

July 26,2021

**Maryland Life Sciences – Martin Rosendale, CEO
Maryland Technology Council**

**Novel Payment Models – Mark Trusheim, MSc, Strategic
Director, NEWDIGS; Visiting Scientist, Sloan School of
Management MIT; President, Co-Bio Consulting**

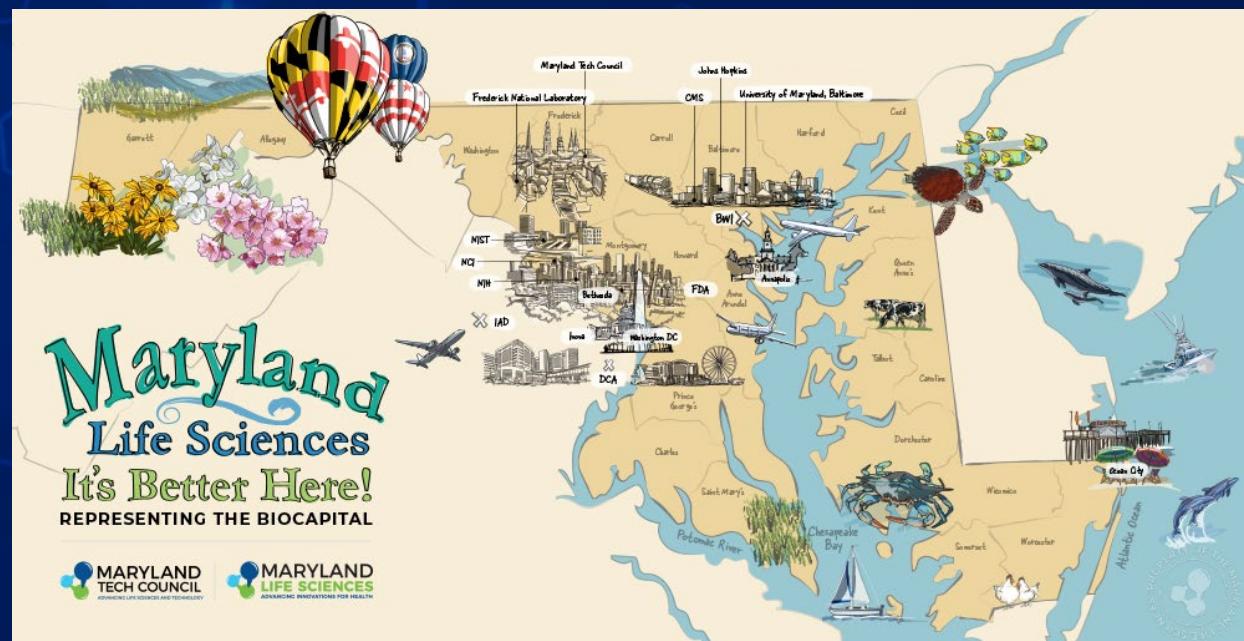




ADVANCING LIFE SCIENCES AND TECHNOLOGY

500 Corporate Members
150,000 Industry Contacts

We support member companies who are saving lives, securing our nation and improving the quality of life through innovation.





**MARYLAND
TECH COUNCIL**
ADVANCING LIFE SCIENCES AND TECHNOLOGY

Maryland Life Sciences:

- 2,500 Life Science Industry Establishments
- 40,000 Industry Employment
- Leading Industry Cluster in Cell and Gene Therapy, Vaccine Development, and Immunotherapies
- First to Map the Human Genome
- Profound Impact in Response to COVID-19

Development of durable medical therapies relies on access to the capital markets, the value of intellectual property and strategic partnerships.

NEW DIGS

Focus

Financing and Reimbursement
of Cures in the US

Novel Payment Models: MD PDAB

July 26, 2021

Mark Trusheim

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MIT CENTER FOR BIOMEDICAL INNOVATION



Outline for Today

- Introduction to FoCUS and the Durable Therapy Pipeline
- New Precision Financing Approaches
- Implementation Challenges

MIT NEWDIGS – Helping the System Catch Up With the Science

- Safe haven “think & do” tank for convening; MIT = trusted neutral intermediary
- Track record of **real world impact**
- Interactive methods/tools for **multi-stakeholder collaboration**
- Bold, transformational system innovations **for ten (10) years**

NEWDIGS “Adaptive Licensing” Project fueled timely action & impact in Europe from regulatory science innovation.....



