CSL889_1001

CSL Behring‘s HEMOPEXIN FIRST IN HUMAN STUDY IN STABLE SICKLE DISEASE

Study Title:

A Phase 1, Multi-Center, Open Label, Single Ascending Dose Study to Evaluate the Safety, Tolerability, and Pharmacokinetics of CSL889 in Adult Patients with Stable Sickle Cell Disease

ClinicalTrial.gov: NCT04285827

Part of the CSL Behring’s Facilities in Marburg, Germany

Date 26-January-2021

Introductory Information

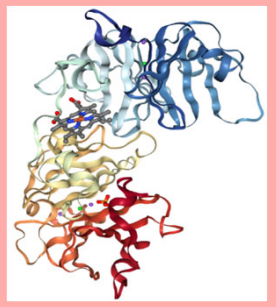
There are many clinical trials available for people living with sickle cell disease, and patient participation plays a pivotal role in the development of new medicines to help treat this devasting condition.

CSL Behring is a global biotherapeutics leader driven by our promise to save lives. We meet patients' need using the latest technologies to develop and deliver innovative biotherapies that are used to treat serious and rare diseases.

CSL Behring is running a Phase 1 study for adults with sickle cell disease.

Visit our Website at WWW.CSLBEHRING.COM

About CSL889 (Hemopexin)



- Vaso-occlusive crisis (VOC) is a common and debilitating complication for those with sickle cell disease (SCD)
- CSL889 is a plasma-derived hemopexin, an important protein produced naturally in the body whose levels are decreased in individuals with SCD
- Hemopexin is important for removing excess heme from the bloodstream, which is thought to play a role in the pain associated with VOC
- CSL889 is a being developed for the treatment of VOC

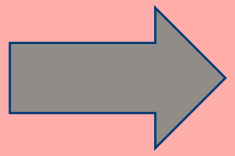
The Study

- This is a clinical research study involving a single administration of the study drug, CSL889 at 6 different dose levels.
- This is the first time that the study drug is being given to humans
- The study product will be given directly into the bloodstream over a period of approx. 1 hour or less
- Participants will be in the study for approximately 6-8 weeks in total
- The study involves 11 visits and a single 24 hrs residential overnight stay

  Depending on the hospital/research unit set up of the study the inpatient visit may require up to 48 hrs
- Eligible patients may receive compensation for their participation in the study, plus reasonable travel expenses
- Approximately 24 participants will take part in the study; each subject will only receive 1 infusion of CSL889

Objectives of the Study

- The study aims to:
  - see how safe and tolerable the drug is in patients with SCD
  - Measure levels of CSL889 in the blood
  - Look for changes in several blood tests related to sickle cell disease to see how the drug might affect these measures.



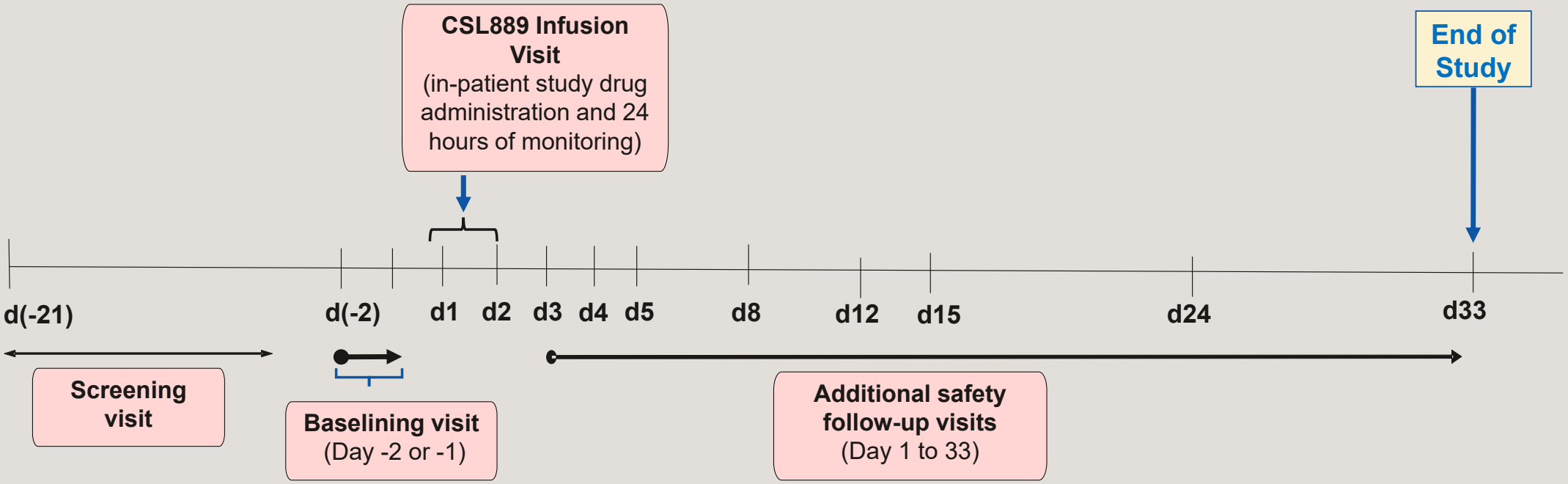
Procedures:

- Drug administration by IV infusion
- Blood tests
- Physical examination
- Measurement of vital signs (blood pressure, breathing rate, heart rate, and body temperature)
- Electrocardiogram (ECG)
- Collection of adverse events

Main Eligibility Criteria

- Aged 18 - 60 years
- Weight above 50 kg and below 110 kg
- Diagnosis of Sickle Cell Disease (HbSS or $HbSB^0$)
- No Sickle Cell Disease Crisis in the 30 days before the study drug is given
- Not taking hydroxyurea and / or L-glutamine, or stable well tolerated regimen of hydroxyurea and / or L-glutamine taken for at least 30 days before dosing that is planned to continue without change throughout the study
- No history of stroke
- Vaccination within 30 days before Day 1, or planned vaccination during the study
- Blood transfusion within the 90 days before Day 1 or expecting blood transfusion during the study

Design – Individual Study Subject Flow



11 visits over 6-8 weeks, including a 24 hrs overnight in-patient stay
Hospital/research unit can offer admission on the day before infusion which will extend the in-patient stay to 48 hrs

Participating countries and Sites

The study is conducted in the United Kingdom, the Netherlands and the United States of America.

United Kingdom

- Collaboration with
  - Five phase I units at either hematology centers or privately owned close to a university hospital where the study is conducted
  - Four Participant Identification Centers in the London area that refer interested patients to the Early Phase Unit at the Guy's and St Thomas' NHS Foundation Trust

Timelines

Planned First Subject enrolled: March 2021
Planned Last Subject enrolled: May 2022

Contact:

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