
Deflazacort, Eteplirsen, and Golodirsen for Duchenne Muscular Dystrophy: Effectiveness and Value

Public Meeting – July 25, 2019



WIFI Network: MIT

Why are we here today?

“The decline was robbing him of his independence. Instead of being an adolescent realizing independence, he was quickly relying on his family (and mobility devices) to help him perform any and every activity of daily life. His confidence and self esteem were also quickly being compromised, as a 10-12 year old who was relying on others for all activities of daily life...he lost his ability to smile around the age of 12.”

-Christine McSherry

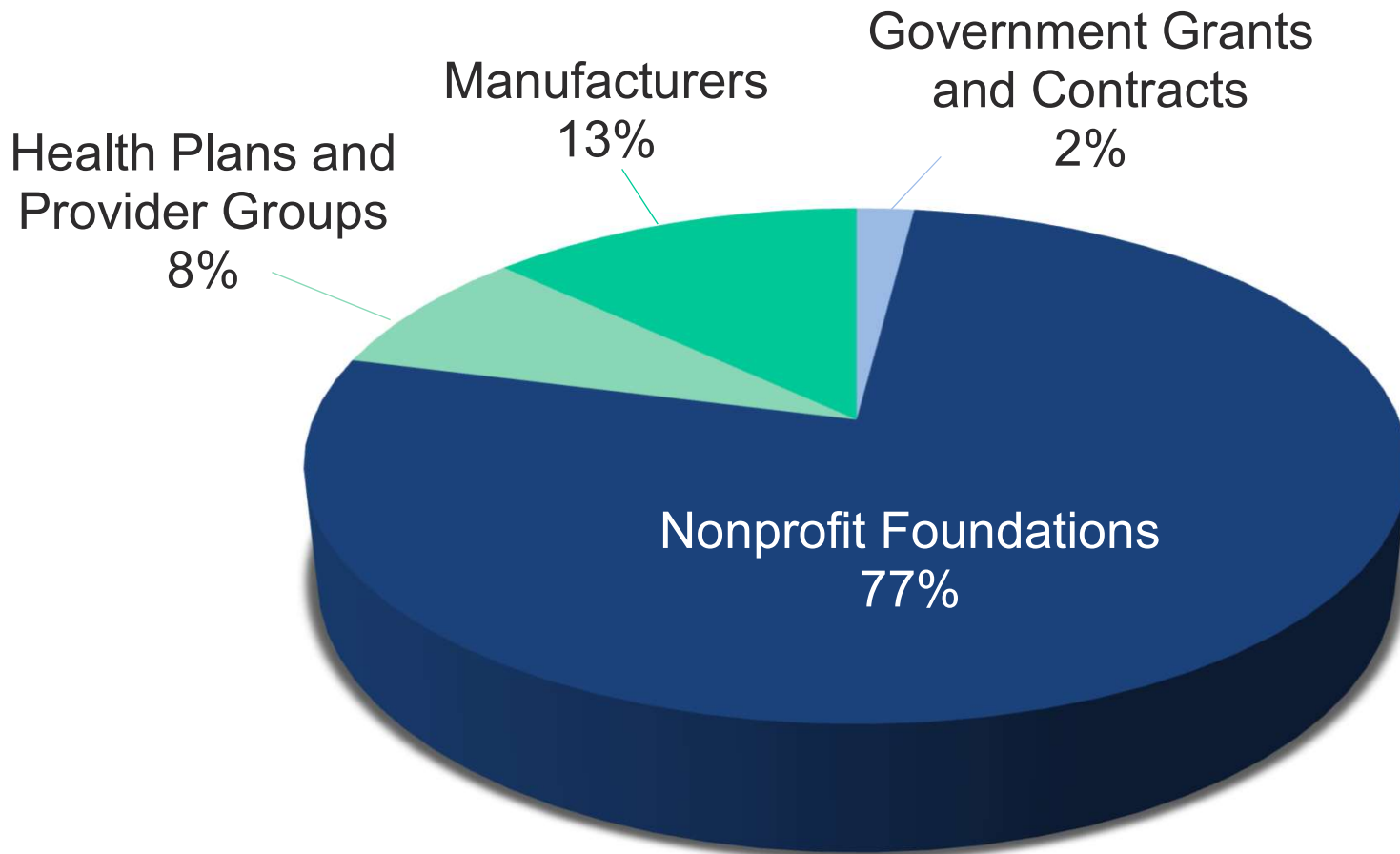
Why are we here today?

- What happens the day these treatments are approved by the FDA?
- The historical context and the challenge we all face today
- Patients can have difficulty accessing drugs
 - Coverage eligibility
- The goals for today's meeting

