Betibeglogene Autotemcel (Beti-cel) for Beta Thalassemia

Public Meeting — June 17, 2022

Meeting materials available at: https://icer.org/beta-thalassemia-2022/#timeline

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Clinical and Patient Experts

Monica Bhatia, MD, Associate Professor of Pediatrics and Director, Pediatric Stem Cell Transplant Program, Columbia University Medical Center

- *No relevant conflicts of interest to disclose*

Nathan Connell, MD, MPH, Associate Professor of Medicine, Harvard Medical School, Brigham and Women's Hospital

- *Dr. Connell has received funding in excess of $5,000 from Takeda Pharmaceuticals.*

Priyanka Kumar, Beta Thalassemia Patient and Advocate

- *No relevant conflicts of interest to disclose*

Eileen Scott, Patient Services Manager, Cooley's Anemia Foundation

- *No relevant conflicts of interest to disclose*

Why Are We Here Today?

[Living with beta thalassemia is] challenging - there's a lot of challenges and appointments. A lot of people don't understand it's not just getting blood, it's a team of doctors (endocrinologist, hematologist, cardiologist). It's a lot of appointments for a young adult. I try to have a healthy balance of work, social life, and medical life. Sometimes it feels like a double life."

Beta Thalassemia Patient

Why Are We Here Today?

- What happens the day these treatments receive FDA approval?
- Questions about:
  - Evidence – 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

Leonard Edloe
Richmond, Virginia

The Whitman family
Bird City, Alaska

The Maccoux family
Brooklyn Park, Minnesota

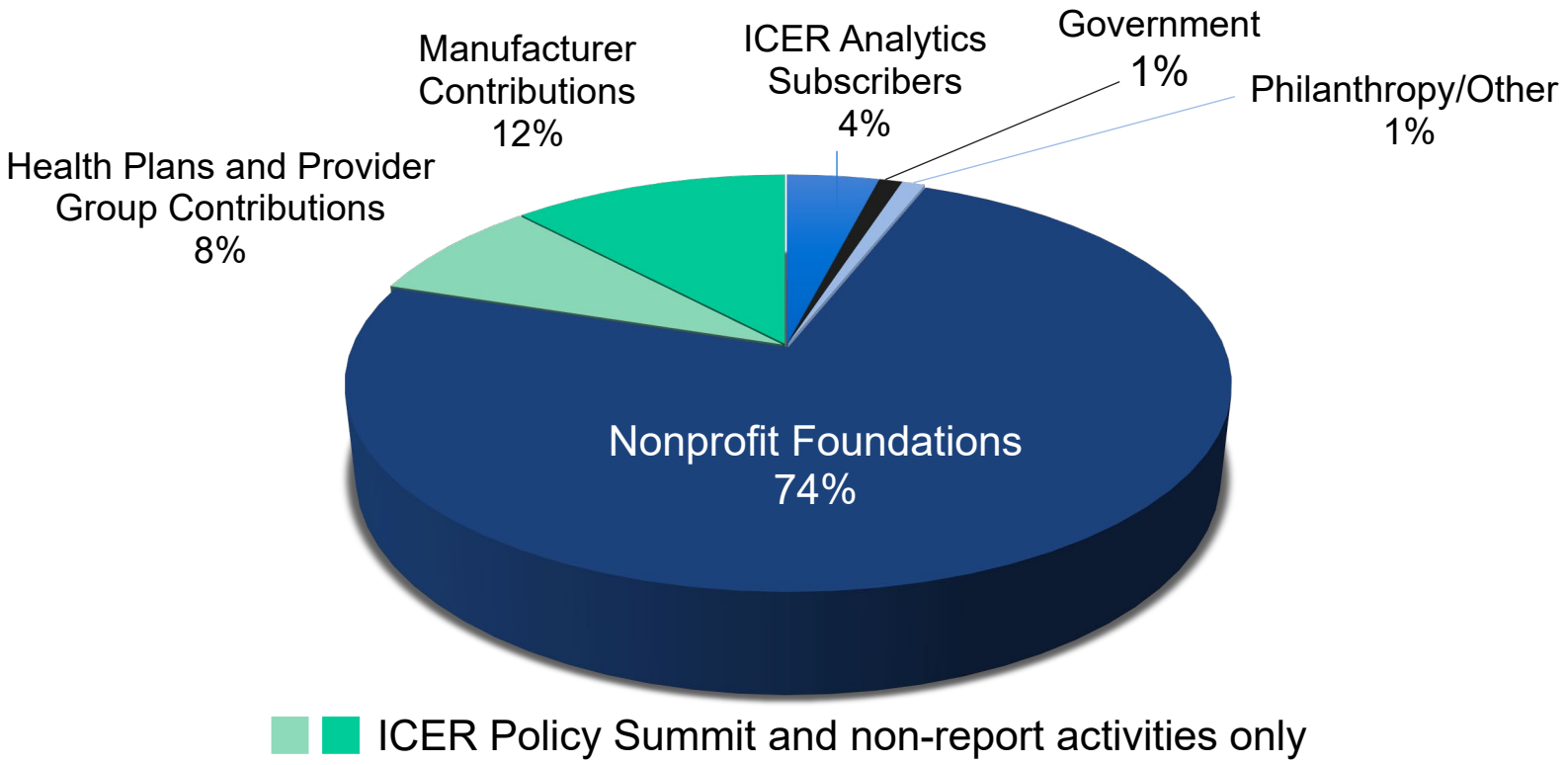
ICER

Organizational Overview

- New England Comparative Effectiveness Public Advisory Council (CEPAC)
- The Institute for Clinical and Economic Review (ICER)

Sources of Funding, 2022

https://icer.org/who-we-are/independent-funding/



How Was the ICER Report Developed?

