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Nanoscope Therapeutics appreciates the opportunity to share new information on the Institute for Clinical and Economic Review's (ICER's) final evidence report of sonpiretigene isteparvovec (MCO-010) for advanced retinitis pigmentosa (RP). With respect to durability of treatment effect, ICER's 2025 report states: "Long-term durability of treatment benefits is difficult to assess." Experts had differing opinions on durability with some expressing concern that the treatment could lead to accelerated death of transfected bipolar cells. Others felt that improving light sensitivity could help preserve retinal pathways. As seen in Figure 3.1, the actual 100-week data could be interpreted in various ways regarding the stability of benefits."[1]

Recent presentations at American Academy of Ophthalmology (AAO) 2025 Annual Meeting and Association for Research in Vision and Ophthalmology (ARVO) 2026 Annual Meeting highlighted new data from the long-term follow-up study REMAIN, further addressing the uncertainty around the duration of treatment effect. Emerging evidence continues to advance our understanding of retinitis pigmentosa and underscores the long-term therapeutic potential of sonpiretigene isteparvovec, with sustained clinical benefit addressing the persistent unmet needs of patients with RP by restoring vision.

Long-Term Efficacy and Safety of Sonpiretigene Isteparvovec (MCO-010): Results from the REMAIN Study[2,3]

REMAIN is a US-based observational follow-up study of adult patients with retinitis pigmentosa and severe vision loss who previously received sonpiretigene isteparvovec in RESTORE. Patients have been monitored for long-term safety outcomes, including any delayed adverse events or serious complications, while also tracking visual improvements in best-corrected visual acuity (BCVA), shape discrimination, and navigation ability.

At week 152 from dosing in RESTORE, sonpiretigene isteparvovec demonstrated robust clinical efficacy, with continued durable vision improvement from baseline of approximately three-line equivalent. Sonpiretigene isteparvovec exhibited a favorable safety profile, with no serious adverse events reported through the observation period, minimizing long-term safety risk and associated monitoring costs. These results establish sonpiretigene isteparvovec as a well-tolerated gene therapy with sustained and clinically meaningful vision restoration in patients with retinitis pigmentosa. Interpreted against the natural history of advanced RP, in which BCVA would be expected to decline greater than one line over the same length of time (~0.04 LogMAR per year[4]), maintenance or restoration of vision over this interval represents a clinically meaningful departure from expected disease trajectory. These observational findings constitute, to our knowledge, the first demonstration of durable vision restoration by an optogenetic therapy for a patient population with severe vision loss and high unmet need beyond a randomized controlled trial setting, empowering retina specialists to offer a new durable therapeutic option for patients with inherited retinal dystrophy upon regulatory approval.

References:

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