Organizational Overview

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

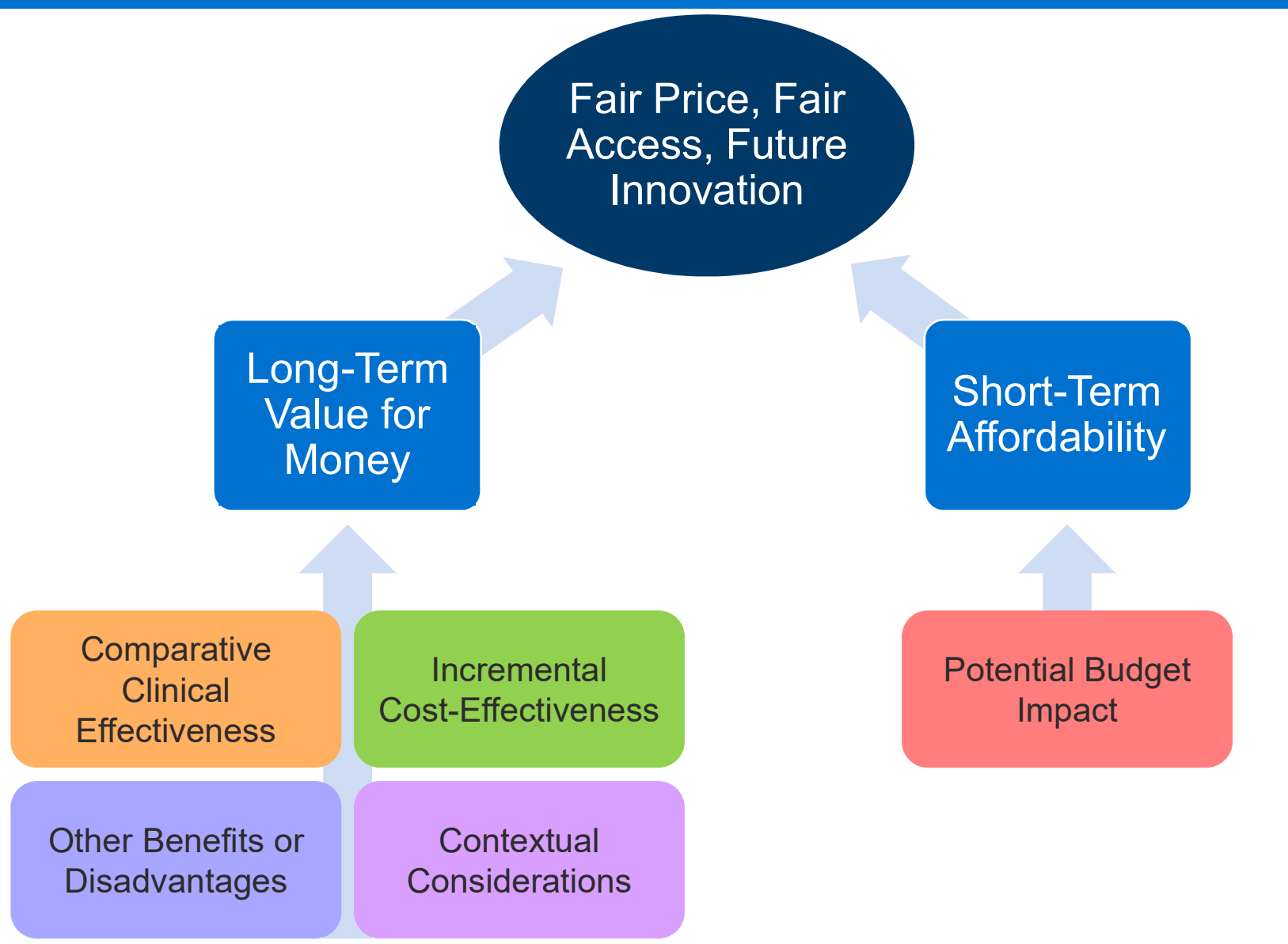
2019 Funding Sources



■ ICER Policy Summit and Non-Report activities only

How was the ICER report developed?

- Scoping with guidance from patient groups, clinical experts, manufacturers, and other stakeholders
- Internal ICER staff evidence analysis
- University of Illinois at Chicago College of Pharmacy cost-effectiveness modeling
- Public comment and revision
- Expert reviewers
 - Emma Ciafaloni, MD, FAAN
 - Peter B. Kang, MD
- How is the evidence report structured to support CEPAC voting and policy discussion?



Agenda

- 10:00 am** Meeting Convened and Opening Remarks
- 10:15 am** Presentation of the Evidence
- 11:20 am** Public Comments and Discussion
- 11:45 am** Lunch
- 12:40 pm** New England CEPAC Panel Deliberation and Vote
- 2:00 pm** Break
- 2:15 pm** Policy Roundtable Discussion
- 3:30 pm** Reflections from New England CEPAC Panel
- 4:00 pm** Meeting Adjourned

Clinical Experts

Emma Ciafaloni, MD, FAAN, Professor of Neurology and Pediatrics, University of Rochester

- *Participated in clinical trials sponsored by PTC Therapeutics and Sarepta.*

Peter B. Kang, MD, Chief, Division of Pediatric Neurology, University of Florida College of Medicine

- *Worked on DMD studies for Catabasis, Pfizer, and Solid Biosciences*
- *Consultant for AveXis and ChromaDex*
- *Served on advisory boards for Sarepta*

Patient Experts

Brian Denger, Patient Advocate

- *No conflicts of interest to disclose.*

Mindy Leffler, MEd, President and Chairman, Casimir

- *Casimir is under contract with Sarepta to facilitate two observational video capture studies.*

Dawn Rezkalla, Little Hercules Foundation

- *Ms. Rezkalla is Vice President of the Little Hercules Foundation.*
- *LHF receives grant funding from life science companies.*
- *Ms. Rezkalla's son has received free drug from Sarepta.*

Evidence Review

David Rind, MD
Chief Medical Officer
ICER



Key Collaborators

- **Grace A. Lin, MD**
- Foluso Agboola, MBBS, MPH
- Noemi Fluetsch, MPH
- Ifeoma Otuonye, MPH

Disclosures:

We have no conflicts of interest relevant to this report.

Background

- Duchenne muscular dystrophy (DMD)
 - X-linked neuromuscular disease
 - Mutations in the dystrophin gene
- Dystrophin
 - Present in skeletal and cardiac muscle
 - Strengthens and connects muscle fibers
 - Lack of dystrophin leads to progressive weakness, wasting, and cardiomyopathy
- Patients with DMD usually have dystrophin levels below 3% of normal
- Severe forms of Becker MD can have levels of 5-20% of normal
- Prevalence of about 6,000 people in the US

Course

- Symptom onset at age 2-3
- Diagnosis often delayed until age 5
- Early symptoms include falls, weakness, difficulty with stairs
- Without treatment, loss of ambulation by age 10-12 (substantial heterogeneity)
- Contractures, scoliosis, fractures
- Dysphagia, gastroparesis
- Respiratory and cardiac failure
- Without treatment (steroids, ventilatory support), death in teens or twenties

Supportive Care

- Physical and occupational therapy
- Skin and pulmonary care
- Assistive devices
- Home and vehicle modifications
- Non-invasive or invasive ventilatory support

Corticosteroids

- Improve strength, prolong ambulation, delay scoliosis and cardiomyopathy, preserve respiratory function
- Mechanism of benefit is uncertain
- Appropriate duration of treatment uncertain
- Usual steroid side effects

Corticosteroid Options

- Prednisone (or prednisolone)
- Deflazacort (Emflaza®) approved by the FDA in 2017

In patients with DMD, what is the comparative efficacy, safety, and effectiveness of deflazacort versus prednisone?