- Scoping with guidance from patients, clinical experts, manufacturers, and other stakeholders
- Internal ICER staff evidence analysis and cost-effectiveness modeling
- Public comment and revision
- Expert reviewers
  - **Monica Bhatia, MD,** Associate Professor of Pediatrics and Director, Pediatric Stem Cell Transplant Program, Columbia University Medical Center
  - **Maria Domenica Cappellini, MD,** Honorary Professor of Internal Medicine, University of Milan
  - **Paul DiLorenzo, PhD,** President, Thalassemia Support Foundation
  - **Sujit Sheth, MD,** Professor, Weill Cornell Medicine
- How is the evidence report structured to support CEPAC voting and policy discussion?

Value Assessment Framework: Long-Term Value for Money

Agenda

Time (ET)	Activity
10:00am – 10:20am	Meeting Convened and Opening Remarks
10:20am – 11:00am	Presentation of the Clinical Evidence
11:00am – 11:40am	Presentation of the Economic Model
11:40am – 11:55am	Public Comments and Discussion
11:55am – 12:50pm	Lunch Break
12:50pm – 2:00pm	New England CEPAC Vote on Clinical Effectiveness and Value
2:00pm – 2:10pm	Break
2:10pm – 3:30pm	Policy Roundtable
3:30pm – 4:00pm	Reflections from Midwest CEPAC
4:00pm	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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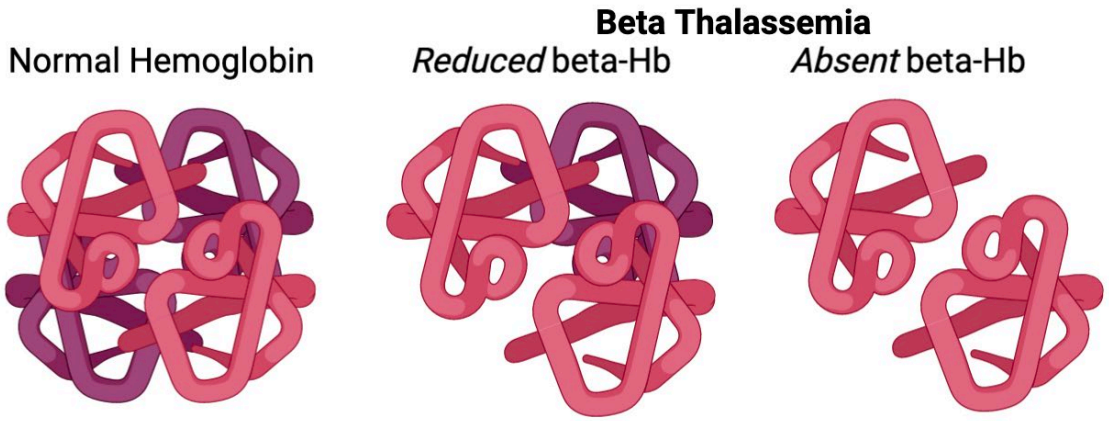
Key Review Team Members

- **Patty Synnott, MS, MALD,** Project Director, Global Health Initiatives, Center for Evaluation of Value and Risk in Health
- **Victoria Lancaster, PharmD, MSc, MBA,** Health Technology Assessment Fellow, ICER
- **Belen Herce-Hagiwara, BA,** Research Assistant, Evidence Synthesis, ICER

Disclosures: We have no conflicts of interest relevant to this report.

Background: Beta Thalassemia

- Inherited disorder of hemoglobin synthesis, autosomal recessive
- Leads to reduced or absent β-globin proteins of hemoglobin
- Varying degrees of anemia
- Most severe form = Transfusion Dependent Thalassemia (TDT)



Background: Epidemiology

- Higher global burden than in the US
- 1.25 million carriers in the US
- US prevalence of TDT is rare
  - ~1000 – 1500 people living with TDT

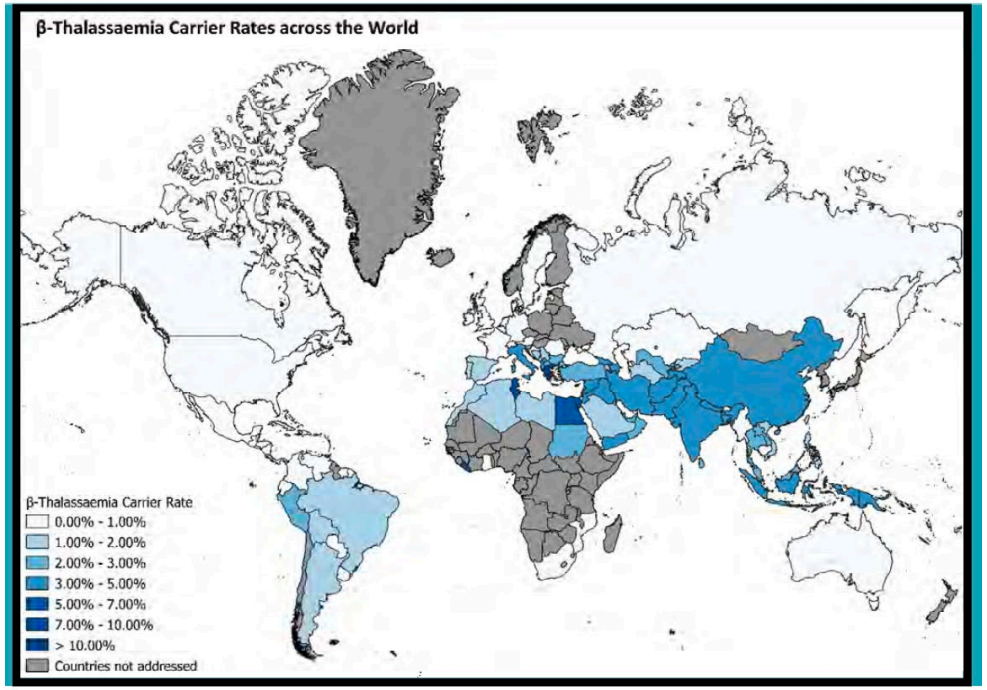
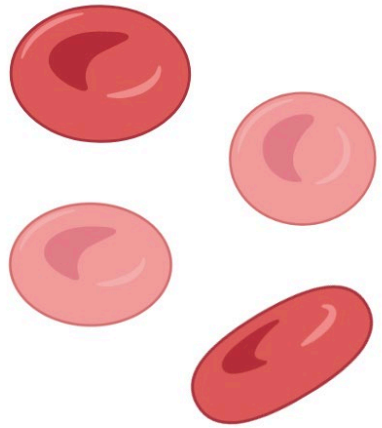


