Protokoltitel	Explorer8
Diagnose	Severe haemophilia A (FVIII <1%) or B (FIX ≤2%)
Protokolnummer	Trail ID: NN7415-4307, Eudra CTnr: 2018-004891-36
Danske centre	Rigshospitalet & Skejby
Formål, design og resume	The trial aims to evaluate the effect and safety of Concizumab 0.25 mg/kg administered daily subcutaneous in patients with haemophilia A (HA) and haemophilia B (HB) without inhibitors. Concizumab is a therapeutic monoclonal antibody that is being developed for the prevention of bleeding episodes, including long-term prophylaxis treatment of bleeding. This is a prospective, multicentre, open label clinical trial with two randomised arms and two non-randomized arms. After the main part of the trial, all patients will be offered to continue in the extension part of the trial and receive treatment with Concizumab for up to an additional 136 weeks. In the extension part patients may increase the dose to 0.35 mg/kg, based on specific criteria.
Randomisering hvordan:	Patients will be randomised to Concizumab prophylaxis or no prophylaxis (Arm 1 + 2) or assigned into the non-randomised treatment arms (Arm 3 + 4), based on their treament regimen before entering the trial. The randomisation between the treatment arms 1 and 2 will be stratified according to haemophilia type and bleeding frequency during the 24 weeks prior to screening section. Patient will be randomised 1:2 to no prophylaxis versus Concizumab prophylaxis.
Hvilke ptt.	Male aged ≥12 years (≥ 18 years in Denmark) Congenital severe haemophilia A (FVIII <1%) or B (FIX ≤2%)
Hvor mange ptt.	Patients planned to be screened in total: 156 Patients planned to be started on trial product in total: 150 Patients on Rigshospitalet: 2
Hvordan	<u>ARM 1 + 2 + 4:</u> Patients who are randomized/allocated to Concizumab prophylaxis will receive a loading dose of 1 mg/kg Concizumab at visit 2 (Arm 2 and 4) or visit 9 (Arm 1) in clinic followed by a maintenance dose of 0.25 mg/kg Concizumab from treatment day 2 and onwards as home treatment. <u>ARM 3:</u> Patients in Arm 3 will not receive a loading dose but continue with Concizumab prophylaxis as there have until enrolment into the trail. The trail products will be administrated in prefilled PDS290 pen-injector - Concizumab 40 mg/ml or Concizumab 100 mg/ml – subcutaneously either in the abdomen or in the thigh.

Hvor længe	The trial consists: - A main part 24 weeks - A extension part in up to 136 weeks - A safety follow-up part for 7 weeks Trial period completion for a patient is defined as when the patient has completed visit 27. Global end of trial date 10 March 2023.
Særlige dosismodifikationer	
Særlige in- eller eksklusions kriterier	**Key inclusion criteria:** Congenital severe haemophilia A (FVIII <1%) or B (FIX ≤2%). **Key exclusion criteria:** Known or suspected hypersensitivity to monoclonal antibodies, known inherited or acquired coagulation disorder other than congenital haemophilia, presence of confirmed inhibitors ≥ 0.6 BU at screening.
Særlige KAT opgaver	Ediary – Treatment with Concizumab, Bleeding episodes including treatment of bleeds, PRO questionnaires and health economic questions PK-sampling ActiGraph – Physical Activity tracker
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