Exon-Skipping Therapies

- Dystrophin is a very long/large protein
- In Becker MD, mutations in the dystrophin gene often lead to a shortened version of the protein that has some function
- Goal of exon-skipping therapies is to allow production of a shortened dystrophin protein

Exon-Skipping Therapies

- Eteplirsen (EXONDYS 51™): Approved by FDA in 2016
- Golodirsen (exon 53 skipping): FDA decision expected in August 2019

In patients with a mutation amenable to exon 51 skipping (~13% of patients) or exon 53 skipping (~9% of patients), what are the comparative efficacy, safety, and effectiveness of adding eteplirsen and golodirsen, respectively, versus supportive care and corticosteroids alone?

Insights from Patients and Patient Groups

- Research concerns
 - Heterogeneity of the DMD population
 - Natural history data
 - Generalizability of results
 - Lack of validated measures for outcomes that reflect function in daily life activities
 - Stabilization and slowing decline are important outcomes
 - Before/after video data may be preferable
 - Durability of treatment effects
 - Side effects

Insights from Patients and Patient Groups

- Financial concerns
 - High costs of therapies and difficulties with coverage
 - Uncovered costs for making homes and vehicles accessible
- Caregiving burden
 - Anxiety, depression, isolation
 - Increased burden with loss of ability to ambulate
- Weight gain
 - Loss of ambulation
 - Increased risk of fractures
 - Increased difficulty for caregivers
 - Psychological harms for patients

Clinical Evidence

Deflazacort versus Prednisone

- Three RCTs
 - Largest and best is Griggs 2016
 - 196 boys with DMD (4% may have had BMD)
 - Karimzadeh 2012
 - 34 boys with DMD
 - Only patients blinded
 - Bonifati 2000
 - 18 boys with DMD
- Seven observational studies
 - Shieh 2018 (114 patients in placebo arm of RCT)
 - McDonald 2018 (440 patients for 10 years)

Muscle Strength in Griggs

- Primary outcome at 12 weeks:
 - Prednisone: +0.27
 - Deflazacort: +0.15
 - No p-value reported
- Exploratory analysis at 52 weeks:
 - Prednisone: +0.23
 - Deflazacort: +0.39
 - No statistical difference
- Bonifati found no difference at one year

Motor Function

(Time supine to standing, time to climb four stairs, time to run/walk/propel a wheelchair 30 feet)

- Griggs did not report 12-week results in a way that deflazacort and prednisone could be compared
- Numerically higher for deflazacort on three outcomes, weeks 12 to 52
- Improvement in time to climb four stairs “greater” with deflazacort at week 52 ($p=0.0461$ unadjusted)
- Karimzadeh better function at 12 months with deflazacort; Bonifati no difference

Loss of Ambulation

- McDonald: age 14 vs. 11.3 ($p=0.01$)
- Similar results in one other observational study
- Minimal or no difference in a third observational study

Harms

- Most evidence suggests greater undesired weight gain with prednisone
 - Griggs 35% vs 28%
- Most evidence suggests greater growth reduction with deflazacort
- Behavior change very important to parents, but evidence is thin that prednisone is worse
 - Griggs 14% vs 9%
- Some suggestion of higher cataract rates with deflazacort

Controversies and Uncertainties

- Observational studies of deflazacort susceptible to bias
- Selective reporting in RCT of deflazacort
- Open-label assessment of behavior change very difficult

ICER Evidence Ratings

Deflazacort versus prednisone:

- Efficacy seems similar or perhaps better; undesired weight gain probably less common; growth retardation probably more common. Moderate certainty of comparable or better net health benefits (C+)

Eteplirsen

- Study 201:
 - RCT in 12 patients
 - Four patients each:
 - Weekly IV eteplirsen 30 mg/kg (FDA-approved dose)
 - Weekly IV eteplirsen 50 mg/kg
 - Weekly placebo
- Three other studies 202, 204, 301
 - None are RCTs

Dystrophin Production

- Study 201:
 - Primary outcome at week 12 for 50 mg/kg group: no significant difference compared to placebo
 - Primary outcome at week 24 for 30 mg/kg group: +23% vs -4%
- FDA requested additional study:
 - 13 patients treated; 12 evaluable
 - After 48 weeks on 30 mg/kg, dystrophin increased from 0.16% of normal to 0.44% of normal

Functional Results

- Study 201 6MWT
 - 50 mg/kg group: -0.3 m
 - 30 mg/kg group: -128 m
 - Placebo group: -26 m
 - Investigators eliminated two patients in 30 mg/kg group with rapid declines and concluded +14 m
- Comparisons with historical controls in other studies found improvements in 6MWT and pulmonary function

Golodirsen

- 25 patient study (interim poster)
 - Baseline dystrophin: 0.095% normal
 - 48 weeks on treatment: 1.019% normal

Harms

- Limited evidence suggests minimal harms with eteplirsen
- No evidence on golodirsen

Controversies and Uncertainties

- Dystrophin increases with eteplirsen and golodirsen seem small
- Measures such as 6MWT may miss important outcomes; broader measurements should have been performed
- Discrepancies between caregiver reports and trial results
- FDA: “A clinical benefit of EXONDYS 51 has not been established”

ICER Evidence Ratings

Eteplirsen and golodirsen:

- Small increases in dystrophin levels; no moderate or high-quality evidence of functional benefits; limited evidence on harms but appear safe. Data are insufficient (“I”)

Potential Other Benefits and Contextual Considerations

- Reduced caregiver burden if effective
- Novel mechanism of therapy for exon-skipping therapies
- High severity and lifetime burden of illness

Public Comments

- Evidence standards for rare diseases
- Timing of evidence review
- Heterogeneity and RWE
- ICER is unable to review treatments for a disease like DMD

Questions?

