

# Value-Based Payment Arrangements for Rx

JP Wieske  
Campaign for Transformative Therapies





A CAMPAIGN FOR  
**Transformative  
Therapies**

- Coalition of Payers, Patients, and Pharma companies
- Achieving Sustainable Access Through Value-Based Payment Arrangements (VBAs)
- Achieving Balance: Access, Cost, and Outcomes
- State-based Solutions

# Gene Therapy 101



Humans have approximately **20,000 genes**, and there are more than **6,000 genetically based diseases**. As our understanding of genetics advances, this number will continue to grow.

## TREATING GENETIC DISEASES WITH GENE THERAPY:

**Gene therapy** is a type of medicine designed to treat a genetic disease by adding the functioning gene or genes into a specific cell (e.g., liver cells, bone marrow cells), which allows the patient's body to return to good health. Gene therapy can also be used to reduce the activity of a harmful gene.

## GENE THERAPY APPLICATIONS:

Currently there are many gene therapies being developed to treat multiple diseases, including **hemophilia, inherited retinal diseases, myeloma, phenylketonuria (PKU), and Huntington's disease**.

These gene therapies are used on **somatic (non-heritable)** cells. Using gene therapy on **non-heritable** cells means the therapy does not change the genes that a person passes on to their children.

# Truly Transformative Treatments

- *Zolgensma* - Spinal Muscular Atrophy:

Cost Under Current Therapies:

Spinraza - \$625,000 - \$750,000 (year 1), \$325,000 (subsequent years)

(weight based)

Cost under NEW: \$2.125 million

- Hemophilia – Multiple Pipeline Candidates:

Cost Under Current Therapies:

\$630,000/yr for just factor replacement

Cost under NEW: TBD

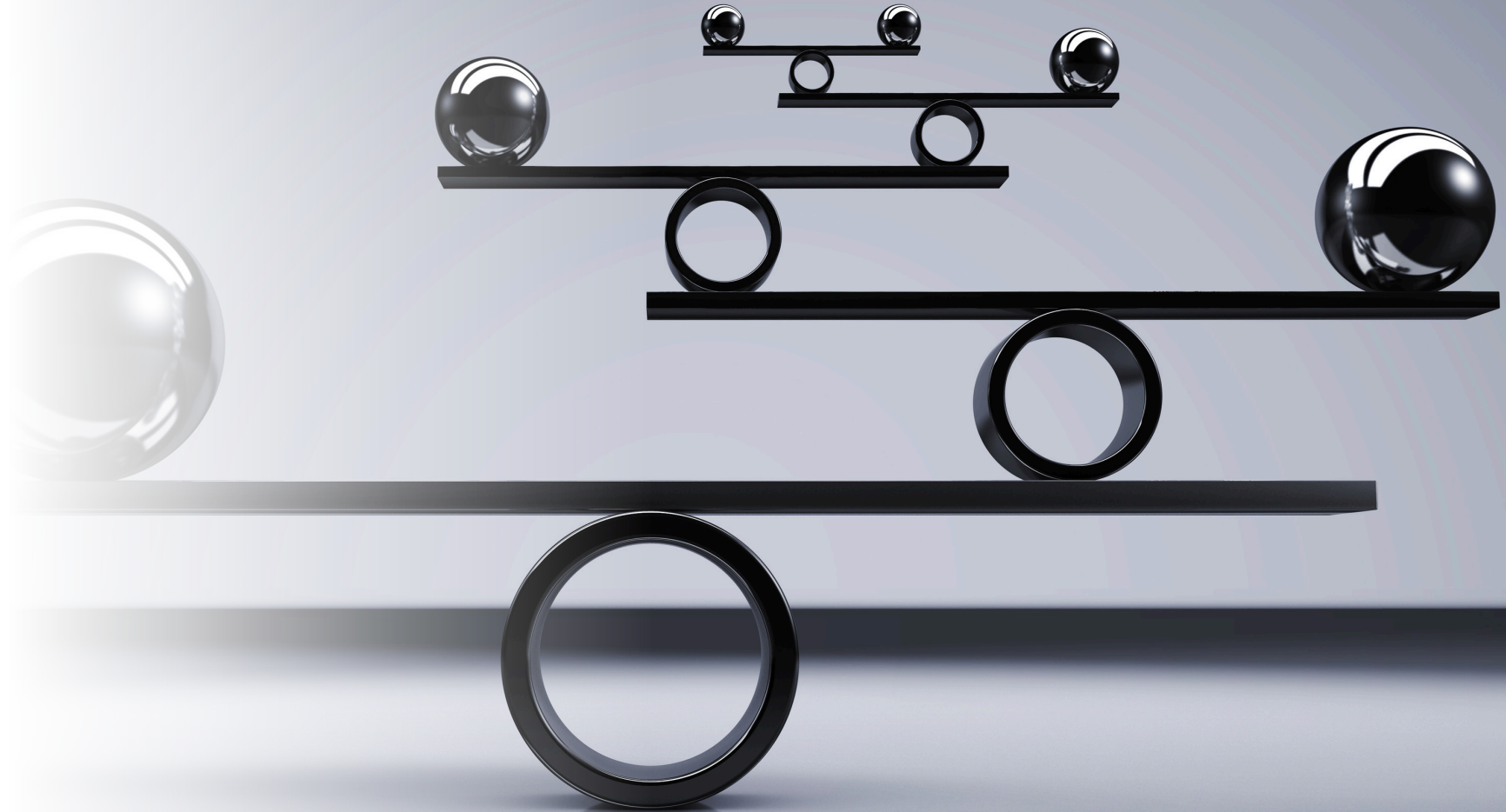


# And more gene therapies are coming

- 2021: 2 gene therapies and multiple cell therapies currently FDA approved
- 2022: Several more, including (potentially) a gene therapy to treat Hemophilia A
- 63 expected to be approved for US market by 2030
- As of July 20, 2021, there were 1,745 gene or cell therapies in development – 254 in phase I, 234 in phase II and 26 in phase III per the American Society of Cell and Gene Therapy

