Kort fortalt om:
ATLAS ALN-AT3SC-003

Rigshospitalet

Protokoltitel	ATLAS-INH: A Phase 3 Study to Evaluate the Efficacy and Safety of Fitusiran in Patients with Hemophilia A or B, with Inhibitory Antibodies to Factor VIII or IX
Diagnose	Hemophilia A or B, with Inhibitory Antibodies to Factor VIII or IX
Protokolnummer	ALN-AT3SC-003
Danske centre	Rigshospitalet
Formål, design og resume	-Routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adult and adolescents. -To evaluate the efficacy of fitusiran compared to on-demand treatment with bypassing agents, as determined by the frequency of bleeding episodes. **-Study Design** The ATLAS-INH trial (ALN-AT3SC-003) is a multicenter, multinational, randomized, open-label phase 3 study designed to evaluate the efficacy and safety of fitusiran in male patients aged ≥12 years, with hemophilia A or B and inhibitory antibodies to factor VIII (FVIII) or factor IX (FIX), who are not receiving prophylactic therapy. Eligible patients will be randomized in a 2:1 ratio to: □ **Fitusiran treatment arm**: Fitusiran 80 mg administered subcutaneously (SC) as prophylaxis once monthly, with use of on-demand bypassing agents for treatment of breakthrough bleeding episodes □ **On-demand arm**: On-demand bypassing agents for treatment of breakthrough bleeding episodes On-demand use of bypassing agents (BPAs) is defined as the use of these agents as needed for episodic bleeding and not on a regular regimen intended to prevent spontaneous bleeding. Throughout the study, patients in the fitusiran treatment arm may receive on-demand treatment for breakthrough bleeding episodes with BPAs, as appropriate. Bleeding events and doses of BPAs administered during the conduct of the study will be recorded in an eDiary. Safety, quality of life, and pharmacodynamic and pharmacokinetic data will also be collected. .
Hvilke ptt.	(≥12 years old) hemophilia A or B patients
Hvor mange ptt.	1-2

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Hvordan	Fitusiran is an SC administered GalNAc-conjugated siRNA targeting liver-expressed messenger RNA (mRNA) for AT. Patients randomized to the fitusiran treatment arm will receive open label fitusiran 80 mg as an SC injection once monthly, for a total of 9 months; dosing will begin on Day 1 of the treatment period. Patients in on-demand arm will receive on-demand BPA therapy per Investigator discretion to treat bleeding episodes from Day 1 through end of study. The protocol will recommend guidance for patients in the fitusiran treatment arm for treatment of breakthrough bleeding episodes during the fitusiran efficacy period.
Hvor længe	**Duration of Treatment** The duration of treatment with fitusiran is 9 months. The estimated total time on study, inclusive of Screening, for each patient is up to 11 months for patients who enroll in the extension study and patients in the on-demand arm. The estimated total time on the study may be up to 17 months in fitusiran treatment arm patients who do not enroll in the extension study due to the requirement for an additional 6 months of follow-up monitoring for AT levels.
Særlige dosismodifikationer	
Særlige in- eller eksklusions kriterier	
Randomisering hvordan:	Eligible patients will be randomized in a 2:1 ratio to: □ **Fitusiran treatment arm**: Fitusiran 80 mg administered subcutaneously (SC) as prophylaxis once monthly, with use of on-demand bypassing agents for treatment of breakthrough bleeding episodes □ **On-demand arm**: On-demand bypassing agents for treatment of breakthrough bleeding episodes
Særlige KAT opgaver	QOL (papir), Bløder-dagbog(e-diary), PK prøver,
Andet	Eudract nr. 2016-001463-36
KAT ansvarlig pro. spl.	Navn: Christel Nielsen Tlf. nr. 5-8340 og Thilda Aarup Tlf. nr. 5-9558
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