Cost-Effectiveness

Surrey Walton, PhD

Associate Professor of Pharmacy Administration

University of Illinois at Chicago College of Pharmacy



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AND ECONOMIC REVIEW

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Key Review Team Members

- Nicole Boyer, PhD, University of Chicago
- Danny Quach, PharmD, University of Illinois at Chicago

Disclosures:

Financial support was provided to the University of Illinois at Chicago and to Nicole Boyer from the Institute for Clinical and Economic Review.

University of Illinois at Chicago researchers and Nicole Boyer have no conflicts to disclose relevant to this report.

Objectives

Estimate the lifetime cost-effectiveness of the following treatments for DMD:

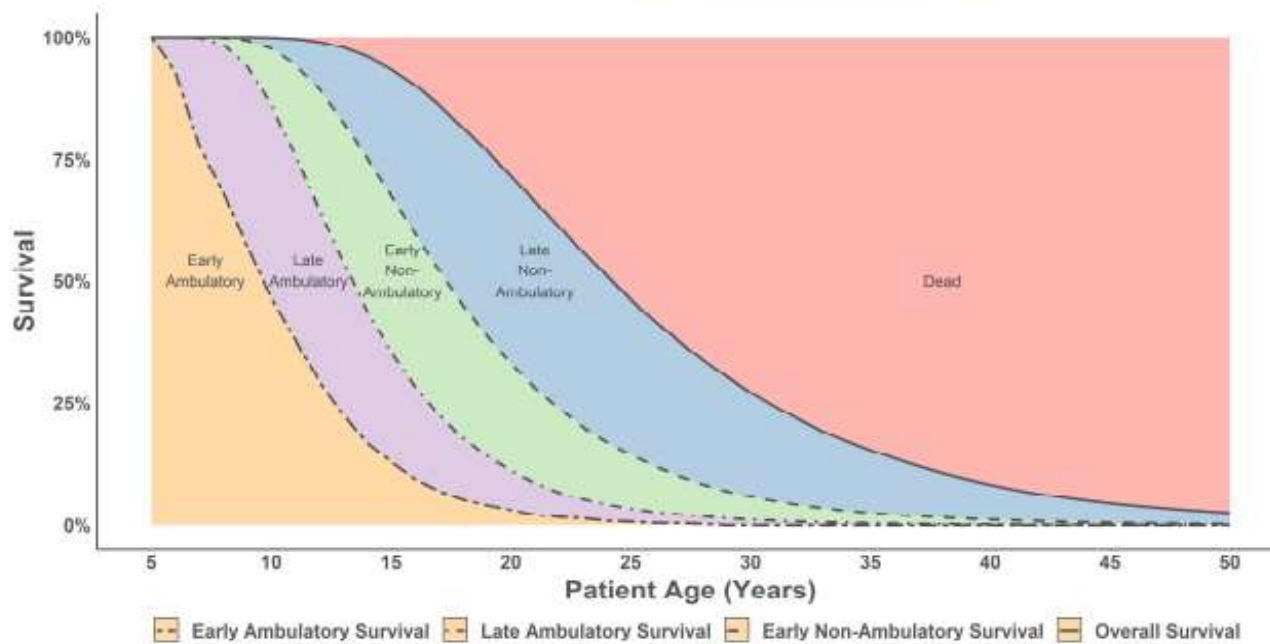
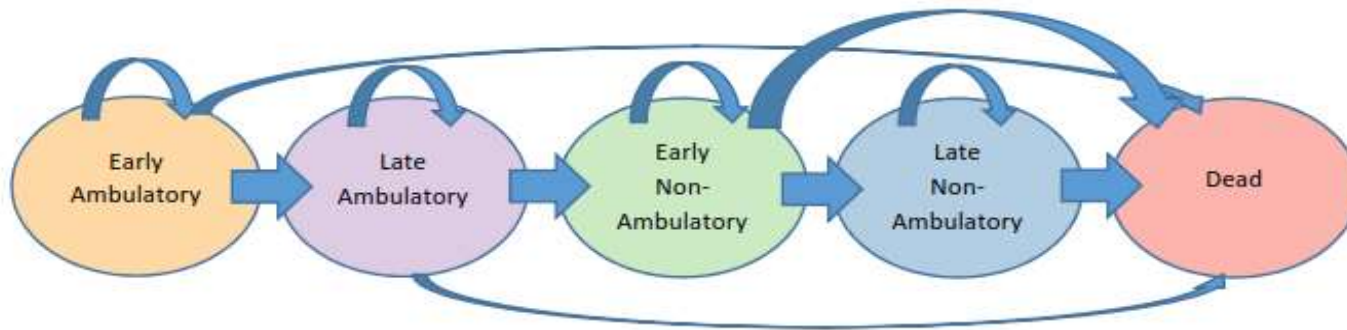
- Deflazacort versus prednisone
- Eteplirsen with corticosteroids versus corticosteroids alone

Methods in Brief

Methods Overview

- **Model:** Multi-State Partitioned Survival Model
- **Setting:** United States
- **Perspective:** Dual Base Case (for deflazacort): Health Care Sector and Modified Societal Perspectives
- **Time Horizon:** Lifetime
- **Discount Rate:** 3% per year (costs and outcomes)
- **Cycle Length:** Annual
- **Primary Outcomes:** Cost per quality-adjusted life year (QALY) gained, Cost per life year (LY) gained

Model Schematic



Informed by previous work (Hill et al., 2018; Ricotti et al., 2013; Goto et al., 2016)