Figure from Thalassaemia International Federation

Background: Clinical Picture

- Rare, but serious – TDT is fatal if untreated:
  - Hemoglobin ~3 – 4 g/dL
  - Extramedullary hematopoiesis → skeletal abnormalities
  - Enlarged liver and spleen → liver failure
  - Heart failure, infection
- Most severe forms present in infancy

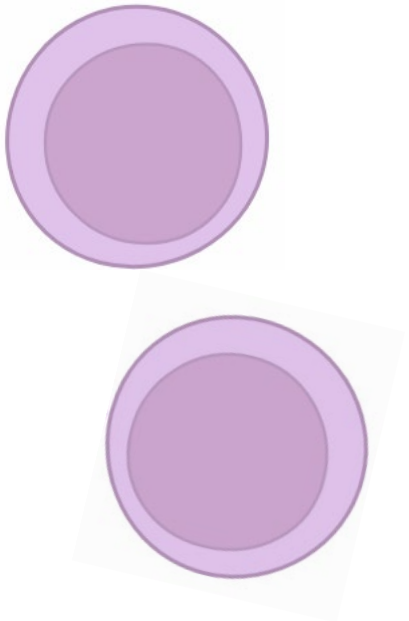


Current Standards of Care for TDT

- Majority of patients = Regular blood transfusions, iron chelation
- Chelation is necessary to treat iron overload
- Even with treatment, numerous health consequences
  - From iron overload: Liver cirrhosis, heart failure, endocrine dysfunction
  - Fertility and pregnancy-related concerns
  - Reduced health-related quality of life
  - Decreased life expectancy despite improved treatment

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 = infection, **GvHD**, **rejection**, failure, infertility

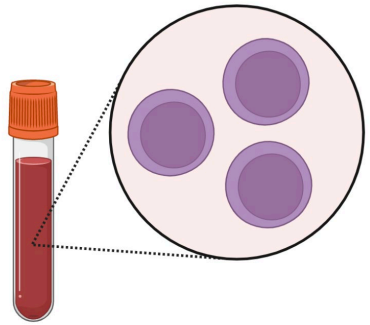


Additional Insights from Discussions with Patients

- “Tethered to the health care system”
- Regular transfusions, frequent specialist visits, blood draws, imaging for monitoring (e.g., MRIs) = “Like having another job”
- Significant fatigue before transfusions
- Difficult with chelation regimens, particularly during adolescence
- Fertility a major concern
- Interested in curative therapy, but aware of / balance risks

New Therapy: Betibeglogene Autotemcel (Beti-cel)

- Utilizes autologous stem cell transplant
- Lentiviral vector used to insert functioning copies of the HBB gene into the patients own stem cells.
- Stem cells modified *ex vivo* and then infused back into the patient
- Requires myeloablative chemo / hospitalization
- FDA decision expected August 2022
- Advisory committee voted unanimously in favor (6/11/22)



Scope of Review

- Population
  - Patients with transfusion dependent thalassemia (TDT), regardless of genotype
  - TDT = 8 or more transfusions per year
- Interventions: Betibeglogene autotemcel (beti-cel)
- Comparators:
  - Standard of care (e.g., iron chelation, blood transfusions)

Scope of Review: Outcomes

- Patient important outcomes:
  - Engraftment
  - Transfusion independence (primary)
  - Complications of iron overload
  - Quality of life, fertility
  - Adverse events
  - Mortality
- Other outcomes: iron studies, hemoglobin, health services

Weighted average hemoglobin ≥9 g/dL without any packed red blood cell transfusions for a continuous period of ≥12 months.

Clinical Evidence

Overview of Beti-cel Clinical Trials for TDT

- **Four open-label trials** and one long-term follow-up cohort:
- HGB-204/-205; Two phase I/II trials (n=22); age range 12 – 35
- **Northstar 2 & 3; Two phase III trials** (n=41); age range 4 – 34

- Manufacturing change occurred *before* the two phase III trials
- Each trial had two years of f/u, ongoing f/u in cohort (+ 13 yrs)
- Last phase III participants have just completed follow-up

Beti-cel and Transfusion Independence

- Among 41 participants from the NorthStar-2 and -3 trials with sufficient follow-up to evaluate the primary endpoint, 37 (90.2%) achieved TI
- Over a median follow-up of 42 months (range 23-87) across Phase I/II and Phase III studies, no patients who achieved TI have lost TI.

Key Trial Results

	Phase I/II	Phase III
Follow-Up, median months (range)	42 (23-88)	
Enrolled, N	23	43
Infused, N	22	41
Successful Engraftment, n (%)	22/22 (100)	41/41 (100)
Time to Neutrophil Engraft., median days (range)	18 (14-30)	25.5 (13-39)
Time to Platelet Engraft., median days (range)	36 (19-191)	46 (13-94)
Hospitalization length†, median days (range)	40 (27-69)†	44 (29-92)
Transfusion Independence, n/N (%)	15/22 (68)	37/41 (90.2)*
Hb level during TI, g/dL, median (range)	10.3 (9.1-13.2)	11.3 (9.3-13.7)

dL: deciliter, g: grams, Hb: hemoglobin, n: number, N: overall number, TI: transfusion independence
*Based on publicly available data from FDA advisor committee meeting 6/2022. † From conditioning through discharge

Harms

- No cases of malignancy in TDT
  - SCD: (1) AML, (1) MDS
  - CALD: (3) MDS with concern for insertional oncogenesis (1)
- No deaths
- Grade ≥3 AEs were common and most often related to myeloablative procedures

Grade ≥3 AEs*

- Most common = thrombocytopenia (96%)
- Stomatitis (55%) and mucositis (20%)
- Neutropenia (78%), febrile neutropenia (45%)
- Veno-occlusive liver disease (12%)
- Other: epistaxis, decreased appetite, fever, anemia

*Data pooled from multiple sources

Beti-cel and Quality of Life

- Modest improvements in quality of life over two years
  - Limitation of pre-/post- design, may fail to capture improvements
  - Adult patients with TDT accustomed to complex regimen
- Across physical, mental, and social domains
- Children/ Adolescents had greater improvements in QoL compared to adults.