... and Illuminated a Broad Set of Principles for Accelerating Sustainable Patient-Centered Innovation



Learning Ecosystems Accelerator for Patient-centered, Sustainable innovation

FoCUS: Dedicated to making innovative cures accessible and sustainable

Durable, potentially curative therapies for genetic disorders and cancer have arrived. Short—even single dose—treatment regimens yield lasting health benefits, but large single payments will challenge the current reimbursement system.

Policy, regulations, and business operations need to evolve to enable emerging solutions. The FoCUS Consortium designs and shares precision financing solutions to ensure patient access and system sustainability.



Focus of FoCUS: An MIT NEWDIGS Consortium

On—

Creating **precision financing solutions** for therapies with large, upfront, acute costs whose benefits accrue over time



Not on—

Assessing or setting value, or negotiating specific prices for specific products



FOCUS: Sustainable Access for Durable Therapies via Downstream Innovation

>100 organizations & 400 individuals engaged



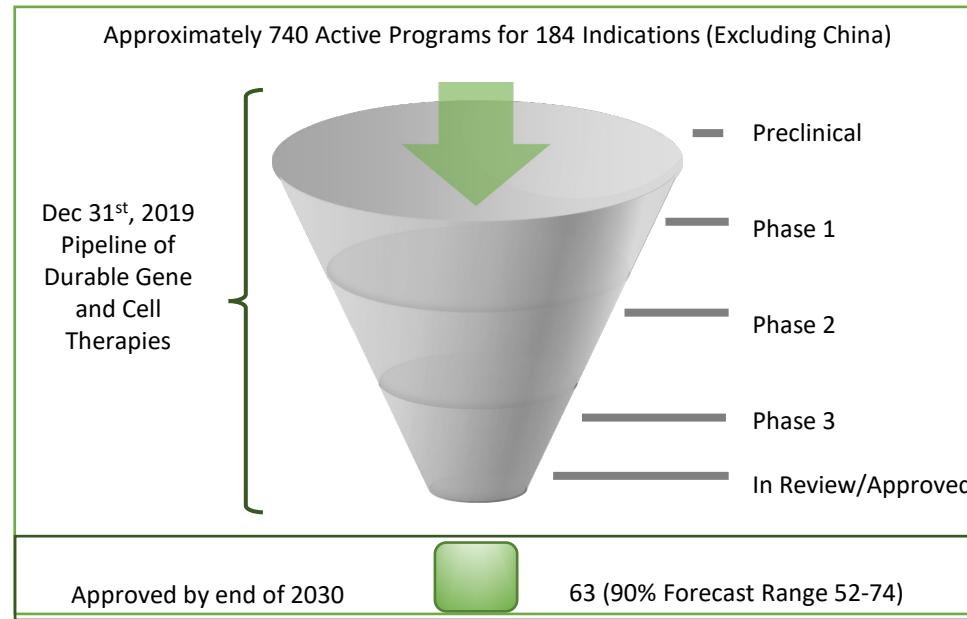
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Image Sources: <https://www.cheapfullcoverageautoinsurance.com>, www.MIT.edu