Treatment Regimens

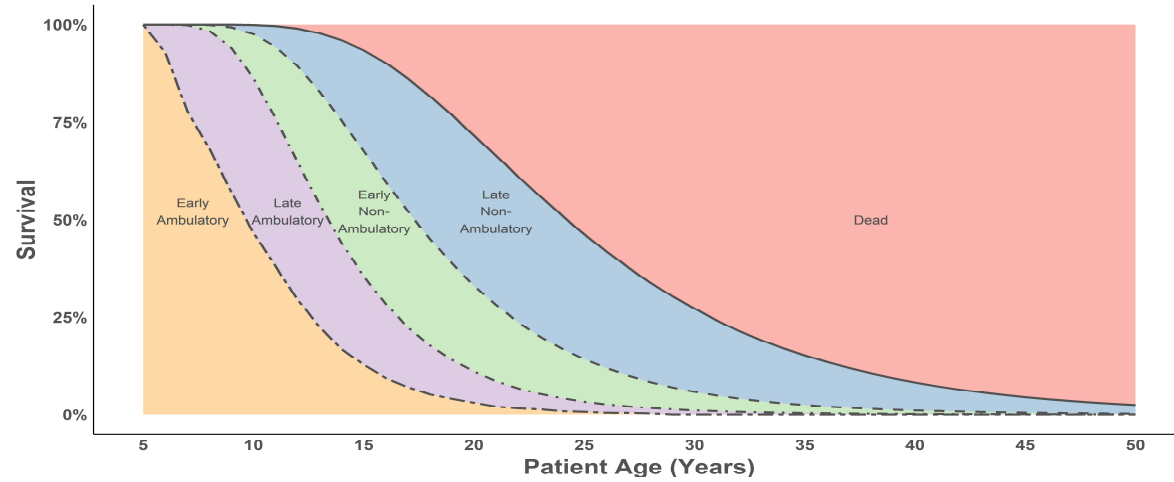
Generic Name (Brand Name)	Dose	Approval status
Prednisone	Approximately 0.75 mg/kg/day administered orally	Generic, Used Off Label
Deflazacort (EMFLAZA®)	Approximately 0.9 mg/kg/day administered orally	Approved
Eteplirsen (EXONDYS 51™)	30 mg/kg/week administered by a 35 to 60 minute IV infusion	Accelerated Approval Contingent on Verification of Clinical Benefit

Key Model Assumptions

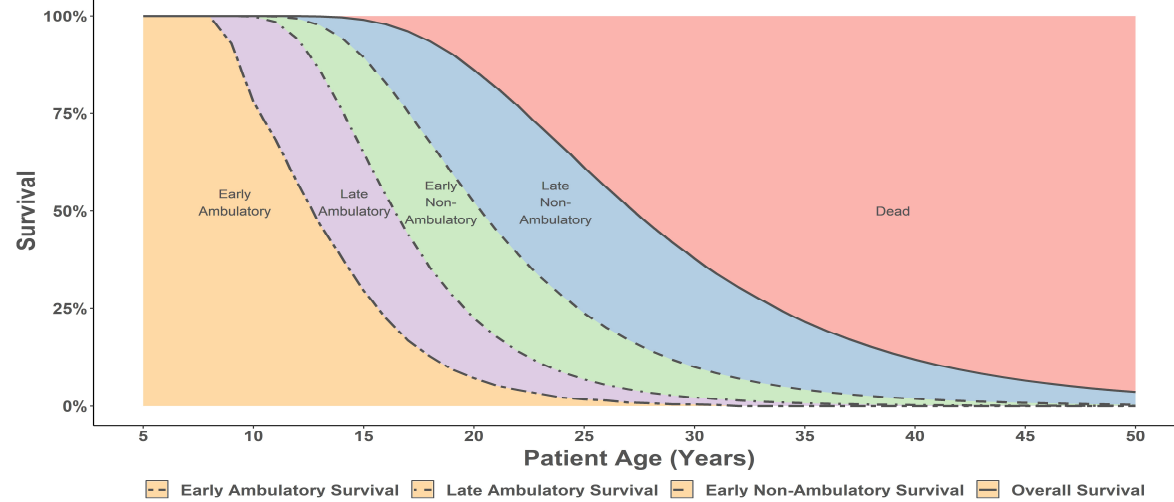
- An evidence-based upper bound treatment effect of a 3-year delay to all subsequent health states was applied to deflazacort
 - Modeled potential treatment effects using parallel rightward shifts in survival curves
- Serious adverse events (SAEs) of corticosteroid use were represented by a disutility of 0.05

Modeled Treatment Effects

Prednisone



Deflazacort



Key Model Inputs

Utilities

Health State	Patient Utility	Caregiver Utility
Early Ambulatory	0.730	0.845
Late Ambulatory	0.640	0.839
Early Non-Ambulatory	0.210	0.784
Late Non-Ambulatory	0.180	0.810

Treatment Costs

Intervention (Dosage)	Annual Treatment Cost
Prednisone (0.75 mg/kg/day)	\$550*
Deflazacort (0.9 mg/kg/day)	\$81,400*
Eteplirsen (30 mg/kg per week)	\$1,002,000*

kg: kilogram, mg: milligram

*These estimates are for a 40 kg patient. Actual costs in the model will vary by expected weight based on the patient's age.

Annual Non-Drug Related Costs

Health State	Health Care Sector Costs	Societal Costs*
Early Ambulatory	\$22,581	\$38,066
Late Ambulatory	\$22,378	\$37,725
Early Non-Ambulatory	\$33,096	\$55,792
Late Non-Ambulatory	\$44,326	\$74,725

*The modified societal perspective costs include all the health sector costs as well as non-medical community services, informal care, indirect costs, and out of pocket costs for DMD related home alterations and other uncovered equipment

Annual Adverse Event Rates and Costs

Adverse Event	Prednisone	Deflazacort	Cost*
Cataracts	0.1%	0.3%	\$75
Cataract Surgery	0%	6.9%	\$3,434
Weight Gain	1.5%	0.6%	\$75
Cushingoid	0.9%	0.7%	\$75
Behavior Change	0.6%	0.3%	\$75
Fractures	0.3%	0.1%	\$7,661

*Cost per event per year

Results

Base-Case Results

Drug	Cost	QALYs	Life Years	Years in Ambulation
Health Care Sector Perspective				
Prednisone	\$464,000	6.88	15.05	7.97
Deflazacort	\$1,010,000	8.40	16.64	10.16
Modified Societal Perspective				
Prednisone	\$1,240,000	6.88	15.05	7.97
Deflazacort	\$1,830,000	8.40	16.64	10.16

