-860.
2. Dias MF, Joo K, Kemp JA, et al. Progress in Retinal and Eye Research (2017), <https://doi.org/10.1016/j.preteyeres.2017.10.004>.
3. Hayes, Inc. Hayes Emerging Technology Report. Luxturna (Voretigene neparvovec-rzyl). Lansdale, PA: Hayes, Inc.; July, 2019.
4. Weleber RG, Pennesi ME, Wilson DJ, et al. Results at 2 Years after Gene Therapy for RPE65-Deficient Leber Congenital Amaurosis and Severe Early-Childhood Onset Retinal Dystrophy. *AAO*. 2016;123:1606-1620.
5. Spark Therapeutics. Phase 1 Follow-on Study of AAV2-hRPE65v2 Vector in Subjects With Leber Congenital Amaurosis (LCA) 2. ClinicalTrials.gov, U.S. National Library of Medicine, 24 Sept. 2010, Phase 1 Follow-on Study of AAV2-hRPE65v2 Vector in Subjects With Leber Congenital Amaurosis (LCA) 2.
6. MICROMEDEX®SOLUTIONS Compendia. 2020. Voretigene Neparvovec-rzyl.
7. Clinical Pharmacology Compendia. [database online]. Tampa FL: Gold Standard, Inc. Voretigene Neparvovec-rzyl. 2020.
8. Naso MF, Tomkowicz B, Perry WL, et al. Adeno-associated virus (AAV) as a vector for gene therapy. *BioDrugs*. 2017;31(4):317-334.
9. Astuti GD, Bertelsen M, Preising MN, et al. Comprehensive genotyping reveals RPE65 as the most frequently mutated gene in Leber congenital amaurosis in Denmark. *Eur J Hum Genet*. 2016;24(7):1071-1079.
10. Kumaran N, Moore AT, Weleber RG, et al. Leber congenital amaurosis/early-onset severe retinal dystrophy: clinical features, molecular genetics and therapeutic interventions. *Br J Ophthalmol*. 2017;101(9):1147-1154.
11. Campa C, Gallenga CE, Bolletta E, et al. The role of gene therapy in the treatment of retinal diseases: a review. *Curr Gene Ther*. 2017;17(3):194-213.
12. Chung DC, McCague S, Yu ZF, et al. Novel mobility test to assess functional vision in patients with inherited retinal dystrophies. *Clin Exp Ophthalmol*. 2017;46(3):247-259.
13. Ashtari M, Zhang H, Cook PA, et al. Plasticity of the human visual system after retinal gene therapy in patients with Leber's congenital amaurosis. *Sci Transl Med*. 2015;7(296):296ra110.

14. Bennett J, Wellman J, Marshall KA, et al. Safety and durability of effect of contralateral-eye administration of AAV2 gene therapy in patients with childhood-onset blindness caused by RPE65 mutations: a follow-on phase 1 trial. *Lancet*. 2016;388(10045):661-672.
15. Ashtari M, Nikonova ES, Marshall KA, et al. The role of the human visual cortex in assessment of the long-term durability of retinal gene therapy in follow-on RPE65 clinical trial patients. *Ophthalmology*. 2017;124(6):873- 883.
16. Ripamonti C, Henning GB, Robbie SJ, et al. Spectral sensitivity measurements reveal partial success in restoring missing rod function with gene therapy. *J Vis*. 2015;15(15):20.
17. Jacobson SG, Cideciyan AV, Roman AJ, et al. Improvement and decline in vision with gene therapy in childhood blindness. *N Engl J Med*. 2015;372(20):1920-1926.
18. Le Meur G, Lebranchu P, Billaud F, et al. Safety and long-term efficacy of AAV4 gene therapy in patients with RPE65 Leber congenital amaurosis. *Mol Ther*. 2018;26(1):256-268.