Product Data Sheet

Product Name: Fitusiran
Cat. No.: GC65060

Chemical Properties

Cas. No.	1499251-18-1		
Formula	$C_{520}H_{679}F_{21}N_{175}O_{309}P_{43}S_{6}$	M.Wt	16248.2
Solubility	H_2O : 50 mg/mL (Need ultrasonic)	Storage	4°C, sealed storage, away from moisture
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.		
Structure			

Fitusiran

Protocol

Animal experiment [1]:

Animal models	FVIII-deficient mice ($F8^{-/-}$ -mice)

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Preparation Method	Fitusiran was administered subcutaneously twice to mice at a dose of 10mg/kg at a 1-week interval. Plasma samples obtained 7 days after the second injection to analyze their thrombin generating capacity. Thrombin generation in platelet poor murine plasma was measured in a microtiter plate fluorometer.
Dosage form	10mg/kg; 14d; qw; s.c.
Applications	Fitusiran increased thrombin generation in mice.

References:
[1] McCluskey G, Maynadié H, Borgel D, et al. Fitusiran treatment modulates the ratio between alpha- and beta-antithrombin isoforms [J]. Hemasphere, 2026, 10: e70376.

Background

Fitusiran is a kind of small interfering RNA (siRNA) therapeutic for hemophilia [1]. Fitusiran's mechanism of action is based on natural cellular interference mechanisms designed to cleave antithrombin messenger RNA (mRNA), thereby reducing antithrombin synthesis in the liver[1]. Fitusiran can enhance thrombin activity to restore hemostasis, and it has been clinically used for the treatment of hemophilia[2].

Fitusiran (10mg/kg; 14d; qw; s.c.) increased thrombin generation in F8 $^{-/-}$ -mice[3]. Fitusiran (10mg/kg; 14d; qw; s.c.) ameliorated the bleeding tendency in coagulation factor X (FX) deficiency mice[4].

References:
[1] Van Thillo Q, Hermans C. Rebalancing agents in hemophilia: knowns, unknowns, and uncertainties [J]. Haematologica, 2025, 110(12): 2902-2912.
[2] Lu D, Dou F, Gao J. Fitusiran: The first approved siRNA therapy for hemophilia via reducing plasma antithrombin levels [J]. Drug discoveries & therapeutics, 2025, 19(2): 131-132.
[3] McCluskey G, Maynadié H, Borgel D, et al. Fitusiran treatment modulates the ratio between

alpha- and beta-antithrombin isoforms [J]. Hemasphere, 2026, 10: e70376.
[4] Verhenne S, McCluskey G, Maynadié H, et al. Fitusiran reduces bleeding in factor X-deficient mice [J]. Blood, 2024, 144(2): 227-236.