

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

**PART 1:  
“FDA APPROVAL, NOW WHAT?”**

**December 6<sup>th</sup>, 2019**





# 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 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 1: FDA APPROVAL, NOW WHAT?

### Today we will discuss:

- FDA's approval process
- Value assessments and their impact on patients

### This webinar series is intended to:

- Identify decisions that are made as new treatments emerge on the market
- Discuss the importance of having the patient community engaged throughout value assessments



# FDA APPROVAL: WHAT IT MEANS

The Food and Drug Administration (FDA) evaluates new drugs before they can be sold. The must ensure that:

- the treatment works correctly
- health benefits outweigh risks
- they are safe and effective

This process can normally take up to 2.5 years, however there are several approaches to making drugs available as rapidly as possible for serious conditions with an unmet medical need, like SCD.





# FDA approves crizanlizumab-tmca for sickle cell disease

[f Share](#) [t Tweet](#) [in LinkedIn](#) [✉ Email](#) [🖨 Print](#)

On November 15, 2019, Food and Drug Administration approved crizanlizumab-tmca (ADAKVEO, Novartis) to reduce the frequency of vaso-occlusive crises (VOCs) in adults and pediatric patients aged 16 years and older with sickle cell disease.

FDA NEWS RELEASE

## FDA approves novel treatment to target abnormality in sickle cell disease

[f Share](#) [t Tweet](#) [in LinkedIn](#) [✉ Email](#) [🖨 Print](#)

For more resources and support related to these new treatments, please visit the websites of the drug manufacturers:

- <https://www.us.adakveo.com/>
- <https://www.oxbryta.com/>

**For Immediate Release:** November 25, 2019

Today, the U.S. Food and Drug Administration granted accelerated approval to Oxbryta (voxelotor) for the treatment of sickle cell disease (SCD) in adults and pediatric patients 12 years of age and older.

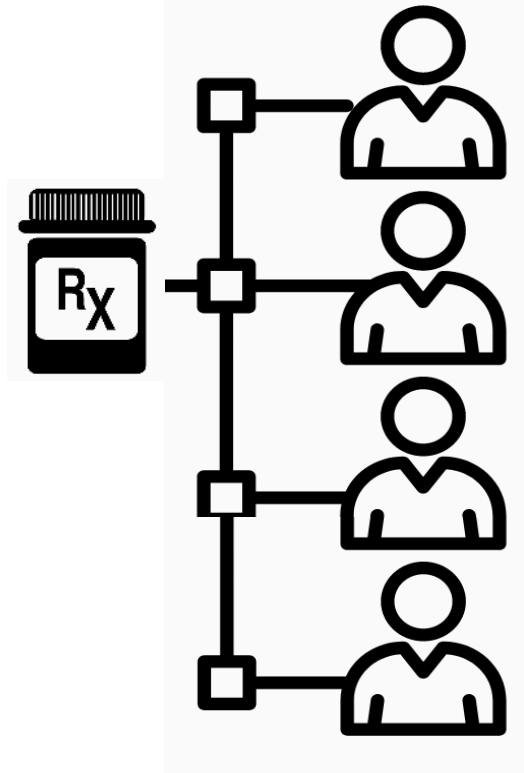
Source: <https://www.fda.gov>



# WHAT COMES AFTER FDA APPROVAL?

## COST, COVERAGE, AND ACCESS

After FDA approval, several other important decisions are being made:



**Drug Manufacturers:** make decisions regarding the price of their drug and how it will be distributed

**Insurance companies and Pharmacy benefit managers:** make coverage decisions and determine the amount they will pay

**Providers:** make decision about what treatment is best for individual patients

**Patients:** make decisions on which of these treatments and other healthcare services are best for them.

One question that everyone needs answered is:

What is the **VALUE** of this new treatment?



# WHAT IS VALUE IN HEALTHCARE?

- Value is defined as **outcomes achieved per dollar spent**.
  - Value depends on results
- Value should always be **defined around the customer**
  - Creation of value for patients should determine the rewards for all other actors in the system.
- Value in health care remains largely **unmeasured** and misunderstood.





# DETERMINING THE VALUE

- Organizations, such as the Institute for Clinical and Economic Review (ICER), develop **value assessment frameworks** and produce reports on the value of treatments.
- Value assessments involve undertaking an assessment of **comparative effectiveness and cost-effectiveness**.
  - **Cost effectiveness** is a health economics analysis that compares the relative costs and outcomes (effects) of treatments.
- Many insurance companies 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**.





