



MEDICAL AND RESEARCH ADVISORY COMMITTEE (MARAC)

MARAC Encourages Clinical Research Studies

September 23, 2022 — The Sickle Cell Disease Association of America (SCDAA) Medical and Research Advisory Committee (MARAC) believes that progress in sickle cell disease (SCD) is tied to clinical trials and comprehensive care. For this reason, we encourage individuals with SCD to consider participating in research. It's because thousands of courageous children and adults with sickle cell disease signed up for research that we have improved survival, developed new medicines and found possible cures.

I've been invited to be in a clinical trial – what should I know before I sign up?

Here are questions to ask if you are invited to be in a clinical trial. Many of these questions are answered in the *informed consent papers* – it will be lot to read, and it may be confusing, but it is worthwhile to pay attention and ask questions. You should always get a copy of the consent papers for your records.

1. What is the purpose of the study?

There are many types of clinical research studies, but the goal of research is the same – to improve the lives and treatment of individuals with SCD. Some studies involve the use of new drugs. Others look at existing approved therapies to see whether they can be useful for SCD. Sometimes studies are simply questionnaires, X-rays and blood draws. Make sure to ask your physician about the trial's purpose.

2. What kind of treatments and tests are involved in the trial?

As mentioned above, the type and number of treatments differ for each clinical trial. During the trial, a lab technician may need to draw blood or perform an electrocardiogram to ensure that you are reacting well to the treatment. Ask your clinical care team about the treatments and tests you can expect throughout the trial process.

3. What are the possible risks or side effects of this treatment?

Side effects vary by trial. You will be closely monitored throughout the clinical trial to ensure that any potential side effects are addressed. It is important to understand all the potential risks or side effects of the treatment before the trial begins.

4. How will the trial affect my daily life?

Asking this question will help you be prepared for your upcoming treatment and plan your personal schedule during the trial. Each trial has a different schedule of care. How often do you have to come to the hospital or clinic? Will you have to stay in the hospital during the clinical trial? If so, how often and for how long? How far will you need to travel to take part in the trial?

5. Do I have to pay for any of the treatments? What costs will my health insurance cover?

Talking to your health care team, as well as facility staff and your insurance company, will help you answer these questions. Your insurance plan should cover the standard of care or routine care associated with the trial. Research-specific tests and procedures will be paid by the sponsor the trial sponsor. Sometimes transportation assistance is provided and you may receive money to compensate for your time and effort.

6. Who will oversee my care while I am participating in the trial?

Your care team will consist of a physician overseeing your participation as well as a team of nurses and research personnel assisting with your treatment and day-to-day care.

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7. What questions should I ask about my other choices?

What are my other treatment choices, including standard treatments? How does the treatment I would receive in this trial compare with other treatment options? What will happen to my SCD without treatment?

8. What questions should I ask about my decision process?

How long do I have to make up my mind about joining this trial? Who can I speak with about questions during and after the trial? Can I talk to who has been in the trial? What if I decide to leave the trial?

9. What questions should I ask about my rights to privacy?

How will my health information be kept private? What happens to my data if I decide to leave the trial? Be cautious about clinical research organizations that might want to sell your data to others and/or claim that you will pay less to participate in their trial.

10. If I benefit from this therapy, will I be allowed to continue receiving it after the trial ends? If I get a placebo, can I switch to the active drug?

The design of some clinical trials allows patients to continue using the therapy as long as they are benefitting. Clarifying this question with your care team is important.

[WATCH: "I participated in clinical trials. I would do it again." \(Video testimony from the National Institutes of Health\)](#)

My friend was asked to participate in a clinical trial, and I wasn't – why not?

- Certain kinds of clinical research study new medications with unknown side effects, so they focus on people who are the least likely to be harmed by the new medication. For example, many studies do not accept older people, or individuals who have kidney/liver problems, asthma, are pregnant, heart problems or a high risk for bleeding.
- If you have not come regularly for sickle cell care or had difficulty taking medications on their prescribed schedule, the research investigators will wonder whether you will follow the rigid schedule of a clinical research study. You may have had valid reasons such as medication side effects or transportation issues. Discuss these barriers with the study team. They may be able to help.
- Advocate for yourself. Ask your doctor(s) whether you are eligible for any clinical research. Some busy doctors simply need a reminder. Others might not have a research study open for enrollment but could refer you to a nearby clinical research group.
- Go to a specialty sickle cell center. Clinical research requires a lot of expertise. You might need to find a specialty center with the right level of resources to offer research studies. Consider going at least once a year to an academic, reputable sickle cell center.
- You might seek out clinical research that does not exclude anybody. Surveys, questionnaires and registries can help to change health care systems or set the stage for future research.