Beti-cel, Chelation, and Iron Overload

- Ferritin levels decreased an average of 50% over 24 months post-treatment. Participants with longer follow-up achieved normalization between 48 – 60 months.
- Liver and cardiac iron did not substantially change within 24 months, but follow-up ongoing. Improvements in LIC, n=3 at 36 months.
- Two-thirds of patients achieving TI discontinued chelation during follow-up.

Controversies and Uncertainties

- No evidence of insertional oncogenesis in TDT, but concerns in other conditions (SCD, CALD).
- Small sample size, insufficient data on long-term outcomes/ durability
  - Earliest trial participants at ~7 years of f/u in the extension cohort
- Real-world adverse effects / mortality from myeloablation
- Single arm trials
  - No comparative effectiveness against current curative therapy
  - Limited inference about secondary endpoints (QoL)

Contextual Considerations

- Moderate risk of long-term morbidity from chronic iron overload
- Short-term risk of death or rapid progression is unlikely with access current standards of care
- Lifelong chronic disease that requires frequent transfusions and chelation as early as infancy

Potential Other Benefits or Disadvantages

- Benefits to curative therapy:
  - More time and greater possible ability to achieve major life goals for patients and caregivers.
  - Reduce burden of complex regimen on patients and caregivers
- Disadvantages:
  - The procedure requires lengthy hospitalization and myeloablation carries risks in the short-term (infection), but also long-term (fertility)

Public Comments Received

- Emerging data – participants in the original trials continue under follow-up providing new data on durability and safety.
- The global perspective – The burden of Thalassemia is greatest outside of the US. We strongly hope that global manufacturers and payers can come to agreements that increase access to curative therapies for patients outside of the US.
- Blood supply – Donated blood is at times a scarce resource. If patients with TDT achieve TI, they will not utilize blood supply resources (societal benefit).

Summary: Beti-cel in TDT

- Evidence for efficacy outcomes was significant enough to suggest that the gene therapy provides a substantial net health benefit.
- Safety outcomes have been consistent with those generally expected from myeloablative conditioning and there have been no deaths in the trials
- No oncogenic events in trials, but too early to dismiss concerns.
- Durability needs to be established in longer-term follow-up.

ICER Evidence Rating for Beti-cel in TDT

Treatment	Comparator	Evidence Rating
beti-cel	Standard of care	B+

Questions?

Beta Thalassemia: Effectiveness and Value

Marina Richardson, MSc

Health Economist

Institute for Clinical and Economic Review

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Key Review Team Members

- **Jon Campbell, PhD, MS**, Senior Vice President for Health Economics, ICER
- **Noemi Fluetsch, MSc, MPH**, (Former) Research Assistant, HEOR, ICER

Disclosures: We have no conflicts of interest relevant to this report.

Objective

Estimate the cost effectiveness of betibeglogene autotemcel (beti-cel) for the treatment of transfusion-dependent beta thalassemia (TDT).

