

**WEBINAR SERIES ON VALUE ASSESSMENTS  
FOR SICKLE CELL DISEASE**

**PART 4:  
“CALL TO ACTION FOR THE SICKLE CELL  
ICER REVIEW”**

**January 10<sup>th</sup>, 2020**

The logo for SICK CELLS is a red circle with a white shadow on the right side, giving it a 3D effect. The words "SICK" and "CELLS" are written in white, bold, sans-serif capital letters, stacked vertically inside the circle. A vertical grey bar is positioned to the left of the top of the circle, and a horizontal grey bar is positioned to the left of the bottom of the circle.

**SICK  
CELLS**



# WHO WE ARE

- **Sick Cells** is a non-profit organization that seeks to **elevate the voices** of the sickle cell disease (SCD) community and their stories of resilience.
- We work with fellow allies across the country to influence policy makers, educators, employers, healthcare administrators and healthcare providers to act to improve treatment and care for the SCD population.





# WEBINAR SERIES

Sick Cells is hosting a four-part webinar series on:

## VALUE ASSESSMENTS FOR SICKLE CELL DISEASE

- PART 1: FDA Approval, Now What? Friday, December 6<sup>th</sup>
- PART 2: Drug Pricing & Coverage Decisions Friday, December 13<sup>th</sup>
- PART 3: Examining the “Cost” of SCD Friday December 20<sup>th</sup>
- PART 4: Call to Action During the ICER Review Friday, January 10<sup>th</sup>



# OBJECTIVES

## PART 4: CALL TO ACTION FOR SCD ICER REVIEW

### Today we will:

- Summarize information shared throughout this webinar series
- Highlight how the community's actions will impact the ICER review

### This webinar session is intended to:

- Get our SCD community engaged during the final months of the ICER review



# ASSESSING VALUE FOR SICKLE CELL DISEASE

## THE ICER REVIEW OF SCD

ICER is assessing the value of **three** treatments for sickle cell disease:

### GBT's Oxbryta (voxelotor)

- FDA Approved in November 2019



### Novartis' Adakveo (crizanlizumab)

- FDA Approved in November 2019



### Emmaus' Endari (L-glutamine)

- FDA Approved in July 2017





# DETERMINING THE VALUE

- The Institute for Clinical and Economic Review (ICER) uses a **value assessment framework** to produce reports on the value of treatments.
- Value assessments **analyze the clinical and economic value** of treatments.
  - They use health economics to compare the costs and outcomes (effects)
- Insurance companies and other stakeholders may use ICER's assessments when determining if they will cover new medicines. This may **impact access and affordability** of these much-needed new medicines **in the future**.





# THE PATIENT VOICE IN VALUE

- Value means different things to different people – what matters to patients is not limited to measured “clinical” outcomes.
- **The patient voice is critical** for understanding how vast the need is for patients to be able to access new medicines.
- Your story ensures that ICER understands the impact that SCD is having in America and the **desperate need for new and different treatments.**

