Gene Therapies for Sickle Cell Disease: Effectiveness and Value

Public Meeting — July 27, 2023

Meeting materials available at: https://icer.org/assessment/sickle-cell-disease-2023/

ICER
INSTITUTE FOR CLINICAL AND ECONOMIC REVIEW

CTAF
CALIFORNIA TECHNOLOGY ASSESSMENT FORUM

Patient Experts

Carley "Elle" Cole, BA, Certified Sickle Cell Medical Advocate, Cleverly Changing, LLC

- *Nothing to disclose*

Jimi Olaghere, Patient Expert

- *Nothing to disclose*

Clinical Experts

Cecelia Calhoun, MD, MPHS, MBA, Assistant Professor of Medicine, Hematology, Yale University School of Medicine

Medical Director, Sickle Cell Program, Smilow Cancer Hospital

- *Nothing to disclose*

Patrick McGann, MD, PhD, Director, Lifespan Comprehensive Sickle Cell Center, Rhode Island Hospital and Hasbro Children's Hospital

Associate Professor of Pediatrics and Medicine, Alpert Medical School of Brown University

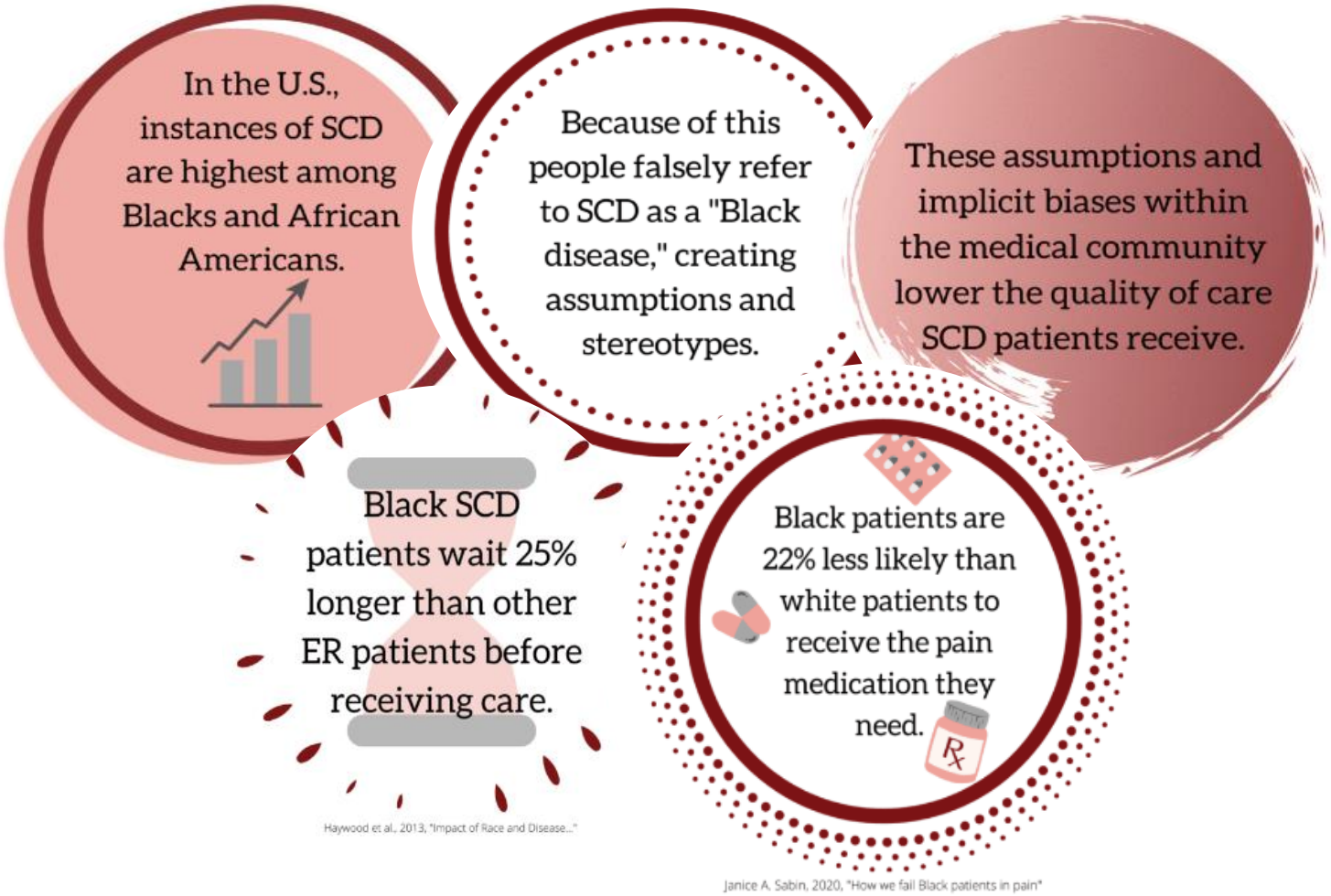
- *Dr. Patrick McGann recieved monetary value in excess of $5,000 after serving on a Novartis Safety Advisory Board*

Why are we here today?

[Sickle cell disease] affects my day-to-day life in pretty much every way. There's really not a minute that you don't feel off, like not your normal self. Being off is a steady state. So some days are good, pain is not too bad and I can do my normal day, whether it be work or hobbies on the weekends. And then some days are not so great where energy is even lower than it normally is, and pain's higher than it normally is, and the day turns into a struggle.

