Sonpiretigene Isteparvovec for Advanced Retinitis Pigmentosa: Effectiveness and Value

Public Meeting — April 11, 2025

Meeting materials available at: https://icer.org/assessment/retinitis-pigmentosa-2025

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NEW ENGLAND CEPAC
COMPARATIVE EFFECTIVENESS PUBLIC ADVISORY COUNCIL

Patient Experts

Todd Durham, PhD, Senior Vice President, Clinical and Outcomes Research, Foundation Fighting Blindness

- *Foundation Fighting Blindness (FFB) has received sponsorships from various health care companies, including Nanoscope Therapeutics, for their scientific conferences, accounting for<25% of their funding. A member of Dr. Durham's household works in the life sciences industry and receives ≥25% of income from the industry. Additionally, the RD Fund, a venture philanthropy subsidiary of the FFB, has equity interests in several life science companies in its portfolio.*

Julie Grutzmacher, MSW, MPH, Director of Patient Advocacy and Population Health Initiatives, Prevent Blindness

- *Prevent Blindness receives >25% of funding from health care companies, including Nanoscope Therapeutics.*

Clinical Experts

Stephen Russell, MD, Professor of Ophthalmology, University of Iowa

- *Dr. Russell has received funds from Spark Therapeutics, ProQR Therapeutics, Novartis, Digital Diagnostics (IDx, LLC) and has stock ownership in Digital Diagnostics (IDx, LLC).*

Vinit B. Mahajan, MD, PhD, Professor Ophthalmology, Stanford University

- *Dr. Mahajan has received funds from Nanoscope Therapeutics, Chigenovo, and Kerna Labs.*

ICER Speakers

Sarah K. Emond, MPP
President & CEO

Anil Makam, MD, MAS
Evidence Author
Associate Professor, UCSF

David Rind, MD, MSc
Chief Medical Officer, ICER

Marina Richardson, PhD, MSc
Lead Modeler, Associate Director, HTA Methods and Health Economics, ICER

Why are we here today?

“This is a life of constant adjustments and adaptations, because once you think you have one level of sight down, you have to learn how to accommodate all over again.”

“The eyes are very important. You pick up all those gestures in the room, or just visual cues. You know you lose that when you have an eye condition, an impairment, it changes the quality of your life. There's not a lot of jobs you can do. People don't want to work with you. They don't understand you. It alters your life course journey, too. So, wherever you were succeeding, you have to now change course and decide. Okay, how do I adapt?"

Individuals living with RP

Why Are We Here Today?

- What happens the day this treatment receives FDA approval?
- Questions about:
  - What are the risks and benefits?
  - How does this new treatment 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

DIAGNOSIS: DEBT

100 Million People in America Are Saddled With Health Care Debt

By Noam N. Levey

JUNE 16, 2022

Why Delaware is eying a 27% premium hike on state employees' health insurance

Amanda Fries

Delaware News Journal

Published 4:35 a.m. ET Feb. 1, 2024 | **Updated 9:29 p.m. ET Feb. 6, 2024**

NBER

National Bureau of Economic Research

WHO PAYS FOR RISING HEALTH CARE PRICES?

100 Million People in America Are Saddled With Health Care Debt (KFF Health News)

Why Delaware is eyeing a 27% premium hike on state employees' health insurance (Delaware Online)

Organizational Overview

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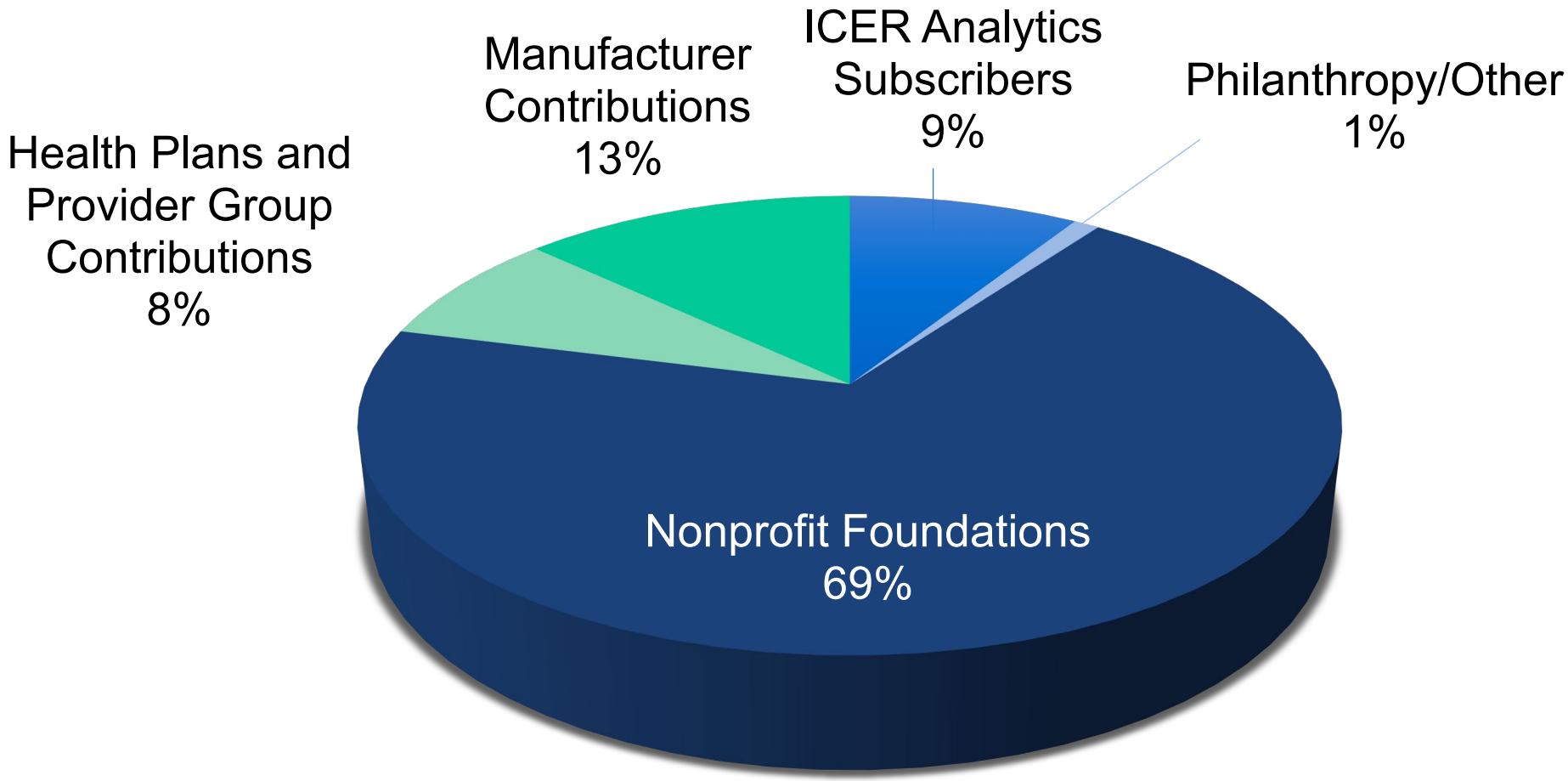
Funding 2025



ICER received significant funding from Arnold Ventures, The California Health Care Foundation, The Patrick and Catherine Weldon Donaghue Medical Research Foundation, and the Peterson Center on Healthcare, LLC. Source: https://icer.org/who-we-are/independent-funding/sources-of-funding/

How Was the ICER Report Developed?

Guidance from patients, clinical experts, manufacturers, and other stakeholders

Evidence analysis in collaboration with the University of California, San Francisco.

