

Eteplirsen

Chemical Properties

CAS No. : 1173755-55-9

Formula:

Molecular Weight:

Keep away from moisture

Storage: Powder: -20°C for 3 years | In solvent: -80°C for 1 year

Actual storage temperature shall be subject to the COA.

Eteplirsen

Biological Description

Description	Eteplirsen (brand name Exondys 51) is an antisense oligonucleotide therapy designed to induce exon 51 skipping in the dystrophin gene, enabling partial restoration of dystrophin protein expression in patients with Duchenne muscular dystrophy carrying specific exon 51 mutations, Eteplirsen is extensively applied in genetic disease research, RNA-targeted therapy development, and translational studies of muscular dystrophy.
Targets(IC50)	Antiviral
In vitro	Eteplirsen functions by enhancing dystrophin synthesis, thereby treating Duchenne muscular dystrophy (DMD). It achieves this by selectively skipping exon 51 in mutated gene variants, which restores the translational reading frame necessary for proper protein production[1].

Reference

Kenji Rowel Q Lim, et al. Eteplirsen in the treatment of Duchenne muscular dystrophy. Drug Des Devel Ther. 2017 Feb 28;11:533-545.

Inhibitor · Natural Compounds · Compound Libraries · Recombinant Proteins

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