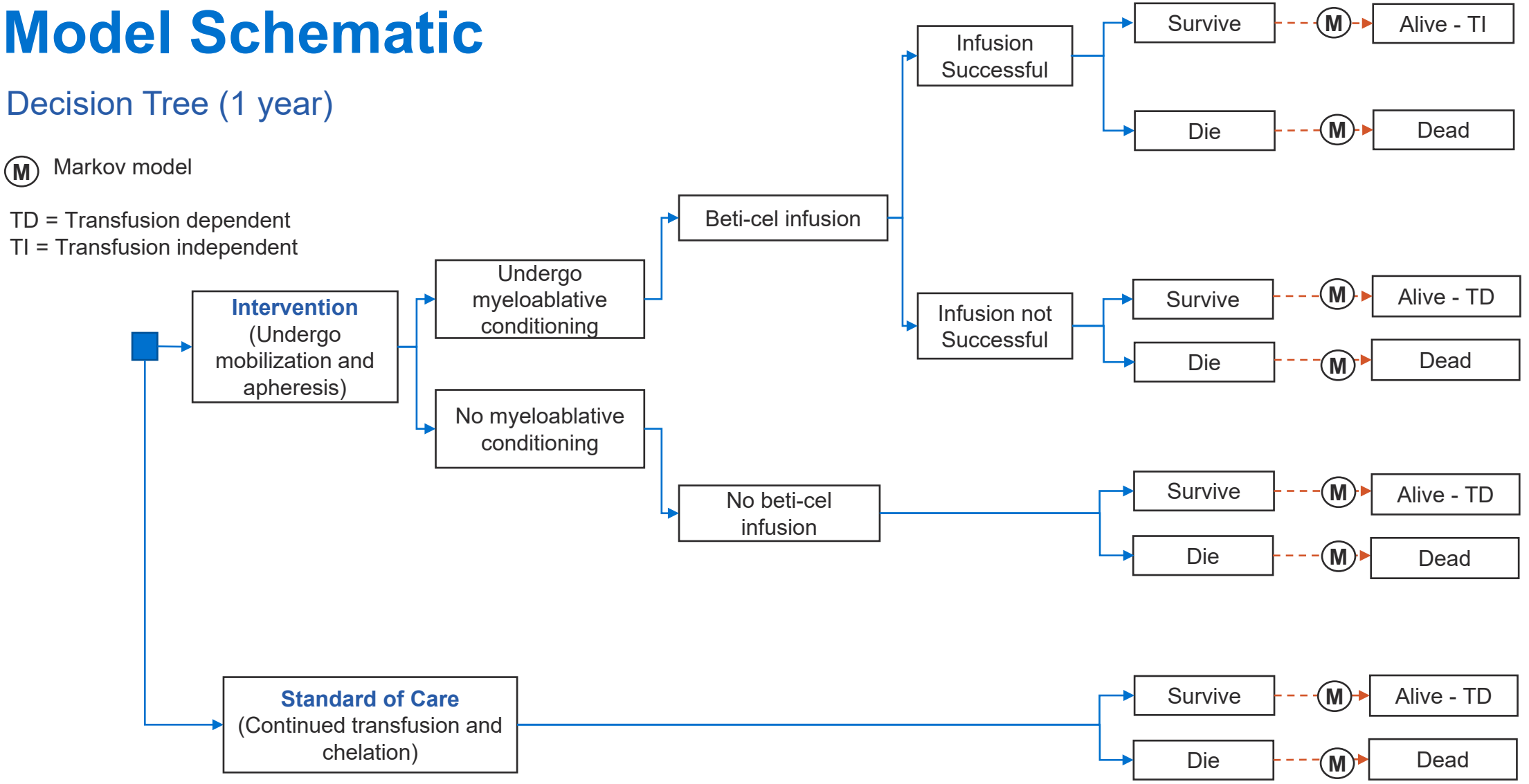
Methods in Brief

Methods Overview

- **Model**: Decision tree followed by a three-state Markov cohort model (transfusion independent [TI], transfusion dependent [TD], and dead)
- **Setting**: United States
- **Perspective**: Health Care Sector Perspective and Modified Societal Perspective
- **Time Horizon**: Lifetime
- **Discount Rate**: 3% per year (costs and outcomes)
- **Cycle Length**: 1 year
- **Primary Outcomes**: QALYs, LYs, evLYs, costs, and TD years averted

Model Schematic

Decision Tree (1 year)



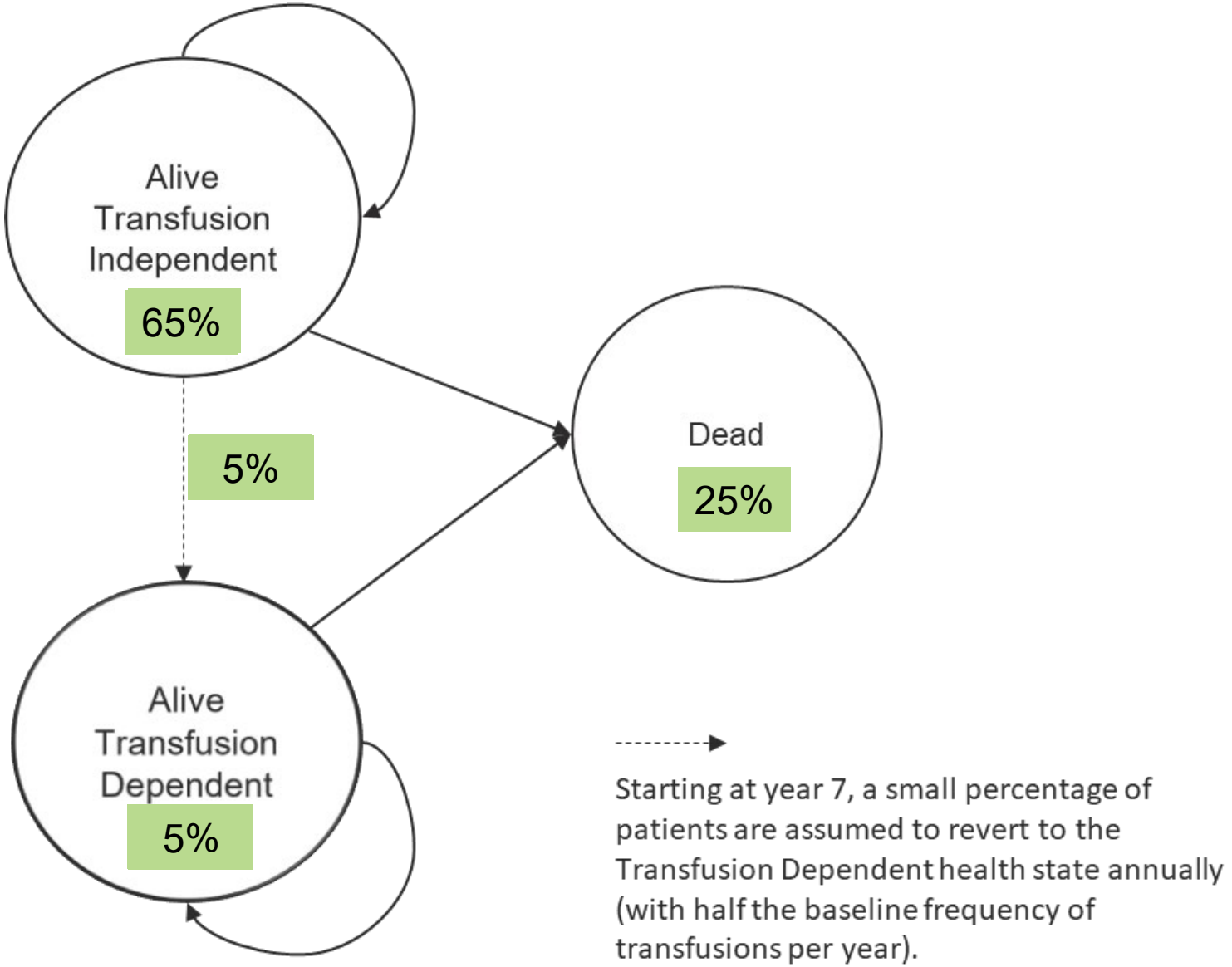
Model Schematic

Markov Model (Lifetime)

Year 2 (Beti-cel arm):
- Mean age : 23 years

Year 10 (Beti-cel arm):
- Mean age : 31 years

Year 40 (Beti-cel arm):
- Mean age : 61 years



Key Model Assumptions

- Risk of death from beti-cel infusion was 1.4%.
- At the end of the lifetime time horizon, 10% of patients achieving TI with beti-cel had reverted to the TD health state with half the baseline frequency of transfusions per year.
- Age-dependent inputs were accommodated when modeling iron overload complications, transfusion and chelation costs, and caregiver costs.
- Disutility, costs, and mortality associated with cardiac, liver, and endocrine complications from iron overload were included.
- Beti-cel retreatment and hematopoietic stem cell transplantation (HSCT) were not included in the model.