QALY: quality-adjusted life year

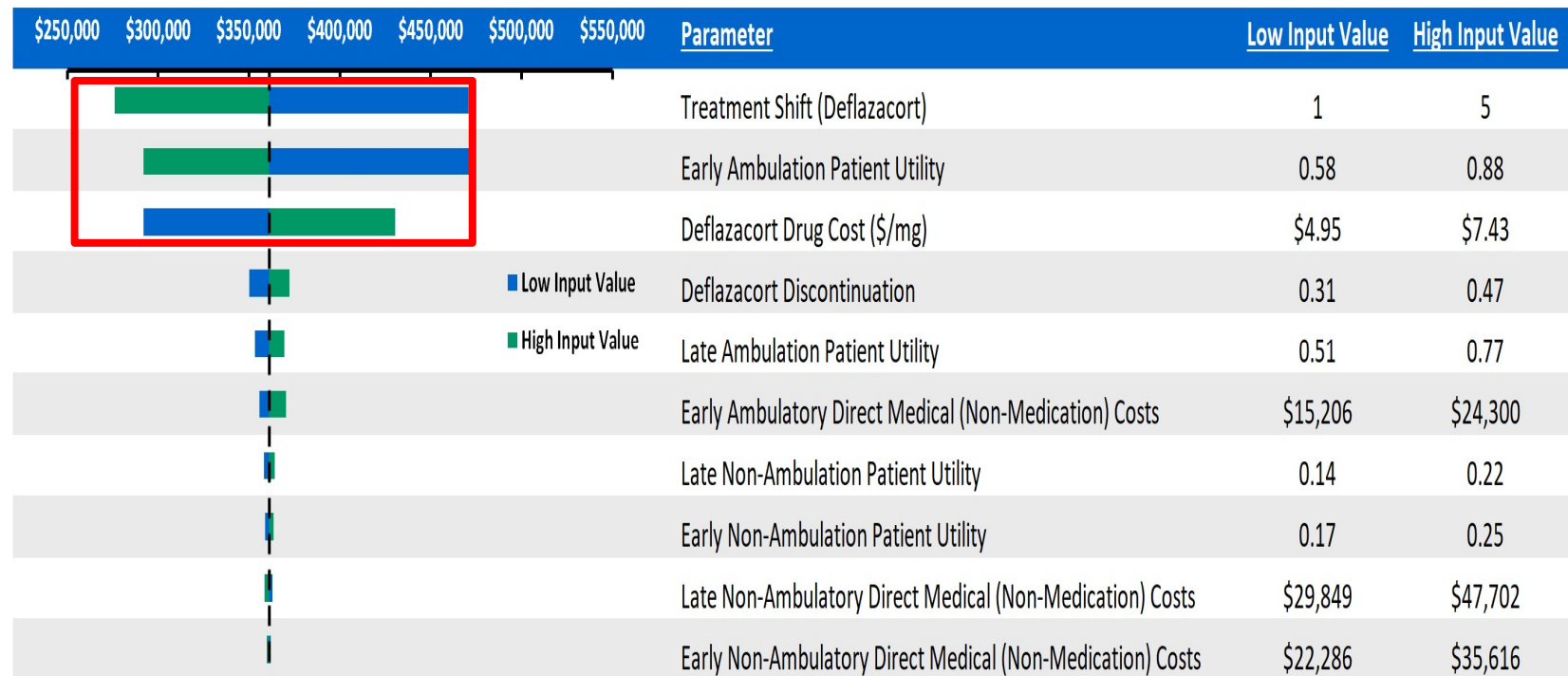
Base-Case Incremental Results

Drug	Incremental Cost per QALY Gained	Incremental Cost per LY Gained	Incremental Cost per Year in Ambulation
Health Care Sector Perspective			
Deflazacort vs. Prednisone	\$361,000	\$343,000	\$250,000
Modified Societal Perspective			
Deflazacort vs. Prednisone	\$390,000	\$371,000	\$269,000

QALY: quality-adjusted life year, LY: life year

One-Way Sensitivity Analyses

Health Sector Perspective



Probabilistic Sensitivity Analysis

Deflazacort	Cost-Effectiveness Thresholds				
	\$50,000 per QALY Gained	\$100,000 per QALY Gained	\$150,000 per QALY Gained	\$300,000 per QALY Gained	\$500,000 per QALY Gained
Percentage of Simulations	0%	0%	0.03%	22.47%	91.77%

QALY: quality-adjusted life year

Scenario Analyses

Scenarios	Total Cost	QALYs	Incremental Cost per QALY Gained
Low dose: prednisone	\$463,000	6.88	--
Low dose: deflazacort	\$892,000	8.40	\$283,000
Added caregiver QALYs: prednisone	\$1,240,000	19.30	--
Added caregiver QALYs: deflazacort	\$1,830,000	22.23	\$202,000

QALY: quality-adjusted life year

Threshold Prices for Deflazacort

	Annual Price to Reach Thresholds					
	Base Case	\$50,000 per QALY Gained	\$100,000 per QALY Gained	\$150,000 per QALY Gained	\$300,000 per QALY Gained	\$500,000 per QALY Gained
Deflazacort (Per Year)*	\$81,400	\$8,200	\$19,900	\$31,700	\$67,000	\$114,000

QALY: quality-adjusted life year

*Price per year is for a 40 kg patient

Threshold Analyses for Eteplirsen

Scenario	Incremental Costs	Incremental QALYs	Incremental Cost per QALY Gained
10 Year Shift	\$12,670,000	4.70	\$2,700,000
20 Year Shift	\$17,510,000	8.20	\$2,140,000
40 Year Shift	\$24,010,000	12.42	\$1,930,000
40 Year Shift Perfect Health	\$23,350,000	28	\$1,110,000

QALY: quality-adjusted life year

Limitations

- There were insufficient clinical trial data for all treatments modeled
- Data were not available to build in additional granularity to the model
- Deflazacort versus prednisone data were limited and mixed → overly optimistic treatment effects
- Several inputs came from cohort studies that could have been subject to selection bias

Comments Received

- The model is too simplistic and does not capture the nuances of DMD
- The societal perspective should include caregiver utilities
- The underlying utility estimates were not suitable for DMD patients

Conclusions

- Evidence for evaluating cost-effectiveness of treatments in DMD remains sparse
- Compared to prednisone, even using upper bound treatment effects, deflazacort is projected to have high costs relative to its benefits for patients and families
- At eteplirsen's current price, no plausible treatment effects made this treatment cost-effective at common thresholds

References

References

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Questions?