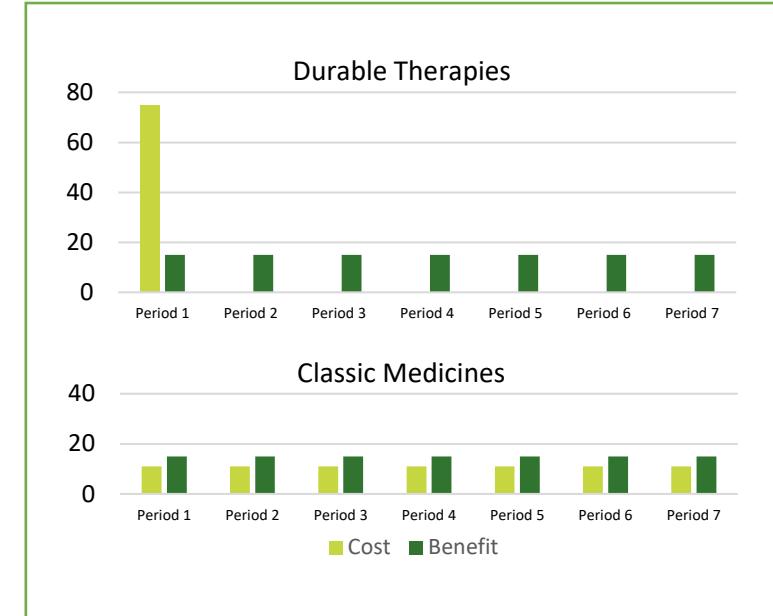
► NEW DIGS ◄

Emerging Durable Therapies Driving New Payment Models

50-75 Therapies Approved for the US Market by 2030



Durable Therapies Distill Payments Upfront



Three financial challenges exacerbated



Payment timing
One-time high cost



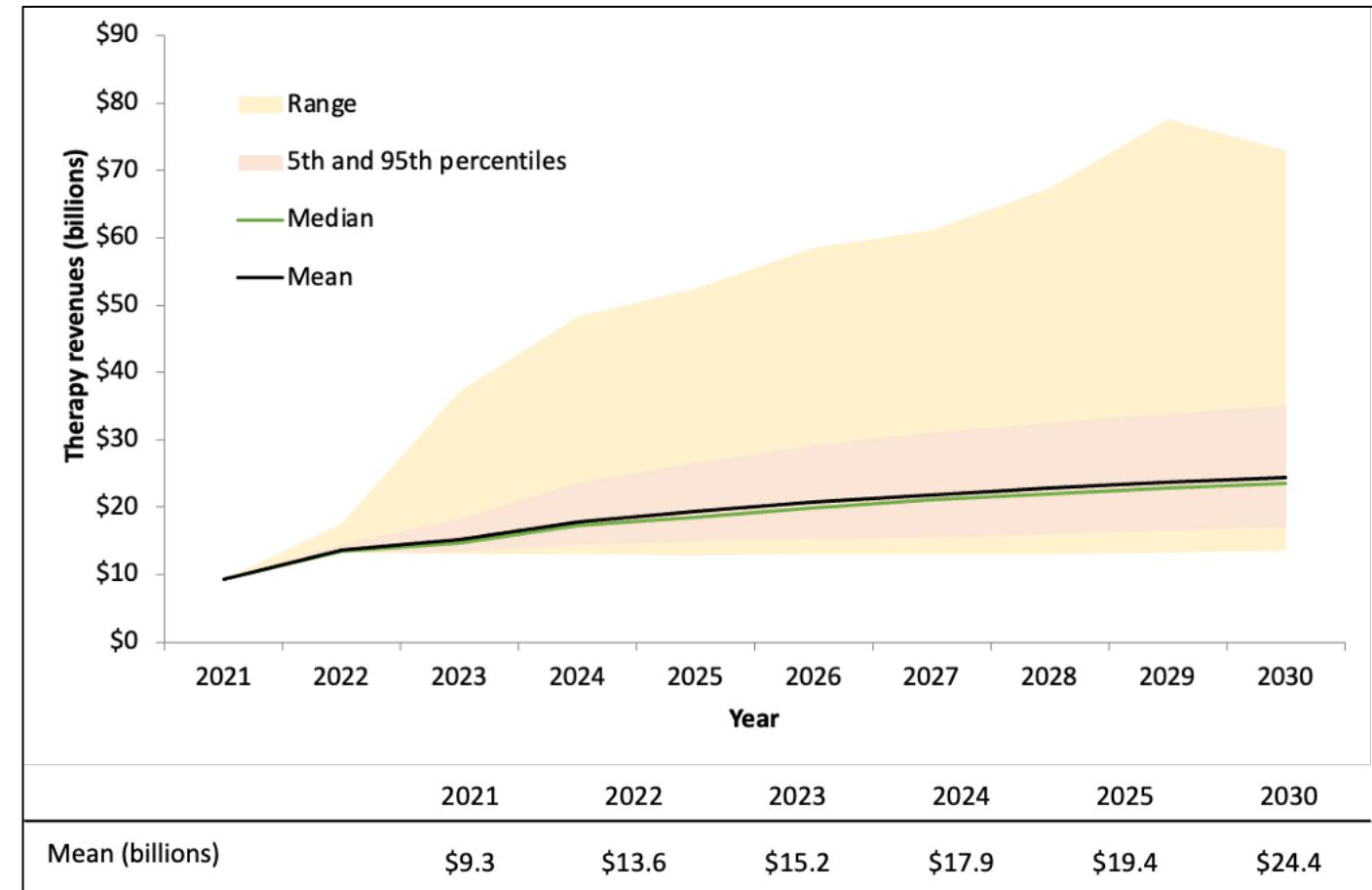
Performance risk:
Effectiveness & durability