Carlyle, Sick Cells Faces of SCD Storytelling Program

Quote collected at the Annual Walk for Sickle Cell in Houston Texas, 10/19/2019



Adapted from 'Racism in Sickle Cell: Why Black Lives in the Healthcare System are Forgotten' by Sick Cells

https://sickcells.org/blog/racism-in-sickle-cell-why-black-lives-in-the-healthcare-system-are-forgotten/

Why Are We Here Today?

- What happens the day these treatments receive FDA approval?
- Questions remain:
  - What are the risks and benefits?
  - How do new treatments fit into the evolving landscape?
  - What are reasonable prices and costs to patients, the health system, and the government?
  - What lessons are being learned to guide our actions in the future?

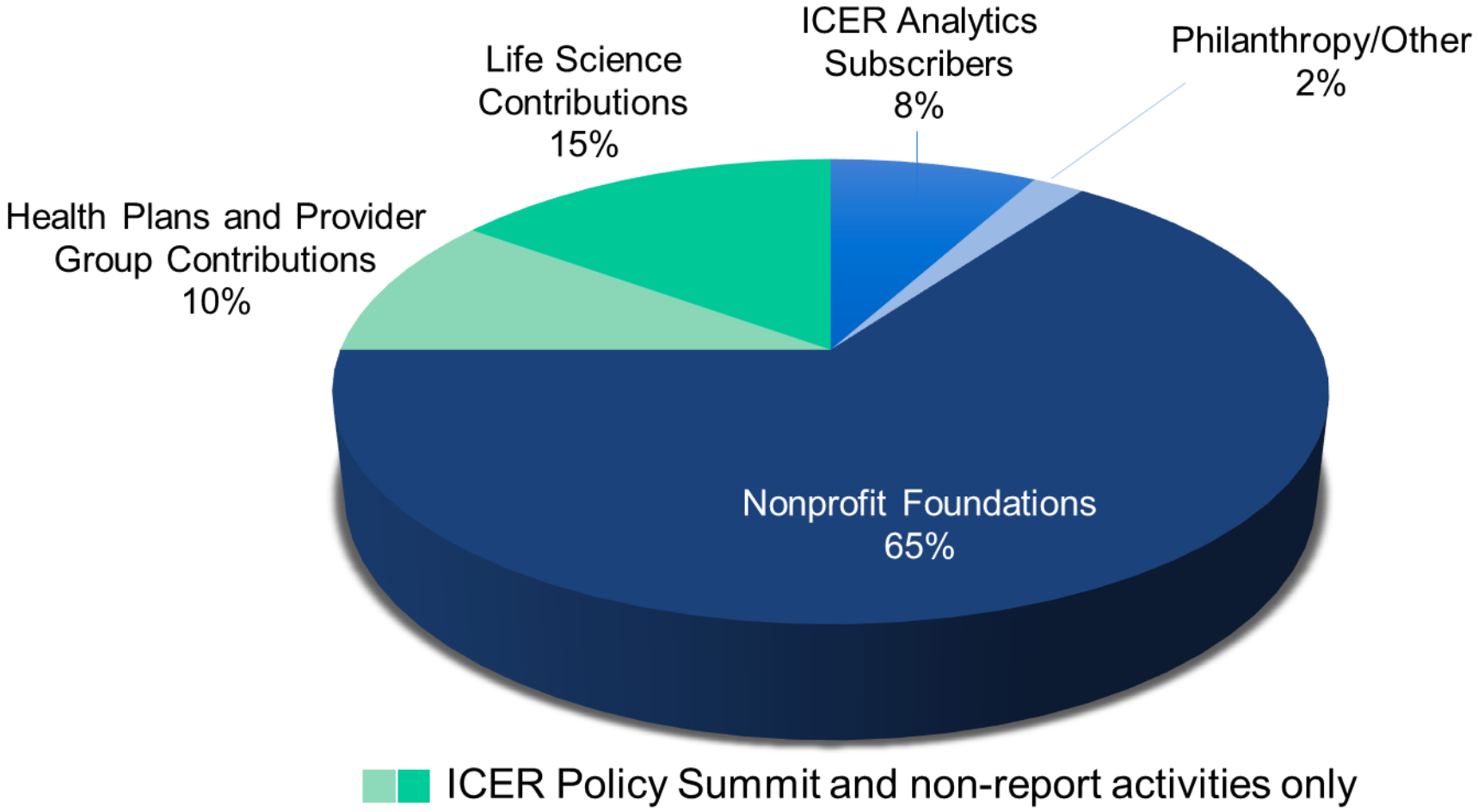
The Impact on Rising Health Care Costs for Everyone

https://khn.org/news/article/diagnosis-debt-investigation-100-million-americans-hidden-medical-debt/

Organizational Overview

- California Technology Assessment Forum (CTAF)
- Institute for Clinical and Economic Review (ICER)

Sources of Funding, 2023



How Was the ICER Report Developed?

- Scoping with guidance from patients, clinical experts, manufacturers, and other stakeholders
- Internal ICER evidence analysis and cost-effectiveness modeling
- Public comment and revision
- Expert reviewers
  - Julie Kanter, MD
  - Patrick McGann, MD, PhD
  - Maia Laing, MBA
- How is the evidence report structured to support CTAF voting and policy discussion?

