Amicus Therapeutics®

Gene Therapy 101: Policy and Principles for Value-Based Contracts

Paul Howard, PhD

Director of Public Policy, Amicus Therapeutics

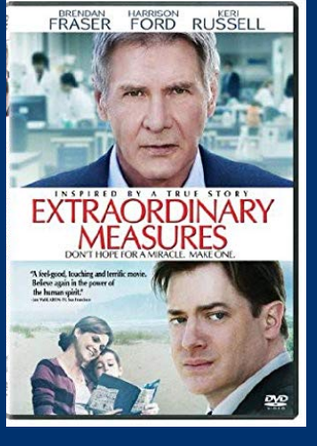
We're a part of the community of rare disease patients

The core values of our company stem from the origins of our organization

John Crowley, Chairman and CEO of Amicus Therapeutics, with his family

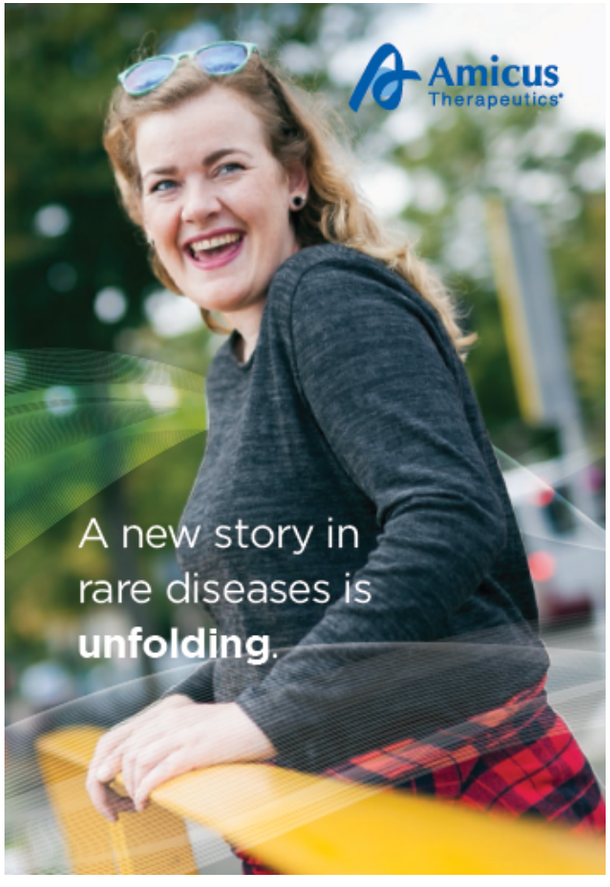
John Crowley's personal journey raising two kids with a rare genetic disorder led him to build a company with a focus on developing advanced therapies to treat a range of serious rare diseases.

A motion picture tells the story of his early experience



- The emotional journey of a family
- His fighting spirit & determination
- The critical link between science & patient dedication

We're dedicated to searching for new therapies that can make a difference



Amicus Therapeutics is a global, patient-dedicated biotechnology company focused on discovering, developing and delivering high-quality medicines for people living with rare metabolic diseases.

As a society, we need to recognize we're at a pivot point in medical science

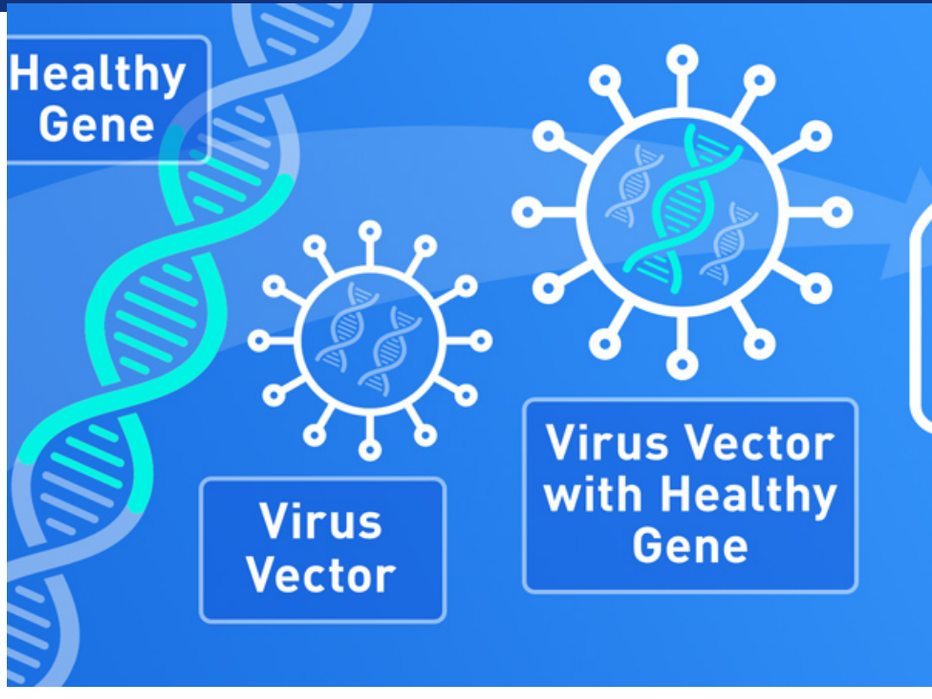
"The FDA is witnessing a surge of cell and gene therapy products entering early development, evidenced by a large upswing in the number of investigational new drug (IND) applications. Based on this activity, we anticipate that the number of product approvals for cell and gene therapies will grow in the coming years, reflecting significant scientific advancement and the clinical promise of these new innovations."