- **Mark Pennesi, MD, PhD,** Professor of Ophthalmology, Oregon Health and Science University
- **Stephen Russell, MD,** Professor of Ophthalmology, University of Iowa
- **Marita Zimmerman, MPH, PhD,** Senior Research Economist, Bill and Melinda Gates Foundation
- **Todd Durham, PhD,** Senior Vice President, Clinical Outcomes Research, Foundation Fighting Blindness
- **Ben Shaberman, MS, MA,** Vice President, Science Communications, Foundation Fighting Blindness

Structured to support **New England CEPAC** voting and policy discussion

Value Assessment Framework: Long-Term Value for Money

Agenda (ET)

10:00 AM	Meeting Convened and Opening Remarks
10:20 AM	Presentation of the Clinical Evidence
11:00 AM	Presentation of the Economic Model
11:40 AM	Public Comments and Discussion
12:00 PM	Lunch Break
12:50 PM	New England CEPAC Deliberation and Vote
1:50 PM	Break
2:00 PM	Policy Roundtable Discussion
3:30 PM	Reflections from New England CEPAC
4:00 PM	Meeting Adjourned

Presentation of the Clinical Evidence

Anil Makam, MD, MAS

Associate Professor of Medicine

University of California, San Francisco (UCSF)

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Key Team Members

Name	Title
Anil N. Makam, MD, MAS	Associate Professor of Medicine, UCSF
Avery McKenna, BS	Research Lead
Belen Herce-Hagiwara, BA	Senior Research Assistant
Sol Sanchez, BA	Research Assistant

Disclosures

UCSF, on behalf of ANM, received funding from ICER for this report.

AM, BHH, and SS are employees of ICER.

We have no conflicts of interest to disclose, financial or otherwise.

Retinitis Pigmentosa (RP) is an inherited disease that causes progressive degeneration of photoreceptor cells

Epidemiology

- Incidence: 1 in 4,000
- US Prevalence: 80-110K
- Not lethal
- Age of onset & rate of progression depend on genetic mutation

Key Symptoms

- Loss of night & peripheral vision
- Difficulty with light changes and seeing colors
- 12% have advanced RP: central vision loss ranging from counting fingers to no light perception

Treatment

- No cures or disease-modifying therapies
- Usual care: vision aids, visual rehabilitation, and managing eye complications

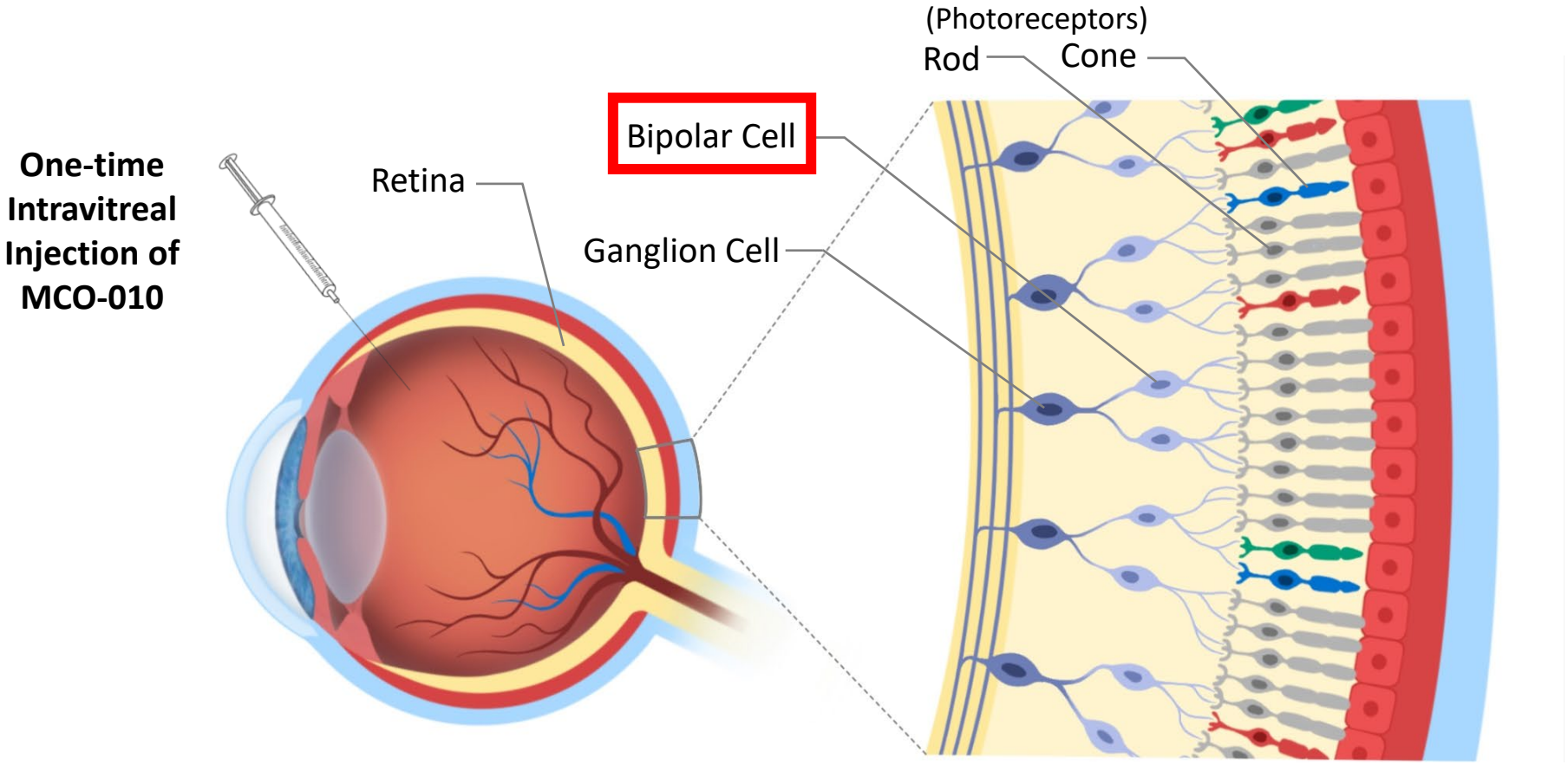
RP: retinitis pigmentosa,
US: United States

Optogenetic therapy is a novel approach to treat RP

- Gene therapy that inserts light-sensitive proteins (opsins) to allow non-photoreceptor cells in the retina to act as photoreceptors to restore vision.
- Mutation agnostic; works across the full range of RP mutations.
- Reserved for the treatment of advanced RP with severe vision loss.

RP: retinitis pigmentosa

Sonpiretigene isteparvovec is the first optogenetic therapy to be considered for approval



Insights from Discussions with Patients

- Slow gradual progression until advanced RP when it becomes more rapid
- Many visual symptoms that vary day-to-day and affect regular activities
- Psychosocial & emotional distress, especially worry if completely blind
- With adaptation can lead meaningful lives, but often contingent on socioeconomic status
- Eager for treatments that restored greater visual functioning, but if completely blind, regaining some light perception would be helpful

RP: retinitis pigmentosa

Primary Outcome: Best-Corrected Visual Acuity (BCVA)

- Measured using a validated computerized tool (FrACT) for low vision
- Scored using LogMAR, where higher scores are worse
- MCID: >0.3 LogMAR change (or three lines on the chart)

FrACT: Freiburg Visual Acuity Test, LogMAR: logarithmic minimum angle of resolution, MCID: minimal clinically important difference
Figure Source: Mones 2024

Primary Outcome: Best-Corrected Visual Acuity (BCVA)

- Ranges from 0 for 20/20 vision to a **floor value of 2.25**

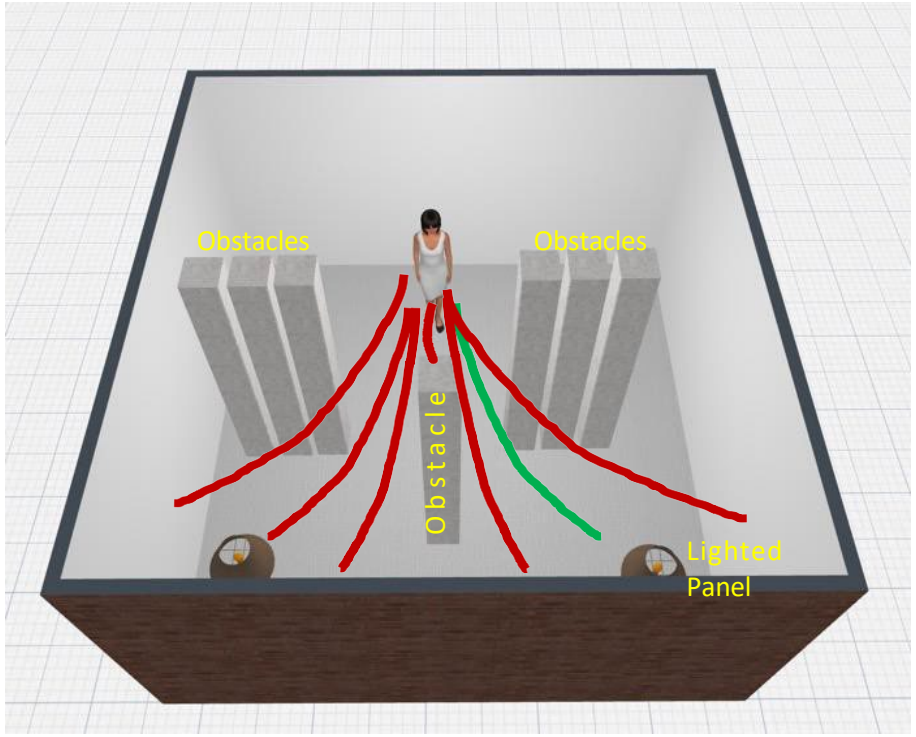
Visual Stage	Better Than Counting Fingers	Counting Fingers	Hand Movement	Light Perception	No Light Perception
LogMAR	~1.4 to 1.8	~1.8 to 2.1	~2.1 to 2.25	Not measurable	Not measurable