Value Assessment Framework: Long-Term Value for Money

Special Social/Ethical Priorities

Benefits Beyond "Health"

Total Cost Overall
Including Cost Offsets

Health Benefits:
Return of Function, Fewer Side Effects

Health Benefits:
Longer Life

Agenda (PT)

9:00 AM	Meeting Convened and Opening Remarks
9:20 AM	Presentation of the Clinical Evidence
10:00 AM	Presentation of the Economic Model
10:40 AM	Public Comments and Discussion
11:20 AM	Lunch Break
12:05 PM	CTAF Deliberation and Vote
1:05 PM	Break
1:15 PM	Policy Roundtable Discussion
2:45 PM	Reflections from CTAF
3:00 PM	Meeting Adjourned

Presentation of the Clinical Evidence

Francesca L. Beaudoin, MD, PhD, MS

Senior Medical Advisor

Institute for Clinical and Economic Review

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Key Review Team Members

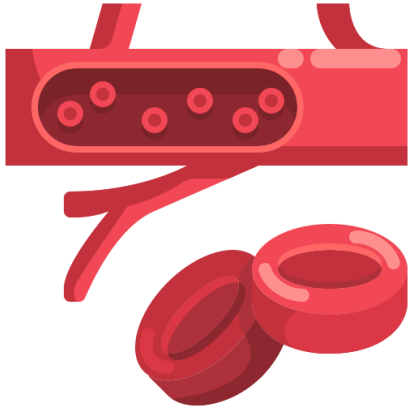
- **Dmitriy Nikitin, MSPH,** Senior Research Lead, Evidence Synthesis, ICER
- **Avery McKenna, BS,** Associate Research Lead, Evidence Synthesis, ICER
- **Emily Nhan, BA,** Senior Research Assistant, Evidence Synthesis, ICER

Disclosures: Financial support provided to Dr. Beaudoin through a contract between Brown University and ICER

Dr. Beaudoin and other members of ICER (Nikitin, Nhan, and McKenna) have no conflicts to disclose.

Background: Sickle Cell Disease

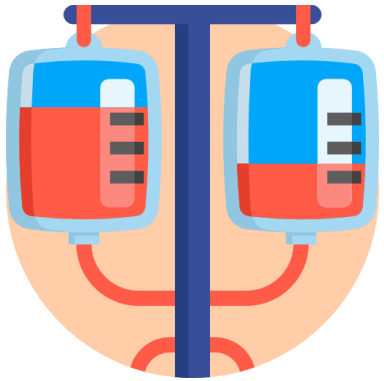
- Genetic mutations in the gene for the beta subunit of Hb leading to structural abnormalities of red blood cells (sickle-shape)
- Sickling leads to vascular obstruction and hemolysis
- Affects ~100,000 Americans
  - Most common in people of African descent
  - Higher prevalence globally
- $3 billion annual in direct health care costs (US)



Hb: hemoglobin

Current Standards of Care for Sickle Cell Disease

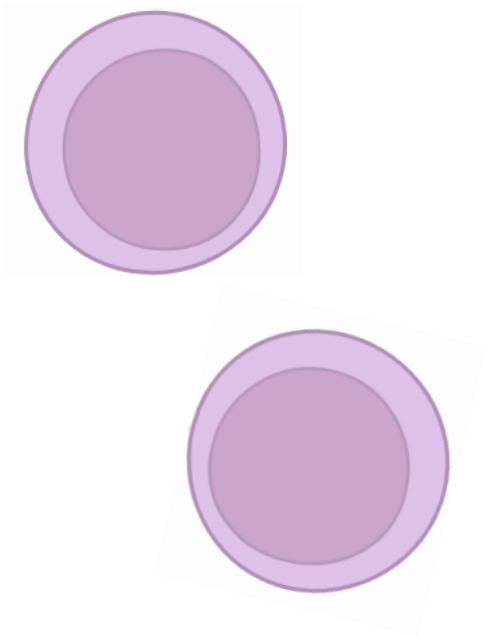
- Hydroxyurea, pain medication, blood transfusions, iron chelation therapy
- Other therapies (l-glutamine, crizanlizumab, voxelotor) have low uptake/ not cost-effective (ICER 2020 SCD Report)
- Even with treatment, numerous health consequences:
  - Severe and recurrent painful crises
  - Acute (e.g., stroke, infection) and chronic complications
  - Fertility and pregnancy-related concerns
  - Reduced health-related quality of life
  - Decreased life expectancy



SCD: sickle cell disease

Current Curative Therapy: HSCT

- Currently, the only **curative** option is hematopoietic stem cell transplant (HSCT *aka* Bone Marrow Transplant).
- HSCT requires a ‘match’, ideally a sibling
- Typically performed in childhood
- Requires myeloablative chemotherapy
- Risks = mortality, infection, GvHD, rejection, failure



GvHD: Graft vs. Host Disease, HSCT: hematopoietic stem cell transplantation

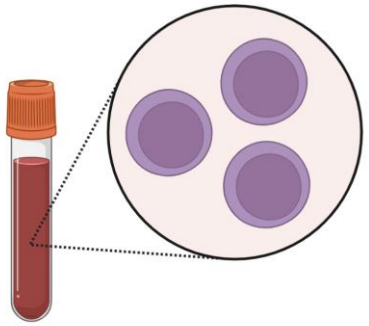
Additional Insights from Discussions with Patients

- An all-encompassing condition
- Social stigma and racial bias
- Importance of multidisciplinary care
- Fertility is a major concern

- Cautious optimism about gene therapies – “joy and apprehension”
- Life-changing improvements in quality of life in people receiving curative treatment

New Therapy: Lovotibeglogene autotemcel (lovo-cel)