Actuarial risk:
Likelihood of encountering a case

Mean Product Revenues Rise to Nearly \$25B

- 5th – 95th percentile range of \$14-35B
- Extreme iterations exceed \$70B
- 2019 US drug spend:
\$509B Payer net
\$356B Manufacturer net
\$ 67B Patient out-of-pocket
(IQVIA:
Medicine Spending and Affordability in the U.S.)



New Precision Financing Solutions To Meet The Challenges



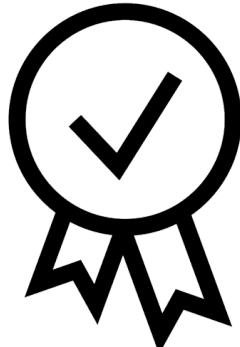
Orphan Reinsurer and
Benefit Manager (ORBM)
and Risk Pools



Short-term
milestone-based
contracts



Multi-year
performance-based
annuities



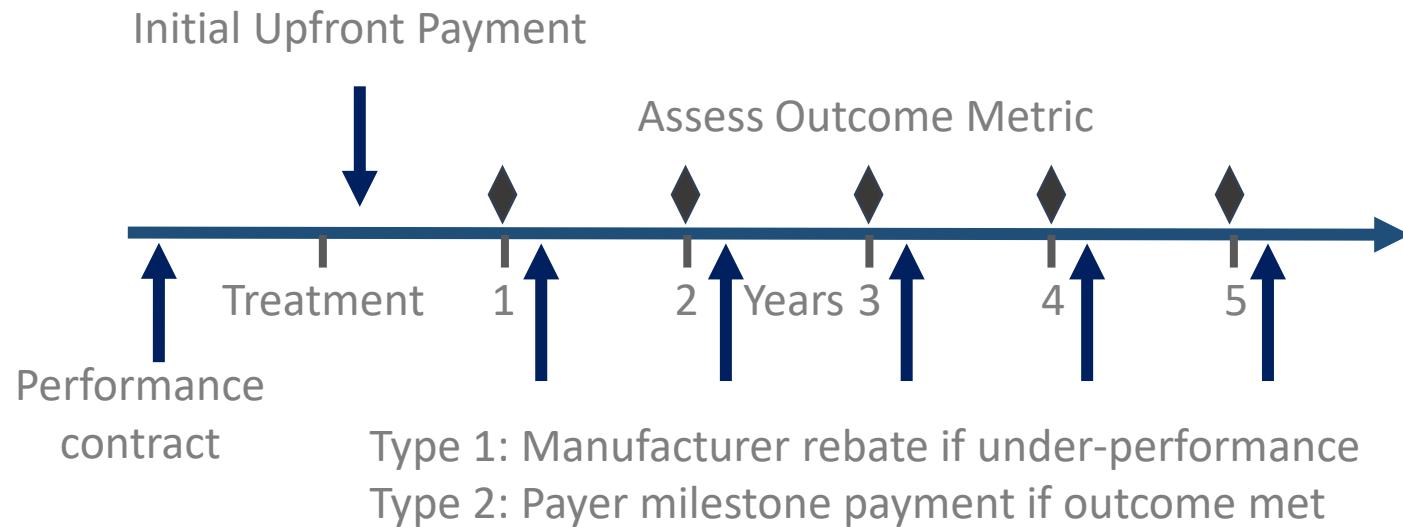
Warranty Model

NETFLIX

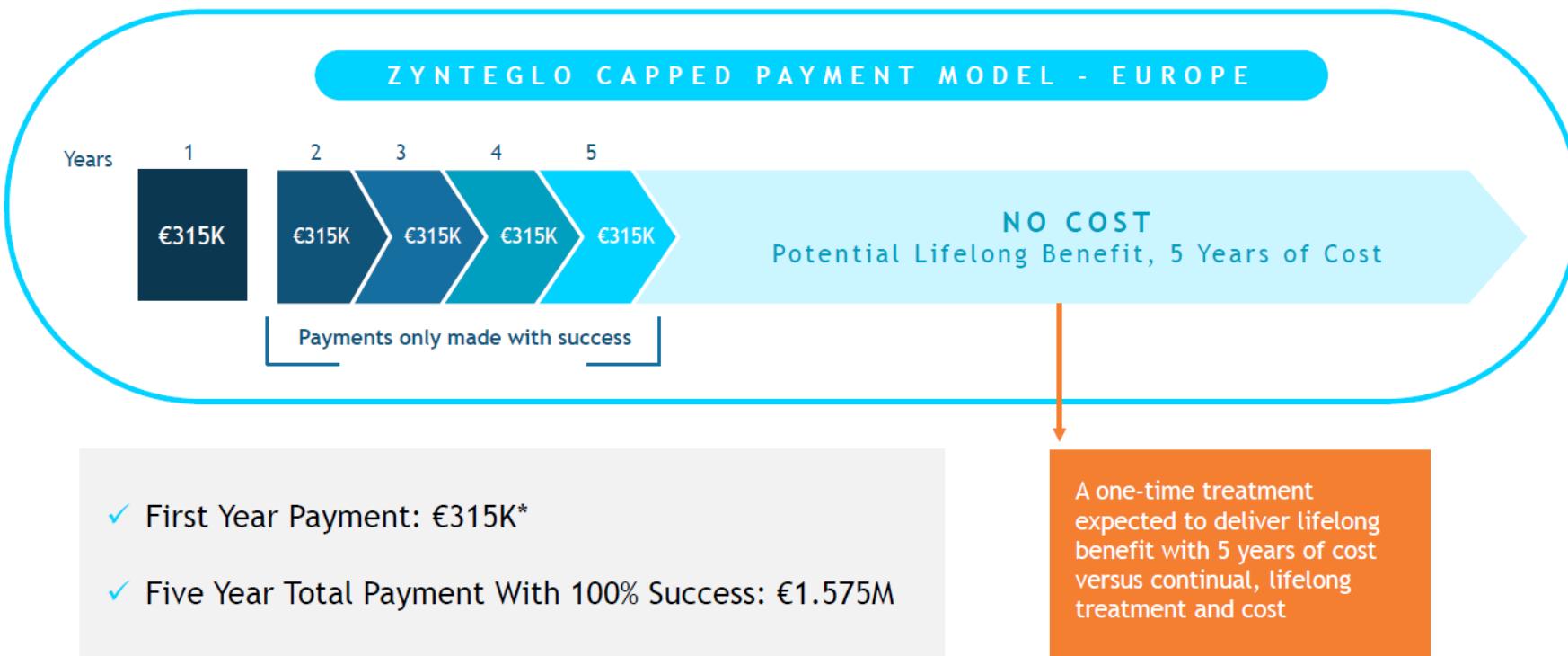
Subscription Model

Performance-based Annuities Addresses Multiple Issues

- **Payment Timing:** Better matches cost avoidance to lower net impact
- **Performance Uncertainty:**
Resolves effect size and durability uncertainty naturally over time
- **Actuarial Uncertainty:** Variable patient numbers smoothed over time
(provides partial patient level reinsurance on demand)



