ISPOR 2021 Issue Panel: Can We All Afford A Cure? The Greatest Challenges Now Facing Payers

May 18, 2021; 1.45–2.45pm

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Introduction

Kate Hanman

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The Issue: Setting the Scene

The cost of gene therapies is unlike anything the healthcare industry has witnessed before

2019

Spinal muscular atrophy gene therapy, Zolgensma, was priced at a record $2.1 million[1]

ICER considered that the price "more fairly aligns with the benefits for these children and their families"[1]

2025

FDA expects to be approving 10 to 20 cell and gene therapies every year[2]

FDA: Food and Drug Administration; ICER: Institute for Clinical and Economic Review.

1. Institute for Clinical and Economic Review. 2021. ICER Comments on the FDA Approval of Zolgensma for the Treatment of Spinal Muscular Atrophy. Available at: https://icer.org/news-insights/press-releases/icer_comment_on_zolgensma_approval/. Last accessed 05.10.21. **2.** U.S. Food and Drug Administration. 2019. Statement from FDA Commissioner Scott Gottlieb, M.D. and Peter Marks, M.D., Ph.D., Director of the Center for Biologics Evaluation and Research on new policies to advance development of safe and effective cell and gene therapies. Available at: https://www.fda.gov/news-events/press-announcements/statement-fda-commissioner-scott-gottlieb-md-and-peter-marks-md-phd-director-center-biologics. Last accessed 05.10.21.

The Issue: Affordability and Access For All

- Payment of more conventional pharmaceuticals usually occurs through up-front costs, where treatments have typically been much cheaper than gene therapies
- The current payer system is generally not used to dealing with, nor is it designed for, such expensive therapies

What are some of the potential affordability issues for gene therapies?

- Management of payments when patients switch health insurers (patient portability)
- Access inequity between public and private healthcare plans
- Feasibility of different payment mechanisms e.g. value-based contracting

What do we want to understand from today's issue panel?

- To date, how have payers responded to these funding and access challenges?
- What are the remaining enduring challenges that must be tackled?

The Panel

Michael Sherman

Executive Vice President & Chief Medical Officer

Harvard Pilgrim Health Care and Tufts Health Plan

Debra Miller

CEO & Founder

CureDuchenne

Ramesh Arjunji

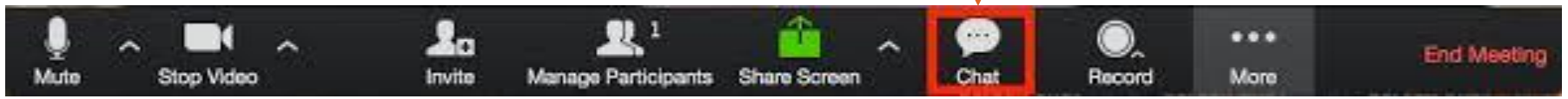
VP, Value and Access

Avrobio

Format of the Issue Panel

- Each panellist will speak for ~10 minutes
- There will then be a brief opportunity for the other panellists to respond (likely to take ~5 minutes in total)
- ~15 minute discussion session

- Questions from the audience are encouraged throughout
- Interactive voting questions will be used throughout your presentation



Questions Posed to the Panel

How are payers managing the access challenges of high-cost gene therapies and what challenges remain?

What are the access inequities between patients on private versus government insurance plans (e.g. Medicaid)?

How has the industry responded to the access challenges faced by payers, and what examples of successful access agreements can we learn from?

How do these access challenges impact patients?

Payer Perspective

Michael Sherman

Executive Vice President & Chief Medical Officer

Harvard Pilgrim Health Care and Tufts Health Plan

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Disclosures: Michael Sherman is a Board Member at Real Endpoints