LogMAR: logarithmic minimum angle of resolution

Secondary Outcome: Mobility

Multi-Luminance Y-Mobility Test (MLYMT):

- Manufacturer-developed
- Six light levels: 100 lux (overcast day) to 0.3 lux (dark night sky)
- Success at each level = correct ID of the lighted panel three times
- MCID: ≥2 light level improvement

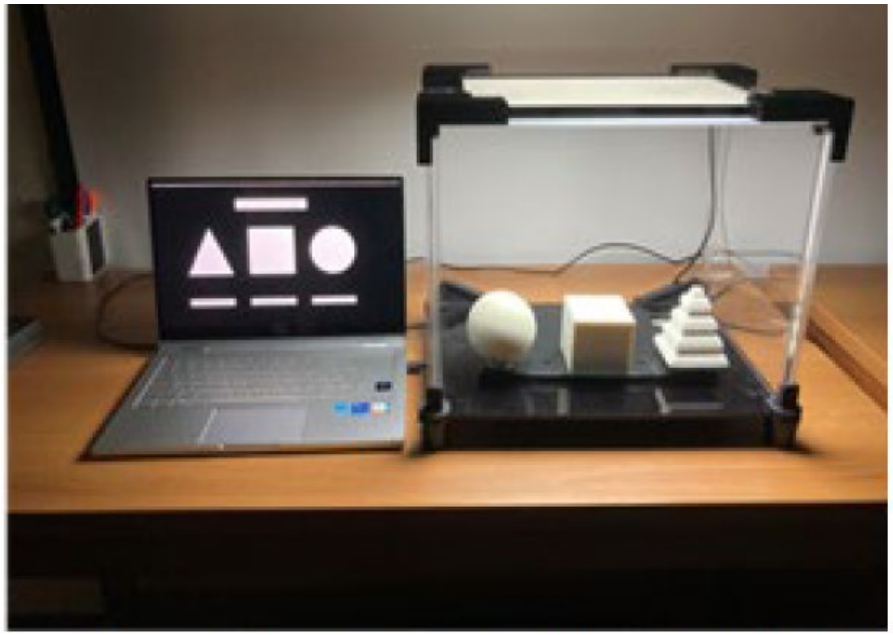


ID: identification, MCID: minimal clinically important difference
Figure Source: Boyer 2023

Secondary Outcome: Shape Discrimination

Multi-Luminance Shape Discrimination Test (MLSDT):

- Manufacturer-developed
- Five light levels: 21 lux (dimly lit room) to 0.2 lux (dark night sky)
- Success at each level = correct ID of the shapes three times
- MCID: ≥2 light level improvement



ID: identification, MCID: minimal clinically important difference
Figure Source: Carlson 2024

Clinical Evidence

Overview of Evidence

RESTORE Trial (N=27)

- Phase 2b randomized, double-masked, multicenter, sham-controlled trial
- 100-week duration; change-from-baseline outcomes assessed at 52 weeks
- Tested two different doses (low and high) vs sham of a single eye
- Adults with advanced RP with BCVA >1.9 LogMAR in the study eye
    - Mean age of 56, 93% White, mean LogMAR of 2.2 (~hand movement)

BCVA: best corrected visual acuity, LogMAR: logarithmic minimum angle of resolution, RP: retinitis pigmentosa

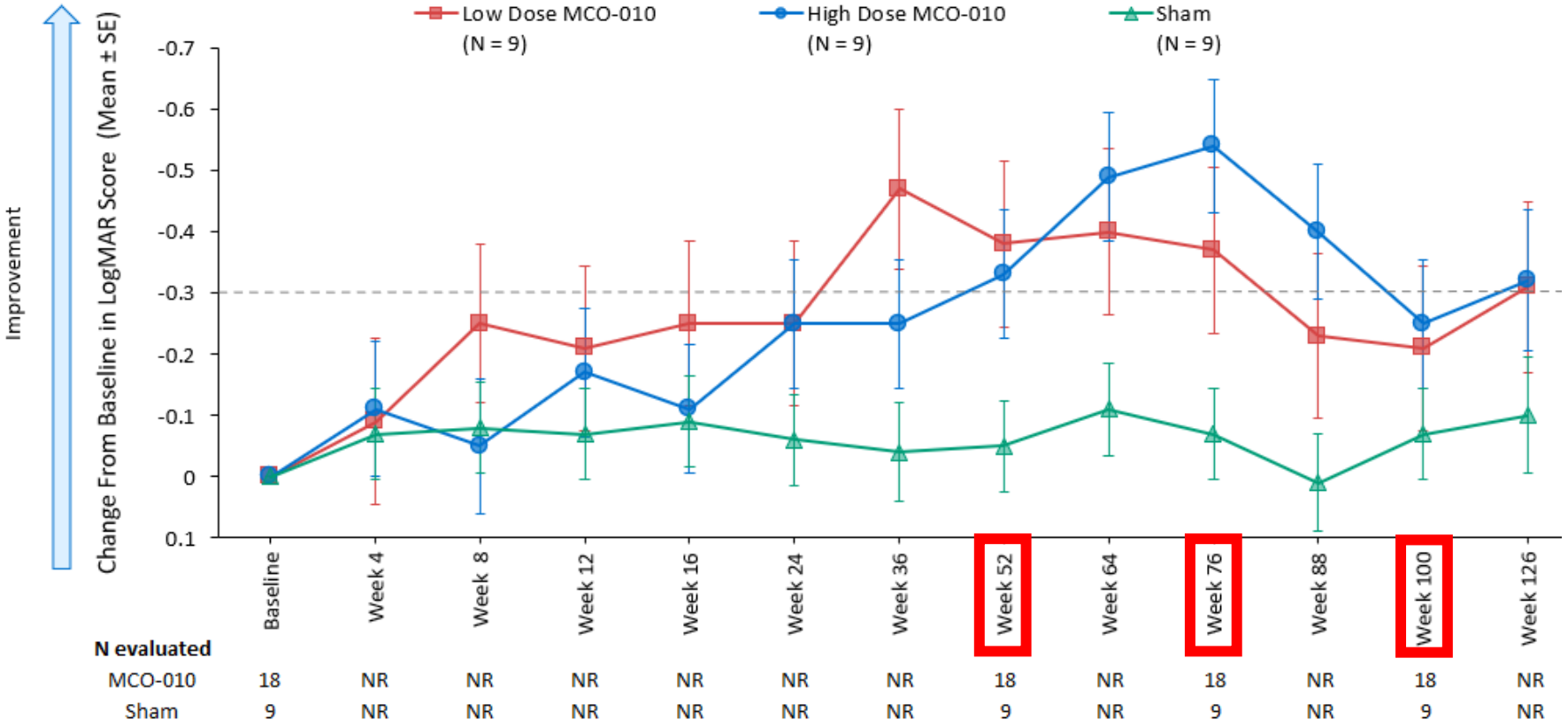
Primary Outcome Results for Visual Acuity (BCVA)

Mean BCVA (LogMAR)	Low-Dose Sonpiretigene (N=9)	High-Dose Sonpiretigene (N=9)	Sham Control (N=9)
Change-From-Baseline (SEM)	-0.38 (0.12)	-0.34 (0.08)	-0.05 (0.07)
P-Value vs Sham	0.029	0.021	-

- Seven (39%) treated participants responded (>0.3 LogMAR) vs one (11%) sham control

BCVA: best corrected visual acuity, LogMAR: logarithmic minimum angle of resolution, SEM: standard error of the mean

