

**LIMITLESS
POSSIBILITY
LIVES
HERE**



**CALIFORNIA
LIFE SCIENCES**

**Value-Based
Arrangements for Gene
and Cell Therapies**

May 3, 2024

Michael Heifetz
Campaign for Transformative Therapies

Campaign for Transformative Therapies

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



Why VBAs?

- Promote Meaningful Access & Equity
- Construct a Sustainable Reimbursement Strategy:
 - Share Risk
 - Hold Manufacturers Accountable
- Voluntary for Both Payers & Manufacturers
- Measure Outcomes
- **Improve Patient Lives**



Truly Transformative Therapies...

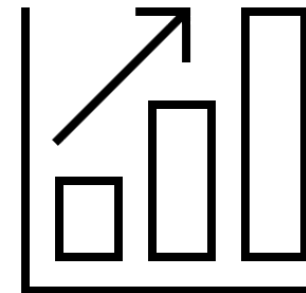
- Casgevy – Sickle Cell Disease
- Lyfgenia - Sickle Cell Disease
- Zolgensma - Spinal Muscular Atrophy (Under Age 2)
- Luxterna – Hereditary Retinal Dystrophy
- Hemegenix – Hemophilia B
- Roctavian – Hemophilia A



Rapidly Changing Environment

- **2017:** First gene therapy approved (Luxturna), multiple cell therapies approved
- **2019:** Zolgensma (SMA) brings total to 2 gene therapies & multiple cell therapies
- **2022:** 3 more approvals
- **2023:** 5 approvals, including 2 for Sickle Cell Disease & 1 for Hemophilia A
- **2024:** 2 approvals already
- 60+ expected to be approved for US market by 2030 (National Institutes of Health)
- As of May 3, 2024: 2,292 gene or cell therapies in development (American Society of Cell and Gene Therapy)

SUSTAINABLE MODELS NEEDED TO MATCH THIS ENVIRONMENT



State Plan Amendments (SPAs)

- 21 States and Washington, D.C.: AL, AZ, AR, CO, DC*, GA, IL, IN, LA, MA, MI, MO, NC, ND, NY, OH, OK, PA, TX, UT, VT, WA
- Multiple others have submitted a SPA (CA) or are moving forward (MN, WI)
- Enables a Path Forward Without a Mandate
- Flexibility for States & CMS (types of therapies, VBAs)