How do I find more clinical research opportunities?

- Ask your doctor(s) whether you are eligible for any clinical research studies.
- Visit [ClinicalTrials.gov](https://www.clinicaltrials.gov), a government sponsored listing of all approved clinical trials in the U.S. and some abroad.

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- Ask your local SCDA member organization (or the patients' organization you belong to) whether they have news about SCD clinical research in your region.
- Seek studies using clinical research finders for sickle cell on the [SCDAA website](#) and [oneSCDvoice.com](#).
- Many registries study SCD using regular questionnaires. The SCDA Get Connected Registry is being set up to serve this function. Registries may also try to relate symptoms with blood tests or genetic tests to lay a foundation for better preventive care in the future.

I'm nervous about participating in clinical research – isn't it risky?

Clinical research participation today looks very different from the past. Many feel nervous about clinical trials after learning about problems with the Tuskegee syphilis project and the HELA cells from Henrietta Lacks. Know that modern clinical trials are set up with layers of safeguards for patients and opportunities to ask questions. Sponsors of clinical trials keep the interest of patients as a priority. Extra cautions are taken to protect children as research participants. Another new feature of clinical research is the community advisory boards for research studies and SCD research networks. Many individuals with SCD now serve on these boards and you can too.

Can clinical trials replace my regular sickle cell care?

All individuals with SCD should have regular health care appointments, but you should make sure check in with your care team *before and after participating in a clinical trial*. Comprehensive sickle cell care is available because of those who participated in past research. Honor those who came before you by taking care of yourself.



(Photo by Lewis Hsu, M.D., August 2022, U.S. Navy Blue Angels at Chicago Air and Water Show)

Enrolling for some early-phase clinical research is like selecting test pilots for new jet planes: you want the pilots with the best vision and the best eye-hand coordination, in case they have to handle a potential emergency.

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LEARN MORE:

Questions to ask – adapted from <https://www.cancer.gov/about-cancer/treatment/clinical-trials/questions>, and from <https://sarahcannon.com/blog/entry/7-questions-to-ask-when-considering-a-clinical-trial> accessed 9/15/22.

Here are links that describe the features of clinical research studies:

[Find a clinical trial near you](#)

[SCDAA](#)



[ASH-CTN](#)



[Proudford Foundation](#)



[CURE Sickle](#)

(focuses on curative therapies clinical research)



[Cayenne Wellness](#)



[British Health Inequalities Program](#)

(requires registration)



G Puppawar, M Mourya, G Kadhe, A. Mane. Conducting clinical trials in emerging markets of sub-Saharan Africa: review of guidelines and resources for foreign sponsors. Open Access Journal of Clinical Trials, 7 (2015), pp. 23-34

*MARAC is a diverse group of SCD providers who have volunteered to provide SCDAA and the sickle cell community with trustworthy advice. The primary advice of SCDAA MARAC is directed toward the sickle cell population in the United States, but we recognize frequent implications for other countries and recognize there is wisdom to gain from other countries.

**SCDAA is the leader in promoting and advancing initiatives focused on people affected by sickle cell conditions worldwide. SCDAA is the largest national community-based organization for sickle cell disease. For 50 years SCDAA and its more than 50 member organizations have demonstrated how community-based organizations can work as partners with medical facilities and local and state government agencies to pursue national health care objectives.

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Who is watching over the study?

IRB (Institutional Review Board) - A special group at a medical center that watches over clinical research safety.

DSMB (Data Safety Monitoring Board) - They regularly go over all study data looking for new problems with safety and whether the study should continue.

DSMC (Data and Safety Monitoring Committee) - Similar responsibilities as the DSMB.

FDA (Food and Drug Administration) – United States government agency that is responsible for drug safety.

THE CIRCLE OF SUPPORT