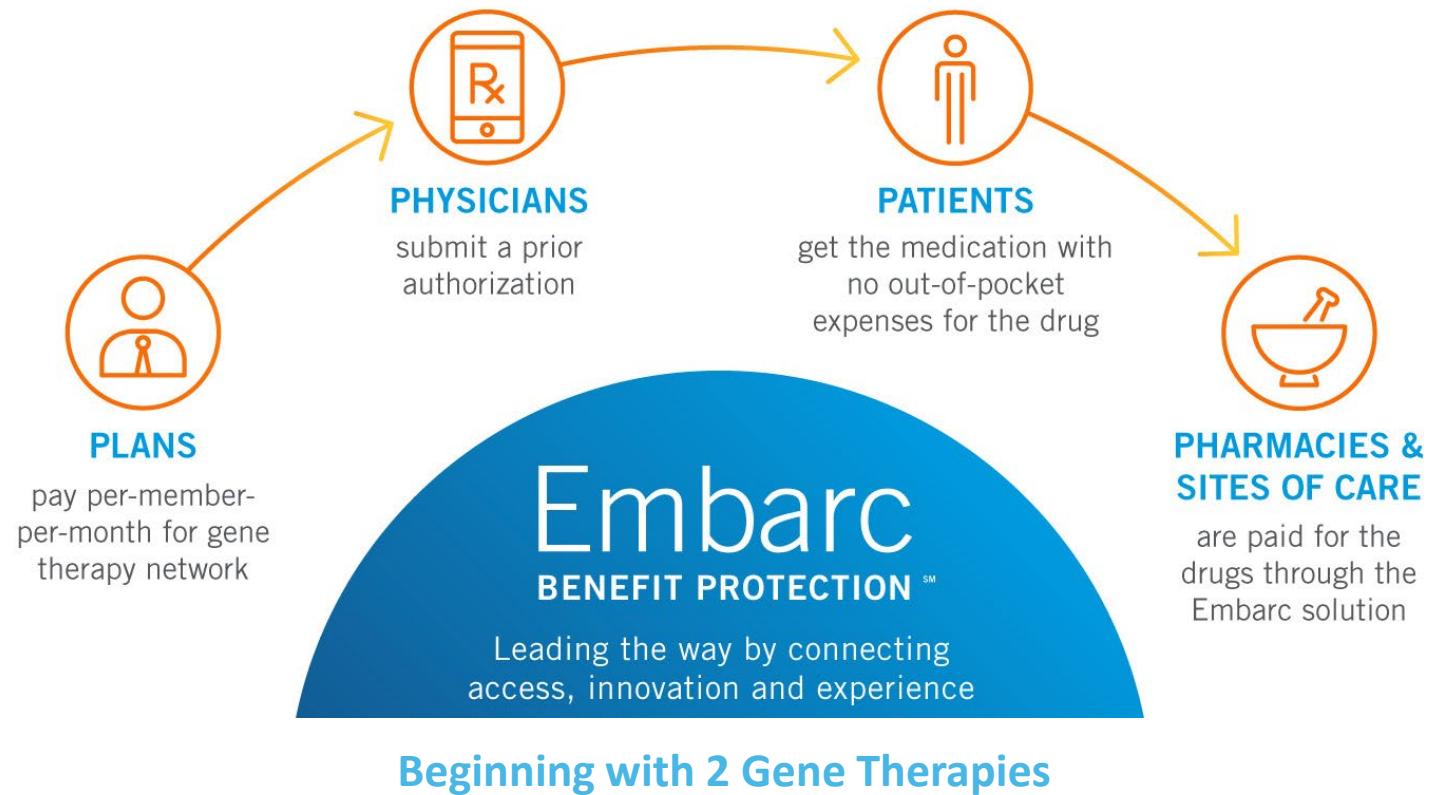
Example Performance-Based Annuity for β -thalassemia



bluebirdbio company presentation, *ready to recode*; September 23, 2019. <http://investor.bluebirdbio.com/static-files/8c4eb7bb-37d6-4fba-941c-1a154c8bbfd6> Accessed November 18, 2019