Model Cohort Characteristics

Baseline Characteristic	Value	Source
Mean age, years	22.2 (SD 12.5)	Kwiatkowski et al., 2012 (Thalassemia Longitudinal Cohort Study)
Female, %	52.3	
Mean number of transfusions per year	14.95 (< 18 years) 16.1 (≥ 18 years)	Market Scan data reported in Kansal et al., 2021
Iron stores (low / moderate / high), %	41 / 40 / 19	
Liver iron (low / moderate / high), %	45 / 32 / 23	
Cardiac iron (low / moderate / high), %	71 / 29 / 0	

Treatment-related Efficacy

- Transfusion independence: 34/38 (89.5%)
  - Phase III trials (NorthStar-2 and -3 trials), n=38 participants had sufficient follow-up to evaluate the primary endpoint of transfusion independence.

Disutilities

Health State	Disutility	Source
Transfusion dependent	-0.22	Shah 2021
Transfusion independent	-0.02	Kansal 2021

Intervention	Disutility	Source
Infusion (1 year)	-0.31	Matza 2021

Complication	Disutility	Source
Cardiac	-0.03	Seyedifar 2015
Liver	-0.03	
Diabetes	-0.04	
Hypogonadism	-0.04	

Beti-cel Acquisition Cost

- Anticipated beti-cel acquisition cost: $2.1 million US (WSJ Press Release, 2019)
- Anticipated outcomes-based agreement included full upfront payment with an 80% payback option.

Other Costs

- Beti-cel associated costs:
  - Beti-cel work up, pre-transplant, and fertility preservation: $27,500
  - Transplant: <18 years $128,300; >18 years $71,400
  - Post-transplant monitoring: $8,500 per year in Years 1-6
  - Iron normalization period:<18 years $29,500; >18 years $50,500
- Annual transfusion and chelation costs: <18 years $44,300; >18 years $88,800
- Annual productivity costs for patient and caregiver associated with transfusion dependence:$18,600

Mortality

Mortality	Value	Source
SMR (TD health state)	3.9	Delea et al. 2007
SMR (TI health state)	1.25	Kansal et al. 2021 (assumption)

SMR: standardized mortality ratio, TD: transfusion dependent, TI: transfusion independent

Results

Base-Case Results

Health Sector Perspective

	Cost	QALYs	evLYs
Beti-cel	$2,730,000	18.70	18.97
SOC	$2,260,000	13.76	13.76

Modified Societal Perspective

	Cost	QALYs	evLYs
Beti-cel	$2,910,000	18.58	18.86
SOC	$2,740,000	13.65	13.65

evLYs: equal value of life years, QALYs: quality-adjusted life years, SOC: standard of care

Base-Case Incremental Results

Health Sector Perspective

Drug	Cost per QALY gained	Cost per evLY gained
Beti-cel vs. SOC	$95,900	$90,800

Modified Societal Perspective

Drug	Cost per QALY gained	Cost per evLY gained
Beti-cel vs. SOC	$35,100	$33,300

SOC: standard of care, QALYs: quality-adjusted life years, evLYs: equal value of life years

Sensitivity Analyses

- One way sensitivity analysis
  - Key drivers
    - Number of transfusions per year
    - Annual cost of chelation therapy
    - Mean starting age
  - Range from $47,000 to $145,000 per QALY gained
- Probabilistic sensitivity analysis
  - Majority of simulations cost effective at $100,00/QALY and $150,000/QALY thresholds

Scenario Analyses

- Optimistic and conservative benefit
  - Modifying the acute risk of death from beti-cel, beti-cel efficacy, and durability of treatment effect.
- No outcomes-based agreement

Scenario Analyses: 50:50 Shared Savings

Health Sector Perspective

Scenario	Intervention	Cost per QALY gained	Cost per evLY gained	Cost per LY gained
50:50 Shared Savings	Beti-cel vs. SOC	$246,400	$233,300	$430,600

Modified Societal Perspective

Scenario	Intervention	Cost per QALY gained	Cost per evLY gained	Cost per LY gained
50:50 Shared Savings	Beti-cel vs. SOC	$215,900	$204,400	$377,400

evLYs: equal value of life years, LY: life year, QALYs: quality-adjusted life years, SOC: standard of care, TD: transfusion dependent

Health Benefit Price Benchmarks (HBPBs)

Price Benchmarks for Beti-cel: 50:50 Shared Savings Analysis

Perspective	Anticipated Acquisition Cost*	Price at $100,000 per QALY gained Threshold	Price at $150,000 per QALY gained Threshold	Discount from Anticipated Acquisition Cost to Reach Threshold Prices
Health Care System	$2,100,000	$1,300,000	$1,570,000	25% - 38%
Societal	$2,100,000	$1,460,000	$1,740,000	17% - 30%

*Excludes beti-cel workup and preparation, transplant, post-transplant monitoring or post-transplant normalization period costs. Unit price represents the full upfront acquisition cost of beti-cel per patient and is based on the full upfront payment for beti-cel with 80% payback option proposed by the manufacturer.

Limitations

- The effectiveness, durability, and safety of beti-cel was based on clinical trial data with small sample sizes and limited follow-up time.
- Limited data was available to inform the duration of the iron normalization period and the probability of complications post beti-cel transplant.
- We used a Markov-based cohort model that required assumptions for modeling age-related iron overload complications and costs.
- Analysis based on an anticipated beti-cel acquisition cost and outcomes-based agreement.