Patient Perspective

Debra Miller
CEO & Founder, CureDuchenne

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No disclosures

Cure Duchenne

Duchenne muscular dystrophy

Cure Duchenne VENTURES

Cure Duchenne BIOBANK

Cure Duchenne CARES

DUCHENNE XCHANGE

CDAccess

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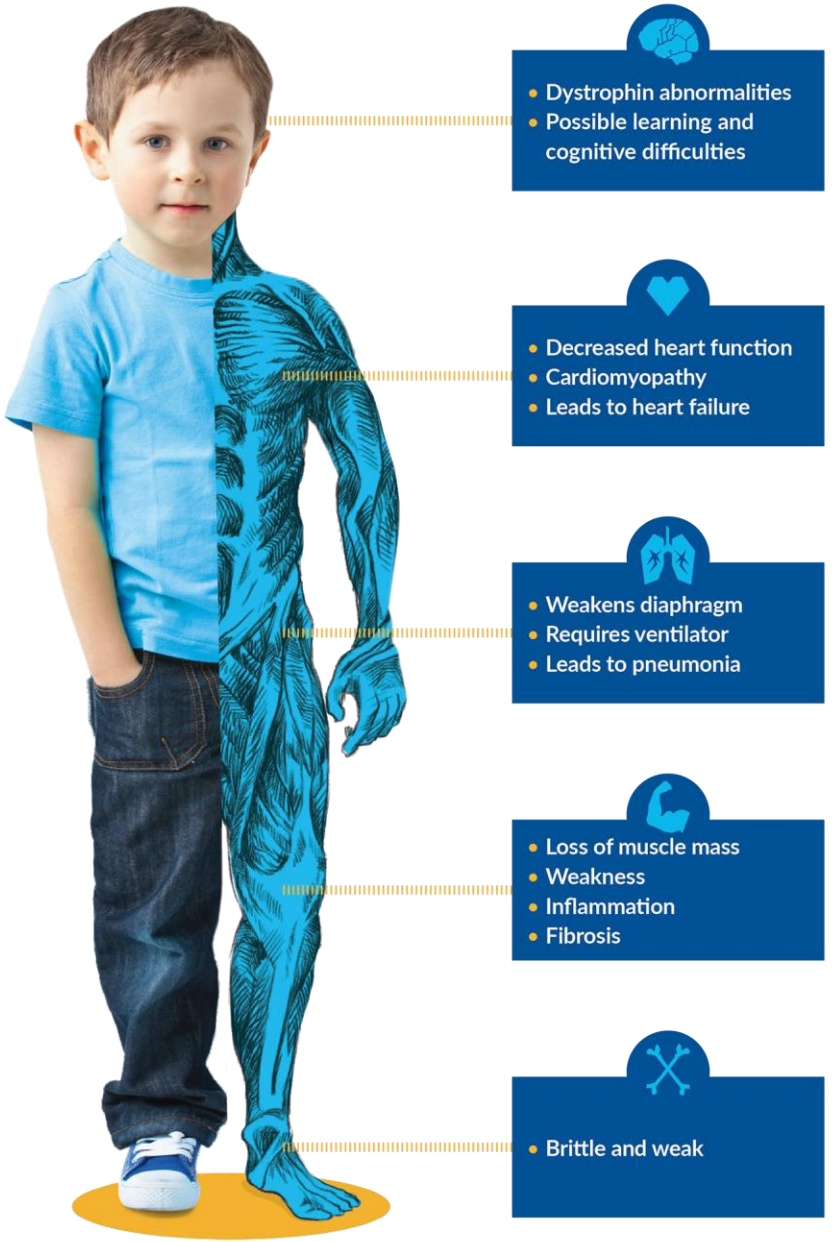
CureDuchenne overview slide

Duchenne is an unrelenting disease where the clock is ticking, and stages of disease are irreversible

The benefit of any drug only happens in a brief window

Once a patient passes through to the next stage of disease, the drug is no longer beneficial, and the damage is done

Duchenne is fatal



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Gene Therapy for Duchenne

- Four companies developing gene therapy for Duchenne
- Massive doses due to amount of muscle mass
- Expensive to manufacture
- Variability in phenotype and disease progression
- Confirmatory trials may require a long follow up period
- 100% fatal
- Only very young patients eligible for clinical trials: safety, cost
- Unclear guidelines from regulators

Status of Reimbursement

- Confusing for drug developers and for patients; how broad should the label be?
- If it doesn’t work, do regulators have the mechanism and the will to reverse an approval
- Rare diseases make large scale trials difficult, need flexibility
- Accelerated approval many times must be made with limited clinical endpoints
- Differences between public and private payers

Rare Diseases Need Answers Now for Gene Therapy Reimbursement

- Gene therapy can halt but not reverse the disease. Every day that is lost equals, lost function and shorter life
- Medicaid can complicate and slow access to gene therapy
- Issues to consider:
  - Value Based Payments
  - CMS support Accelerated Approvals
  - Adherence to confirmatory trials
  - Reimbursement to full FDA label

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Cure Duchenne

Thank you!

