



Clinical Trials/Studies

Novartis Research Study to Gain Insight into How Adult Patients Are Impacted by Sickle Cell Disease

The purpose of this study is to gain insight into how adult patients are impacted by sickle cell disease. Investigators are looking for volunteers who were diagnosed with sickle cell disease. The study is sponsored by Novartis Pharmaceuticals Corporation, and will be conducted by Optum's Patient Insights division. The study was approved by an Independent Review Board (IRB). Starting on Friday, Nov. 9, patients, with sickle cell disease will be recruited, who live in the United States and are 18 years or older, to participate in the online study. The survey will be open for 4 months or until 325 patients complete the survey. The one-time survey takes between 30 and 45 minutes.

Participation is completely voluntary and involves taking part in a web-based survey. If you are eligible to participate and complete the survey, you will be compensated for your time with a \$75 prepaid Visa gift card, which will be mailed to you. No medical treatment is provided.

Please visit the following web address to learn more:

www.amihealthy.com/custom/standard/SCD/SCDHome.aspx

Sickle Cell Trait Study by the Consortium for Health and Military Performance (CHAMP) at the Uniformed Services University of the Health Sciences (USUHS)

The Consortium for Health and Military Performance at the Uniformed Services University of the Health Sciences has launched a genomics study to better understand the association between exertion-related events (EREs) and Sickle Cell Trait (SCT). The study is approved by the Institutional Review Board (IRB). We are actively enrolling African American men and women between the ages of 18-80 with SCT, who have experienced EREs such as Exertional Rhabdomyolysis, Exertional Heat Stroke/Illness and Exertional Collapse. Findings from the study will provide much needed evidence to guide diagnosis, treatment and policy decisions that will impact the SCT population and individuals with EREs. Findings will also help develop genetic markers to determine the susceptibility to ERE prior to an event.

The SCT study plan:

- 300 SCT carriers who have had Exertional-Related Events (EREs)
- 150 SCT carriers who have NEVER had EREs
- 150 Immediate family members who have or have NEVER have EREs

The inclusion criteria for the study are:

- Must be SCT positive
- Must be African American
- Must be able to read and speak English
- Must be between the ages of 18 and 80
- Are NOT being treated for any mental disorders

Click on the links below to download their documents:

[SCT White Paper](#)

[IRB-Reviewed-SCT Online Newsletter](#)

[2019 SCT Flyer](#)

Rivipansel: Evaluating Safety, Efficacy and Time to Discharge (RESET) Study

Pfizer, Inc. is conducting a randomized, double-blinded study evaluating the safety and efficacy of rivipansel (previously GMI-1070) in treating painful crises (VOC) in individuals with Sickle Cell Disease (SCD) requiring hospitalization. Click here for more information about the study.

The study is “randomized” meaning that, in addition to their usual pain medications and other treatments, approximately one half of the patients will receive rivipansel while the other half will get an IV fluid which looks like rivipansel. “Double-blinded” means that neither the patient nor the physician knows which group each patient is in.

Therapeutic Approach

Modulation of the course of the VOC through a Pan-Selectin Inhibitor (Inhibitor of E-, P- and L-selectins). Selectins are adhesion molecules which cause blood cells to stick to one another and to the endothelial cells that line the blood vessels, causing blockages in blood flow (which result in a VOC).

Status

Rivipansel has demonstrated safety in studies so far. It has shown the ability to reduce cell adhesion/stickiness and to improve blood flow. In a Phase 2 study in patients admitted for a VOC, rivipansel shortened the length of hospital stay and reduced the amount of opioid pain medication used. The Phase 3 RESET trial is currently being conducted in patients admitted to the hospital with a VOC.

News & Other Links

<https://clinicaltrials.gov/ct2/show/NCT02187003>

<http://press.pfizer.com/press-release/pfizer-announces-enrollment-first-patient-phase-3-trial-sickle-cell-disease>

Jason Carter Clinical Trials Program

Every year, thousands of patients are diagnosed with blood cancers and other life-threatening diseases. For many patients, treatment through clinical trials provides hope and a potential cure.

But finding clinical trials can be challenging. The Jason Carter Clinical Trials Program was created to help patients with blood cancers or blood disorders and their families find and join clinical trials.

- Help you and your family learn about and access clinical trials.
- One-on-one telephone support from a clinical trial nurse to help navigate and search for clinical trials.
- Easy-to-use, web-based search tool to find relevant clinical trials.
- Easy-to-read educational resources for patients and families to learn about sickle cell treatment options and clinical trials.

Financial assistance for travel expenses related to clinical trials.

(888) 814-8610

JCCTP.org

clinicaltrials@jcctp.org

National Minority Quality Forum Joins NIH in Launching the All of Us Research Program to Advance Precision Medicine

On May 6, 2018, the National Institutes of Health opened national enrollment for the All of Us Research Program in collaboration with the National Minority Quality Forum and other partners. All of Us is a unique effort to advance individualized prevention, treatment and care for people of all backgrounds. The overall aim of All of Us is to enroll 1 million or more volunteers and oversample communities that have been underrepresented in

research to make the program the largest, most diverse resource of its kind. Individuals ages 18 and older will be able to enroll, regardless of health status.

Precision medicine is an emerging approach to disease treatment and prevention that considers differences in people's lifestyles, environments and biological makeup, including genes. By partnering with 1 million diverse people who share information about themselves over many years, the All of Us Research Program will enable research to more precisely prevent and treat a variety of health conditions.

All of Us seeks to transform the relationship between researchers and participants, bringing them together as partners to inform the program's directions, goals and responsible return of research information. In service of this objective, NIH has funded more than 100 organizations throughout the U.S. to be partners in the program, including the National Minority Quality Forum.

Participants who enroll in All of Us will be able to access their own health information, summary data about the entire participant community and information about studies and findings that come from All of Us. Participants are asked to share different types of health and lifestyle information, including through online surveys and electronic health records (EHRs), which will continue to be collected over the course of the program. At different times, some participants will be asked to visit a local partner site to provide blood and urine samples and to have basic physical measurements taken, such as height and weight. In the future, participants may be invited to share data through wearable devices and to join follow-up research studies, including clinical trials. In future phases of the program, children will be able to enroll, and the program will add more data types, such as genetic data.

To learn more about the All of Us Research Program and how to join, please visit <https://www.JoinAllofUs.org>. For additional information about the National Minority Quality Forum's initiatives, please contact Gretchen C. Wartman, Vice President for Policy and Program, at 202-223-7560 or gwartman@nmqf.org.

"All of Us" is a registered service mark of the U.S. Department of Health & Human Services (HHS).

Sickle Cell Disease Association of America, Inc. in no way endorses any drugs, treatments, clinical trials, or studies reported on this website. Information is provided to keep the readers informed. Because the manifestations and severity of sickle cell vary among individuals, personalized medical management is essential. Therefore, it is strongly recommended that all drugs and treatments be discussed with the reader's physician(s) for proper evaluation and treatment.