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Evaluation of safety and efficacy of OCU400 gene therapy for retinitis Pigmentosa: Phase 1/2 study results

Abstract:

Retinitis pigmentosa (RP) is a group of rare genetic disorders that causes retinal degeneration, leading to vision loss and blindness. Mutations in over 100 different genes can lead to RP, and the only FDA- approved gene therapy addresses merely 2% of cases, leaving most patients without therapeutic options. Gene-agnostic treatment approaches are viable alternatives to traditional gene-specific therapies. Nuclear hormone receptor (NHR)-based novel modifier gene therapy utilizes NR2E3 overexpression as a gene- agnostic approach to modulate retinal cell homeostasis through the regulation of multiple transcriptional networks. A total of 18 adult RP subjects with autosomal dominant or biallelic autosomal recessive NR2E3 mutations or autosomal dominant RHO mutations enrolled in the Phase 1/2, open-label clinical trial (NCT05203939). Subjects received a unilateral, single subretinal injection of OCU400 (AAV5- hNR2E3) with a low (5 x 109 vg/eye), medium (1 x 1010 vg/eye), or high (5 x 1010 vg/eye) dose in the eye with poorer vision. The primary safety endpoints included identifying study-related adverse events and ophthalmological changes. Efficacy endpoints included changes from baseline in Best Corrected Visual Acuity (BCVA), Low-Luminance Visual Acuity (LLVA), and the Multi-Luminance Mobility Test (MLMT). After 12 months post-OCU400 dosing, analysis demonstrated that OCU400 treatment was safe and well tolerated. Efficacy results demonstrated stabilization or improvement in 89% (16/18) of subjects in the treated eye as assessed through BCVA or LLVA or MLMT compared to baseline. Importantly, 78% (14/18) of subjects displayed stabilization or improvement in MLMT scores. A Phase 3 clinical trial for OCU400 is ongoing (NCT06388200).

Biography

Murthy Chavali brings over 20 years of experience in the biotechnology sector and academia, with a focus on innovation, discovery, drug development, and clinical research. Currently, he oversees the clinical development of various ophthalmological therapies at Ocugen, concentrating on conditions such as inherited retinal disorders, diabetic macular edema, and age-related macular degeneration. He has extensive experience in drug development, including expertise across a range of therapeutic modalities such as cell and gene therapies, small molecules, biologics, and stem cell therapies. His clinical development skills include creating Phase I-III clinical protocols, executing clinical trial studies, regulatory interactions and managing medical affairs teams.