

Product Data Sheet

Product Name: Tofersen
Cat. No.: GC64897

Chemical Properties

Cas. No. 2088232-70-4

Formula M.Wt 7128

Solubility Water : 100 mg/mL (14.03 mM; Need ultrasonic) Storage -20°C, stored under nitrogen

General tips For obtaining a higher solubility , please warm the tube at 37 °C and shake it in the ultrasonic bath for a while. Stock solution can be stored below -20°C for several months.

Shipping Condition Evaluation sample solution : ship with blue ice All other available size: ship with RT , or blue ice upon request.

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

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Address: 10292 Central Ave. #205, Montclair, CA, USA

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Structure

Tofersen

Background

Tofersen (BIIB067) is an antisense oligonucleotide that mediates RNase H-dependent degradation of superoxide dismutase 1 (SOD1) mRNA to reduce the synthesis of SOD1 protein. Tofersen can be used for the research of amyotrophic lateral sclerosis (ALS)[1].

Tofersen targets the SOD1 mRNA using antisense oligonucleotides (ASOs) that bind to the SOD1 mRNA by Watson-Crick base pairing. Tofersen activates RNase H1 that destroys the targeted RNA[2].

[1]. Miller T, et, al. Phase 1-2 Trial of Antisense Oligonucleotide Tofersen for SOD1 ALS. N Engl J Med. 2020 Jul 9;383(2):109-119. [2]. McCampbell A, et, al. Antisense oligonucleotides extend survival and reverse decrement in muscle response in ALS models. J Clin Invest. 2018 Aug 1;128(8):3558-3567.

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