US Insurer Cigna Offering ORBM-Lite: Embarc



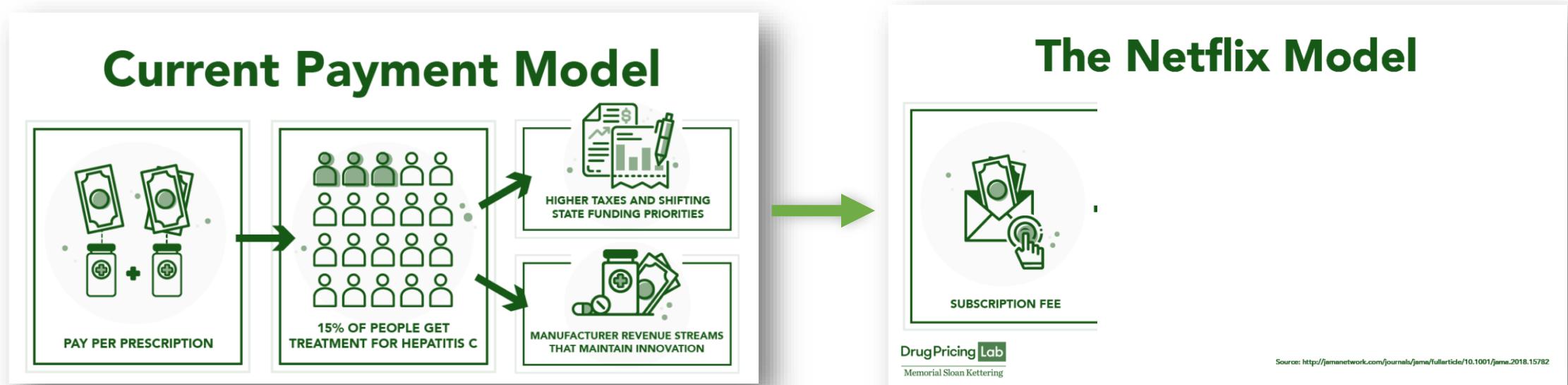
Warranty Model



- Health plan approves of and pays for therapy
- Developer provides warranty policy
- Health plan holds warranty in the event that the therapy fails to deliver on promise
- Warranty pays for supplemental factor needed to get the patient to proper circulating factor level
- Health plan submits claim to Outcome Based Contract Administrator (OBCA)
- Value delivered based on covered healthcare costs not a rebate

Netflix / Subscription Model

- Fixed fee for each year
- Unlimited drug supply
- Increase access
- Align public health, payer and manufacturer incentives



US Implementation Just Beginning for Durable Therapies

| | Payment Timing | Performance | Actuary | US Status |
|--|-------------------|-------------|---------|-----------|
| Short-term milestone contracts | | | | |
| Multi-year performance annuities | | | | |
| ORBM and Risk Pools | | | | |
| Subscription Model | | | | |

Key Challenges: Nearly All with Workable Solutions

- **Patient mobility and performance data collection**
- **Risk sharing**
 - Participation or exclusion of providers
 - Interaction with reinsurance and stop-loss insurance
- **Legal & Regulatory**
 - **Medicaid drug price reporting and rebate** with exception of unit pricing
 - **Anti-Kickback Statute to define explicit safe harbor**
 - **FDA communication guidelines to enable appropriate performance metrics** Clinical trial endpoints often not practical for clinicians or present in data systems

Summary

- Introduction to FoCUS and the Durable Therapy Pipeline:
 - Dozens of products with \$10s of billions in 2030 list price revenue
- New Precision Financing Approaches and Players
 - 5 major models
 - Many offerings emerging
- Implementation Challenges
 - Regulatory to outcomes tracking
 - Solvable

FoCUS Open Resources

FoCUS

Go to: <https://payingforcures.MIT.edu>

Research Briefs and Peer-Reviewed Publications

ScienceDirect
Content last available at [sciencedirect.com](https://www.sciencedirect.com)
Journal homepage: www.sciencedirect.com/science/journal

Themed Section: Curative Therapies

Are Payers Ready, Willing, and Able to Provide Access to New Durable Gene Therapies?

June P. Barlow, MD, MSc, MBA,* Mo Yang, PhD, J. Russell Teague, MA, DMH
Center for Biomedical Innovation, Massachusetts Institute of Technology, Cambridge, MA, USA

ABSTRACT

Objective: To explore reasons unique to the study design, quality, methods, study, and performance-based model was acceptable to payers for durable gene therapies.

Results: One third of payers were willing to provide access to new durable gene therapies based on a performance-based model. Payers cited four models.

Conclusion: Access to new durable gene therapies was impacted first. Cost models, dampening reimbursement, payers performance.

Value Health 2018; 21(1): 83-90.

