

# The ICER Draft Evidence Report on Sickle Cell Disease: An Overview, Next Steps, and Expectations



# Introductions

- Welcome

**Dr. Lakiea Bailey**

*The Sickle Cell Community Consortium*

- Overview of the ICER Timeline, Draft Report, and Next Steps

**Ashley Valentine and Maggie Jalowsky**

*Sick Cells*

- Sickle Cell Community Collaboration and Questions

**Beverley Francis-Gibson**

*Sickle Cell Disease Association of America, Inc.*

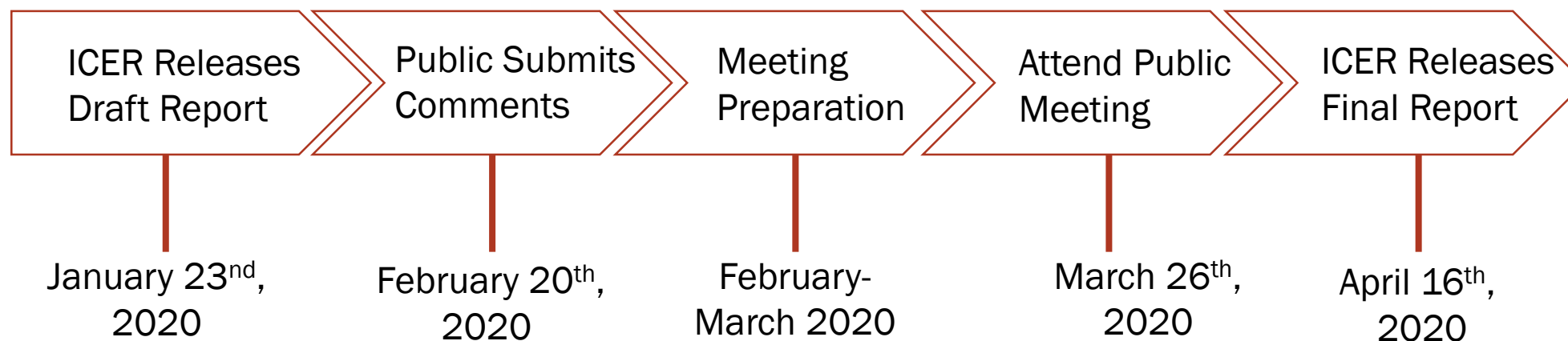


# Where Are We Now?

## ICER's Timeline for SCD Review



# ICER'S TIMELINE FOR SCD REVIEW



- ICER released their **draft evidence report** on January 23, 2020
- The report, along with more information about the timeline for the review, can be found on their website: <https://icer-review.org/topic/sickle-cell-disease/>



# ICER'S TIMELINE FOR SCD REVIEW



## The Draft Evidence Report: January 23<sup>rd</sup>, 2020

- **What is ICER's Draft Evidence Report?**
  - Includes background and context about sickle cell disease
  - Reviews the evidence and data that ICER uses in its report
  - Provides preliminary “cost-effectiveness analyses”
  - Recommends a price that ICER believes is fair for these SCD treatments
- This is a **draft version** of the report, though rarely will ICER make major changes to the model after the draft report is published.



# ICER'S TIMELINE FOR SCD REVIEW

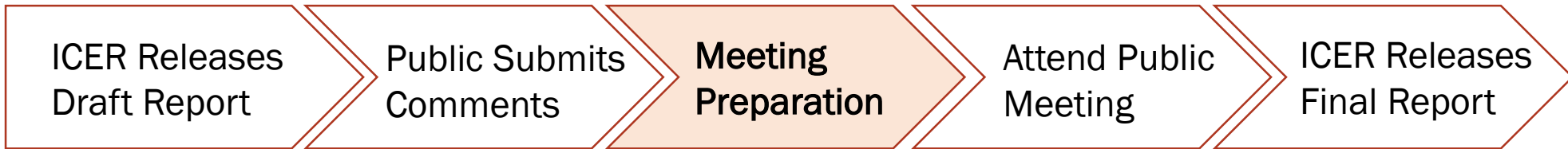


**Public Comments Period:**  
**Jan 23<sup>rd</sup> – Feb 20<sup>th</sup>, 2020**

- The **public comment period is now open**, and will remain open for **four weeks**.
- Public comments are due on February 20, 2020 at 5pm.
- The Public Comment Period ensures all stakeholders have the opportunity to provide input to ICER's evidence report.
- Written comments, including those submitted by individual patients, will be posted publicly to the ICER website.