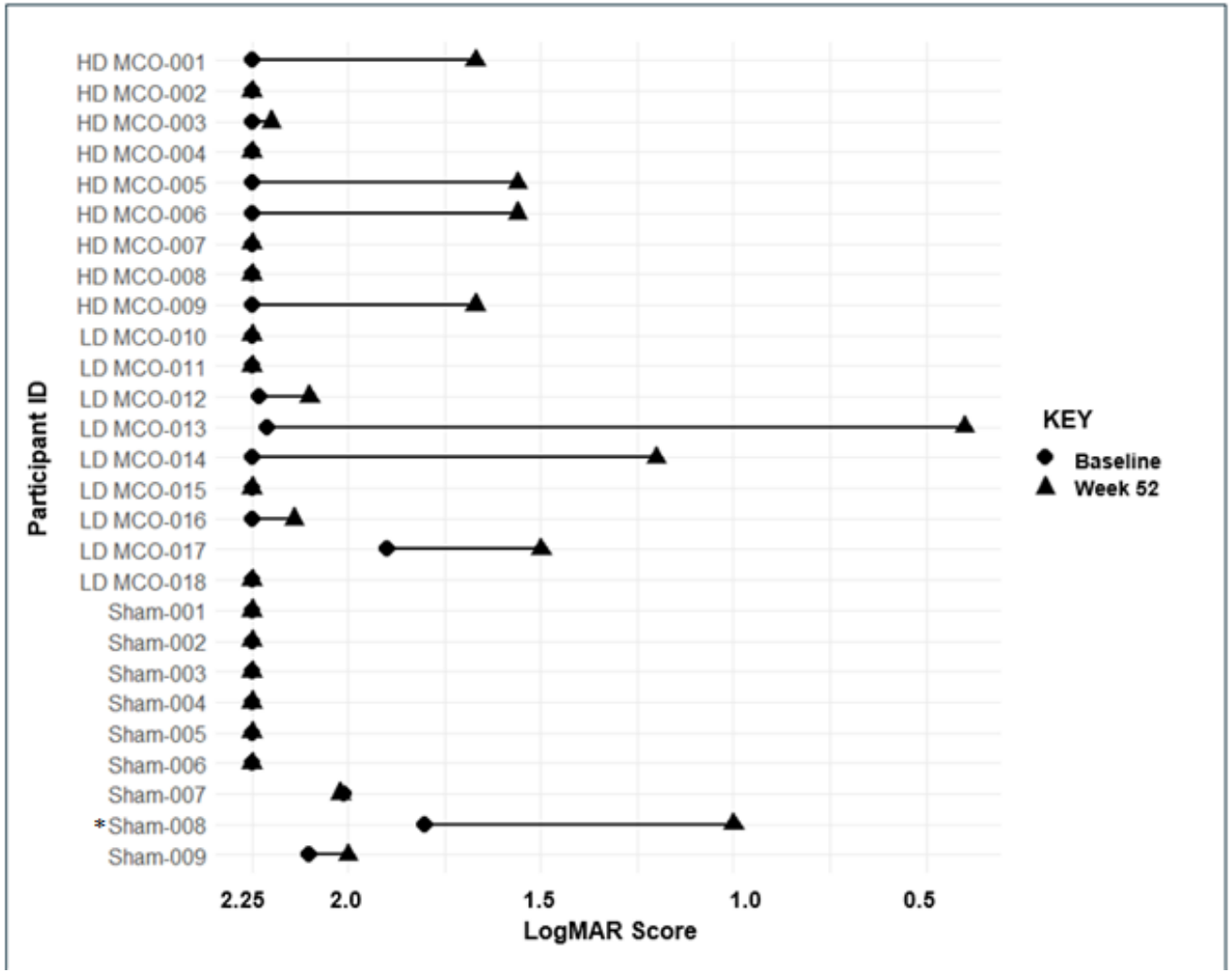
Change in Visual Acuity Over Time



MCID Threshold

LogMAR: logarithmic minimum angle of resolution, MCID: minimal clinically important difference, N: number, NR: not reported, SE: standard error
Figure adapted with permission.

Individual Participant Response for Visual Acuity



- Ten sonpiretigene-treated participants had detectable improvement, but wide range (-0.04 to -1.83)
- Eight sonpiretigene-treated and six sham-control participants had **no** detectable improvement
  - All at floor (2.25) at baseline
- Sham-008 Participant: Major protocol deviation related to incorrect recording of BCVA

BCVA: best corrected visual acuity, HD: high-dose, LD: low-dose, LogMAR: logarithmic minimum angle of resolution

Secondary Outcomes

Light Level Improvement (Mean)	Mobility		Shape Discrimination	
	Combined Sonpiretigene (N=18)	**Sham Control** (N=9)	**Combined Sonpiretigene** (N=18)	**Sham Control** (N=9)
at Baseline	1.17	1.0	0.83	1.67
at Week 52	4.17	3.0	2.44	1.89
Change-from-baseline (SEM)	**+3.0 (0.59)**	**+2.0 (1.0)**	**+1.94 (0.59)**	**+0.22 (0.86)**
P-Value vs Sham	0.20	-	0.17	-
Responders*, n (%)	12 (67%)	3 (33%)	10 (56%)	2 (22%)

* Responders defined as ≥2 light-level improvement, although some were near or at the ceiling

N: number, SEM: standard error of the mean

Composite Responder Analysis at Week 52

Outcome(s)	Combined Sonpiretigene (N=18)	Sham Control (N=9)
Response in 1 outcome	18 (100%)	5 (56%)
Response in 2 outcomes	10 (56%)	1 (11%)
Response in 3 outcomes	1 (6%)	0 (0%)

Response Definitions

Visual acuity: >0.3 LogMAR
Mobility: 2+ light levels
Shape: 2+ light levels

LogMAR: logarithmic minimum angle of resolution

Harms

- Most (94%) experienced mild-moderate eye adverse events (vs 67% sham)
  - Ocular inflammation, elevated ocular pressure, conjunctival hemorrhage
- Similarly low use of topical steroid therapy between groups at week 52
- No serious harms or deaths

Controversies and Uncertainties

- Emerging evidence base consists of one small trial of short-duration not yet published
- Visual outcomes in advanced RP difficult to measure, hard to interpret (floor & ceiling effects), & inconsistency across the three outcomes
- Long-term durability is unknown
- Some experts questioned the biologic plausibility
- Potential for unmasking & reporting bias
- Evidence for treating both eyes is uncertain; potential gains may be less than one eye

Public Comments Received

- Durability of effect & safety was established in mouse models
  - *Longer term evidence in humans & to a lesser degree, larger mammals, is preferred*

- Health equity considerations for adaptation to severe loss include financial means, time flexibility, and social support

Benefits Beyond Health and Special Ethical Priorities

Key Points

- Potential for health equity gains if can restore vision for people least able to adapt to severe vision loss
  - Less caregiver burden

Summary

- Sonpiretigene appears well tolerated and could potentially have clinically meaningfully improvements in vision, at least in the short-term.
- Tempered by several uncertainties
  - Emerging evidence base with a single small trial
  - Unknown longer-term harms
  - Challenges with outcome measurement, interpretation, and consistency
  - Unknown durability of effect with uncertain biologic plausibility

ICER Evidence Rating for Sonpiretigene Isteparvovec

Treatment	Comparator	Population	Evidence Rating
Sonpiretigene Isteparvovec	Usual Care	Adults with advanced RP	**P/I**

P/I: promising but inconclusive

Questions?

Presentation of the Economic Model

Marina Richardson, PhD

Associate Director, HTA Methods and Health Economics

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Key Team Members

Name	Title
Marina Richardson, PhD	Associate Director, HTA Methods and Health Economics
Woojung Lee, PhD	Associate Director, Health Economics and Decision Modeling
Marie Phillips, BA	Health Economics Research Assistant

Disclosures

MR, WL, and MP are employees of the Institute for Clinical and Economic Review (ICER) and have no conflicts to disclose.

Objective

- To evaluate the lifetime cost-effectiveness of sonpiretigene isteparvovec (sonpiretigene) compared to usual care for the treatment of patients with advanced retinitis pigmentosa and severe vision loss.