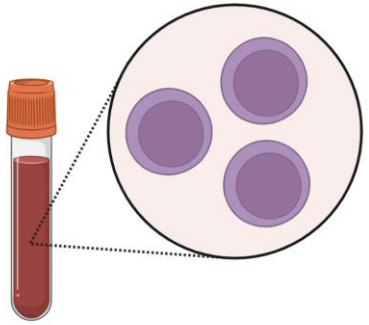
- Utilizes autologous stem cell transplant
  - Stem cells modified *ex vivo* and then infused back into the patient
- Lentiviral vector used to insert functioning copies of the *HBB* gene into the patients own stem cells
  - Production of modified anti-sickling adult hemoglobin, HbA^{T87Q}
- Requires myeloablative chemo/hospitalization
- FDA decision expected December 20, 2023



FDA: Food and Drug Administration

New Therapy: Exagamglogene autotemcel (exa-cel)

- Utilizes autologous stem cell transplant
- CRISPR/Cas9 gene-edited cell infusion therapy targeting *BCL11A* to increase fetal hemoglobin (HbF)
  - HbF≥30% considered to achieve clinical cure
- Requires myeloablative chemo / hospitalization
- FDA decision expected December 8, 2023



FDA: Food and Drug Administration

Scope of Review

- Population:
  - Adolescents and adults with severe SCD (i.e., minimum of four severe vaso-occlusive events/ crises in the prior two years).
  - No access to or ineligible for HSCT (e.g., no available match)
- Interventions:
  - Lovotibeglogene autotemcel (lovo-cel)
  - Exagamglogene autotemcel (exa-cel)

- Comparators:
  - Standard of care (e.g., hydroxyurea, iron chelation, blood transfusions)

HSCT: hematopoietic stem cell transplant, SCD: sickle cell disease

Scope of Review: Outcomes

- Patient important outcomes:
  - Frequency of acute pain crisis (VOC/VOE)
  - Hospitalization
  - Ability to maintain education/employment
  - Quality of life, fertility
  - Adverse events (malignancies)
  - Mortality

-No universal definition of VOE/VOC
-All definitions included: acute chest syndrome, splenic sequestration, and priapism
-Visit to a medical facility part of the definition of severe VOC and VOEs

- Other outcomes: Hemoglobin levels, hemolysis markers, measures of gene editing durability, health services

VOC: vaso-occlusive crisis, VOE: vaso-occlusive event

Clinical Evidence

Overview of Lovo-cel and Exa-cel Clinical Trials for Severe SCD

Intervention & Key Trial	Design	Follow-up	Primary Outcome	Age, Years (Range); % Female	Annualized Rate of VOCs/VOEs (Range)
lovo-cel **HGB-206** N=36 (Group C)*	Phase I/II, single-arm, open-label	24 months Current follow-up: 20.9 months (as of July 2021)	Proportion of participants free of (severe) VOEs	Median: 24 (12-38); 37% female	Median: 3 (0.5-13.5)
exa-cel **CLIMB-121** N=35	Phase I/II/III, single-arm, open-label	24 months Current follow-up: 11.6 months (as of September 2022)	Proportion of participants free of severe VOCs	Mean: 22.1 (12-34); 45% female	Mean: 4.2 (2-18.5)

SCD: sickle cell disease, VOC: vas-occlusive crisis, VOE: vaso-occlusive event
*earlier cohorts included Group A (N=7) and Group B (N=2), with a different manufacturing and administration process.

Key Trial Results: Lovo-cel

- Reduction in occurrence of vaso-occlusive events (VOEs)
  - 30/31 trial participants were free of severe VOEs between 6 and 18 months of follow-up
  - Median # of severe VOEs/year was reduced from 3 → 0
  - Reduction in the number of annual hospital admissions and days
  - Non-severe VOEs only reported in a small sample (n=10) with 90% free of any VOE

Lovo-cel Results

- Improvements in quality-of-life measurements
  - Reductions in pain intensity, improved Health Utility Index
  - Reduction in hours of work missed due to health problems
- Favorable Hematological Response
  - Increase in total Hb levels (8g/dL → 12g/dL, baseline to month 12)
  - Increase in levels of modified adult hemoglobin HbA^{T87Q}
  - Reduction in markers of hemolysis

dL: deciliter, g: grams, g: grams, Hb: hemoglobin, PRO: patient-reported outcome

Harms: Lovo-cel

- 100% of participants reported AEs
- >50% SAEs (stomatitis, thrombocytopenia, neutropenia)
- One death in Group C Cohort 20 months post-infusion, cardiac fibrosis deemed unrelated to lovo-cel.
- Two deaths related to hematologic malignancy in earlier cohort, no evidence of oncogenic insertion (Group A).
- Two cases of suspected MDS determined to be anemia from co-occurring alpha-thalassemia mutation

AE: adverse event, MDS: myelodysplastic syndrome, SAE: serious adverse event

Key Trial Results: Exa-cel

- Reduction in occurrence of vaso-occlusive crises (VOC)
  - 16/17 trial participants (94.1%) who had at least 12 months of follow-up were free of *severe* VOCs (Sept. 2022)
    - No *hospitalization* for severe VOC during follow-up
  - Baseline average of 4.6 VOCs per year over two-year period before treatment

VOC: vaso-occlusive crisis

Key Trial Results: Exa-cel

- Improvements in quality-of-life measurements
  - Greater than minimum clinically important difference (MCID) in QoL measures (EQ VAS, FACT-G) by month six, sustained over 18 months
- Favorable Hematological Response
  - Increase in total Hb levels (9.1g/dL → 12.1g/dL, baseline to Month 3, → 11.0 g/dL during remainder follow-up)
  - Mean proportion of fetal Hb>30% by month three and through 24 months of follow-up

dL: deciliter, EQ VAS: EuroQol visual analog scale, FACT-G: Functional Assessment of Cancer Therapy-General, g: grams, Hb: hemoglobin, QoL: quality of life

