



# **RISE UP** **SICKLE CELL** **WARRIOR**

BLAZE,  
Sickle Cell  
Warrior



**LEARN ABOUT THIS NEW  
CLINICAL TRIAL INSIDE  
VISIT [RISEUPSTUDY.COM](https://riseupstudy.com)**

Warriors were compensated and may not have taken part in RISE UP.  
Sickle Cell Warrior is a term coined and used by the sickle cell community.  
It refers to individuals living with sickle cell disease.

# FELLOW WARRIOR,

We take on sickle cell disease together every single day. Now, Agios is standing alongside us to take this fight to the next level with the RISE UP Sickle Cell Clinical Trial.

New teammates can bring uncertainty, but Agios is here giving you the straight facts and providing support every step of the way—if you choose to enroll.

The Warrior community is strong and this trial is a new opportunity to challenge sickle cell through research.

So let's do what we do and **RISE UP**.

## — THE SICKLE CELL WARRIORS

(L to R) GOLIE, PHILL, TRISTIAN, TEONNA, DOMINIQUE, DEMITRIOUS, BLAZE



## A MOVEMENT WITH MOMENTUM

Recently, there've been exciting developments in sickle cell therapies thanks to those in the #SickleCellWarrior movement, who've risen to the challenge, demanded progress, and enrolled in clinical trials.

**Because of Warrior participation**, new research and potential treatments have emerged, each one helping the Warrior community battle this disease.

- In the past 5 years, the number of sickle cell advocates and advocacy groups has grown nearly 5-fold, from 8 to almost 40 today
- In 2017, the first new treatment in 20 years became available for children with sickle cell
- In 2019, 2 more therapies became available
- In 2020, the Food and Drug Administration (FDA) made approving new sickle cell therapies a priority. Now there are over 20 different therapies being studied



## WHY WE'RE FIGHTING NOW

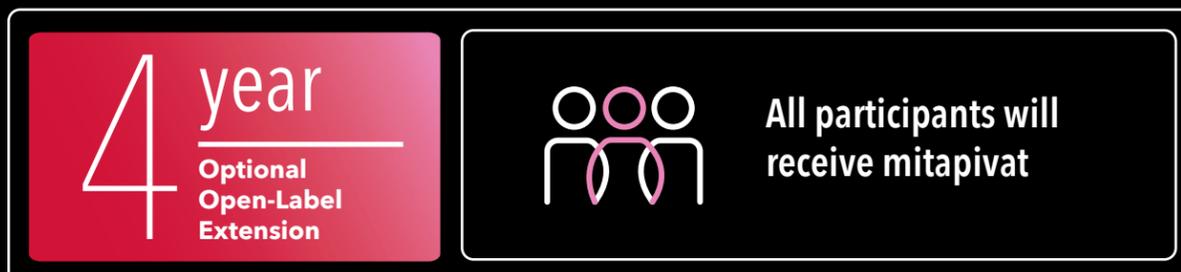
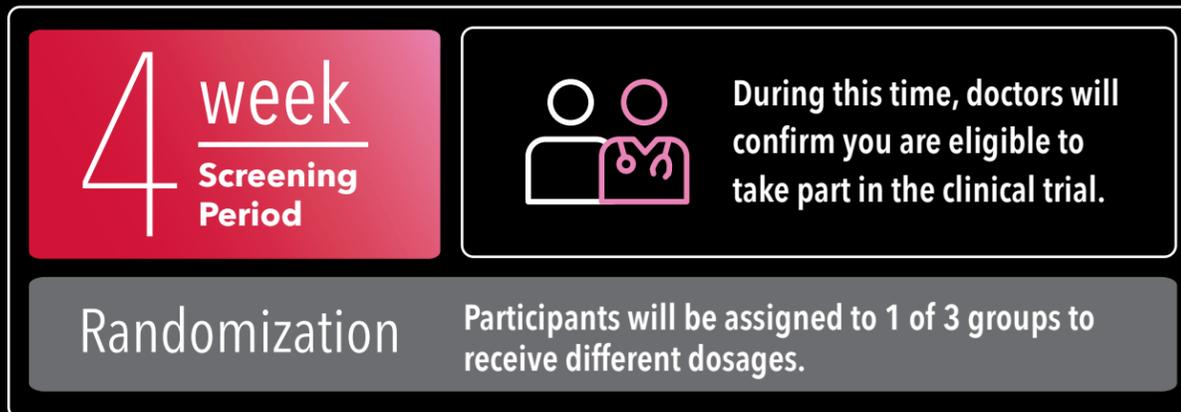
After successfully completing smaller studies, Agios is ready to take the fight against sickle cell disease to a much larger clinical trial. If RISE UP is successful, Agios will seek FDA approval for a new drug called mitapivat for people with sickle cell disease.



## WHAT TO EXPECT IN RISE UP

RISE UP is a phase 2 clinical trial that takes place over a 3-month period.

Participants who complete phase 2 have the option to move into a 4-year extension of the study. Of course, enrollment in this long-term extension period is completely optional.