**FDA**  
**REGULATORY REVIEW**  
(federal agency)

**“Is this medicine safe for the public?”**

- Determines the safety and efficacy of treatments
- Measures efficacy from the **primary outcome** in clinical trials
- Utilizes clinical trial data
- Compares new drug to **placebo**
- Results in **approval** for drugs coming to the market

**ICER**  
**VALUE ASSESSMENT**  
(independent nonprofit)

**“Is this medicine worth its cost?”**

- Determines the value and cost effectiveness of treatments
- Prioritizes outcomes that are **most meaningful to patients**
- Utilizes clinical trial data in combination with real-world data
- Compares drugs to other **“usual care” treatments**
- Determines a **price range** that they believe is fair for new drugs



# 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

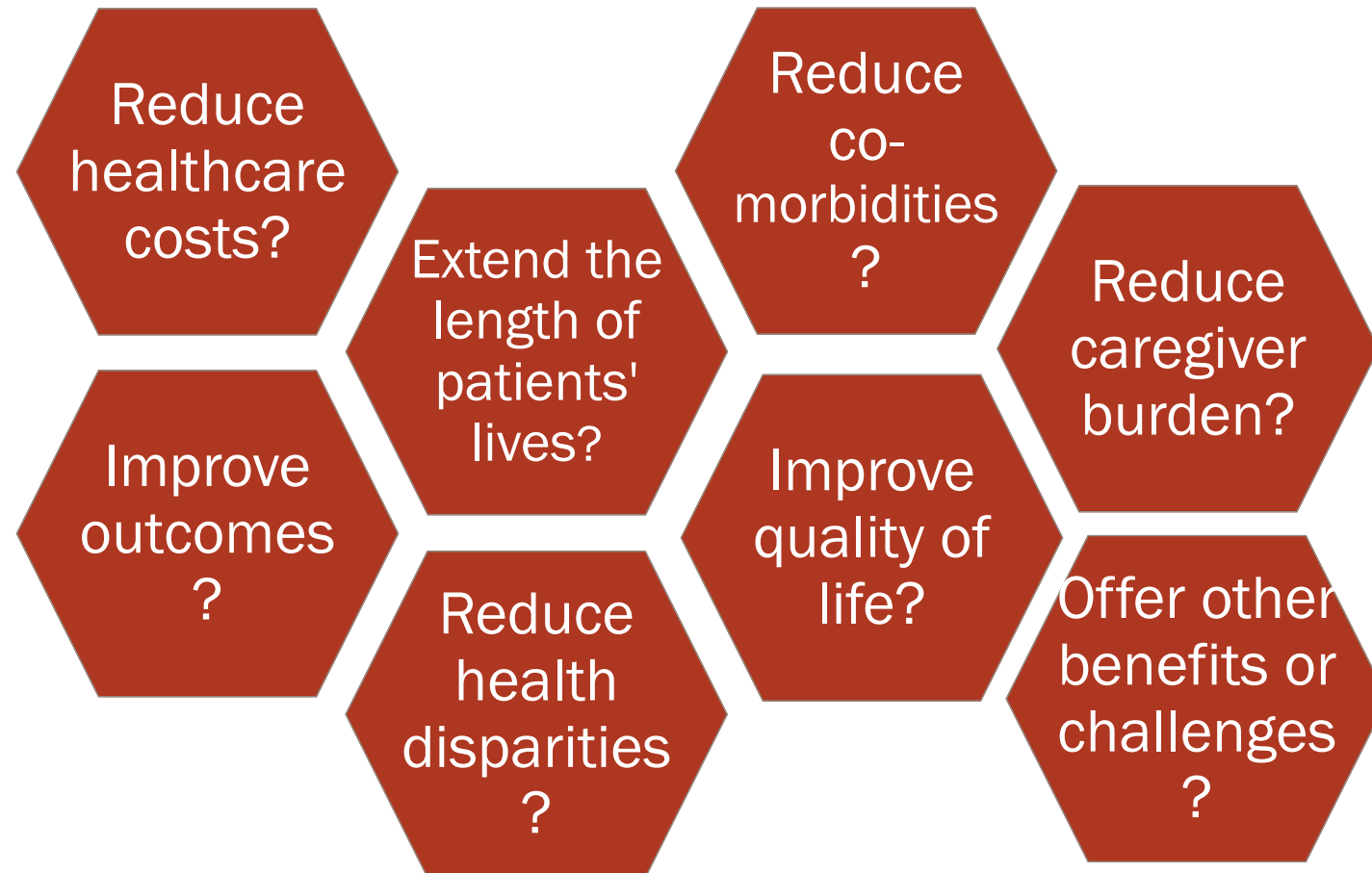




# VALUE AND COST EFFECTIVENESS

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

How effectively can each drug...





# 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 painful and disabling SCD is, how limited in effectiveness current treatments are, and 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.**



# STAY ENGAGED

- Join us during the next part of our webinar series:  
**PART 2: “Drug Pricing & Coverage Decisions”**  
**Friday, December 13<sup>th</sup>**
- Stay tuned to our website and social media pages for more information including registration links: [www.sickcells.org/engage](http://www.sickcells.org/engage)
- Reach out to us at [mjalowsky@sickcells.org](mailto:mjalowsky@sickcells.org)



# SUMMARY

- As new treatments for sickle cell disease emerge on the market, stakeholders must focus on determining the value of treatments.
- Value assessments impact patient's access to and the affordability of new treatments.
- Input from patients and advocates is critical during ICER's value assessment of three sickle cell disease drugs.



# Q&A SESSION