Unmet Need

Condition	Absolute evLY Shortfall	Proportional evLY Shortfall
Retinitis Pigmentosa	14.9	71%
Other Example Conditions		
Myelodysplastic Syndrome-Induced Anemia	8.7	73%
Multiple Sclerosis	18.9	52%
Osteoporosis	2.6	19%

evLY: equal value of life years gained

Methods in Brief

Patient Pilot Project

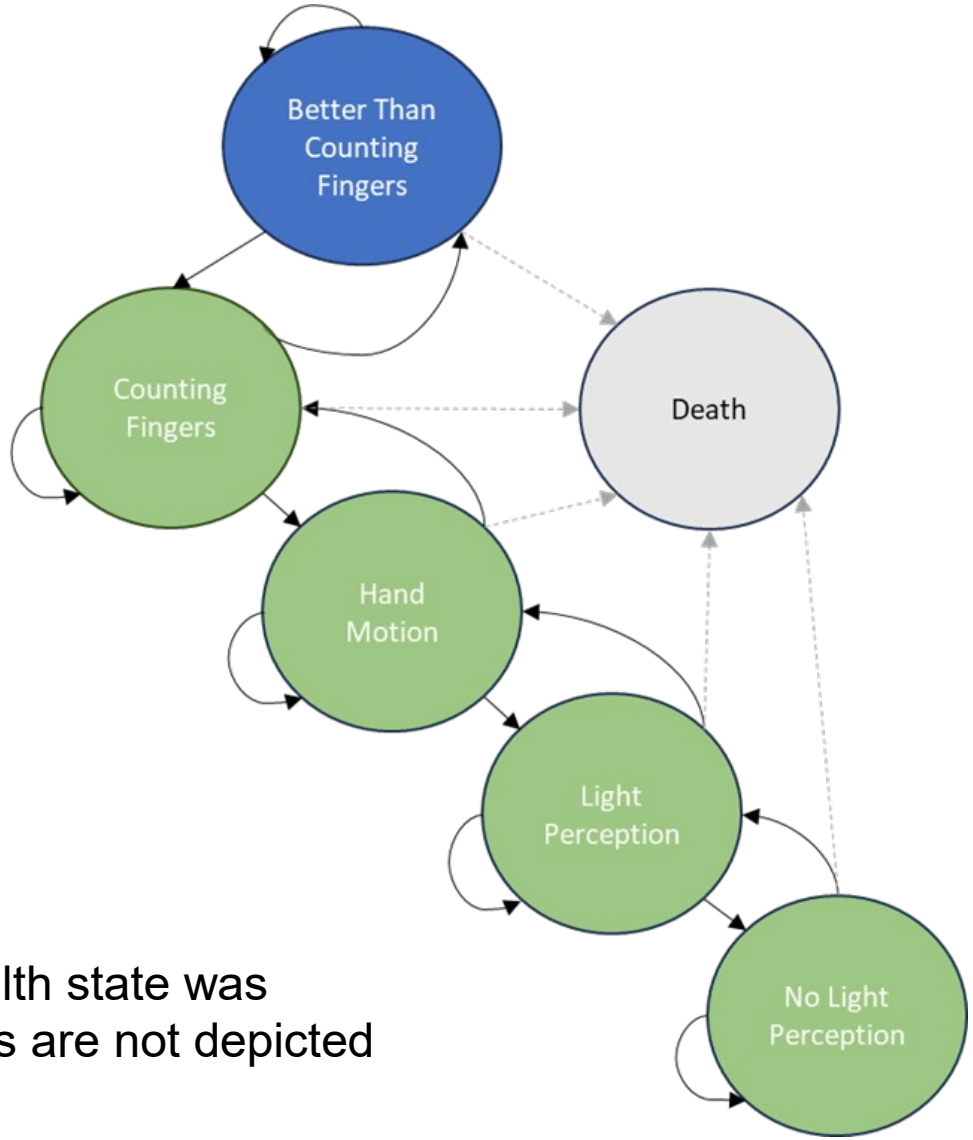
- **Aim**: To **work with the patient community** to ensure that the patient experience and goals of treatment are reflected in the cost-effectiveness analysis.
- **Structure**: Conducted **three focused sessions** with four patients from the retinitis pigmentosa community.
- The **impact** of these sessions included:
  - Choice of outcome measures selected
  - Additional scenario analysis conducted
  - Refinement and validation of data inputs
  - Highlighted important deliberative considerations

Methods Overview

Domain	Approach
Model	Markov Model
Setting	United States
Perspective	Health Care Sector Perspective*
Time Horizon	Lifetime
Discount Rate	3% per year (costs and outcomes)
Cycle Length	1 year
Primary Outcome	Cost per quality-adjusted life year (QALY) gained; equal value of life year (evLY) gained; life-years gained; **years with vision better than counting fingers gained and years with light perception gained**

* Modified Societal Perspective analysis was undertaken as a scenario analysis.

Model Schematic



Note: Movement of more than one health state was possible in the model. These transitions are not depicted in the model schematic for simplicity.

Model Characteristics

- Target Population
  - Mean age (years): 56.4
  - Percent female: 37%
  - Baseline level of visual functioning: counting fingers or worse

Key Assumptions

- **Treatment effectiveness** of sonpiretigene was modeled based on a **composite endpoint at** Week 52:
  - Best corrected visual acuity, multi-luminance y-mobility testing, multi-luminance shape discrimination testing.
  - *A priori* method to address limitations in the data.
- **Treatment effect was assumed to last for five years** followed by progressive decline in visual functioning over another five years.
  - Limited efficacy data beyond Week 100 of the RESTORE trial.

Key Model Inputs: Baseline Health State Distribution

Health State	Percentage	Source
Better Than Counting Fingers	0%	RESTORE trial†
Counting Fingers	Confidential Data Provided by the Manufacturer*	
Hand Motion		
Light Perception		
No Light Perception		

* Based on documented medical history
† One patient was removed because of a major protocol deviation. Percentages in each health state calculated using a weighted average based on the number of patients in each arm of the trial.

Key Model Inputs: Treatment Effect (Year 1)

Health State	Improved	Worsened	Stayed the Same	Source
Better Than Counting Fingers	Confidential Data Provided by the Manufacturer*			RESTORE trial
Counting Fingers				
Hand Motion				
Light Perception				
No Light Perception				

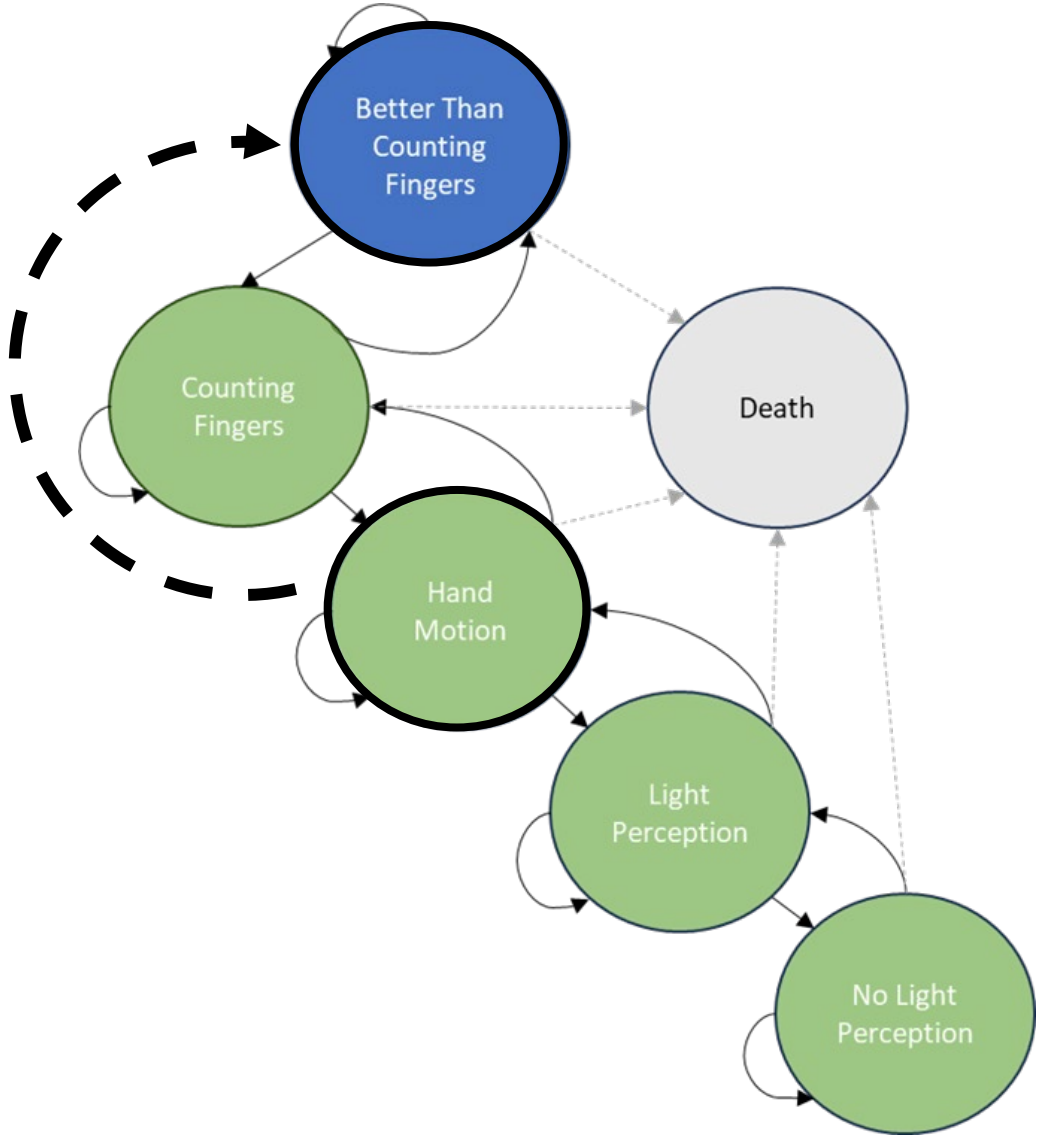
* And ICER calculation using a composite endpoint of three outcomes measures (BCVA, shape discrimination and mobility testing) and Week 52 BCVA