# ICER'S TIMELINE FOR SCD REVIEW



## Public Meeting Preparation February and March 2020

- The **Meeting Agenda** and **Discussion Questions** are posted on ICER's website.
- Patients and community members will be invited to speak at the public meeting.
- If you are interested in getting involved in advocacy effort and storytelling, reach out to [thayer@pipcpatients.org](mailto:thayer@pipcpatients.org)



# ICER'S TIMELINE FOR SCD REVIEW



## Attend the Public Meeting: March 26<sup>th</sup>, 2020

- The Public Meeting will be hosted in Boston on March 26<sup>th</sup>, 2020.
- The meeting will consist of:
  - a review of the report's findings
  - a policy roundtable discussion
  - a vote on the cost-effectiveness of the treatments
- The meeting is **open to the public** and we are gathering advocates to attend in person. ICER will also live stream the meeting.





# ICER'S TIMELINE FOR SCD REVIEW



## ICER Releases Final Evidence Report: April 16<sup>th</sup>, 2020

- The final report will summarize the overall findings of the report and meeting.
- This will be released on April 16, 2020.
- This is the report that may be utilized by payers or other stakeholders.



# Overview of the Draft Evidence Report



# Draft Evidence Report:

## Patient Perspectives

- The robust summary of patient perspectives demonstrates ICER's efforts to include the patient voice.
- It is not clear this has impacted the pricing/cost models.
- Some analyses will be updated using data from the **“My Life with Sickle Cell Disease” Survey**
  - Current Total is **231 respondents** (The goal is 300!)
  - Take Survey here: : <https://www.surveymonkey.com/r/mylifewithSCD>



# Draft Evidence Report:

## Cost-Effectiveness Analyses

- Based on current analyses, it's likely for ICER to determine the three sickle cell disease treatments are not cost effective.



# Draft Evidence Report:

## Scope of the Assessment

- The majority of the evidence was abstracted from randomized controlled trials
- Given the lack of long-term outcome and safety data, there is significant uncertainty about the ICER's ability to understand the benefits that patients will receive from these new treatments.



# Draft Evidence Report:

## Comparators

- The analysis compare each intervention to “optimal usual care” as provided in clinical trials.
- The usual care provided in the trials differs from the real-world usual care experienced by most patients with SCD.
  - American Society for Hematology has clear guidelines on transfusions for patients with SCD. These do not appear to be captured in the report.



# Draft Evidence Report:

## Key Measure of Benefit

- Clinical trials of new therapies focused on outcomes, such as VOCs, that can be easily measured to demonstrate clinical effectiveness.
- Without meaningfully reflecting the other acute and chronic outcomes that matter to patients, a value assessment is incomplete.



# Draft Evidence Report:

## Definition of Pain Crises

- Report replies on measuring acute pain crises in which the patient sought medical interventions, like going to an emergency department to receive treatment.
- Most sickle cell disease patients will acknowledge that it is standard to manage most pain crises at home.





# Draft Evidence Report:

## Lifespan assumptions

- Report assumes that the average lifespan of a SCD patient under usual care is 45 years of age and then proceeds to make assumption about gained years from these new treatments.
- At this juncture there is not adequate data to support these assumptions.



# Draft Evidence Report:

## Use of the Quality-Adjusted Life Year (QALY)

- We discussed the QALY in depth on our January 16 webinar. If you would like to refresh the webinar can be found: <http://www.pipcpatients.org/voh-sickle-cell-webinar.html>
- Given the complex nature of SCD, its severity, and the fact that the burden falls primarily on minority populations, the QALY is a poor method for evaluating any valued accrued from interventions aimed at its treatment.
- Disease-specific metrics would have better captured value to patients.



# What's Ahead?

## Next Steps for Patients and Advocates



# What's Ahead:

## Submit Your Comments!

- Please take some time to digest what you have learned today, and review the report: [View the Report Here](#)
- How To Submit Comments:
  1. Advocates may submit comments directly to ICER by emailing comments to [publiccomments@icer-review.org](mailto:publiccomments@icer-review.org). Comments should be attached as a word document in 12-pt Times New Roman font
  2. Advocates may also submit comments using Sick Cells' Public Comment Submission Form. Sick Cells will then format and submit all comments directly to ICER. Go to [sickcells.org/shareyourstory](http://sickcells.org/shareyourstory)

\*Comments are due by Thursday, February 20<sup>th</sup>.



# How to Stay Engaged:

- Follow [valueourhealth.org/sicklecell](http://valueourhealth.org/sicklecell)
- Plan to attend the Public Meeting on March 26th
- Join PIPC and other SCD advocates to **elevate the patients' voice through storytelling** and other efforts
- Reach out to [Thayer@pipcpatients.org](mailto:Thayer@pipcpatients.org) if you are interested in getting involved.



# Questions?