Public Comment and Discussion

Fleur Chandler, PhD

Health Economist, Duchenne UK
Chair of Project HERCULES

Conflicts of Interest:

- Serves in a contract role with Sanofi
- Owns stock in GlaxoSmithKline
- Project HERCULES is partially joint funded by a pharma consortium and Duchenne UK. Dr. Chandler does not take any payment from Duchenne UK or Project HERCULES.

Annie M. Kennedy

Senior Vice President, Legislation & Public Policy, Parent Project Muscular Dystrophy

Conflicts of Interest:

- PPMD receives 36% of its funding from health care companies, including PTC Therapeutics and Sarepta.

Mindy Leffler, MEd

President and Chairman, Casimir

Conflicts of Interest:

- Casimir is under contract with Sarepta to facilitate two observational video capture studies.



Christine McSherry, RN
Founder, Jett Foundation
Co-Founder and CEO, Casimir

Conflicts of Interest:

- Jett Foundation has received educational grants from Sarepta and PTC.
- Casimir is under contract with Sarepta to facilitate two observational video capture studies.

Lunch

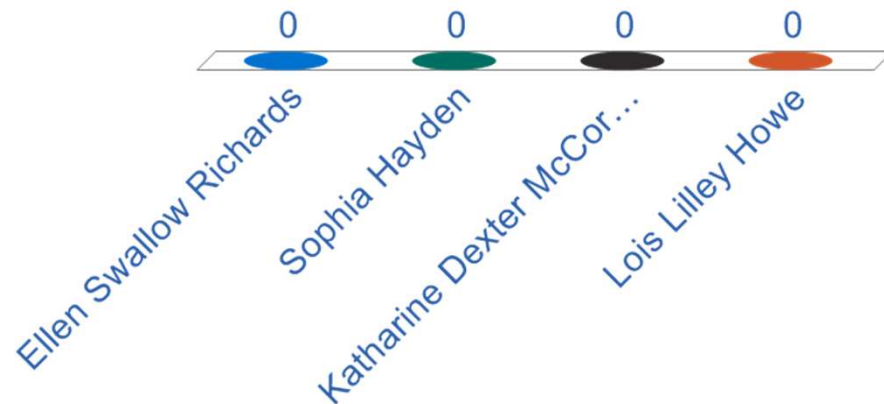
Meeting will resume at 12:40pm

Voting Questions

WIFI Network: MIT

0. Admitted in 1870, who was the first woman to graduate from MIT?

- A. Ellen Swallow Richards
- B. Sophia Hayden
- C. Katharine Dexter McCormick
- D. Lois Lilley Howe



1. For patients with DMD, is the evidence adequate to demonstrate that the net health benefit of deflazacort (Emflaza® PTC Therapeutics) is superior to that provided by prednisone?

- A. Yes
- B. No



2. For patients with DMD amenable to exon 51 skipping, is the evidence adequate to demonstrate that the net health benefit of eteplirsen (EXONDYS 51™, Sarepta Therapeutics) added to corticosteroids and supportive care is superior to that provided by corticosteroids and supportive care alone?

A. Yes

B. No



3. For patients with DMD amenable to exon 53 skipping, is the evidence adequate to demonstrate that the net health benefit of golodirsen (SRP-4053, Sarepta Therapeutics) added to corticosteroids and supportive care is superior to that provided by corticosteroids and supportive care alone in patients with DMD?

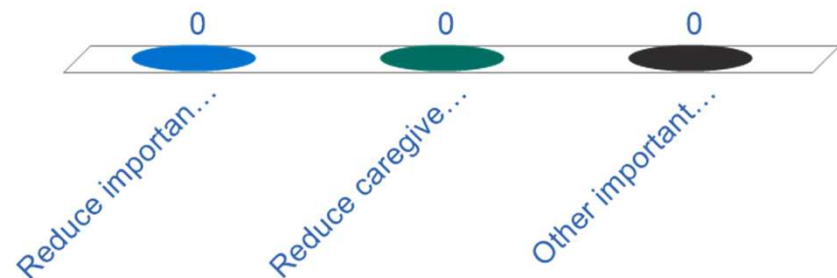
A. Yes

B. No



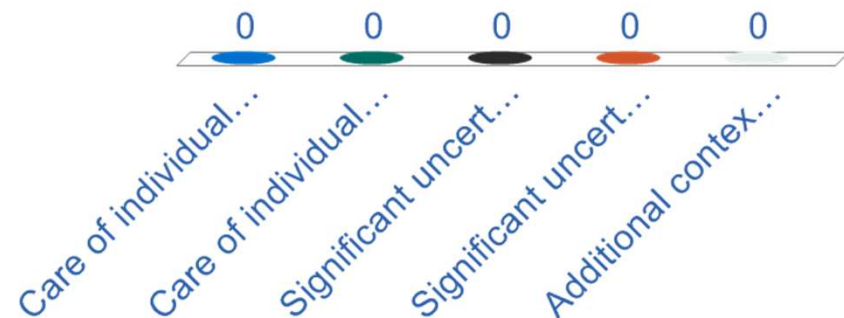
4. Is it likely that treatment with deflazacort offers one or more of the following potential “other benefits” that are not adequately captured in the base-case cost-effectiveness model? (select all that apply)

- A. Reduce important health disparities
- B. Reduce caregiver/family burden
- C. Other important benefits or disadvantages