Industry Perspective

Ramesh Arjunji
VP, Value and Access, Avrobio

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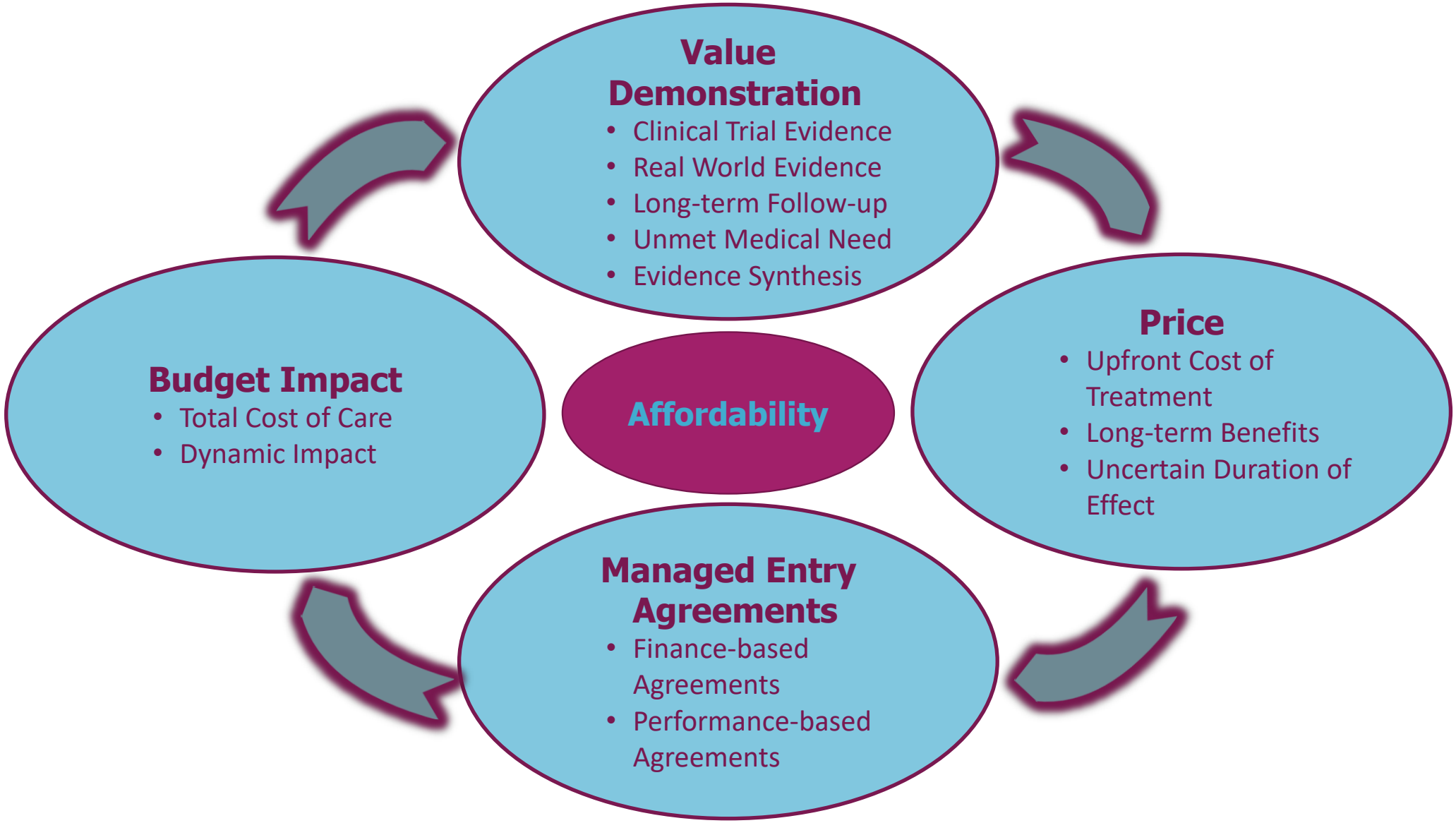
Disclosures