Hypothetical Example

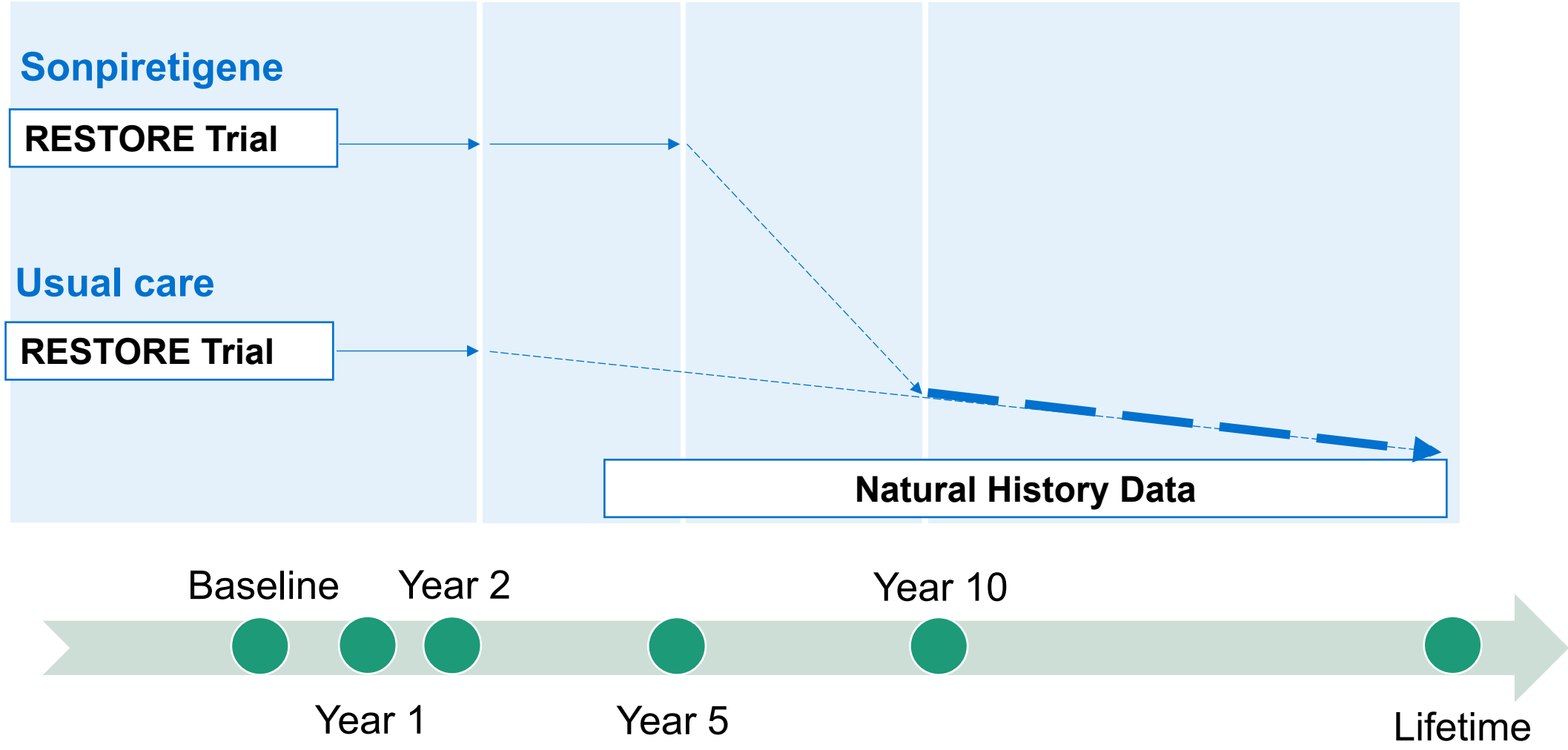
- **Baseline Health State** for Hypothetical Patient A: classified as “Hand Motion”
- **At Year 1:** Patient A experienced improvements on BCVA (LogMAR 1.75, “Better than Counting Fingers”) and Mobility testing.

BCVA (LogMAR)	Mobility Test	Shape Discrimination
2.25 to 1.75 (Improved)	Improved	No change

- **Treatment Effect at Year 1:** Patient A would be considered improved by 2 health states.



Key Model Inputs: Treatment Effect and Durability



Key Model Inputs: Treatment and Related Costs

Placeholder price for sonpiretigene: **$437,500**

- Assumes treatment in **one eye**.
- Represents half of the midpoint of the range predicted by IPD Analytics ($750,000 to $1,000,000) which assumes treatment of both eyes.

Key Model Inputs: Other Direct and Indirect Costs

Health State	Annual Direct Heath Care Costs, Mean (SD)	Annual Direct, Non-Health Care Costs, Mean (SD)	Annual Indirect Costs, Mean (SD)
Better Than Counting Fingers	$19,327 ($48,935)	$51,349 ($10,270)	$12,587 ($21,977)
Counting Fingers			
Hand Motion		$52,499 ($10,500)	
Light Perception			
No Light Perception			
Source	Frick 2012 (related and unrelated health care costs)	Brown 2016 (assisted living, low vision services and devices)	Brown 2016 (paid and unpaid labor)

SD: Standard deviation

Key Model Inputs: Utilities

Health State	Mean (SD)	Source
Better Than Counting Fingers	0.54 (0.26)	O'Brien 2023 and calculation*
Counting Fingers	0.43 (0.28)	O'Brien 2023
Hand Motion	0.38 (0.27)	O'Brien 2023 and calculation†
Light Perception	0.33 (0.26)	O'Brien 2023
No Light Perception	0.26 (0.08)	Brown 2001

* Using a weighted average of patients reaching better than counting fingers and achieving profound impairment (0.50) and severe impairment (0.65).
† Mid-point of counting fingers and light perception utilities from O'Brien 2023.

Results

Base-Case Results

Drug	QALYs	evLYs	Life Years	Years in Better Than Counting Fingers	Years with Light Perception
Sonpiretigene	6.88	6.88	17.70	3.41	15.24
Usual Care	6.17	6.17	17.70	0.18	14.67
Incremental	**0.72**	**0.72**	**0**	**3.23**	**0.57**

Note: Incremental values may not match individual intervention values due to rounding.