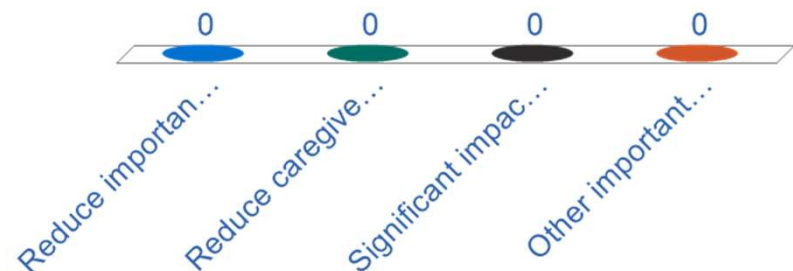
5. Are any of the following contextual considerations important in assessing deflazacort's long-term value for money? (select all that apply)

- A. Care of individuals with condition of high severity
- B. Care of individuals with condition with high lifetime burden of illness
- C. Significant uncertainty about long-term risk of serious side effects
- D. Significant uncertainty about magnitude or durability of the long-term benefits
- E. Additional contextual considerations



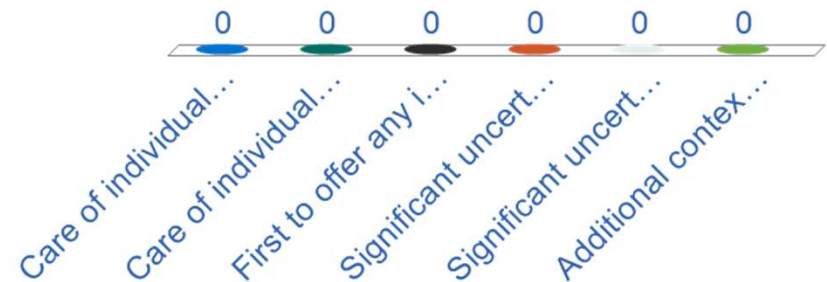
6. Is it likely that treatment with eteplirsen or golodirsen offers one or more of the following potential “other benefits” that are not adequately captured in the base-case cost-effectiveness model? (select all that apply)

- A. Reduce important health disparities
- B. Reduce caregiver/family burden
- C. Significant impact on improving return to work/overall productivity
- D. Other important benefits or disadvantages



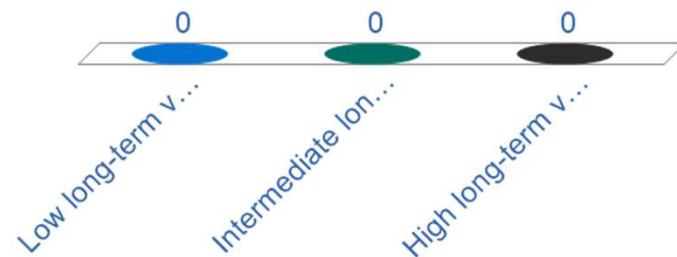
7. Are any of the following contextual considerations important in assessing eteplirsen's and golodirsen's long-term value for money? (select all that apply)

- A. Care of individuals with condition of high severity
- B. Care of individuals with condition with high lifetime burden of illness
- C. First to offer any improvement
- D. Significant uncertainty about long-term risk of serious side effects
- E. Significant uncertainty about magnitude or durability of the long-term benefits of this intervention
- F. Additional contextual considerations



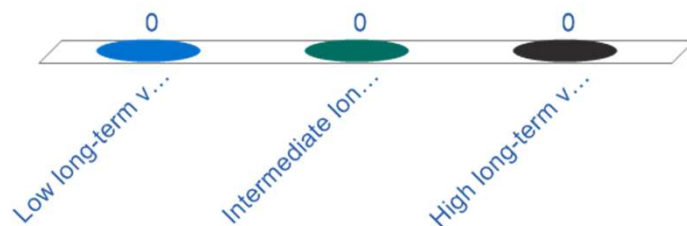
8. Given the available evidence on comparative effectiveness and incremental cost-effectiveness, and considering other benefits, disadvantages, and contextual considerations, what is the long-term value for money of treatment with deflazacort versus prednisone?

- A. Low long-term value for money
- B. Intermediate long-term value for money
- C. High long-term value for money



9. Given the available evidence on comparative effectiveness and incremental cost-effectiveness, and considering other benefits, disadvantages, and contextual considerations, what is the long-term value for money of treatment with eteplirsen versus supportive care and corticosteroids alone?

- A. Low long-term value for money
- B. Intermediate long-term value for money
- C. High long-term value for money



Break

Meeting will resume at 2:15pm

Policy Roundtable

Policy Roundtable Participants

Participant	Affiliation	Conflict of Interest
Emma Ciafaloni, MD, FAAN	Professor of Neurology and Pediatrics, University of Rochester	Participated in clinical trials sponsored by PTC Therapeutics and Sarepta.
Brian Denger	Patient Advocate	None disclosed.
Peter B. Kang, MD	Chief, Division of Pediatric Neurology, University of Florida College of Medicine	Worked on DMD studies for Catabasis, Pfizer, and Solid Biosciences; consultant for AveXis and ChromaDex; served on advisory boards for Sarepta.
Mindy Leffler, MEd	President and Chairman, Casimir	Casimir is under contract with Sarepta to facilitate two observational video capture studies.
Dawn Rezkalla	Little Hercules Foundation	Ms. Rezkalla is VP of LHF, which receives grant funding from life science companies. Ms. Rezkalla's son has received free drug from Sarepta.
Erik Schindler, PharmD, BCPS	Clinical Pharmacy Manager, UnitedHealthcare	Full-time employee of UnitedHealthcare.
John Watkins, PharmD, MPH, BCPS	Formulary Manager, Premera Blue Cross	Full-time employee of Premera Blue Cross.

New England CEPAC Panel Reflections

Next Steps

- Meeting recording posted to ICER website next week
- Final Report published on or around August 15
 - Includes description of New England CEPAC votes, deliberation, policy roundtable discussion

- Materials available at:

<https://icer-review.org/topic/duchenne-muscular-dystrophy/>



Adjourn