## REASONS WHY RISE UP MIGHT BE A GOOD FIT

### Eligibility criteria:

- You're 16 years of age or older
- You're diagnosed with sickle cell
- If you or your partner are capable of becoming pregnant, you must agree to use 2 forms of contraception
- You've had at least 2 but not more than 10 pain crises in the past year
  - Defined as pain requiring medical attention and treatment caused by acute chest syndrome, sustained erection, or red blood cell blockages in the liver, spleen, or elsewhere in the body
- You have a hemoglobin level between 5.5 and 10.5 g/dL
- If you are taking hydroxyurea, your dosage must be stable for at least 90 days before starting this study drug



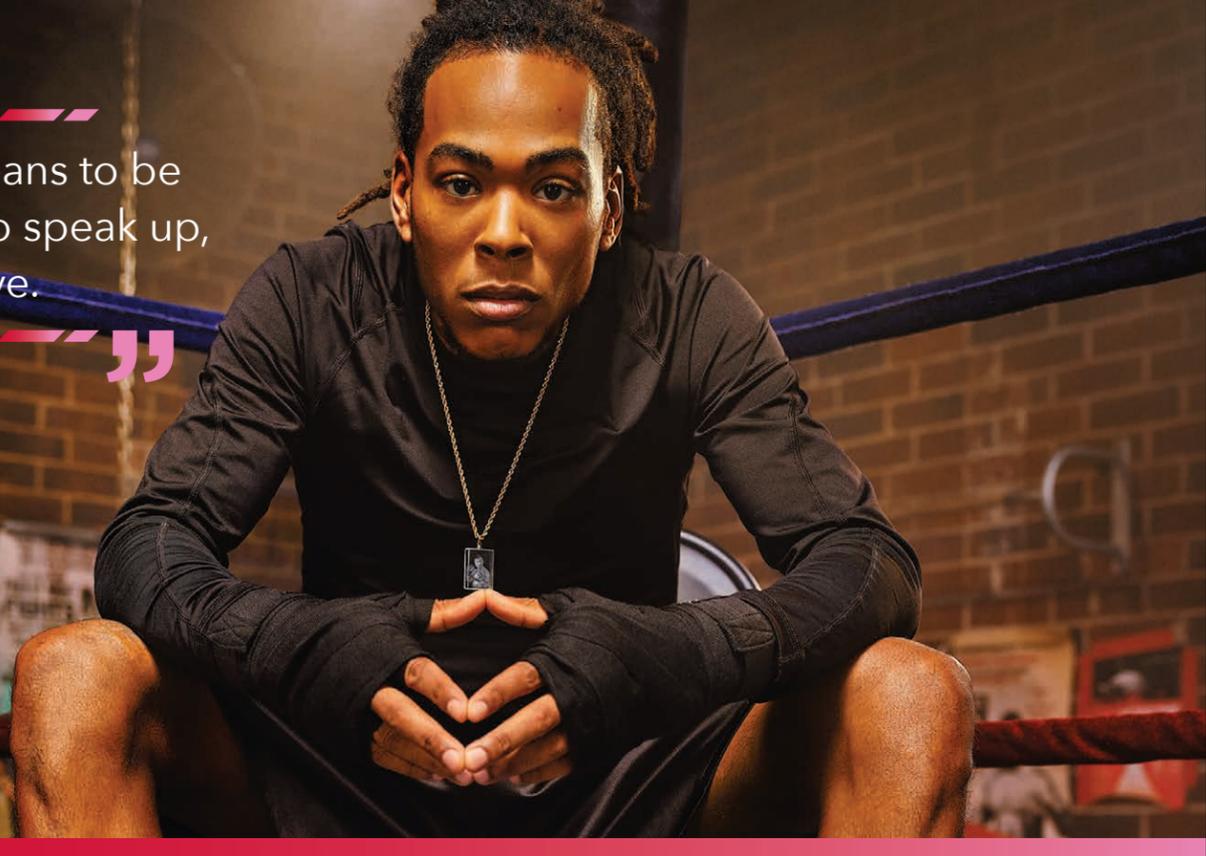
## REASONS RISE UP MIGHT NOT BE A GOOD FIT

- If you're pregnant or breastfeeding
- If you're receiving regularly scheduled transfusions
- If you have any hepatobiliary disorders including liver or gallbladder disease
- If you have severe kidney disease
- If you've undergone gene therapy or had a prior bone marrow or stem cell transplant
- If you're currently receiving treatment for sickle cell disease (such as voxelotor, crizanlizumab, or L-glutamine), with the exception of hydroxyurea
- If you're currently receiving therapies that stimulate blood production such as erythropoietin

**Deciding to enroll in RISE UP is an important decision. Talk with your doctor, your family members, and fellow Warriors about joining the fight.**

“ To RISE UP means to be courageous, to speak up, to be pro-active. ”

DEMITRIOS,  
Sickle Cell Warrior



## GET THE TRUTH

There's a lot of information out there; feel free to ask questions so you're comfortable joining a clinical trial. And if you decide to join, your safety is the number one priority. Learn more in the Myths vs Facts section on the next page.



