

# THE EPIC STUDY

EVALUATING A NEW STUDY DRUG  
FOR A SICKLE CELL CRISIS



INFORMATION FOR FAMILIES

## What Is A Clinical Trial?

- Medical research study for new treatments
- Conducted to determine if a treatment is safe and effective
- U.S. federal agencies oversee medical research
- Participation is always voluntary



## The EPIC Study of MST-188

- MST-188 is a new investigational agent given during sickle cell crisis
- Aim of the EPIC Study is to evaluate if MST-188 can shorten the duration of a crisis
- Individuals 4 to 65 years old
- MST-188 is given in addition to regular pain management and treatment
- No cost to participants



## If MST-188 is Successful ...

It will be a new drug to help treat a painful sickle cell crisis.

- May improve blood flow
- Crisis may end sooner
- Could shorten time in the hospital





## Normal vs Sickle Red Blood Cells

### Normal red blood cells

- Soft and flexible
- Deliver oxygen well
- Live 30-90 days



Normal red  
blood cell

### Sickled red blood cells

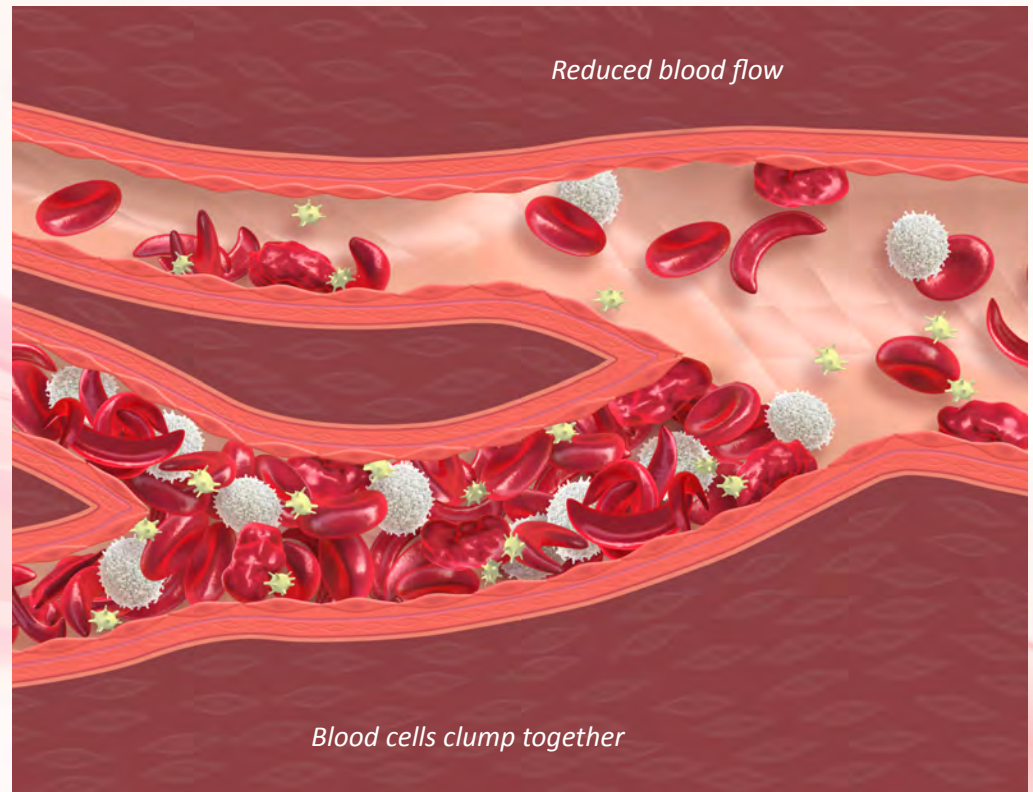
- Rigid and sickle-shaped
- Deliver oxygen poorly
- Sticky surfaces
- Live 10-12 days



Sickled red  
blood cell

## What is Sickle Cell Crisis?

- Also known as “Vaso-Occlusive Crisis”
- Red blood cells clump together
- Cells block blood flow



ARTIST ILLUSTRATION

## The Most Common Symptom of Sickle Cell Crisis is Pain



### Complications of Inadequate Blood Flow

#### Short Term

- Acute chest syndrome
- Anemia
- Fatigue/tiredness

#### Long Term

- Bone disease
- Lung disease
- Organ failure
- Stroke



- Current medications for sickle cell crisis are primarily for pain management
- New therapies that shorten the duration of a crisis and improve blood flow are needed

## Common Therapies:

### Medications

- Bronchodilators
- Oral pain medicine and patient-controlled analgesia
- Steroids