Harms: Exa-cel

- 34.3% reported adverse events related to exa-cel treatment
- 40% reported SAEs (thrombocytopenia, neutropenia)
- No malignancies as of September 2022
- One death attributed to SARS-CoV-2 infection and potentially related to busulfan lung injury
- One patient treated required therapeutic phlebotomy

SAE: serious adverse event, SARS-CoV-2: severe acute respiratory syndrome coronavirus 2

Controversies and Uncertainties

- Small sample size, insufficient data on long-term outcomes/ durability
  - Experts suggested that long-term follow-up (>15 years) is required to establish precision around durability of treatment effect
- Effects in real-world settings and broader SCD populations
- Comparative Effectiveness
  - Single arm trials; no comparison against HSCT or each other

HSCT: hematopoietic stem cell transplant, SCD: sickle cell disease, VOC: vaso-occlusive crisis

Contextual Considerations

- SCD risk of many acute, severe complications (e.g., infection, stroke, myocardial infarction, blood clots, renal infarctions) that can lead to significant disability and death
- The cumulative burden of SCD disease is substantial

SCD: sickle cell disease

Potential Other Benefits or Disadvantages

- Benefits to potentially curative therapy:
  - QoL, ability to achieve major life goals related to education, work, or family life
  - High likelihood of improvement in caregivers' ability to return to school and/or work and overall productivity
  - Reduce the need for other long-term medical therapies (standard of care)
  - Addressing health inequities in a vulnerable population impacted by bias
- Disadvantages:
  - Requires lengthy hospitalization and myeloablation carries risks in the short-term (infection), but also long-term (fertility)

QoL: quality of life

Public Comments Received

- Challenges in classifying severity of SCD and VOCs
- Comparison of gene therapies to HSTC
- Emerging data – participants in the original trials continue under follow-up providing new data on durability and safety.

HSCT: hematopoietic stem cell transplant, SCD: sickle cell disease, VOC: vaso-occlusive crisis

Summary: Gene therapies for Sickle Cell Disease

- Both **lovo-cel** and **exa-cel** demonstrate good efficacy in reducing severe VOE/VOCs.
- Safety outcomes have been consistent with those generally expected from myeloablative conditioning
- Careful monitoring required for risk of malignancy
- Durability and long-term safety need to be established over years of follow up, greater uncertainty with exa-cel as it would be first in class

VOC: vaso-occlusive crisis, VOE: vaso-occlusive event

ICER Evidence Ratings for Lovo-cel and Exa-cel for SCD

Treatment	Comparator	Evidence Rating
Lovo-cel	Standard of care	B+
Exa-cel	Standard of care	C++
Lovo-cel	Exa-cel	I

B+: Incremental or Better – Moderate certainty of a small or substantial net health benefit, with high certainty of at least a small net health benefit
C++: Comparable or Better – Moderate certainty of a comparable, small, or substantial net health benefit, with high certainty of at least a comparable net health benefit
I: Insufficient – Any situation in which the level of certainty in the evidence is low

SCD: sickle cell disease

Questions?

Presentation of the Economic Model

Praveen Thokala, MASc, PhD

Senior Research Fellow

University of Sheffield

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Key Contributors

- **Praveen Thokala, PhD**, University of Sheffield
- **Eldon Spackman, PhD**, University of Calgary
- **Jon Campbell, PhD, MS** Senior Vice President of Health Economics, ICER

Disclosures:

Financial support provided through a contract between University of Sheffield and ICER.

Drs. Campbell, Thokala, and Spackman have no conflicts to disclose.

Objective

- To evaluate the lifetime cost-effectiveness of lovo-cel and exa-cel, each compared to standard care for the treatment of patients with severe SCD