Base-Case Results

Drug	Anticipated Intervention Acquisition Costs*	Intervention-Related Costs†	Non-Intervention Costs	Total Costs
Sonpiretigene	$437,500	$26,600	$342,200	$806,000
Usual Care	$0	$0	$342,200	$342,200
Incremental	**$437,500**	**$26,600**	**$0**	**$464,000**

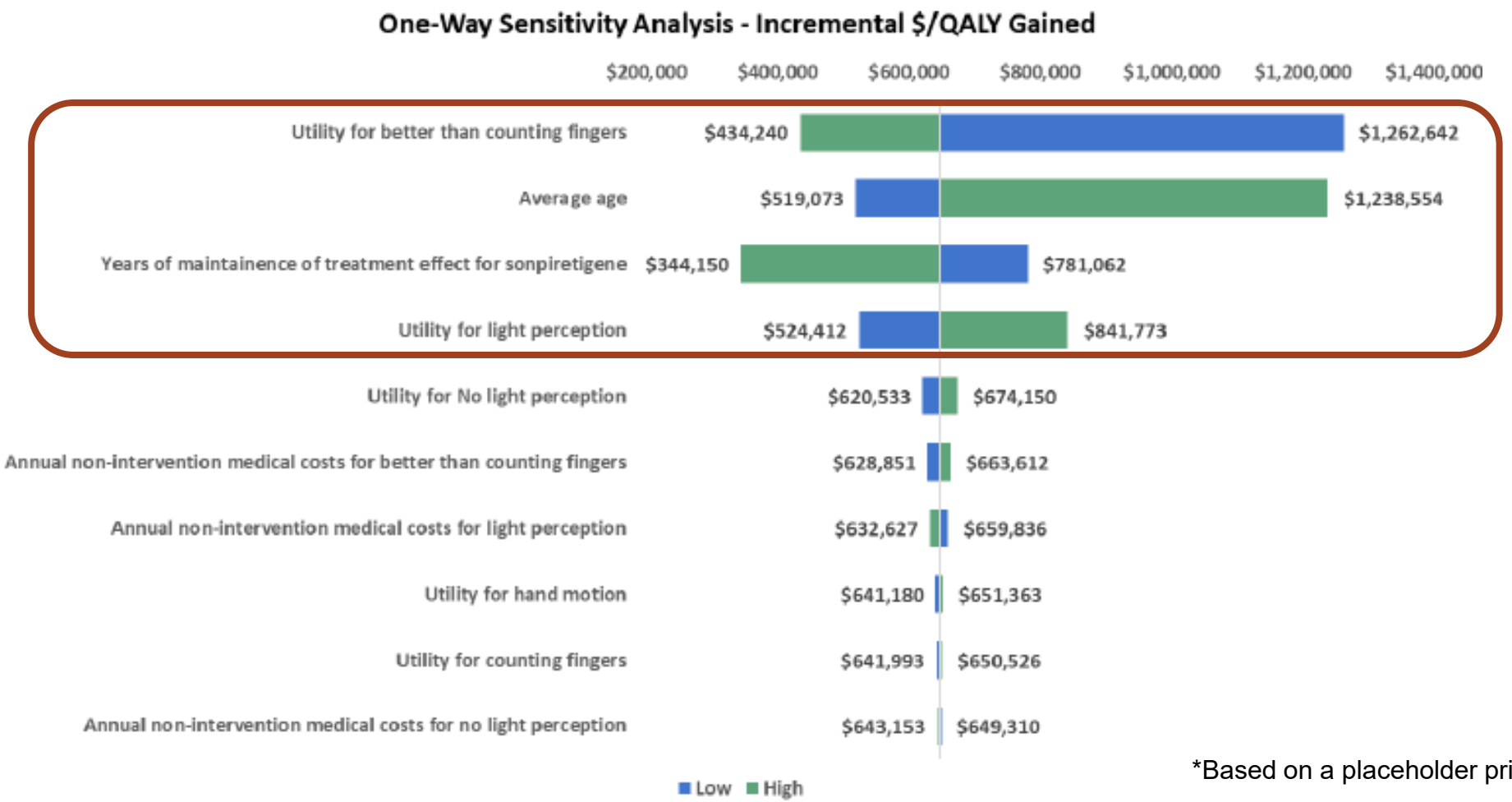
* Based on a placeholder price
† Based on a 6% mark-up
Note: Incremental values may not match individual intervention values due to rounding.

Base-Case Incremental Results

Drug	Cost per QALY Gained*	Cost per evLY Gained*	Cost per additional year in better than counting fingers*	Cost per additional year with light perception*
Sonpiretigene vs. Usual Care	$646,000	$646,000	$144,000	$811,000

*Based on a placeholder price

One Way Sensitivity Analyses



*Based on a placeholder price

QALY: quality-adjusted life years

Probabilistic Sensitivity Analysis

Drug	Cost-Effective at $50,000 per QALY and evLY Gained*	Cost-Effective at $100,000 per QALY and evLY Gained*	Cost-Effective at $150,000 per QALY and evLY Gained*
Sonpiretigene vs. Usual Care	0%	0%	0%

*Based on a placeholder price

Key Scenario Analyses

	Cost per QALY and evLY Gained*			
Drug	**Base Case**	**Lifetime Durability of Treatment Effect**	**Optimistic Benefit Scenario†**	**Conservative Benefit Scenario‡**
Sonpiretigene vs. Usual Care	$646,000	$312,000	$481,000	$664,000

* Based on a Placeholder Price
† Optimistic Benefit Scenario: assumes a 10-year durability of treatment effect
‡ Conservative Benefit Scenario: requires improvement in 3/3 outcome measures to be considered “improved” at 1-year

Health Benefit Price Benchmark (HBPB)

Price Benchmark for Sonpiretigene: one-time treatment in the worse seeing eye

Drug	Placeholder Price*	Price at $100,000 per QALY or evLY Threshold*	Price at $150,000 per QALY or evLY Threshold*
Sonpiretigene	$437,500	$67,400	$101,300

* For treatment in one eye

Key Limitations

- The clinical data used to model the primary treatment effect (small sample sizes, outcome measures limited) and unable to fully capture in sensitivity analyses.
- Very limited data beyond Week 100 of the RESTORE trial to inform estimates for the durability of treatment effect.
- No data to inform the additional benefit that could be achieved from treating both eyes.
- Variability in health state utility values available.

Comments Received

- Baseline distribution of patients across health states for sonpiretigene and usual care.
- Health state utility value for “Better than Counting Fingers”.
- Mismatch between placeholder price used and clinical trial results.
- Assumptions about durability of treatment effect.

Conclusions

- Patients treated with sonpiretigene experienced gains in QALYs and evLYs and a greater number of years with vision better than counting fingers compared to patients receiving usual care.
- At a placeholder price that assumes treatment in one eye, our analysis suggests that treatment with sonpiretigene would exceed commonly used cost-effectiveness thresholds.
- When assuming a lifetime durability of treatment effect, cost-effectiveness would improve but results remain above commonly used cost-effectiveness thresholds.

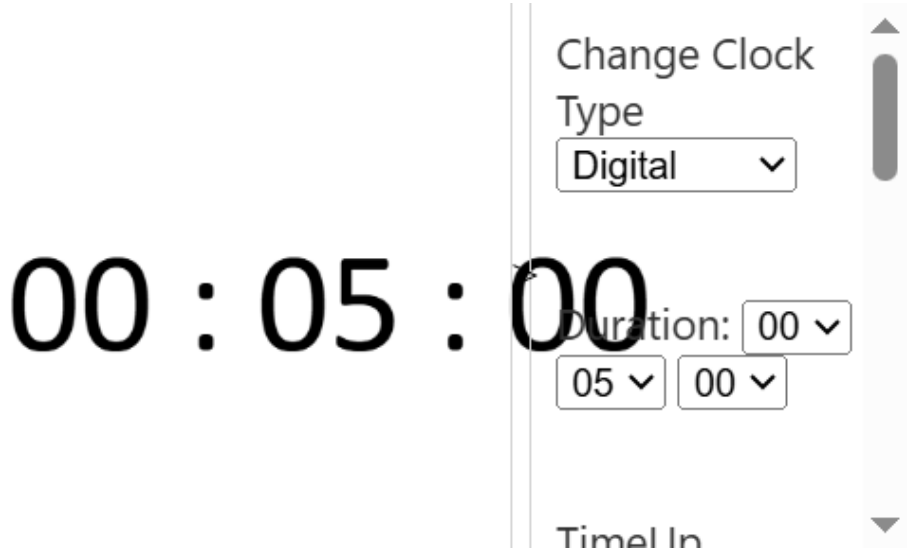
Questions?