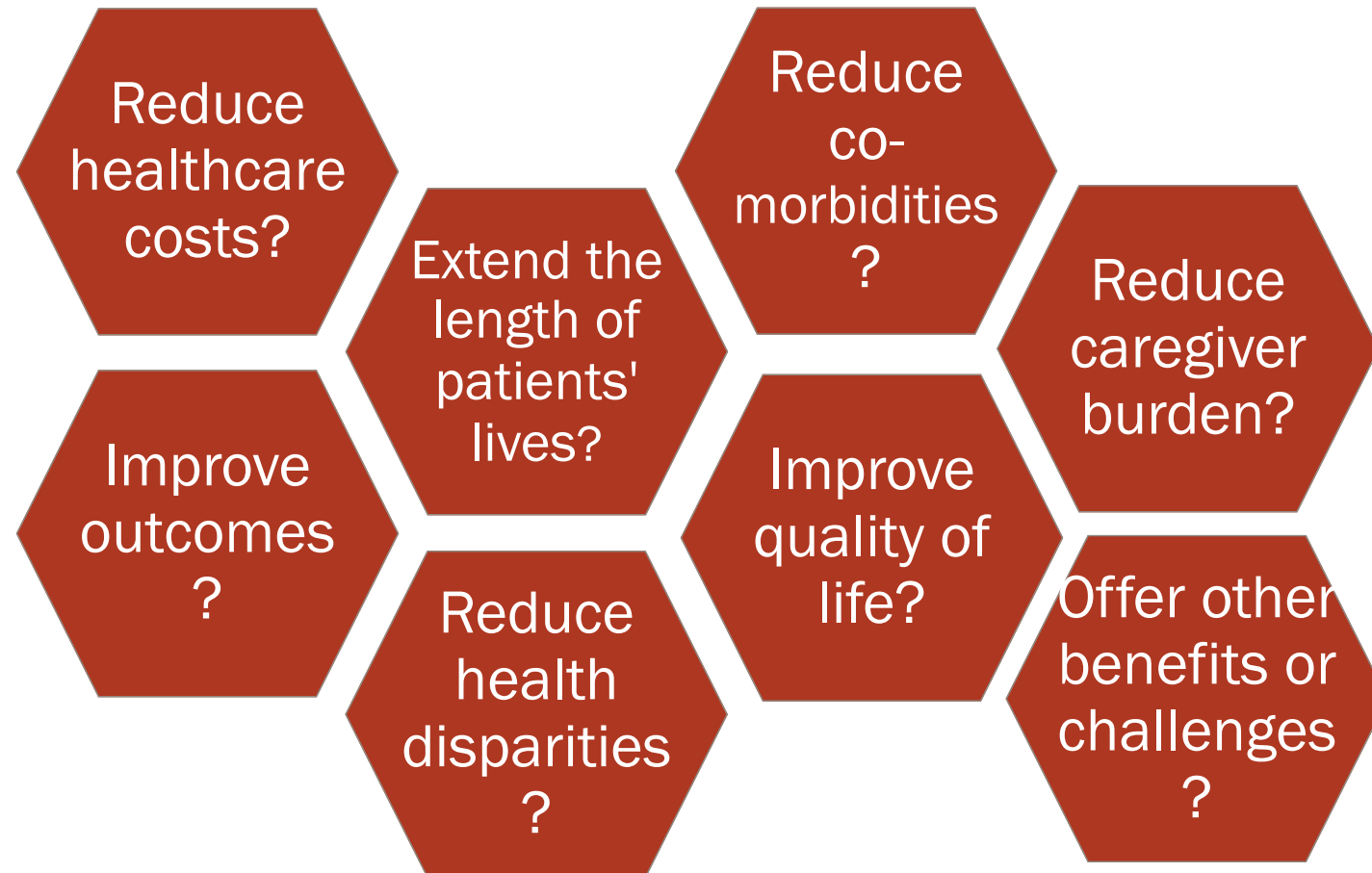




# VALUE AND COST EFFECTIVENESS

What is the **VALUE** of these new treatments?

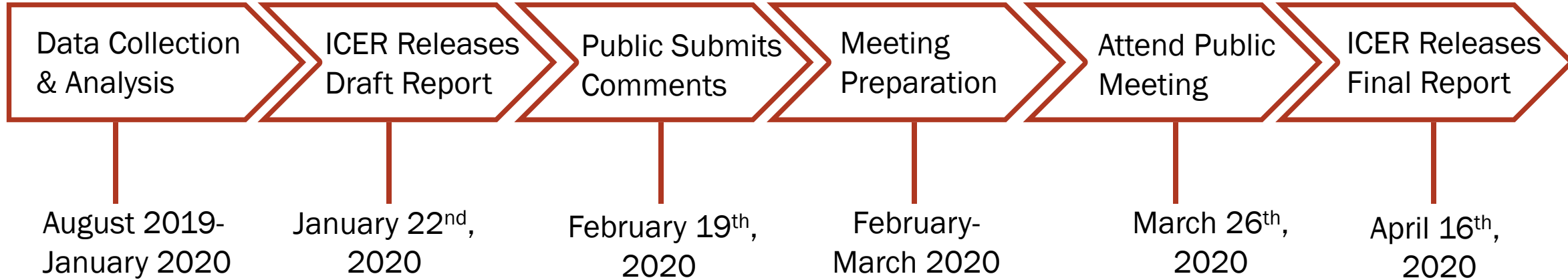
How effectively can each drug...







