

## Sepofarsen

Cat. No.:	HY-153496
CAS No.:	2227173-68-2
Molecular Weight:	5962
Target:	Others
Pathway:	Others
Storage:	Please store the product under the recommended conditions in the Certificate of Analysis.

# Sepofarsen

### BIOLOGICAL ACTIVITY

#### Description

Sepofarsen (QR-110) is an RNA antisense oligonucleotide targeting to the p.Cys998X mutation (also known as the c.2991+1655A>G mutation) in the CEP290 gene.

### REFERENCES

- [1]. Russell SR, Drack AV, Cideciyan AV, et al. Intravitreal antisense oligonucleotide sepofarsen in Leber congenital amaurosis type 10: a phase 1b/2 trial. *Nat Med.* 2022;28(5):1014-1021.
- [2]. Cideciyan AV, Jacobson SG, Ho AC, et al. Durable vision improvement after a single treatment with antisense oligonucleotide sepofarsen: a case report. *Nat Med.* 2021;27(5):785-789.

**Caution: Product has not been fully validated for medical applications. For research use only.**

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