Kort fortalt om:

CT-AMT-061-02 HOPE-B

Rigshospitalet

Protokoltitel	Phase III, open-label, single-dose, multi-center multinational trial investigating a serotype 5 adeno-associated viral vector containing the Padua variant of a codon-optimized human factor IX gene (AAV5-hFIXco-Padua, AMT-061) administered to adult subjects with severe or moderately severe hemophilia B
Diagnose	Hemophilia B severe or moderately severe
Protokolnummer	**CT-AMT-061-02**
Danske centre	Rigshospitalet
Formål, design og resume	**Objectives:** **Primary:** To demonstrate the effect of AMT-061 on endogenous FIX activity 6 months after a single AMT-061 treatment. **Secondary:** To demonstrate the non-inferiority of AMT-061 as compared to standard of care continuous routine Factor IX prophylaxis in terms of prevention of bleeding and to demonstrate additional efficacy and safety aspects of systemic administration of AMT-061. **Trial Design/Methodology:** This is an open-label, single-dose, multi-center, multinational trial, with a screening period, a lead-in phase, a treatment + post-treatment follow-up phase, and a long-term follow-up phase.
Hvilke ptt.	Male subjects with severe or moderately severe hemophilia B.
Hvor mange ptt.	56
Hvordan	**Trial Design/Methodology:** This is an open-label, single-dose, multi-center, multinational trial, with a screening period, a lead-in phase, a treatment + post-treatment follow-up phase, and a long-term follow-up phase.
Hvor længe	First Subject Screening Q2 2018 Last Subject Last Visit Q3 2024
Særlige dosismodifikationer	The single administered dose of AMT-061 will be 2 x 1013 gc/kg. All Danish patients will be dosed in Germany
Særlige in- eller eksklusions kriterier	NA
Randomisering hvordan:	NA
KAT ansvarlig pro. spl.	Navn: Tina Bjerg Tlf.nr. 35457860 Thilda Aarup Tlf. nr. 3535 9558
Forsøgsansvarlig læge	Navn: Peter Kampmann Tlf. nr. 35451733