# ICER'S TIMELINE FOR SCD REVIEW



- More information about ICER's timeline for Sickle Cell Disease review can be found on their website: <https://icer-review.org/topic/sickle-cell-disease/>



# ICER'S TIMELINE FOR SCD REVIEW



## Data Collection and Analysis:

**August 2019 – January 2020** (*currently underway*)

- Once a topic has been announced, ICER begins collecting data for their report
- Drug manufacturers or other stakeholders may have evidence that they share with ICER to help ensure that the review contain the best possible information
- Sick Cells and ICER have worked together to identify areas where data may be missing

### HOW TO GET INVOLVED:

- Complete the “My Life with Sickle Cell Disease: Patient and Caregiver Survey”
- **Survey will go live next Thursday (January 16)** and will be available online.



# ICER'S TIMELINE FOR SCD REVIEW



## ICER Releases the Draft Evidence Report: January 22<sup>nd</sup>, 2020

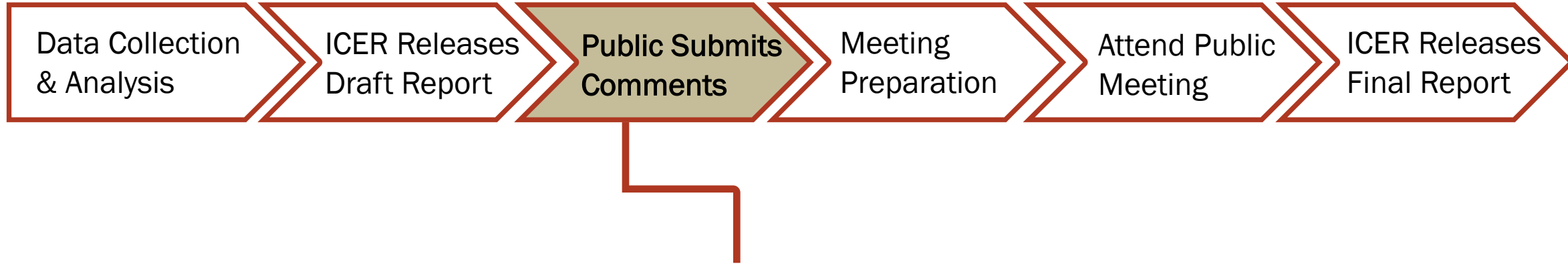
- The **Draft Evidence Reports** will include the “cost-effectiveness analyses” and will recommend a price range that ICER believes is fair for these new drugs.
- This is a draft version of the report.

### HOW TO GET INVOLVED:

- Join webinars hosted by the SCD Community and PIPC to help explain details included in the Draft Evidence Report (**the PIPC Webinar is scheduled for next Thursday, January 16<sup>th</sup>**)



# ICER'S TIMELINE FOR SCD REVIEW



## Public Submits Comments on Draft Evidence Report: Jan 22<sup>nd</sup> – Feb 19<sup>th</sup>, 2020

- The Public Comment Period ensures all stakeholders have the opportunity to provide input to ICER's evidence report.
- Report are open to public comment for **four weeks**.
- Written comments, including those submitted by individual patients, will be posted publicly to the ICER website

### HOW TO GET INVOLVED:

- Write a comment to ICER during this time period. **We need you to share your story!**



# ICER'S TIMELINE FOR SCD REVIEW



## Public Meeting Preparation February and March 2020

- The **Meeting Agenda** and **Discussion Questions** will be posted in February, in preparation for the public meeting.
- Patients and community members are invited to speak at the public meeting. Those wishing to speak at the meeting must contact ICER via email.