Manufacturer Public Comment and Discussion

Samarendra Mohanty, PhD
President and Chief Scientific Officer, Nanoscope

Conflicts of Interest:

- *Dr. Mohanty is a full-time employee of Nanoscope.*

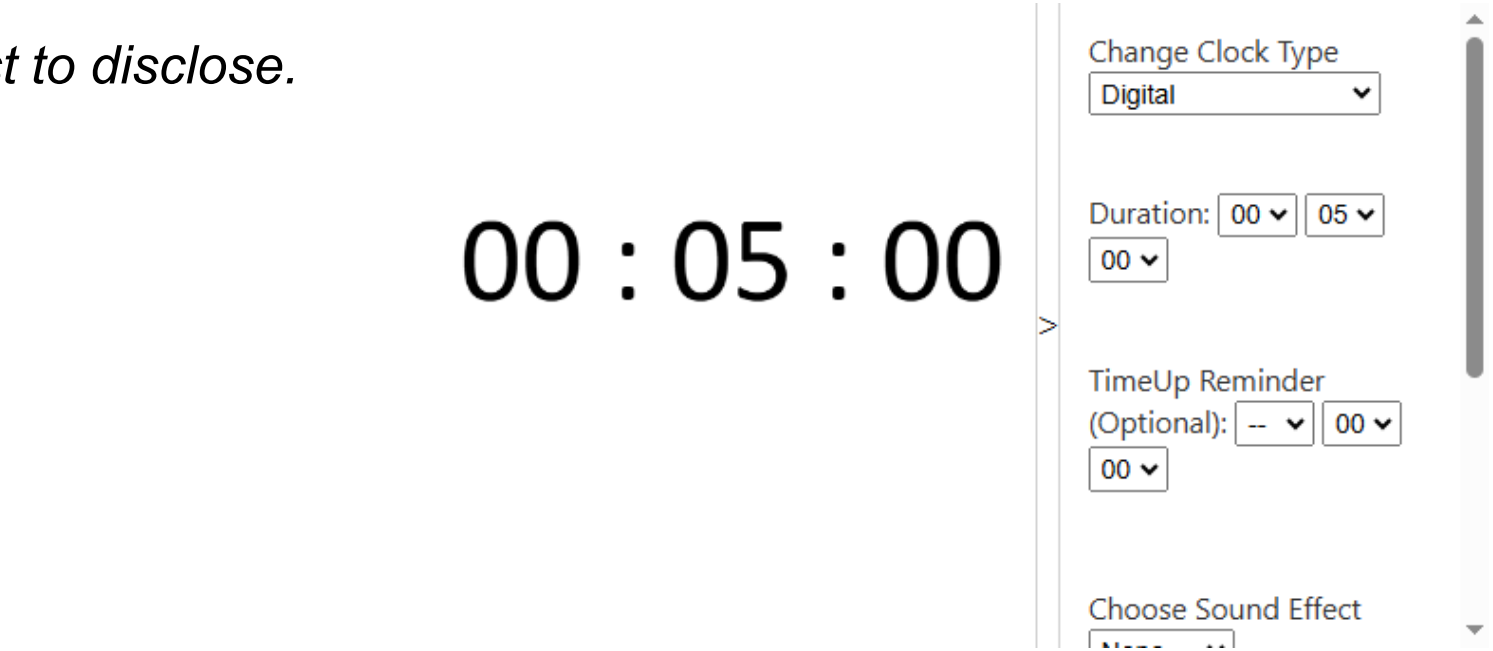


Public Comment and Discussion

Becky Andrews, LCMHC
Owner/Therapist at Resilient Solutions, Inc / Founder, Daring Sisters

Conflicts of Interest:

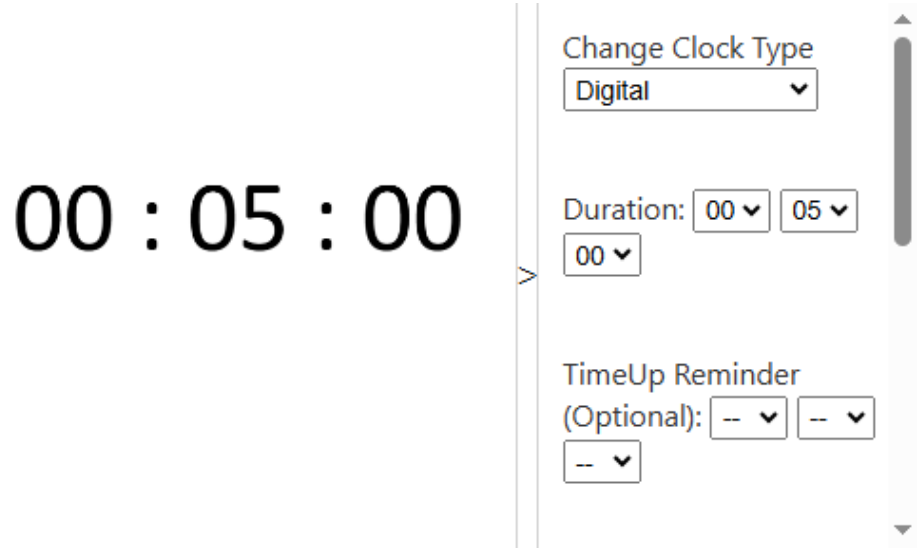
- *No conflicts of interest to disclose.*



Griffin Pinkow
CEO and Founder, Foreseeable Future Foundation

Conflicts of Interest:

- *No conflicts of interest to disclose.*



Lunch

Meeting will resume at 12:50PM ET

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Voting Questions

Patient Population for all questions: *People with advanced retinitis pigmentosa (RP) with severe vision loss.*

Note for all questions: *Usual care may include low vision aids, mobility training and support, and vision related rehabilitation.*

Clinical Evidence

1. For patients with advanced RP, is the current evidence adequate to demonstrate that the net health benefit of sonpiretigene is greater than that of usual care?

Benefits Beyond Health and Special Ethical Priorities

To help inform judgments of overall long-term value for money, please indicate your level of agreement with the following statements about advanced RP:

2. There is substantial unmet need despite currently available treatments.

3. This condition is of substantial relevance for people from a racial/ethnic group that have not been equitably served by the healthcare system.

To help inform judgments of overall long-term value for money, please indicate your level of agreement with the following statements based on the relative effects of sonpiretigene versus usual care:

4. The treatment is likely to produce substantial improvement in caregivers' quality of life and/or ability to pursue their own education, work, and family life.

5. The treatment offers a substantial opportunity to improve access to effective treatment by means of its mechanism of action or method of delivery.

Break

Meeting will resume at 2:00PM ET

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Policy Roundtable

Policy Roundtable

Participant	Conflict of Interest
Todd Durham, PhD, Senior Vice President, Clinical and Outcomes Research, Foundation Fighting Blindness	Foundation Fighting Blindness (FFB) has received sponsorships from various health care companies, including Nanoscope Therapeutics, for their scientific conferences, accounting for<25% of their funding. A member of Dr. Durham's household works in the life sciences industry and receives ≥25% of income from the industry. Additionally, the RD Fund, a venture philanthropy subsidiary of the FFB, has equity interests in several life science companies in its portfolio.
Julie Grutzmacher, MSW, MPH, Director of Patient Advocacy and Population Health Initiatives, Prevent Blindness	Prevent Blindness receives >25% of funding from healthcare companies including Nanoscope Therapeutics.
Hemant Hora, MD, FACP, Vice President, Medical Affairs, Senior Medical Director, Point32Health	Dr. Hora is a full-time employee of Point32Health.

Policy Roundtable

Participant	Conflict of Interest
Vinit B. Mahajan, MD, PhD, Professor of Ophthalmology, Stanford University	Dr. Mahajan has received funds from Nanoscope Therapeutics, Chigenovo, and Kerna Labs.
Samar Mohanty, PhD, Co-Founder and President, Nanoscope Therapeutics	Dr. Mohanty is a full-time employee of Nanoscope Therapeutics.
Lindsay Rippelmeyer, PharmD, Senior Director, Supply Chain Finance, Express Scripts by Evernorth	Dr. Rippelmeyer is a full-time employee of Express Scripts.
Stephen Russell, MD, Professor of Ophthalmology, University of Iowa	Dr. Russell has received funds from Spark Therapeutics, ProQR Therapeutics, Novartis, Digital Diagnostics (IDx, LLC) and has stock ownership in Digital Diagnostics (IDx, LLC).

New England CEPAC Council Reflections

Next Steps

- Meeting recording posted to ICER website next week
- Final Report published on or around May 15th, 2025. Includes description of New England CEPAC votes, deliberation, policy roundtable discussion
- Materials available at: https://icer.org/assessment/retinitis-pigmentosa-2025

Adjourn

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