

Title: A Prospective, Multicenter, Open Enrollment Study of Human Plasma-Derived Factor XIII Concentrate in Subjects with Congenital Factor XIII Deficiency

IRB# 2010-039

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Synopsis:

The investigators and CSL Behring LLC, the Sponsor of this study, are conducting a research study to evaluate the safety and recommended (best) amount or level of Factor XIII in patients with FXIII deficiency. Factor XIII Concentrate works by assisting the blood in the usual clotting process, thereby preventing bleeding.

Factor XIII Concentrate is made by collecting blood samples from multiple donors and concentrating the human clotting Factor XIII that is required to enable blood to clot normally. Factor XIII Concentrate is currently licensed in 14 countries as Fibrogammin® P, for the prevention of bleeding in patients lacking Factor XIII. Fibrogammin® P is not licensed in the United States, Canada or Spain.

The purpose of this study is to evaluate the safety and recommended (best) amount or level of Factor XIII Concentrate required to prevent bleeding so that it can be licensed and sold in the United States.

Blood samples will be collected to measure baseline level of red blood cells, white blood cells, and platelets, and to measure how well blood clots (how "thin" it is) and the level of clotting factors before patients receive any study medicine. Blood samples will also be taken to see if the patient has had an infection with certain viruses in the past and to determine if patients have developed any immunity to Factor XIII. A blood sample will also be drawn for any future testing. The total amount of blood drawn at this visit will be approximately 40 ml or 8 teaspoons.