Aplastische Syndrome

Alle Patienten: Telomeropathie-Register
Eine nicht-interventionelle epidemiologische Beobachtungsstudie mit zeitlich versetzten Eintrittspunkten der Patienten in die untersuchte Kohorte

PNH

PNH-Register
Collection of data to evaluate safety and characterize progression of Paroxysmal Nocturnal Hemoglobinuria

Patients treated with Eculizumab

BP39144
Phase I/II
to assess safety, efficacy, pharmacokinetics and pharmacodynamics of RO7112689 in healthy volunteers and patients with PNH

PEGASUS
Phase III
to Evaluate the Efficacy and Safety of APL-2; patients treated with Eculizumab, but Hgb <10,5g/dl

ALXN1210-303
Phase III
noninferiority Study of Ravulizumab administered SC vs IV in Patients currently treated with Eculizumab

AA

Moderate Aplastic Anemia

EMAA
Phase II/III,
Efficacy and Safety of Thrombopoetin-Receptor Agonist Eltrombopag in Combination With Ciclosporin A in Moderate Aplastic Anemia

Inclusion:
- Current diagnosis of a Moderate Aplastic Anemia requiring standard treatment with CSA without prior specific therapy.
- MAA is defined as Aplastic Anemia fulfilling the following criteria:
  - no evidence for other disease causing marrow failure
  - hypocellular bone marrow for age
  - depression of at least two out of three peripheral blood counts
  - without fulfilling the criteria for SAA
  - Transfusion-independent MAA

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