### HOW TO GET INVOLVED:

- Volunteer to speak at the public meeting in March or plan to attend as an advocate for the community.



# 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.
- ICER will present the evidence report to a board composed of provider groups, public and private payers, patient groups, and purchasers. They will vote on the overall strength of ICER's report.
- The meeting will be live streaming.

### HOW TO GET INVOLVED:

- Join us (or tune in) for the public meeting



# ICER'S TIMELINE FOR SCD REVIEW



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

- Final Evidence Report and Meeting Summary will be published to ICER's website.
- These are the final materials that may be utilized by payers and other stakeholders.

### HOW TO GET INVOLVED:

- Stay engaged for future value assessments and new treatments in the pipeline



# CALL TO ACTION

Community Action	Impact on ICER Review
<b>1.</b> Complete the Patient and Caregiver Survey	<ul style="list-style-type: none"><li>• <b>Measures the impact</b> SCD has on your school, work, and family life, including caregivers</li><li>• Calculates how much you spend each year to try and manage your disease (<b>out-of-pocket costs</b>)</li></ul>
<b>2.</b> Submit your story during the Public Comment Period	<ul style="list-style-type: none"><li>• Emphasizes the negative impact that <b>fatigue</b> and other complications of SCD have on your life</li><li>• Provides an estimate the <b>direct and indirect costs</b> (missed work days, missed school, etc.) that result from your condition</li></ul>
<b>3.</b> Attend the Public Meeting in Boston	<ul style="list-style-type: none"><li>• Informs the final report and the policy implications</li><li>• Influences the voting from ICER's evidence report</li></ul>





# OUT-OF-POCKET COSTS

- **Out-of-pocket costs** include deductibles, co-pays, co-insurance for covered services plus all costs for non-covered expenses.
- On average, how much do you spend out-of-pocket **per month** for sickle cell disease and its complications on the following?
  - Medications (both prescription and over the counter)
  - Vitamins or nutritional supplements
  - Paid caregivers or support services
  - Medical supplies (such as wheelchairs, canes, bandages)
  - Accessibility improvements to your home
  - Transportation and parking for appointments
  - Pain management techniques (such as message, yoga, meditation)
  - Other costs



# OUT-OF-POCKET COSTS

## EXAMPLE:

- On average, how much do you spend out-of-pocket **per month** for sickle cell disease and its complications on the following?
  - Medications (both prescription and over the counter) = **\$84**
  - Vitamins or nutritional supplements =
  - Paid caregivers or support services =
  - Medical supplies (such as wheelchairs, canes, bandages) = **\$3,936.56**
  - Accessibility improvements to your home =
  - Transportation and parking for appointments = **\$62**
  - Pain management techniques (such as message, yoga, meditation) = **\$60**
  - Other costs = **\$650**



# STAY ENGAGED

- Stay tuned to the Sick Cells website and social media pages!
- Fill out the “**My Life with Sickle Cell Disease: Patient and Caregiver Survey**” opening next Thursday (January 16<sup>th</sup>)
- Join us and other SCD community members on PIPC’s “An Introduction to ICER and Their Implications for New Sickle Cell Treatments” - January 16, 2020, 2:00-3:00 PM ET
  - **[Webinar Details](#)**
  - RSVP to **[dgleason@pipcpatients.org](mailto:dgleason@pipcpatients.org)**
- Reach out to **Sick Cells at [mjalowsky@sickcells.org](mailto:mjalowsky@sickcells.org)**



# Q&A SESSION