- *FDA Commissioner Scott Gottlieb and CBER Director Peter Marks* (Jan 2019)

- **Policy decisions we make today will dictate how rapidly new scientific advances can be translated into effective therapeutics**
- **And how accessible they are given other ongoing health care costs and burdens**
- **In other words, we have a chance to get this right**

That starts with a new reimbursement model for potentially curative technologies

- Old paradigm: **Pay by the pill** or injection
- **Maintenance model** requires continuous dosing
- Payments align with **value over time**
- **Genomic technologies are disruptive**
- Potential for **cures in a single dose**
- Entire **value is delivered upfront**
- Benefits continue **long after dosing ends = durability**



Genomic technologies can help us treat and cure diseases that were previously untreatable or uncurable.

Volume to Value: Gene Therapies

Bipartisan consensus that reimbursement should reflect real world outcomes and value to patients.

The largest share of U.S. healthcare costs are physician and hospital costs.

Gene therapies offer an opportunity to dramatically reduce those costs over time

Potential to increase productivity by reducing burdens on families and caregivers

Goldilocks Public Policy: Value-Based Agreements (VBAs)

The Right Policy Mix for the Next 10+ Years

- Gene therapies are still an evolving science, with FDA approvals based on small trials
- Evidence on durability will only emerge over time
- Pricing should reward innovation and reflect the value delivered to patients and society
- Risk should be shared and linked to patient outcomes
- Value Based Agreements (VBAs) align risk based on patient outcomes
- Future Innovations: Dosing, Manufacturing, Durability, Safety

Federal Policy Changes Are Needed to Encourage Experimentation, Evolution of VBAs as Technology Advances

Federal statutes inhibit the evolution of VBA's, particularly the Medicaid Prescription Drug Rebate Program (MDRP)

Congress requires manufacturers to offer state Medicaid programs the "best price" granted to any other payer (exceptions: DoD, VA, IHS); generally a minimum rebate of 23.1%

This is a unit level pricing measure based on all discounts…so in a VBA, it could be triggered by a single non-responding patient for all 50 state Medicaid programs

Problem: If a 100% rebate is offered for a patient who doesn't respond to a gene therapy, the national Medicaid Best Price would drop to $0**.**

***Solution*:** Unleash both the commercial market and Medicaid programs to experiment with value-based agreements.

5 Principles for Congressional Reforms to Expand VBAs

1) **Preserve the Medicaid Best Price** rebate for genomic therapies (23.1%) **as the floor** for gene therapies. For a $2m product paid over 5 years at $400k/yr that would be a minimum annual discount of $92,400 relative to price charged private payers.

2) **Require manufacturers to offer state Medicaid programs the same VBA agreement(s)** for their product that they have negotiated with any commercial payer, **on the same outcomes-based terms**. The state **could choose** the most appropriate agreement.

3) **States can still negotiate supplemental rebates and collect their own outcomes data.**

4) For **non-responding patients**, states would be eligible to receive the highest pre-specified commercially negotiated rebate. **But even if every patient responded (an ideal outcome) the minimum Medicaid rebate (or supplemental negotiated rebates) would still apply.**

5) **Rebated discounts under VBAs should be excluded from Medicaid Best Price calculation.**

Objections and Concerns

Q: States won't be able to negotiate complex VBAs.

A: They can simply **opt for the most attractive commercially negotiated contract terms**.