Comments Received

- Suggestion for beti-cel treatment effectiveness to be based on the outcomes for all 41 patients included in the Phase 3 trials (which included 3 non-evaluable patients) and durability should be considered life long.
- Concern that more appropriate methodology would be to use a patient-level model for analysis compared to our cohort-based approach.

Conclusions

- Our findings suggest that beti-cel provides net health benefits to patients with transfusion dependent beta thalassemia.
- At an anticipated price of $2.1 million with an 80% payback option we found that beti-cel meets commonly accepted value thresholds.
- Under a shared savings scenario where half the cost offsets are returned to society, the health benefit price benchmark range is $1.3 to $1.8 million.

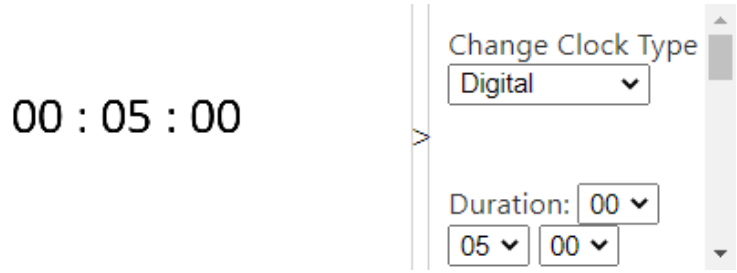
Questions?

Public Comment and Discussion

Clark Paramore, MSPH
Head of Value Demonstration, bluebird bio

Conflicts of Interest:

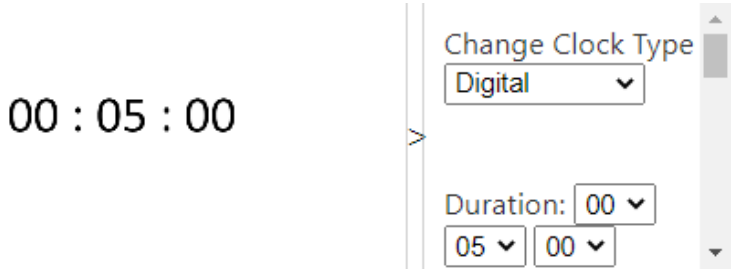
- *Clark is a full-time employee of bluebird bio.*



Craig Butler
National Executive Director, Cooley's Anemia Foundation

Conflicts of Interest:

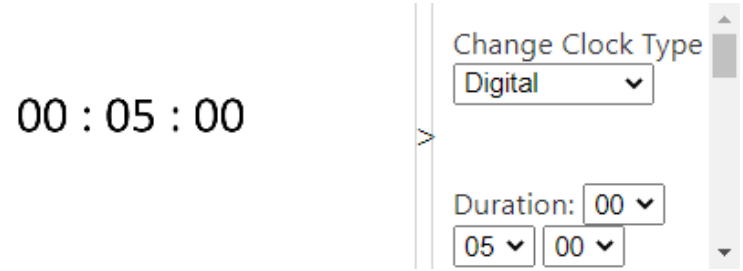
- *The Cooley's Anemia Foundation receives grants from several companies to support activities such as their Patient-Family Conference Fundraising Gala and Care Walk. Companies are not involved in determining or directing content for any activities.*
  - *bluebird bio (20% of funding, Chiesi Global Rare Diseases (20% of funding), Vertex (20% of funding), Bristol Myers-Squibb (15% of funding), Merck (15% of funding), Agios (5% of funding), Hemanext (5% of funding)*



Lily Cannon
Operations Manager, Thalassaemia International Federation

Conflicts of Interest:

- *The Thalassaemia International Federation is a non-profit, non-governmental patient organization that receives financial support from several stakeholders. Financial supporters have no say in the development of our programs, projects, publications or any other activities of the Federation, as per our Code of Ethics.*
  - *bluebird bio (9.22% of funding), Novartis (6.97% of funding), Bristol Myers Squibb (3.84% of funding), SVifor Pharma (2.77% of funding), Pharmapal (2.32% of funding), Agios (2% of funding), Hemanext (1.67% of funding), Resonance Health (0.58% of funding)*



Lunch

Meeting will resume at 12:50pm

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Voting Questions

Patient population for all questions: *Patients living with transfusion-dependent thalassemia, typically defined as eight or more transfusions per year.*

Clinical Evidence: Given the currently available evidence, is the evidence adequate to demonstrate that the net health benefit of betibeglogene autotemcel is superior to that provided by standard clinical management (e.g., transfusion and chelation)?

A. Yes

B. No

Contextual Considerations and Potential Other Benefits or Disadvantages

Please vote on the following contextual considerations:

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

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

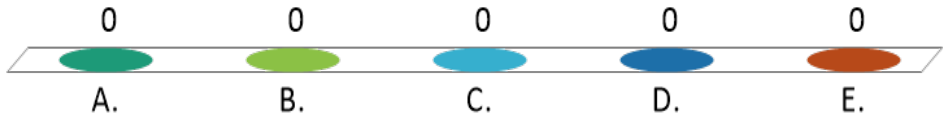
A. Very low priority

B. Low priority

C. Average priority

D. High priority

E. Very high priority



Please vote on the following contextual considerations:

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

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

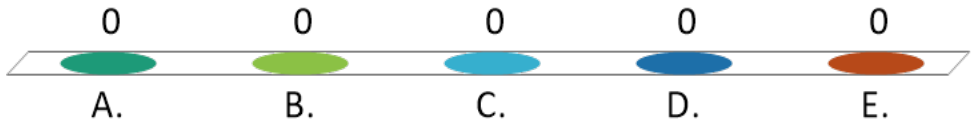
A. Very low priority

B. Low priority

C. Average priority

D. High priority

E. Very high priority



Please vote on the following potential other benefits or disadvantages:

What are the relative effects of betibeglogene autotemcel versus standard clinical management on the following outcomes that inform judgement of the overall long-term value for money of betibeglogene autotemcel?