ScienceDirect
Content last available at [sciencedirect.com](https://www.sciencedirect.com)
Journal homepage: www.sciencedirect.com/science/journal

Themed Section: Curative Therapies

Estimating the Clinical Pipeline of Cell and Gene Therapies and Their Potential Economic Impact on the US Healthcare System

Casey Quinn, PhD,* Cole Young, PhD, Jonathan Thomas, BS, Mark Trusheim, MSc, and the MIT NEWDIGS FoCUS Writing Group
Center for Biomedical Innovation, Massachusetts Institute of Technology, Cambridge, Massachusetts, USA

ABSTRACT

Objective: To estimate the number of durable gene and cellular therapies new product launches in the United States through 2030 and the number of treated patients.

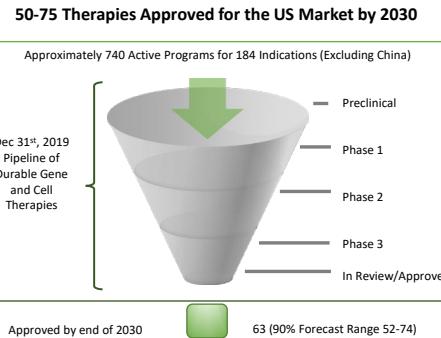
Methods: A statistical analysis of clinical trial pipeline data, disease incidence, and prevalence was conducted to estimate the impact of new cell and gene therapies. Myriad Genetics®'s Pheochromogly® database to estimate the rates and timing of new product launches on the basis of the phase of development, duration in phase, and probability of progression. Disease incidence and prevalence were estimated from the National Institutes of Health's National Institutes of Health.

Conclusion: Access to new durable gene and cellular therapies will be impacted first. Cost models, dampening reimbursement, payers performance.

Value Health 2018; 21(1): 91-100.

FoCUS
Precision Financing Solutions for Durable / Potentially Curative Therapies

Unique Gene and Cell Therapy Pipeline Impact Modeling



Public Speaking Engagements

FoCUS Financing and Reimbursement of Cures in the US

Speaking Engagements

- Jan 29 MassBio Policy Breakfast (M. Trusheim; Boston)
- Feb 6-7 Blue Cross Blue Shield Association / Aspen Institute (M. Trusheim; DC)
- Feb 7 Medicaid Innovation Summit (D. Rollman; Orlando)
- Feb 13 TBD - Milken Institute FasterCures Workshop (M. Trusheim; DC)
- Feb 21 American Society for Transplantation and Cellular Therapy (ASTCT)/CIBMTR TCT Meeting (Trusheim; Orlando)
- Mar 23-25 MedImpact 2020 (J. Barlow; Dan Mytelka - Carlsbad, CA)
- Mar 30-Apr 2 Hanson-Wade 4th Annual Gene Therapy for Rare Disorders (M. Trusheim; Boston)
- April 7-9 Alliance for Healthcare Research and Quality? (AHRQ) (M. Trusheim; LA)
- April 15-16 Eye for Pharma Philadelphia 2020 (M. Trusheim; Philadelphia)
- April 21 National Cooperative Rx Annual Meeting (J. Barlow; Madison, WI)
- May 1 Terrapin World Orphan Drug Congress (M. Trusheim; DC/MD)
- TBD Mellon Financial 'Double Take' Podcast (D. Mytelka)

On-line Toolkit to Educate and Support Practitioners Developing Financing Solutions

Paying for Cures Toolkit
FoCUS Project at MIT NEWDIGS

Home Overview Guided Assessments Planning Tools Bibliography

Paying for Cures Toolkit Home

Toolkit purpose
What is FoCUS?
How to navigate
For more detail...

Step 2: Ratio the total US population to your plan

Your Plan Size (# of members) US Population: 328,000,000

Clinically Relevant Population

Plan-specific Total Incidence Estimate **Plan-specific Total Prevalence**

Incidence Modifier **Prevalence Modifier**

Estimated Treated Population

The FoCUS Project at MIT NEWDIGS has developed this Toolkit as a resource for practitioners facing the financial challenges of offering, choosing, reimbursing or supporting an advanced durable therapy. This toolkit enables you to:

Educational Events

MIT CBI
Center for Biomedical Innovation

Paying for Cures Events Research and tools Media

Paying for cures: Ensuring patient access and system sustainability
February 12, 2019 • Washington, DC

Design Labs



NEW DIGS

NEW DIGS

Focus

Financing and Reimbursement
of Cures in the US

THANK YOU

July 26, 2021

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