*Sustainable Model Needed to Match This Environment*

**Can a multi-million dollar gene therapy deliver value?**



# Yes.

Over the last four months, an independent organization (ICER) has compared BioMarin's Roctavian for hemophilia A and CSL Behring's etranocogene dezparovec for hemophilia B against commonly used therapies for the bleeding disorders.

**Roctavian and etranocogene dezparovec are not only more effective, ICER said, but in the long run they're also less expensive.**

"The gene therapies have large cost savings associated with them, with very large lifetime costs associated with both the treatments and the comparators," ICER wrote. "In addition, the gene therapies are associated with higher (quality of life scores) and lower bleeds."

[https://icer.org/wp-content/uploads/2022/05/ICER\\_Hemophilia\\_Draft-Evidence-Report\\_091322.pdf](https://icer.org/wp-content/uploads/2022/05/ICER_Hemophilia_Draft-Evidence-Report_091322.pdf)

# Medicaid Financing: Drug Spend

## Access

- States use a variety of methods to limit access to high-cost drugs
  - Utilization review / Managed Care
  - PBM Contracting / Preferred Drug Lists

## Cost

- States have access to a variety of cost limits including best price
- Risk sharing
  - Carving out high-cost drugs from MCO contracts
  - Reinsurance or risk pool mechanisms to share high costs

## Quality

- Medicaid requires coverage of all medications





# Medicaid Financing – Gene Therapy Issues

- Gene therapies will
  - Revolutionize the treatment of many diseases
  - Create significant healthcare financing issues
- Medicaid will be required to cover these treatments
  - High costs will create efforts to limit access

The explosion of patient transforming treatments requires Medicaid to start looking at these issues now.



State policymakers need  
solutions that balance  
access **AND** cost

# Value Based Payments (VBP) can help control both access and cost by aligning the interests of the drug company and Medicaid

- Broad Patient Access
- Sustainable Reimbursement Strategy:
  - Share Risk
  - Hold Manufacturers Accountable
  - Measure Outcome(s)
  - Did the drug do what it was supposed to do?
- Improve Patient Lives
- Interchangeable terms:
  - OBA: Outcomes-Based Arrangement
  - VBA: Value-Based Arrangement (can be used for both prescription drugs and medical services)
  - VBP: Value-Based Purchasing Arrangements (the term CMS uses)

# How do Value-Based Payments work?

- Medicaid contracts with drug manufacturer setting terms of the agreement
  - Access
  - Cost
  - Risk Structure
- Agreement provides broad access only to eligible patients
- Patient outcomes are tracked to determine success of therapy
  - May use claims history, medical records, or specific testing
- The gene therapy's effectiveness is measured. Contracts vary but may look at the population or down to the individual patient level.



# Types of VBPs

- Outcomes Determine Reimbursement: Clinical Metrics, Evaluation
- Warranty Model
- Payment Over Time: “Installment Plan”
- Reinsurance: Limit Costs to Payers

A stack of five dark, rounded stones is balanced on a weathered wooden post. The stones are arranged in a pyramid-like structure: one stone on the post, two on the first stone, and two on the second stone. The background shows a beach with waves and a clear blue sky.

**VBP**s ensure broad access and control costs by requiring drug manufacturers to share risk.

# Value-Based Payments have challenges

- Administration
  - Each VBP requires a separate agreement
  - Medicaid staff needs to administer
- Objective Outcome Measurement
  - Set of patient measures need to be agreed to
  - Data availability can be an issue
- Third Party Evaluation – Requires agreement and trust
- Operational – MCO & Medical Providers
- Federal ambiguity over best price
  - *The Anti-Kickback Statute & Stark*: Under current statute, some “pay for results” discounts negotiated under a value-based contract might be construed as an unlawful inducement to use a manufacturer’s drug.

# ... And Opportunity

- Access
  - Contracts broaden access to patients
- Risk sharing
  - Medicaid receives rebates when the treatment is not as effective as promised
  - Rebates often apply on a per patient basis
- Cost Management
  - Many agreements provide more effective financing arrangements
  - Only paying when it works as promised
- Outcome and Data Analytics
  - Medicaid tracks the effectiveness of the treatment ensuring effectiveness over time.



# State Plan Amendments

- 11 States: AL, AZ, CO, LA, MA, MI, NC, OH, OK, TX, WA
- Multiple others exploring the issue
- Enables a path forward; NOT a mandate
- Flexibility for states & CMS (types of therapies, VBAs)
- In some cases, your Medicaid agency may have the authority in others the agency will need legislative approval.

# Will Medicaid be ready for these transformative therapies?

- Status Quo: Out of Date
- Overarching Federal Policy: Unlikely in Near-term; Administratively Burdensome
- State Plan Amendment Process: Flexible & Practical



# Thank You!

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Questions?



**QR Code for CTT's Paper  
on VBA/OBA/VBP**