SCD: sickle cell disease

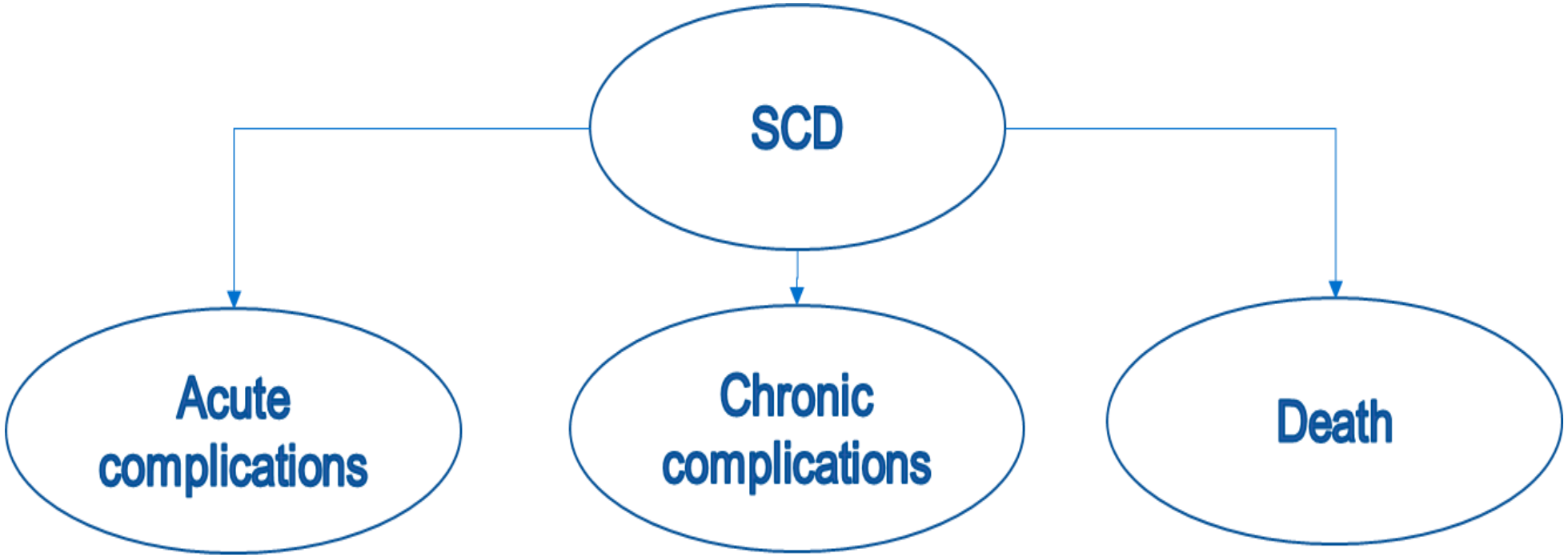
Methods in Brief

Methods Overview

- **Model**: Markov
- **Setting**: United States
- **Perspective**: Co-base with health care sector perspective and modified societal perspective
- **Time Horizon**: Lifetime
- **Discount Rate**: 3% per year (costs and outcomes)
- **Cycle Length**: 1 year
- **Outcomes**: Cost per QALY gained; cost per LY gained; cost per evLY gained; cost per VOC avoided

evLY: equal value life year, LY: life year, QALY: quality-adjusted life year; VOC: vaso-occlusive crisis

Model Schematic



SCD: sickle cell disease

Key Model Assumptions

- VOC rates impact risk of acute and chronic complications, and death
- Acute complications (except VOCs), chronic complications, and death modeled independently
- Treatment effect is measured as patients without VOCs
  - Patients with VOCs after treatment are assumed to have same rate of complications as those on standard care
  - After year seven, the model assumes loss of efficacy for a very small proportion of patients each year (annual rate of 0.27%)

VOC: vaso-occlusive crisis

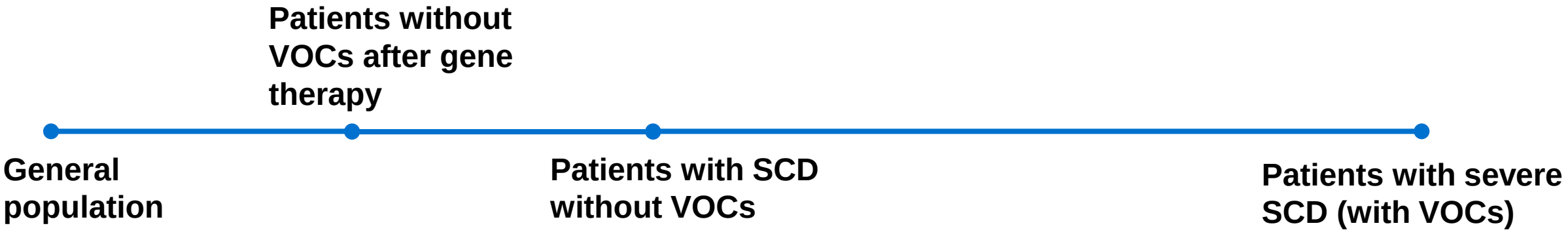
Key Model Inputs: Treatment-Related Efficacy

- Treatment success measured as proportion of patients without VOCs

Treatment	Proportion of patients without VOCs, % (Mean values)
Lovo-cel	96.8
Exa-cel*	96.8*

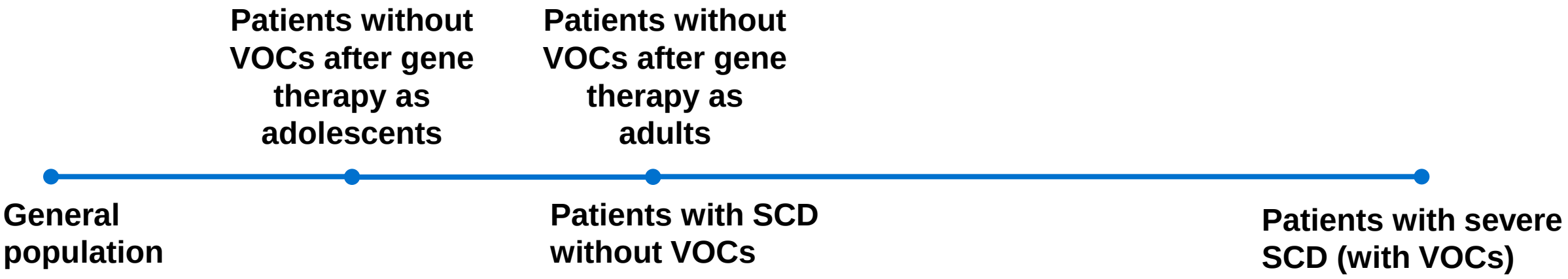
VOC: vaso-occlusive crisis
*Given the smaller sample size in the exa-cel trial, higher uncertainty for exa-cel was reflected in the parametric distribution used for probabilistic sensitivity analysis.

