

Value-Based Arrangements for Rx

Michael Heifetz
Campaign for Transformative Therapies



Campaign for Transformative Therapies

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



A CAMPAIGN FOR Transformative Therapies

The Campaign for Transformative Therapies believes that encouraging value-based arrangements for gene therapies in federal health programs and the private sector is necessary to ensure patients can access affordable, innovative treatment.

OHIO Medicaid Basic Stats

- Members: 3.0 million
- Managed Care Members: 2.7 million
- Overall Spend \$31.7 Billion
- Drug Spend: Approx. 15%

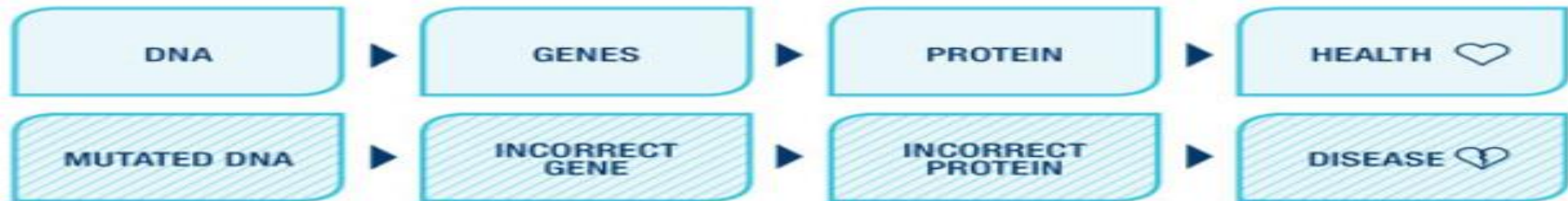
OHIO Initiatives

- Value Based Arrangements (State Budget)
- MCO Procurement
- PBM Reform & Accountability
- Redetermination & FMAP Tapering
- Covid...

Why VBAs?

- Promote Meaningful Access
- Build Sustainable Reimbursement Strategies:
 - Share Risk
 - Hold Manufacturers Accountable
- Measure Outcomes
- Improve Patient Lives

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.

For additional information on other types of technologies used to treat disease, such as somatic gene editing, please visit [BIO.org](https://www.bio.org) and [BIO.org/GenomeEditing](https://www.bio.org/genomeediting), where you can also find BIO's position on human germline editing

Truly Transformative Therapies

- Zolgensma - Spinal Muscular Atrophy (Under Age 2)
(Abigail Wexner Institute, Nationwide Children's Hosp.)
- "SRP-9001" - Duchenne Muscular Dystrophy
- *Luxterna* – Hereditary Retinal Dystrophy
- Hemophilia – Multiple Pipeline Candidates; No FDA Approvals yet



Truly Transformative Therapies



- *Zolgensma* - Spinal Muscular Atrophy:

Cost Under Current Therapies: *Spinraza* - \$625,000 - \$750,000 (year 1), \$325,000 (subsequent years)

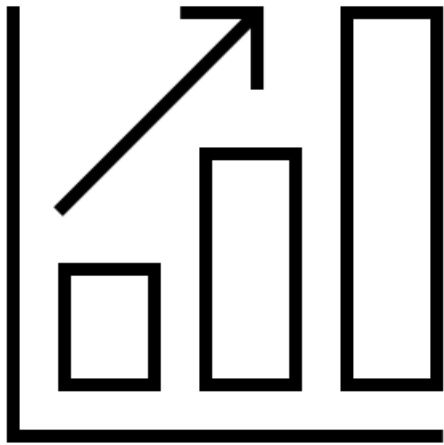
Cost under NEW: \$2.125 million

- Hemophilia – Multiple Pipeline Candidates:

Cost Under Current Therapies: \$630,000/yr. for factor replacement

Cost under NEW: TBD

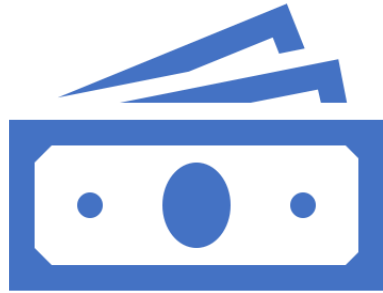
Rapidly Changing Environment



- 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

Current Policy Barriers



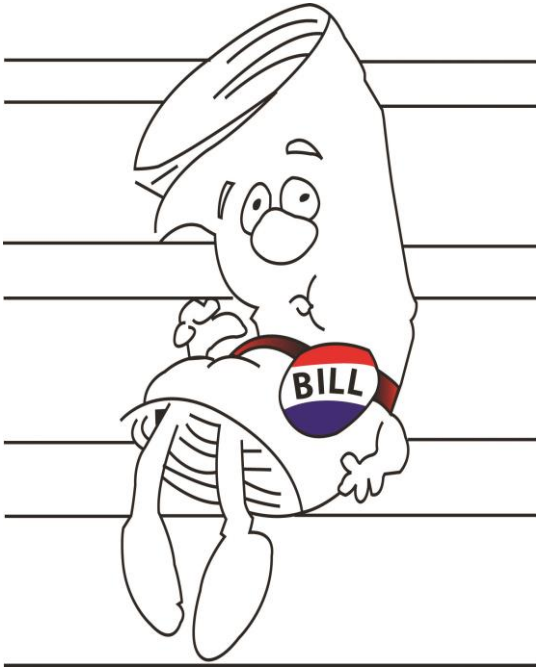
Federal Health Program Drug Price Regulations:
Medicaid Best Price & AMP



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.

Policy Solutions ?

LEGISLATION



- Lower Costs More Cures Act (S.2164)

REGULATION

- December 2020 CMS Final Rule on Medicaid VBPs
 - Multiple Best Price solution

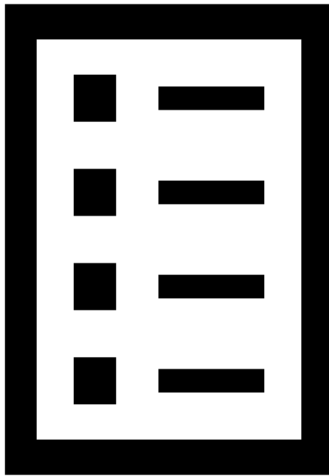
PATH FORWARD?

State Plan Amendments



- 10 States: AL, AZ, CO, LA, MA, MI, NC, OK, TX, WA
- Multiple Others Exploring the Issue
- OHIO: Requires SPA Submission by 12/01/2021
- Enables A Path Forward; NOT a mandate
- Flexibility for States & CMS (types of therapies, VBAs)

Types of OBAs



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

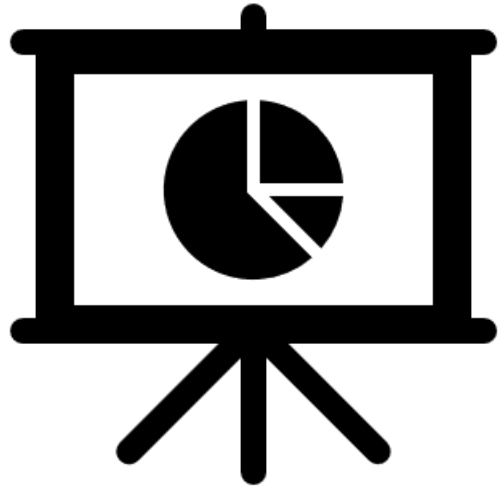
Challenges

- Administration of VBAs: Bandwidth & Expertise
- Objective Outcomes Criteria & Data
- Third Party Evaluation
- Operational: MCOs, Providers
- Federal Ambiguity

Opportunities

- Access
- Risk-Sharing
- Cost Management
- Outcomes Data/Analytics

Conclusion



- Status Quo: Out of Date
- Overarching Federal Policy:
Unlikely in Near-term;
Administratively Burdensome
- SPA Process: Flexible & Practical
- OHIO: Solid Foundation for New Approach

Thank You!

Q & (maybe) A