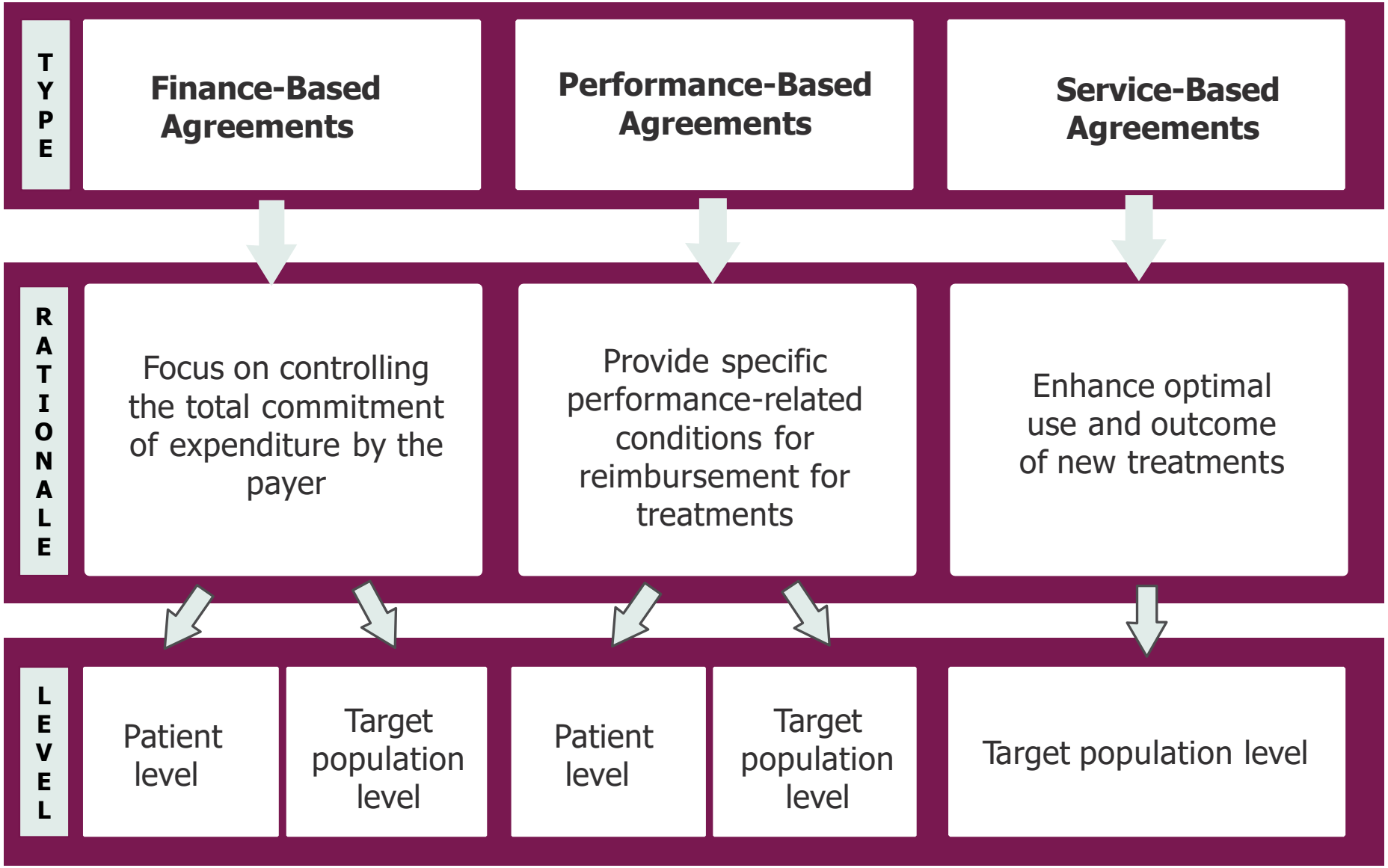
Ramesh Arjunji is an employee of AVROBIO, and may own AVROBIO stock or other equities

The opinions expressed here are my own and may not express the views or opinions of my employer

Manufacturers' response to affordability challenges for short-term curative therapies



Managed entry agreements[1]



1. Dabbous, M et al. Managed Entry Agreements: Policy Analysis From the European Perspective. Value Health. 2020; 23(4):425-433

Discussion & Questions

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