### Treatments

- Blood transfusions
- IV fluids
- Oxygen



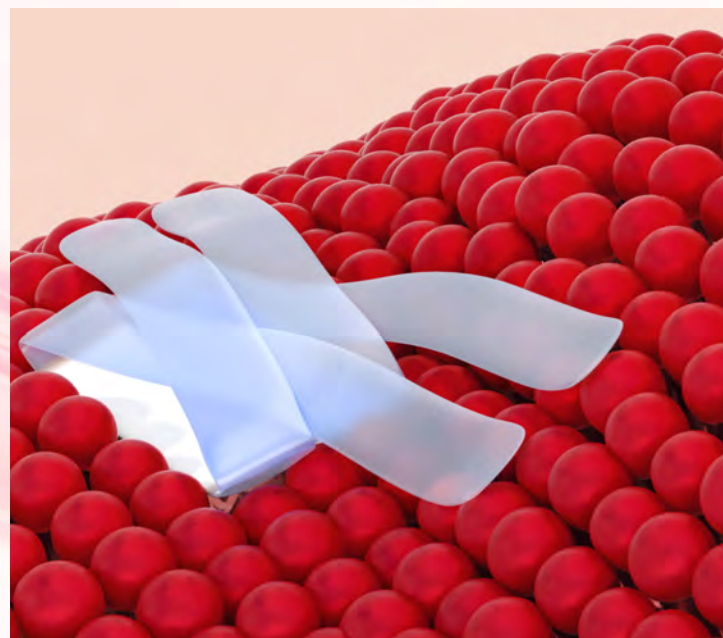


## What Does MST-188 Do?

MST-188 attaches to damaged areas of cell surfaces

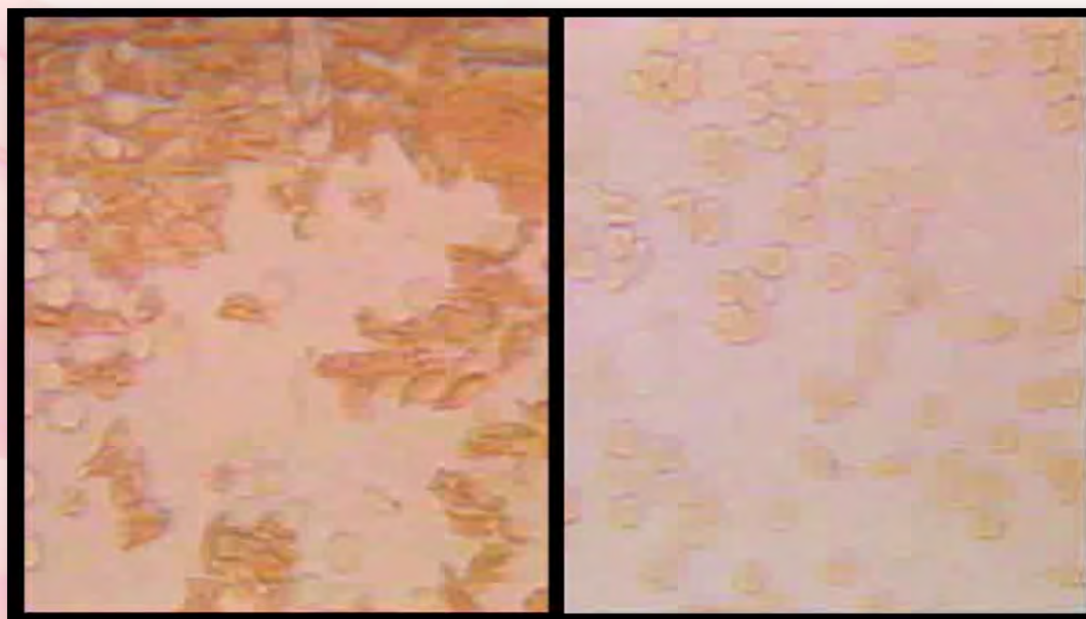


MST-188  
(Purified Poloxamer 188)



