# Study to Evaluate Sepofarsen in Subjects With Leber Congenital Amaurosis (LCA) Type 10 (HYPERION)

NCT06891443

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| **Status** | RECRUITING |
| **Phase** | Phase 3 |
| **Sponsor** | Laboratoires Thea |
| **Enrollment** | 32 participants |

## Key Eligibility Criteria

### Inclusion (5)

- Confirmed clinical diagnosis of LCA10 and a molecular diagnosis of homozygosity or compound heterozygosity for the c.2991+1655A\>G mutation in CEP290.
- Adults: \>=18 years / Minors: 6 to \<18 years.
- BCVA (FrACT) equal to or worse than logMAR +0.4 (approximate Snellen equivalent 20/50) to +2.9 logMAR based on quantifiable, reliable FrACT. LP subjects with documented evidence of prior better vision eligible.
- Symmetrical disease between the two eyes as defined by a BCVA (FrACT) within 0.2 logMAR at baseline.
- Detectable ONL in the macular area as determined by the CRC at Screening.

### Exclusion (5)

- Mutations in genes other than the CEP290 gene associated with other IRD diseases or syndromes.
- Presence of any ocular pathology in either eye that may make comparison of the eyes not feasible.
- Presence of unstable concurrent CME, or subject started on (or changed dose of) topical or systemic carbonic anhydrase inhibitor treatment in the 3 months prior to enrollment. CME is allowed if stable for 3 months (with or without treatment).
- Presence of any clinically significant lens opacities/cataracts based on the AREDS lens grading scale.
- Any prior receipt of genetic (RNA or DNA therapy) or stem-cell therapy for ocular or non-ocular disease, including sepofarsen.

## Locations (14 total)

UCSF Wayne and Gladys Valley Center for Vision, San Francisco, California, United States
University of Miami - Bascom Palmer Eye Institute, Miami, Florida, United States
University of Iowa, Iowa City, Iowa, United States
... and 11 more locations