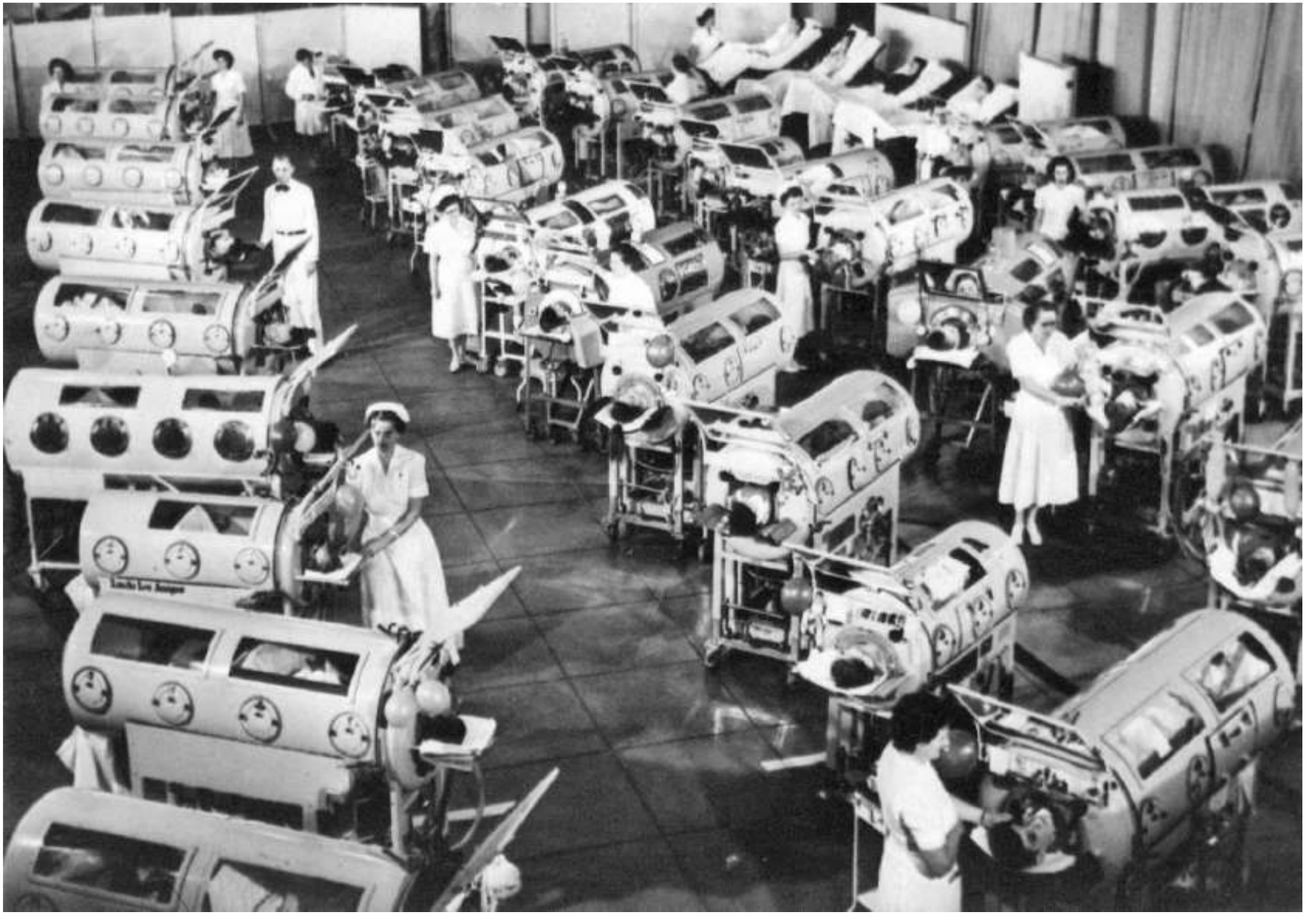
Q: But aren't outcomes-based contracts complex, and difficult to administer?

A: States and commercial insurers could recognize manufacturer and payer designated **"Centers of Excellence" (COEs) for dosing and collecting patient outcomes data over time**. Pooling outcomes over larger patient cohorts should reduce uncertainty and costs, but payers and manufacturers should have the freedom to identify critical outcomes (clinical, non-clinical, or patient-reported) in transparent contract terms.

Q: If we exempt gene therapies from Medicaid Best Price, won't prices and costs simply go up?

A: **We need to consider all-in costs.** That is why it is important to think about COEs, and all the associated treatment costs over time. **Disability, home care, physician care, physical therapy, and other drug treatments should all be considered as part of economic analysis.**

What if we could cure crippling childhood diseases with a single injection?



Thank you.

“We have a duty to obsolete our own technologies”

- Amicus Belief Statement