A DEVELOPING APPROACH TO BREAKTHROUGH THERAPIES: THE VIRGINIA MEDICAID EXPERIENCE

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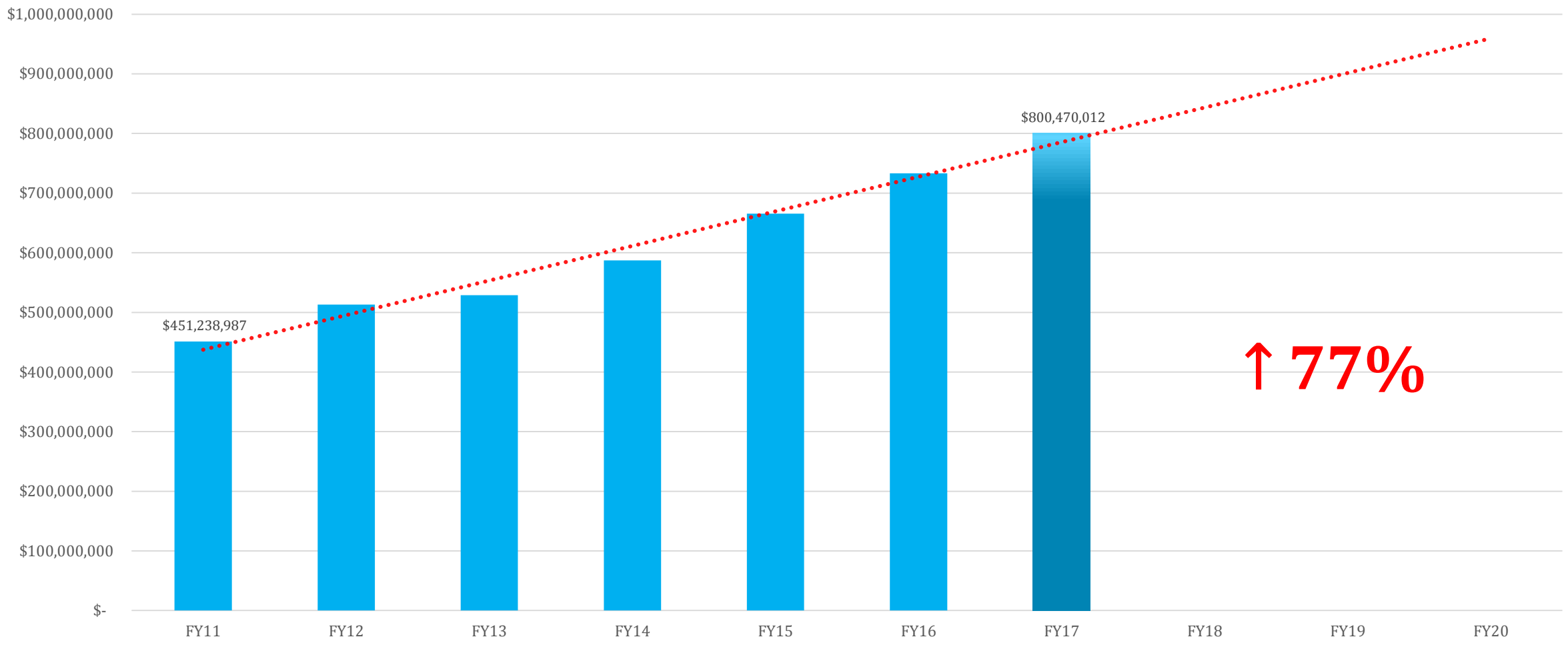
DMAS Pharmacy Spend 2011-17