“ I'm all about expanding access to clinical trials because my family is Nigerian. And so I fight for myself, for my children, and for the broader community. ”

PHILL,  
Sickle Cell Warrior

## MYTHS

## FACTS

**Once you join a trial, you're a human guinea pig.**

**In clinical trials, transparency is key. That's why every trial ensures that the rights of participants are protected through informed consent.**

The informed consent document describes trial activities, visits, and potential risks and benefits. Reading it can help you decide whether or not you want to join. You're not a guinea pig. You're an informed participant.

**Clinical trials aren't transparent about your safety.**

**Open communication and transparency about trial safety is the top priority.**

All clinical trials are reviewed for safety by the Food and Drug Administration (FDA) and an Institutional Review Board (IRB) made up of doctors, scientists, and community members.

These groups work to ensure transparency with participants about both the benefits and risks of study medications.

**Enrolling in a trial is without risk.**

**In clinical trials, participants receive a potential new treatment so that its impacts and risks, if any, can be studied.**

**If there is a clinical trial that I could enter, my doctor will tell me about it.**

**Your doctor may not know about every clinical trial.**

The National Institutes of Health has an online database where you can search for appropriate trials: [clinicaltrials.gov](https://clinicaltrials.gov).

For help navigating your options, talk to your doctor. You can also contact an advocacy group or visit an informative website such as [SCDstudies.com](https://SCDstudies.com).

**If I join a clinical trial, I won't get the same level of care that I receive from my doctor.**

**Trial participants not only receive care from trial staff, but their own doctor as well.**

Trials include very detailed procedures, and often include extra tests and extra visits.



TRISTIAN,  
Sickle Cell Warrior



## WHAT DOES “PLACEBO-CONTROLLED, DOUBLE-BLIND” MEAN?

A placebo is a pill that looks exactly like the study medication but does not contain any medicine. Placebos provide a way to compare the results produced by the investigational medicine. “Double-blind” means that neither you nor your doctor will know whether you’re taking placebo or the study medication, so as not to influence the results or evaluations.



## MY PRIVACY IS IMPORTANT. HOW WILL IT BE PROTECTED?

Throughout the study, your name and all your personal medical information will be kept completely confidential. Each participant in the study will be assigned a unique identifier. Records and data collected throughout the study will not contain your name or personal identifying information, but rather the unique identifier.



## WHAT’S INVOLVED WITH PHASE 2 OF RISE UP IF I JOIN?

You will receive either mitapivat (50 mg or 100 mg) or placebo tablets to take twice a day, every day, for 3 months. Placebos look just like the study drug, but will have no mitapivat in them. Approximately every 2 weeks, you’ll be evaluated by a doctor to measure for changes in anemia, changes in pain crises, and your overall health and safety. After the initial 3 months, you may choose to continue taking mitapivat, or begin taking mitapivat if previously on placebo, for up to approximately 4 years (216 weeks).



## WHAT HAPPENS FIRST IF I DECIDE I WANT TO JOIN?

Before you are fully enrolled as a participant in RISE UP, a medical team will assess your medical history and health to ensure you can participate. See the “Reasons why RISE UP might be a good fit” section to get a better idea if the trial is right for you.



One of my biggest motivations to fight is the people in the sickle cell community.



TEONNA,  
Sickle Cell Warrior

 @sicklequeent



## WHAT HELP CAN I EXPECT DURING THE STUDY?

You have other things happening in your life, and battling sickle cell disease every day is a huge challenge. If you need help to participate in the study, support may be available for:

- Caregivers
- Childcare
- Travel
- Transportation
- Stipends
- Nurses for remote visits
- Virtual visits

The above services depend on local and national regulations. Please check with your local investigator to confirm which services are available in your area. More information about support will be available on your specific enrollment website.



DOMINIQUE,  
Sickle Cell Warrior



“ Having higher participation in clinical trials helps give clarity on the options and opportunities available to us. It’s beneficial for the greater good. ”

GOLIE,  
Sickle Cell Warrior

@golielorenzo



## HOW TO GET STARTED

Like all US clinical trials, RISE UP is registered at [clinicaltrials.gov](https://clinicaltrials.gov). Enrollment begins there.

Visit [clinicaltrials.gov/ct2/show/NCT05031780](https://clinicaltrials.gov/ct2/show/NCT05031780).

Have questions? Call Agios Pharmaceuticals Medical Information at **1-833-228-8474**, Mon-Fri, 9 AM to 7 PM ET, or email [medinfo@agios.com](mailto:medinfo@agios.com).

# JOIN RISE UP TODAY!

DEMITRIOUS,  
Sickle Cell Warrior

GOLIE,  
Sickle Cell Warrior

TEONNA,  
Sickle Cell Warrior

# NOW WE RISE **#SICKLECELLWARRIOR**

STAND ALONGSIDE YOUR FELLOW WARRIORS AND JOIN THE **RISE UP** CLINICAL TRIAL

Connect with fellow Warriors online. Use the #SickleCellWarrior hashtag and visit [RiseUpStudy.com/Warrior-Stories](https://RiseUpStudy.com/Warrior-Stories).



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