Every day, research uncovers new information about medical conditions and their treatment.

Volunteer involvement in clinical studies is a key part in the development and advancement of future therapies.

Results collected from clinical studies have led to thousands of medications becoming available to patients all over the world.

How can I learn more about the study?

To ask questions or find out if you could participate, please contact:

ViSion SERENITY

VIT-2763-SCD-202_Patient Brochure_v3.0_26Apr2022_United Kingdom English

JOIN OUR SICKLE CELL STUDY NOW

BE PART OF A RESEARCH PROGRAMME

LEARN MORE ABOUT RESEARCH OF A POTENTIAL TREATMENT FOR SICKLE CELL DISEASE

ViSion SERENITY

What is a clinical study?

A clinical study (also known as a clinical trial) is designed to learn more about a drug's ability to treat a specific disease or condition. Regulatory agencies and health authorities use the results of clinical studies to decide if a drug should be made available to patients. A drug used in a clinical study has not yet been approved for the treatment of a disease, but it is being tested in clinical trials to see if it should be approved as a future treatment option.

Clinical studies are conducted by experienced and trained medical professionals who monitor the health of participants throughout the study. Also, every clinical study is reviewed by an independent review board (IRB) or ethics committee (EC), which helps ensure that the study is conducted safely and that the rights of study participants are protected.

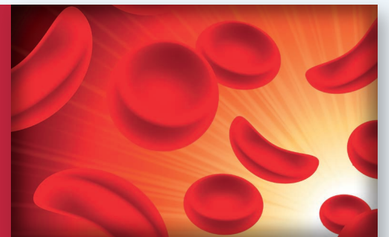
What is the purpose of this study?

The purpose of this study is to explore the safety, tolerability and effectiveness of a study drug called VIT-2763 for sickle cell disease (SCD). Specifically, we want to investigate its effects on the breakdown of red blood cells and inflammation associated with sickle cell disease.

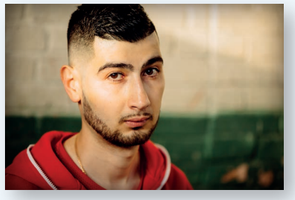
What is the study drug?

VIT-2763 targets and blocks a protein in the body called ferroportin. Ferroportin allows the body to transport iron to the blood stream. Iron is important in the production of haemoglobin, the protein that transports oxygen in red blood cells. It is expected that, by blocking ferroportin, VIT-2763 may lower the iron level in the blood. This might reduce the concentration of abnormal haemoglobin (sickle cells) in the red blood cells, and it may reduce the destruction of red blood cells caused by SCD.

CLINICAL STUDIES ARE THE ONLY WAY WE CAN DEVELOP NEW AND BETTER TREATMENTS AND IMPROVE PATIENT CARE.



VIT-2763 is being developed by Vifor International Inc., the sponsor of this study. It has been tested before in healthy volunteers at multiple dose levels and was well-tolerated by study participants. VIT-2763 is also being tested in people with another blood disorder called thalassemia.



What is a placebo?

Placebos are used in clinical studies so that researchers can understand what effect a new investigational product might have on a condition. A placebo looks like the study drug, but does not contain any active substance. In this study if you qualify, you have a 1 in 4 chance of receiving placebo.

Who can be in this study?

To take part, you must:

- Be 18 to 60 years of age.
- Have SCD, including HbS/S or HbS/βT0 genotype (but **NOT** HbS/βT+ genotype or HbSC disease). The study doctor can tell you what kind of SCD you have if you are not sure.
- Have had 1-10 vaso-occlusive crises within 12 months prior to study screening. These are also called 'sickle cell pain crises' or 'VOC episodes'.
- Not have chronic liver disease or a history of liver cirrhosis.

Other criteria will also be reviewed to see if you can take part.

What does it mean to be involved in this study?

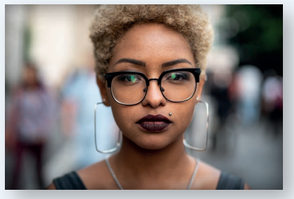
Approximately 24 patients will take part in this study, at institutions located in a number of countries including the United States of America.

The study lasts about 16 weeks and is divided into 3 main periods:

- **Screening/Baseline (up to 4 weeks):** Your medical history will be reviewed, and other tests will be done to see if you can take part in the study.
- **Study Treatment Phase (8 weeks):** If you qualify, you will be randomly assigned (like rolling dice) into 1 of 4 different study treatment groups with various doses of VIT-2763 or placebo. You will have a 3 in 4 chance of receiving VIT-2763 and a 1 in 4 chance of receiving placebo. You will take the oral study treatment twice or three times daily and attend 5 visits for study-related testing.
- **Follow up (4 weeks):** You will return to the study clinic 28 days after the end of study treatment for one final health check.

Why participate in the study?

Treatment options for people with sickle cell disease are limited. This study will contribute to the development of a potential new treatment. Participants will also receive medical monitoring throughout the study that may provide additional health insights.



Taking part in a clinical study is completely voluntary. If you enrol, you can choose to leave the study at any time and for any reason and it will not affect your usual care.

What is the Informed Consent Form?

Before joining the study, the research staff will review a document with you called the Informed Consent Form (ICF). This document provides much more detail about the study, the tests being done, possible risks and side effects, and your rights as a participant. You should read the ICF carefully, ask questions, and talk to the study doctor about any concerns you might have.

Will the study cost anything?

The study treatment, visits, tests and procedures that are needed in this study are provided at no cost to participants. The ICF or the study staff can tell you about any reimbursement that may be available.