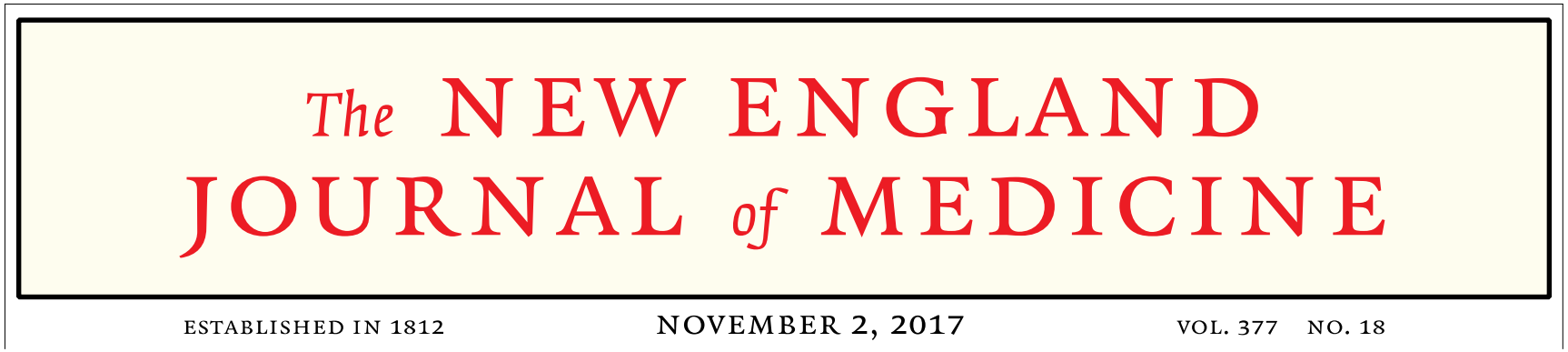
The (Optional) Pharmacy Benefit

- Defined by Social Security Act 1927
- Medicaid programs are required to cover all drugs that are
  - FDA approved
  - Medically necessary
  - Manufactured by a pharmaceutical company participating in the Medicaid Drug Rebate Program
- The Act allows Medicaid program to develop preferred drug lists (PDLs) and exclude drugs from the PDL as long as a service authorization (SA) process is established

Case Study: Zolgensma

- Gene-replacement therapy for Spinal Muscular Atrophy, a debilitating condition with few therapeutic options
- Affects 1:10,000 newborns with ~9 infants with SMA born in Virginia each year
- Most expensive drug in history at $2.1 million
- Received FDA approval May 24

Zolgensma: Clinical



Single-Dose Gene-Replacement Therapy for Spinal Muscular Atrophy

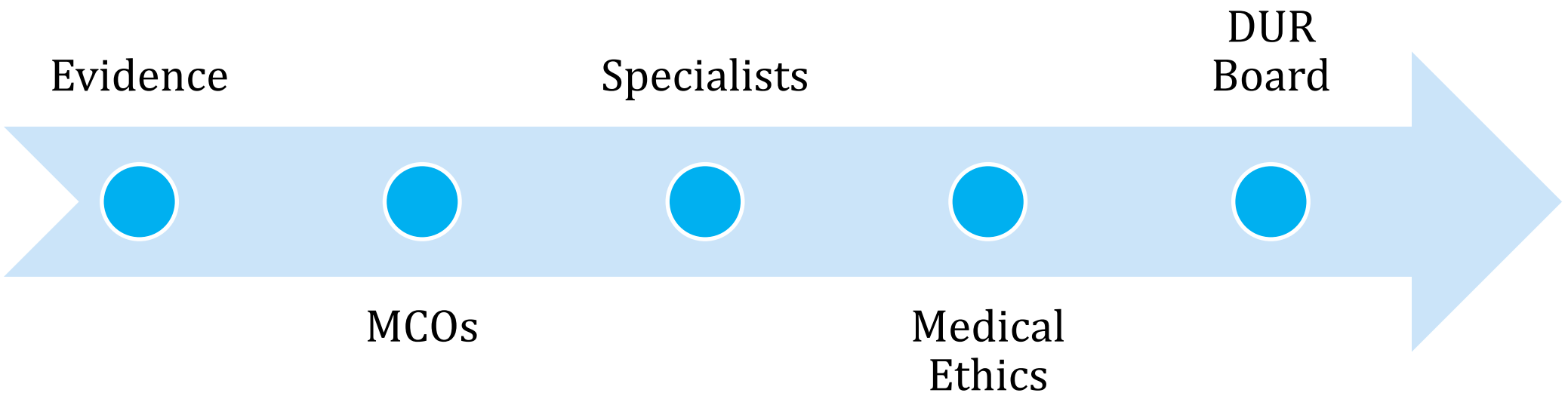
- 1 non-randomized peer-reviewed clinical trial of 15 patients SMA T1 and 2 copies of SMN2
- 100% survival at 20 months compared to historical control of 8% survival
- Handful of small unpublished clinical trials with similar results

Zolgensma: Clinical

FDA Insert: ZOLGENSMA (onasemnogene abeparvovec-xioi) is an adeno-associated virus vector-based gene therapy indicated for the treatment of pediatric patients less than 2 years of age with spinal muscular atrophy (SMA) with bi-allelic mutations in the *survival motor neuron 1* (*SMN1)* gene.

