

CASE STUDY

Haemophilia A

Study

Inhibitor Development in Previously Untreated Patients (PUPs) or Minimally Blood Component-Treated Patients (MBCTPs) when Exposed to plasma-derived von Willebrand Factor-Containing Factor VIII (VWF/FVIII) Concentrates and to Recombinant Factor VIII (rFVIII) Concentrates: An Independent, International, Multicentre, Prospective, Controlled, Randomised, Open Label, Clinical Trial.

Study objective

To assess the immunogenicity of VWF/FVIII and of rFVIII concentrates by determining the frequency of inhibitor development in PUPs and MBCTs in the first 50 EDs or in the first 3 years from enrolment, whichever comes first.

Investigational drug

rFVIII products and plasma-derived VWF/FVIII products.

Study Aim

This study aims to test the hypothesis that plasma-derived VWF/FVIII products are less immunogenic than rFVIII products.

Study details

Patients will be randomised to be treated exclusively with a single FVIII product either plasma-derived or recombinant, and followed up until inhibitor development or until 50 exposure days (EDs) or 3 years from enrolment have elapsed, whichever comes first. Participating sites are in Europe, USA, South America, South Africa, India, Middle East.

Sintesi Research Services

Project management, regulatory, monitoring and site coordination, investigators and CRA meetings, central laboratory samples management, data management, QA QC, Statistics, final reports.

Key challenges

The study is globally involving 80 sites worldwide, the recruitment started one year ago and despite the rarity of the disease under study, 33% of expected case study was enrolled in this short timeframe.

Due to local requirements and limitations in using these classes of products in PUPs, some of the initial countries were timely replaced with new sites.

Keys of project success

Sintesi Research organized investigators meetings on a regular basis in order to keep investigators motivated and maintain the study profile high: metrics of the study, together with preliminary interim results were frequently discussed during dedicated meetings at the most important medical congresses.

To keep constantly update the sponsor / investigators about screenings and screening results Sintesi Research offered a project dedicated website.

Sintesi Research team worked closely with each clinical site encouraging the investigators to complete enrolment and determining what resources were needed to carry out trial related activities, which might be a critic issue in emerging countries.



To discover Sintesi Research services contact us.

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