ARTIST ILLUSTRATION

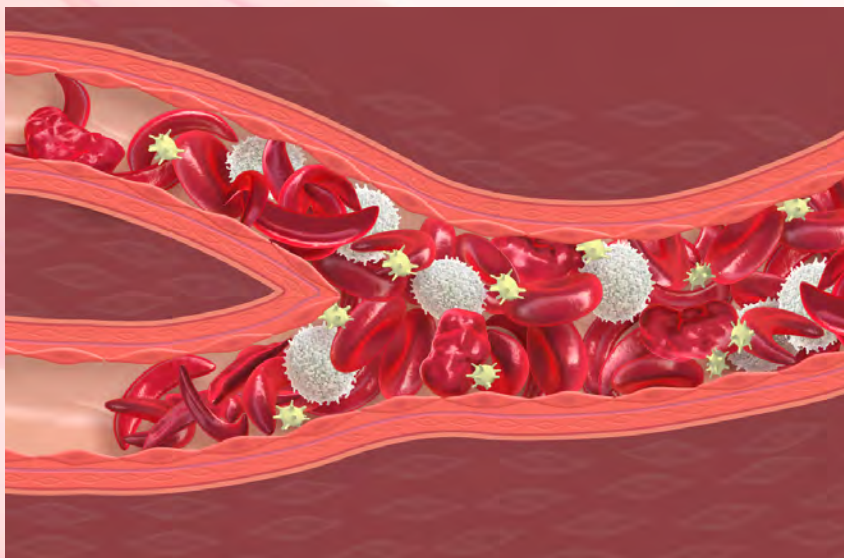
## Laboratory Studies of MST-188 Show it Increases Blood Flow



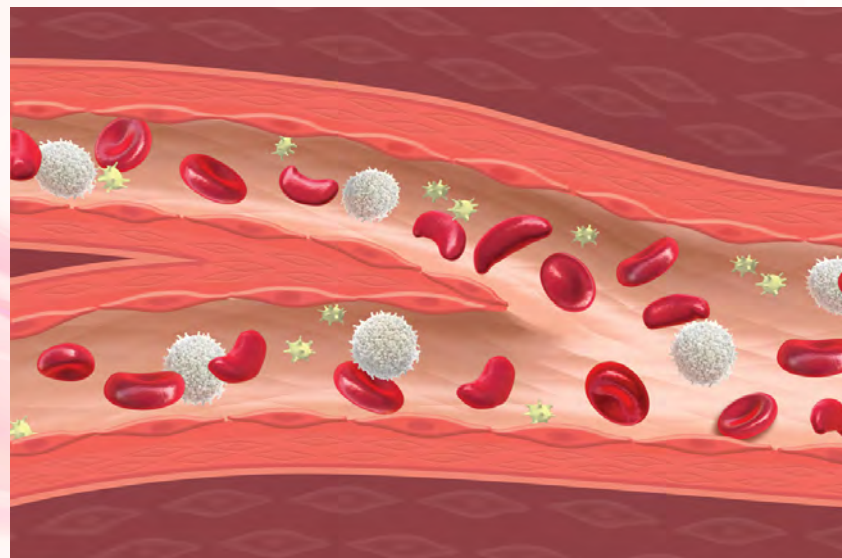
*Without MST-188*

*With MST-188*

## MST-188 May Shorten the Length of a Sickle Cell Crisis



Before MST-188



After MST-188

ARTIST ILLUSTRATION



## Previous MST-188 Experience in Sickle Cell Disease

- More than 200 sickle cell patients
- Over 70 children with sickle cell disease
- MST-188 was generally well tolerated in prior clinical trials
- Speak with your doctor for information about specific safety findings and adverse events observed in prior clinical trials



## Who Can Participate?

- Patients with sickle cell disease
- At least 4 years old and no older than 65
- Hospitalized for a crisis no more than 6 times in the past 6 months

*Talk to your doctor about other criteria*



## What Will Happen During Participation?

- All patients receive the standard care for crisis
- Regular pain management will be given
- Half of the patients will ALSO receive MST-188
- No cost or obligation to participate
- Blood samples will be taken at designated times

*Talk to your doctor about specific safety findings  
and adverse events observed in prior clinical trials*





## How Many Patients Will Participate in the EPIC Study?

- 388 patients will be treated in a hospital setting in the U.S. and other countries
- 194 patients will receive MST-188



## Why Participate in the EPIC Study?

- All participants will receive standard pain management
- May be able to leave the hospital sooner
- No cost to participate
- Your participation may help others with sickle cell disease



## How to Sign Up for the EPIC Study

Talk to your doctor and study nurse  
about the consenting process





## What To Do If A Crisis May Be Starting

- Immediately contact your doctor
- Go to the study hospital as soon as your doctor advises
- It is important to go to the study hospital within 24 hours of the start of pain



## Who to Call

### Who to Call:

Name : Dr. Charles Quinn

Phone: 513-636-6770

### Where to go:

Name: Cincinnati Children's Hospital Medical Center

Address: 3333 Burnet Avenue, Cincinnati, OH 45229



## When You Need Information about EPIC

- Always call the study contact number first
- The study team is your primary source of information
- Call your doctor for additional information about the study or your situation





## Information About Sickle Cell Disease



[TheEPICstudy.com](http://TheEPICstudy.com)



Centers for Disease Control and Prevention



Children's Sickle Cell Foundation



National Human Genome Research Institute



National Institute of Health



Sickle Cell Disease Association of America



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Thank You