WEBINAR SERIES ON VALUE ASSESSMENTS FOR SICKLE CELL DISEASE

PART 1:
"FDA APPROVAL, NOW WHAT?"

December 6th, 2019

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 6th
- **PART 2:** Drug Pricing & Coverage — Friday, December 13th
- **PART 3:** Examining the “Cost” of SCD — Friday December 20th
- **PART 4:** Call to Action During the ICER Review — Friday, January 10th

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.

SICK CELLS

FDA approves crizanlizumab-tmca for sickle cell disease

Share | Tweet | 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

Share | Tweet | Linkedin | Email | Print

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.

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

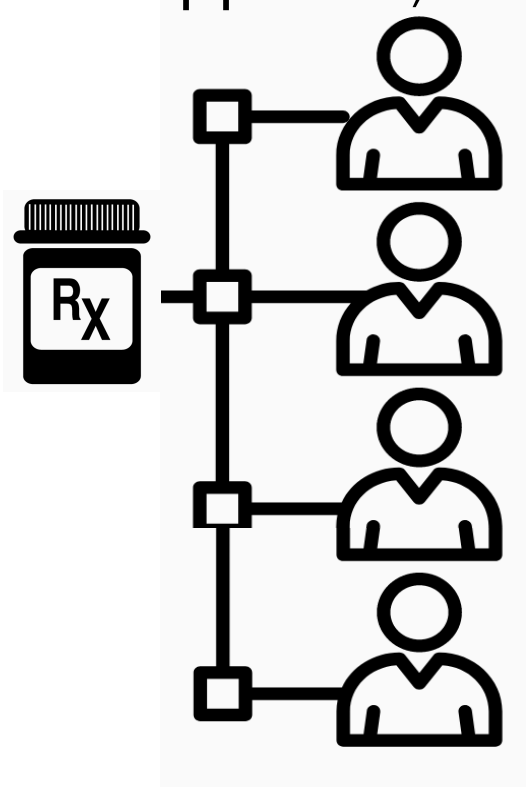
- https://www.us.adakveo.com/
- https://www.oxbryta.com/

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.**

ICER
INSTITUTE FOR CLINICAL AND ECONOMIC REVIEW

SICK CELLS

FDA REGULATORY REVIEW (federal agency)	ICER VALUE ASSESSMENT (independent nonprofit)
"Is this medicine safe for the public?"	**"Is this medicine worth its cost?"**
• 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	• 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

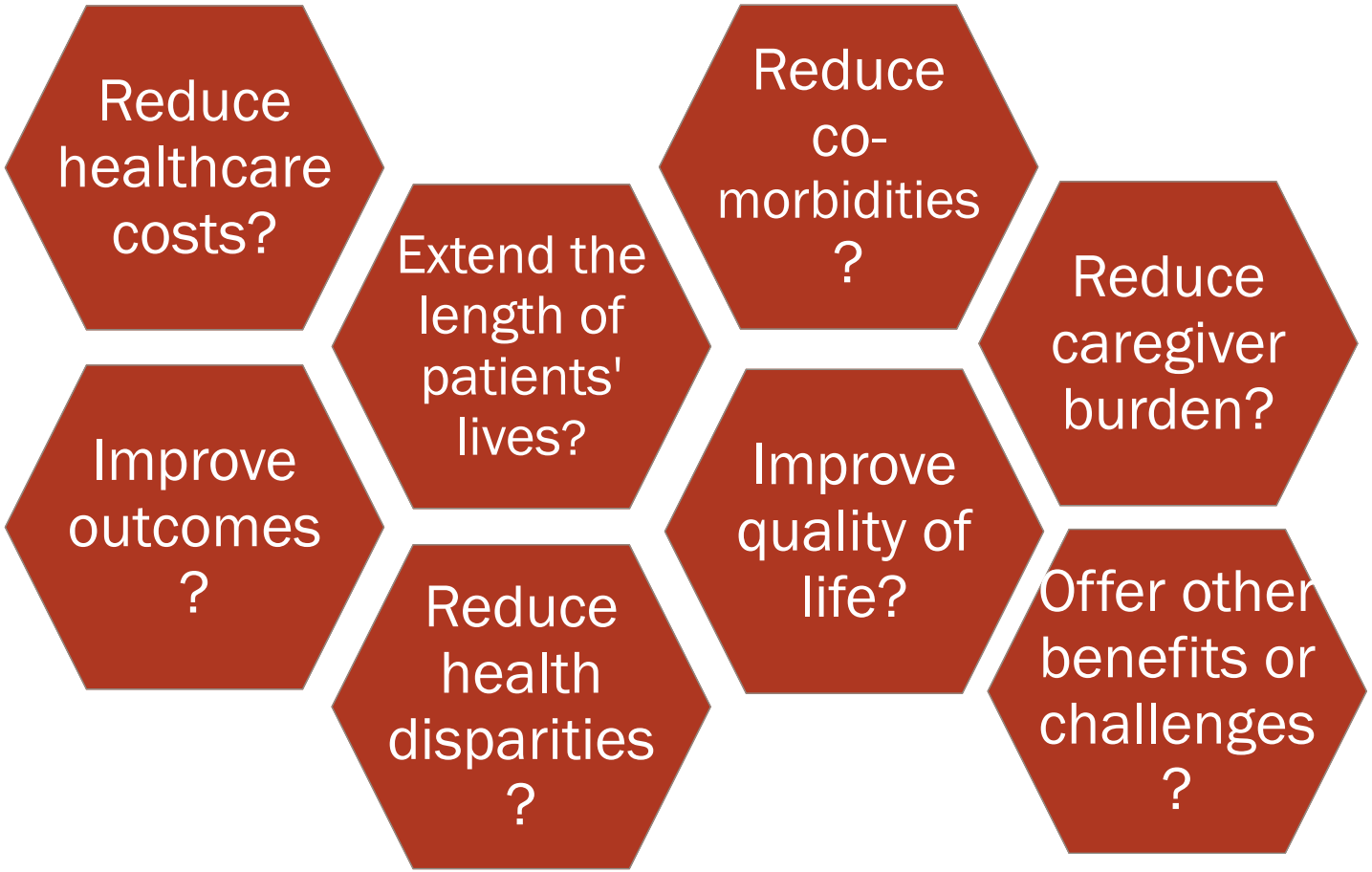
Emmaus' Endari (L-glutamine)

- 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 13th

- Stay tuned to our website and social media pages for more information including registration links: www.sickcells.org/engage

- Reach out to us at 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