



# ELEVATING COMMUNITY VOICES IN THE ICER REVIEW:

## THE VALUE OF GENE THERAPIES IN SICKLE CELL DISEASE

TOOLKIT TO PROMOTE  
ADVOCACY DURING THE  
**2023 ICER SCD REVIEW**

# OVERVIEW

Sickle cell disease (**SCD**) is a rare blood disorder that affects over 100,000 individuals in the United States. SCD is a debilitating chronic disease that affects patients and their families mentally, physically, and financially. After many years of research, **two emerging gene therapies**, lovo-cel and exa-cel, offer hope for a potential clinical cure for SCD.

The Institute for Clinical and Economic Review (**ICER**) is a healthcare nonprofit that measures how well prescription drugs work and suggests a price for those drugs through a **value assessment**. In April 2023, ICER released a Draft Evidence Report on Gene Therapies for Sickle Cell Disease. Read the report [here](#).

Given how the ICER report could greatly impact SCD warriors, such as changing the cost or accessibility to gene therapies, there is a need for the SCD community to make our voices heard.

**This toolkit will help provide information on the ICER process and how to bring your voices and opinions to ICER.**

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# 2023

## ICER SCD TIMELINE

MAY  
09

### Public Comment Period

Join the community in **submitting comments** to ICER to share your perspective about the Draft Evidence Report.

**SUBMIT A PUBLIC COMMENT!**

JUL  
13

### Updated Evidence Report

Review updates to ICER's clinical and economic analysis and any additional insights included from the patient community.

**SHARE WITH SCD ADVOCATES!**

JUL  
27

### ICER Public Meeting

Watch the **full-day virtual event**, including the ICER presentation and community testimonies.

**PARTICIPATE IN ICER'S MEETING!**

AUG  
21

### Final Evidence Report

Use the final ICER report to **engage with payers** and ensure that patient perspectives are at the center of coverage decisions

**SHARE YOUR EXPERIENCE!**

Review ICER's full timeline and materials [here](#).



# WAYS TO ENGAGE

1

## SUBMIT PUBLIC COMMENTS TO ICER

During the **Public Comment Period**, stakeholders can submit comment letters to ICER. Letters are due to ICER by **May 9th, 2023**. Email your letter to ICER at [publiccomments@icer.org](mailto:publiccomments@icer.org).

2

## REGISTER FOR THE PUBLIC MEETING

ICER will host a virtual public meeting on **July 27, 2023**. Register [here](#) to join the meeting and hear the results.

3

## ENCOURAGE YOUR COMMUNITY TO PARTICIPATE

Tell others in your networks about the ICER review and opportunities to engage. **Share this toolkit** and spread the key messages.

4

## ENGAGE WITH PAYER AND POLICY DECISION-MAKERS

Discuss the **limitations of the ICER report** with other payer stakeholders. Learn more about advocacy opportunities through the [Sick Cells Ambassador Program](#).

5

## SHARE ON SOCIAL MEDIA

Engage in social media **to raise awareness** about key issues and gather support from other community members. Check out the guide below.

## SOCIAL MEDIA GUIDE

#Sicklecell

#Advocacy

#access

#ICER

#SCDAdvocates

#genetherapy

#ICERSicklecell

### Twitter Tags:

@SickCells @icer\_review

### Messages for Social Media:

ICER is assessing the value of #genetherapy for SCD. They need you to #advocate for accessible treatment options. #sicklecell #ICERSicklecell #SCDadvocates

Let @ICER\_review know what it's like to live a day in your shoes and why there is an urgent need for a #SCDcure. #sicklecell #genetherapy #ICERSicklecell

Share your #sicklecellstory with #ICER and help ensure warriors can equitably access new treatments. #genetherapy #ICERSicklecell #advocacy #healthequity

# KEY MESSAGES

Join the community in **urging ICER** to incorporate the **community perspectives** in their economic analysis and express concerns on the following **key messages**:



## Urgent Need for Treatment Options

- There is a **high unmet need** for medicine to treat SCD, as current treatment is insufficient.

### Recommendation:

Curative treatments are needed to improve **longevity and quality of life**.



## Societal Costs

- Sickle cell disease causes billions of dollars in **lost wages and productivity yearly** and many other **financial impacts** on individuals and families.

### Recommendation:

ICER needs to include these **societal costs** in the base analysis.



## Value Defined By Patients

- **Improvement in quality of life** is the outcome patients **value most**, however, ICER defined efficacy by reduction in VOCs.

### Recommendation:

Value needs to be measured based on **what matters to patients**.



## Annual Pain Crises

- ICER's model assumes that patients have **four pain crises** per year. Studies suggest this is likely low and **missing pain events treated at home**.

### Recommendation:

ICER's model must reflect **treatment of pain outside the hospital**.



## Lack of Evidence to Support Conclusion

- Important **evidence is missing**, such as the impact of treatment on chronic pain, fatigue, anxiety, and depression.

### Recommendation:

ICER needs to hold the review until **adequate evidence is available**.



## Opportunity to Promote Health Equity

- Historically ICER's assessments for SCD give payers justification to **create access barriers and cause inequities**.

### Recommendation:

ICER must advance methods that **support health equity** in this review.

# RESOURCES

- 1 • [ICER Draft Evidence Report on Gene Therapy for SCD](#)
- 2 • [ICER Requirements to Submit Public Comments](#)
- 3 • [Registration for ICER Public Meeting on SCD](#)
- 4 • [Sick Cells' Definitions of Patient-Important Disease Impacts](#)

## Check out these additional resources from Sick Cells:

To learn more about getting involved in advocacy programs and opportunities:

[\*\*Ambassador Program\*\*](#)

To learn more about value assessments and the 2019 ICER Review for SCD:

[\*\*Value & Cost\*\*](#)

To learn more about promoting health equity in value assessments:

[\*\*"Finding Equity in Value" whitepaper\*\*](#)

## ABOUT SICK CELLS

Sick Cells is a national advocacy organization for sickle cell disease. Sick Cells' mission is to elevate the voices of the SCD community and stories of resilience. In highlighting the community's grave disparities, we influence decision makers and propel change.



**Questions? Contact us at [info@sickcells.org](mailto:info@sickcells.org)**

