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
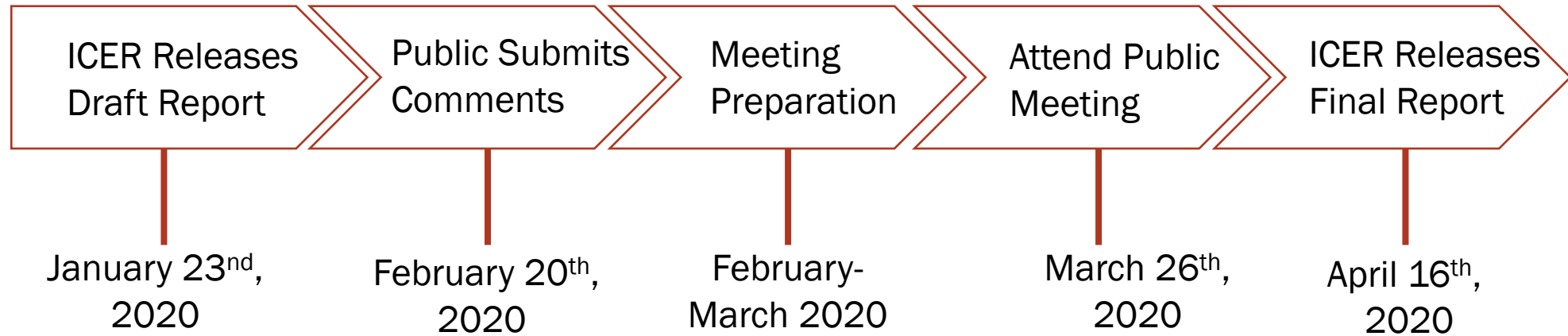 *Sickle Cell Disease Association of America, Inc.*

Where Are We Now?
ICER's Timeline for SCD Review

ICER'S TIMELINE FOR SCD REVIEW

ICER Releases Draft Report	Public Submits Comments	Meeting Preparation	Attend Public Meeting	ICER Releases Final Report
January 23nd, 2020	February 20th, 2020	February-March 2020	March 26th, 2020	April 16th, 2020

- 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/

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The Draft Evidence Report:
January 23rd, 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.

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ICER Releases Draft Report › Public Submits Comments › Meeting Preparation › Attend Public Meeting › ICER Releases Final Report

Public Comments Period:
Jan 23rd – Feb 20th, 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

ICER Releases Draft Report → Public Submits Comments → **Meeting Preparation** → Attend Public Meeting → ICER Releases Final Report

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

ICER'S TIMELINE FOR SCD REVIEW

ICER Releases Draft Report → Public Submits Comments → Meeting Preparation → **Attend Public Meeting** → ICER Releases Final Report

Attend the Public Meeting:
March 26th, 2020

- The Public Meeting will be hosted in Boston on March 26th, 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 Draft Report | Public Submits Comments | Meeting Preparation | Attend Public Meeting | ICER Releases Final Report |

ICER Releases Final Evidence Report:
April 16th, 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. 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

*Comments are due by Thursday, February 20th.

How to Stay Engaged:

- Follow 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 if you are interested in getting involved.

Questions?