Modelling Acute Complication rates



SCD: sickle cell disease, VOC: vaso-occlusive crisis

Modelling Chronic Complication Rates and Death



SCD: sickle cell disease, VOC: vaso-occlusive crisis

Key Model Inputs: Health State Utilities

Health State	Utility	Source
SCD Without Complications	0.80	Anie et al. 2012
Disutility Due to Gene Therapy (for One Year)	-0.11	Matza et al. 2020
Additional Utility for Patients Who Have no VOCs	0.05	Assumption

SCD: sickle cell disease, VOC: vaso occlusive crises

Key Model Inputs: Costs

- Placeholder price for gene therapies $2,000,0000
- Cost per VOC: $5,762

Modified societal perspective

- Patient productivity losses: $19,250 per year
- Caregiver costs: $19,662 per year

SCD: sickle cell disease, VOC: vaso-occlusive crisis

Results

Base-Case Results

Treatment	Treatment Cost*	Total Cost*	VOCs	Life Years	evLYs	QALYs
Health Care System Perspective						
Lovo-cel or Exa-cel	$2,000,000	$2,827,000	4.18	21.87	17.31	16.38
Standard of Care	--	$1,490,000	119.26	15.80	9.44	9.44
Incremental Cost-Effectiveness Ratios			$11,600	$220,000	$170,000	$193,000
Modified Societal Perspective						
Lovo-cel or Exa-cel	$2,000,000	$2,837,000	4.18	21.87	17.31	16.38
Standard of Care	--	$1,714,000	119.26	15.80	9.44	9.44
Incremental Cost-Effectiveness Ratios			$9,800	$185,000	$143,000	$162,000

evLY: equal value life year, QALY: quality-adjusted life year, SCD: sickle cell disease, VOC: vaso-occlusive crisis

* Using Placeholder value price of $2 million for lovo-cel based on analyst estimates.

Scenario Analyses – Lovo-cel or Exa-cel

Optimistic scenario

- Assumed better treatment durability and greater reduction in complications
- ~30% decrease from base case incremental cost-effectiveness ratios

Conservative scenario

- Lower utility bump and lower reduction in complications
- ~30% increase from base case incremental cost-effectiveness ratios

Sensitivity Analysis

Key drivers from one way sensitivity analyses

- Annual numbers and costs of VOCs
- Utility after gene therapy
- Treatment success rate for exa-cel

Probabilistic Sensitivity Analyses

- Greater uncertainty for exa-cel due to smaller sample size

VOC: vaso-occlusive crisis

Limitations

Uncertainty around:

- Frequency and cost of VOCs
- Effectiveness of the therapies in reducing complications
- Risk of malignancy, long-term durability and harms

VOC: vaso-occlusive crisis

Comments Received

- Feedback on Draft report: VOCs
  - Change: the number of VOCs per year in the model was changed to 5.1 per year for patients on standard care (rather than the four VOCs per year used in the draft report).

VOC: vaso-occlusive crisis

Conclusions

Although uncertainties about durability and harm remain, both lovo-cel and exa-cel are likely to substantially improve quality and length of life among patients with SCD. Ultimately, cost effectiveness will depend on the actual prices for these therapies

SCD: sickle cell disease

Questions?

Manufacturer Public Comment and Discussion

Anjulika Chawla, MD
Senior Medical Director, Clinical Research and Development, bluebird bio

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Duration: 00 05 00

TimeUp Reminder

Conflicts of Interest:

- *Dr. Chawla is a full-time employee of bluebird bio*
- *Dr. Chawla collaborated with Real Chemistry, a third-party entity to directly compose public comments at ICER's public meeting*

Jaime Rubin Cahill, MA, MPH
Vice President, HEOR, Vertex Pharmaceuticals

Conflicts of Interest:

- *Jaime Rubin Cahill is a full-time employee of Vertex Pharmaceuticals*

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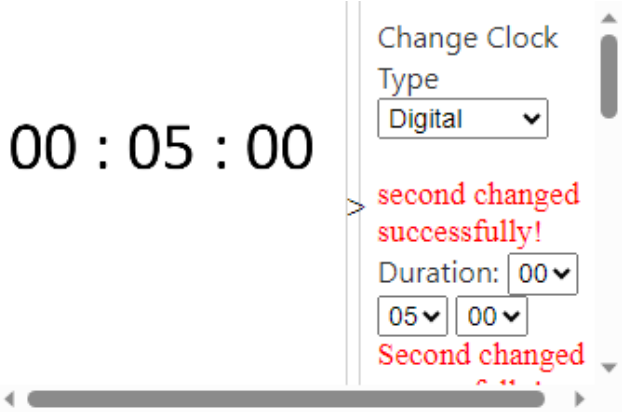
TimeUp Reminder

Public Comment and Discussion

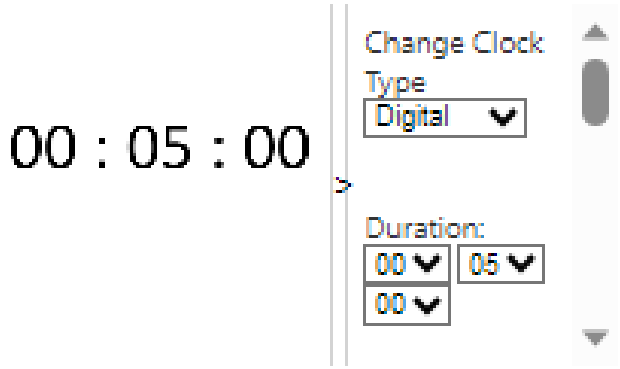
Yvenalda Guillaume, Caregiver

Conflicts of Interest:

- *Nothing to disclose*



Maggie Jalowsky, Patient Advocate
Director of Advocacy, Sick Cells



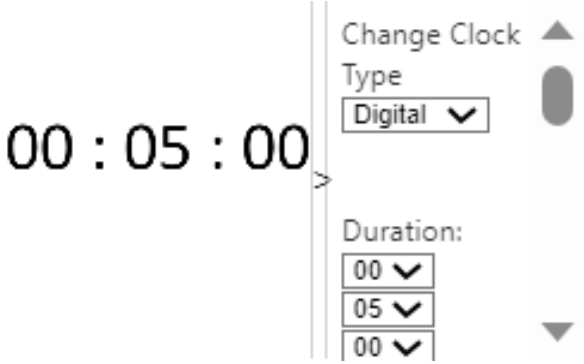
Conflicts of Interest:

- *Sick Cells has received sponsorship funding and charitable contributions in excess of $5,000 from companies developing in sickle cell. These funds are not in any way connected to, or conditioned upon, any past, present or future prescribing, purchasing, or recommending product manufactured or marketed by these companies.*
- *Maggie Jalowsky holds status as an employee of Sick Cells, which has received sponsorship funding and charitable contribution from companies developing in sickle cell (>25%).*

Genesis Jones, BS, Sickle Cell Warrior

Conflicts of Interest:

- *Genesis Jones received monetary value in excess of $5,000 from Be the Match.*



Jeannie Kittrell, Caregiver

Conflicts of Interest:

- *Nothing to disclose*

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Tesha Samuels, Patient Advocate
Journey to ExSCellence

Conflicts of Interest:

- *Nothing to disclose*

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Elinam Joe Tsogbe, Patient

Conflicts of Interest:

- *Elinam Joe Tsogbe collaborated with Sick Cells to directly compose public comments delivered at the ICER public meeting.*

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Lunch

Meeting will resume at 1:05 pm PT

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Voting Questions

Patient population for all questions:

Adolescents and adults with severe sickle cell disease (SCD) who do not have access to, or cannot receive, hematopoietic stem cell transplantation (HSCT) from a matched sibling or haploidentical donor.

slido

Given the currently available evidence, is the evidence adequate to demonstrate that the net health benefit of exagamglogene autotemcel (exa-cel) is superior to that provided by standard of care (i.e., hydroxyurea, chronic blood transfusions, pain medicati

ⓘ Start presenting to display the poll results on this slide.

slido

Given the currently available evidence, is the evidence adequate to demonstrate that the net health benefit of lovotibeglogene autotemcel (lovo-cel) is superior to that provided by standard of care (i.e., hydroxyurea, chronic blood transfusions, pain medic

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Given the currently available evidence, is the evidence adequate to distinguish the net health benefit between exa-cel and lovo-cel?

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If 'yes' to the last question: given the currently available evidence, which product is superior based on its net health benefit?

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When making judgments of overall long-term value for money, what is the relative priority that should be given to *any* effective treatment for SCD, on the basis of the following contextual considerations:

slido

Acuity of need for treatment of individual patients based on short-term risk of death or progression to permanent disability

ⓘ Start presenting to display the poll results on this slide.

slido

Magnitude of the lifetime impact on individual patients of the condition being treated

ⓘ Start presenting to display the poll results on this slide.

What are the relative effects of exa-cel/lovo-cel versus standard of care on the following outcomes that inform judgments of the overall long-term value for money of exa-cel/lovo-cel?

slido

Patients’ ability to achieve major life goals related to education, work, or family life

ⓘ Start presenting to display the poll results on this slide.

slido

Caregivers’ quality of life and/or ability to achieve major life goals related to education, work, or family life

ⓘ Start presenting to display the poll results on this slide.

slido

Patients’ ability to manage and sustain treatment given the complexity of regimen

ⓘ Start presenting to display the poll results on this slide.

slido

Society’s goal of reducing health inequities

ⓘ Start presenting to display the poll results on this slide.

Break

Meeting will resume at 1:15 pm PT

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Policy Roundtable

Policy Roundtable Participants

Participant	Conflict of Interest
Cecelia Calhoun, MD, MPHS, MBA	Nothing to disclose
Jaime Rubin Cahill, MA, MPH	Jaime Rubin Cahill is a full-time employee of Vertex Pharmaceuticals
Elle Cole, BA	Nothing to disclose
Michelle A. Gourdine, MD	Dr. Gourdine receives monetary value in excess of $5,000 from CVS Health, receives equity interest in excess of $10,000 from CVS Health and Agilon Health, had status as Agilon Health board director and received >25% funding, had status as Horizon BCBS NJ board director ad received >25% funding, had status at CVS Health and received >25% funding, and had status as the University of Maryland Medical System receiving >20% funding.
Jimi Olaghere	Nothing to disclose
Patrick McGann, MD, PhD	Dr. McGann received monetary value in excess of $5,000 after serving on a Novartis Safety Advisory Board
Clark Paramore, MSPH	Clark Paramore is a full-time employee of bluebird bio
John Watkins, PharmD, MPH, BCPS	Nothing to disclose

CTAF Council Reflections

Next Steps

- Meeting recording posted to ICER website next week
- Final Report published on or around August 21, 2023
  - Includes description of CTAF votes, deliberation, policy roundtable discussion
- Materials available at: https://icer.org/assessment/sickle-cell-disease-2023/

Adjourn

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