

## A Research Study for Children (12 and older) and Adults with Sickle Cell Disease- Zileuton

### What is the purpose of this study?

We find that patients with sickle cell disease (SCD) have inflammation in their blood even when they are well, and their lung function tests suggest a tendency to have asthma. Inflammation and asthma increase the number of acute sickle events. Our laboratory research shows that both inflammation and asthma could be blocked by the drug Zileuton. Zileuton is a drug that has been approved by the Food and Drug Administration (FDA) to treat asthma. The FDA has not approved Zileuton for the treatment of SCD, therefore it will be studied as an investigational drug. The purpose of this research is to study the effects (good and bad) on children and adults with SCD. This study will examine

1. the safety of Zileuton
2. how Zileuton is used by your body at different dose amounts
3. if Zileuton improves the inflammation and tendency to asthma in children and adults with SCD.

### What is involved?

You will be in the research study for approximately 10 weeks in which you would be asked to take Zileuton twice daily for six weeks.

Participation in this research study will involve a total of 6 visits.

### What are the benefits?

If you agree to take part in this research study, you may or may not receive a direct medical benefit. Potential benefits for you may include the possibility of less chronic inflammation that is present even when you are not having an acute sickle event. This chronic inflammation has been associated with increased number of acute sickle cell events. Your lung function studies may show improvement which may prevent you from having frequent sickle events. However, until we do the research study, we cannot guarantee any benefits. Information learned from this research study may benefit other patients with sickle cell disease in the future.

### What are risks?

A detailed list of side effects will be provided to those patients interested in knowing more about the study.

### Will I be paid for participating in this study?

You will receive compensation for your time and efforts associated with your participation in the research study up to \$455.

### Who can participate?

Children and adults who are 12 years or older and have been diagnosed with moderate to severe sickle cell disease (HbSS, HbSC, HbS $\beta^{\text{thal}}$  or HbS $\beta^0$  thalassemia) may be eligible to participate.

### Who should not participate?

You should not be in this study if you have any of the following:

- A history of active hepatitis or active liver disease
- Are HIV positive
- Are pregnant or nursing a child
- Are unable or unwilling to use contraception
- Have taken an investigational drug within the past 4 weeks
- Are currently taking one of the following medications or have taken one in the past 4 weeks:
  - Hydroxyurea
  - A leukotriene antagonist (Singulair) or steroids (Prednisone)
  - Theophylline
  - Coumadin
  - Terfenadine
  - Propranolol
- Require chronic transfusion therapy
- Anything that in the opinion of the investigator would compromise your participation in the study
- Have difficulty swallowing tablets or pills

## What are my rights as a participant in a research study?

Your participation in this research study is completely voluntary. Your decision whether or not to participate in this study will not result in any penalty or loss of benefits. If you decide not to participate in the study, or if you decide to leave the study before it is completed, standard medical care will still be available to you.

## Whom should I contact for more information?

The Comprehensive Sickle Center

Phone: 513-636-6770

E-mail: [sicklecell@cchmc.org](mailto:sicklecell@cchmc.org)

Or

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