1. Patients' ability to achieve major life goals related to education, work, or family life

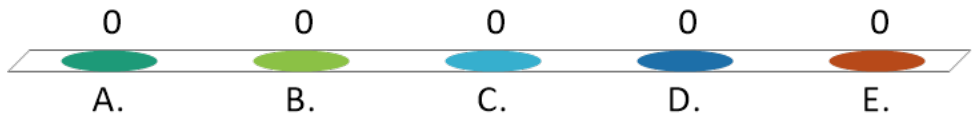
A. Major negative effect

B. Minor negative effect

C. No difference

D. Minor positive effect

E. Major positive effect



Please vote on the following potential other benefits or disadvantages:

What are the relative effects of betibeglogene autotemcel versus standard clinical management on the following outcomes that inform judgement of the overall long-term value for money of betibeglogene autotemcel?

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

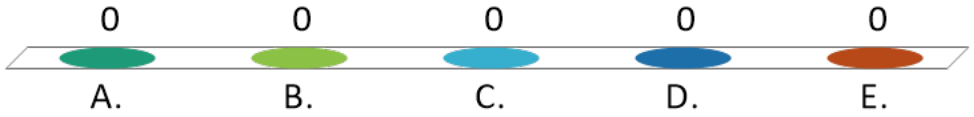
A. Major negative effect

B. Minor negative effect

C. No difference

D. Minor positive effect

E. Major positive effect



Please vote on the following potential other benefits or disadvantages:

What are the relative effects of betibeglogene autotemcel versus standard clinical management on the following outcomes that inform judgement of the overall long-term value for money of betibeglogene autotemcel?

3. Patients' ability to manage and sustain treatment given the complexity of regimen

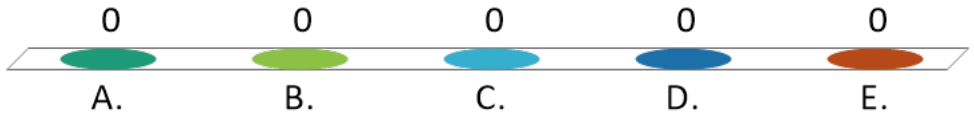
A. Major negative effect

B. Minor negative effect

C. No difference

D. Minor positive effect

E. Major positive effect



Please vote on the following potential other benefits or disadvantages:

What are the relative effects of betibeglogene autotemcel versus standard clinical management on the following outcomes that inform judgement of the overall long-term value for money of betibeglogene autotemcel?

4. Society's goal of reducing health inequities

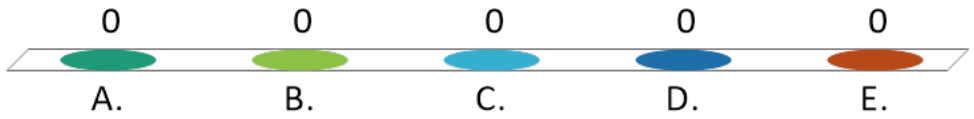
A. Major negative effect

B. Minor negative effect

C. No difference

D. Minor positive effect

E. Major positive effect



Please vote on the following potential other benefits or disadvantages:

What are the relative effects of betibeglogene autotemcel versus standard clinical management on the following outcomes that inform judgement of the overall long-term value for money of betibeglogene autotemcel?

5. A potential advantage for therapies that offer a new treatment choice with a different balance or timing of risks and benefits that may be valued by patients with different risk preferences.

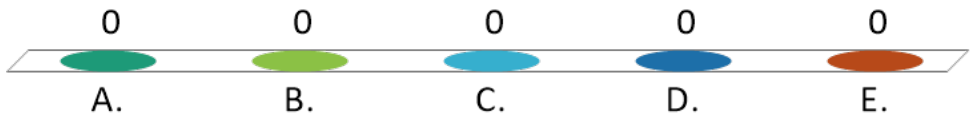
A. Major negative effect

B. Minor negative effect

C. No difference

D. Minor positive effect

E. Major positive effect



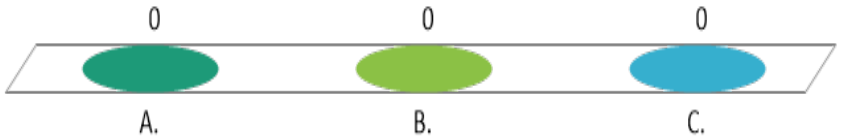
Long-term Value for Money

1. Assuming the pricing and outcomes-based arrangement presented in the report, what is the long-term value for money of betibeglogene autotemcel versus standard clinical management?

A. Low long-term value for money at assumed pricing and outcomes-based arrangement

B. Intermediate long-term value for money at assumed pricing and outcomes-based arrangement

C. High long-term value for money at assumed pricing and outcomes-based arrangement



Break

Meeting will resume at 2:10pm

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Policy Roundtable

Policy Roundtable Participants

Participant	Conflict of Interest
Monica Bhatia, MD, Associate Professor of Pediatrics and Director, Pediatric Stem Cell Transplant Program, Columbia University Medical Center	None.
Nathan Connell, MD, MPH, Associate Professor of Medicine, Harvard Medical School, Brigham and Women's Hospital	Dr. Connell has received funding in excess of $5,000 from Takeda Pharmaceuticals.
Leslie Fish, PharmD, Senior Vice President, IPD Analytics	None.
Priyanka Kumar, Beta Thalassemia Patient and Advocate	None.
Clark Paramore, MSPH, Head of Value Demonstration, bluebird bio	Mr. Paramore is a full-time employee of bluebird bio.
Erik Schindler, PharmD, BCPS, Director, Emerging Therapeutics and Outcomes-Based Contracting, UnitedHealthcare Pharmacy	Dr. Schindler is a full-time employee of UnitedHealthcare.
Eileen Scott, Patient Services Manager, Cooley's Anemia Foundation	None.

CEPAC Council Reflections

Next Steps

- Meeting recording posted to ICER website next week
- Final Report published on or around July 19, 2022
  - Includes description of New England CEPAC votes, deliberation, policy roundtable discussion
- Materials available at: https://icer.org/beta-thalassemia-2022/#timeline

Adjourn

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