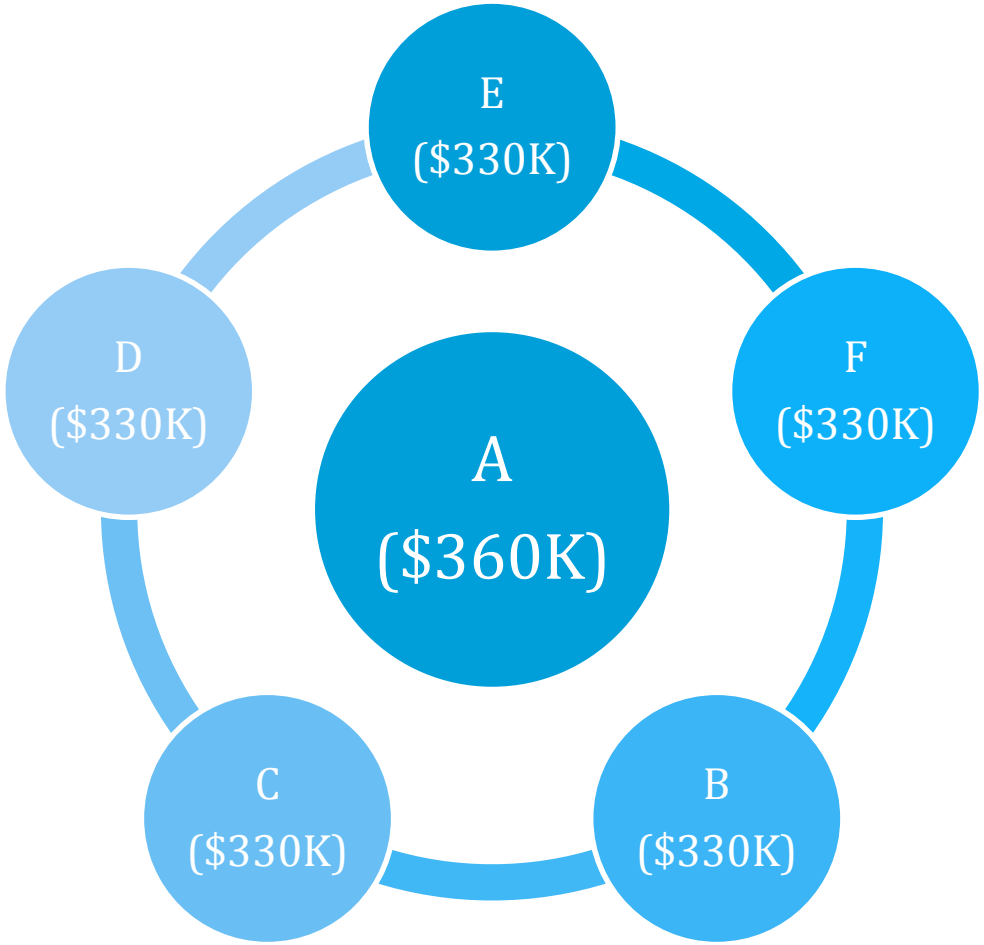
Zolgensma: Clinical

How do you proceed when the FDA approval is broader than the published clinical evidence?



Zolgensma: How Cost and Risk are Managed

For drug costs > $175K, the managed care organizations (MCOs) share in the cost. Above this amount, MCO A is responsible for 10% of costs, and MCOs B-E are responsible for 90% of costs (proportional to their enrollment).

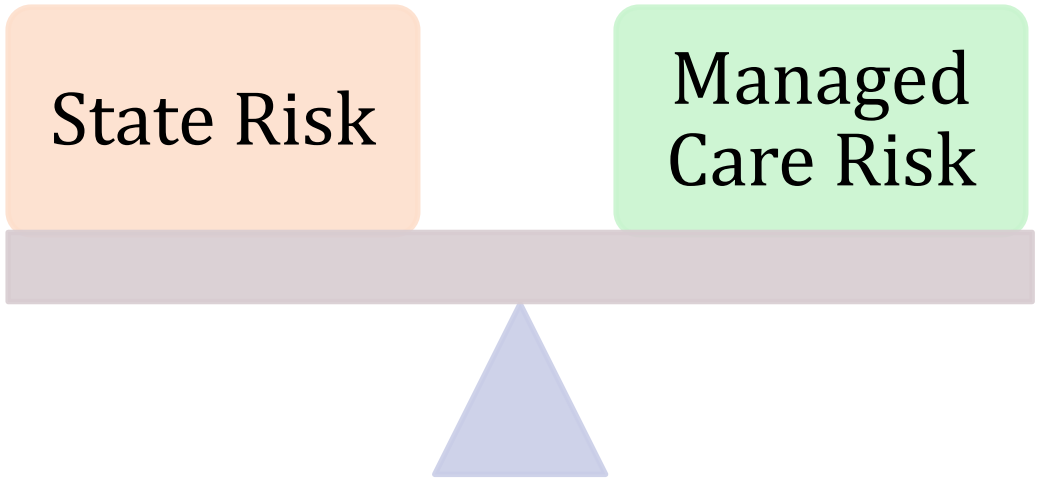


Key Considerations

- While this attenuates the impact of adverse selection, it does not eliminate it.
- It is beneficial to the state because it is budget-neutral each year.
- It is acceptable to the MCOs if the rate-setting process accurately predicts drug costs each year.

Zolgensma: Cost and Risk Considerations

- Who receives the medication?
- Who pays? Over what time?
- How are the medical costs (savings) accounted for?
- Who holds the risk?



Key Points

- Pharmacy costs are on a steep and unsustainable trajectory
- Medicaid is required to cover all FDA-approved, medically necessary drugs with a negotiated rebate
- High-cost breakthrough therapies require focused attention and innovation from both a clinical and financial perspective