

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 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 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



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



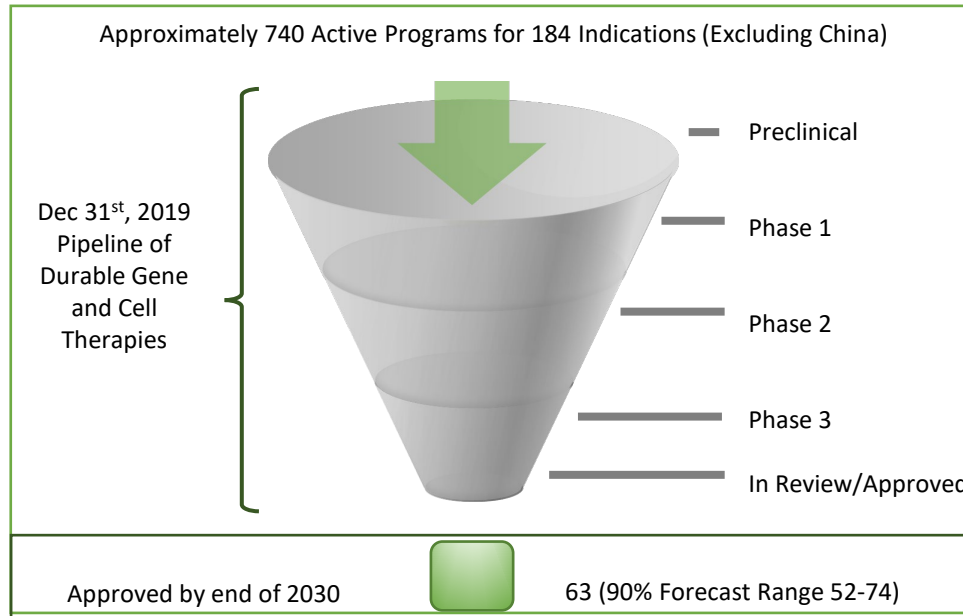
Not on—

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

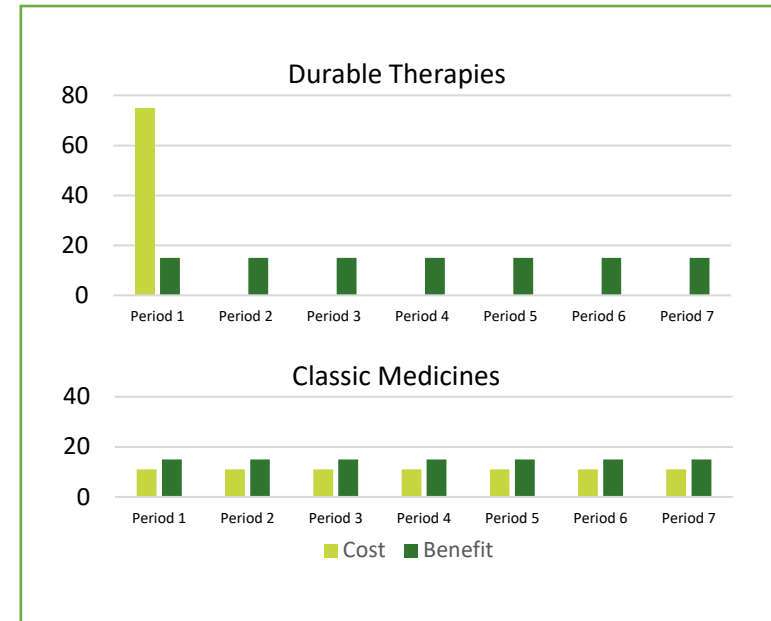


Emerging Durable Therapies Driving New Payment Models

50-75 Therapies Approved for the US Market by 2030



Durable Therapies Distill Payments Upfront



Three financial challenges exacerbated

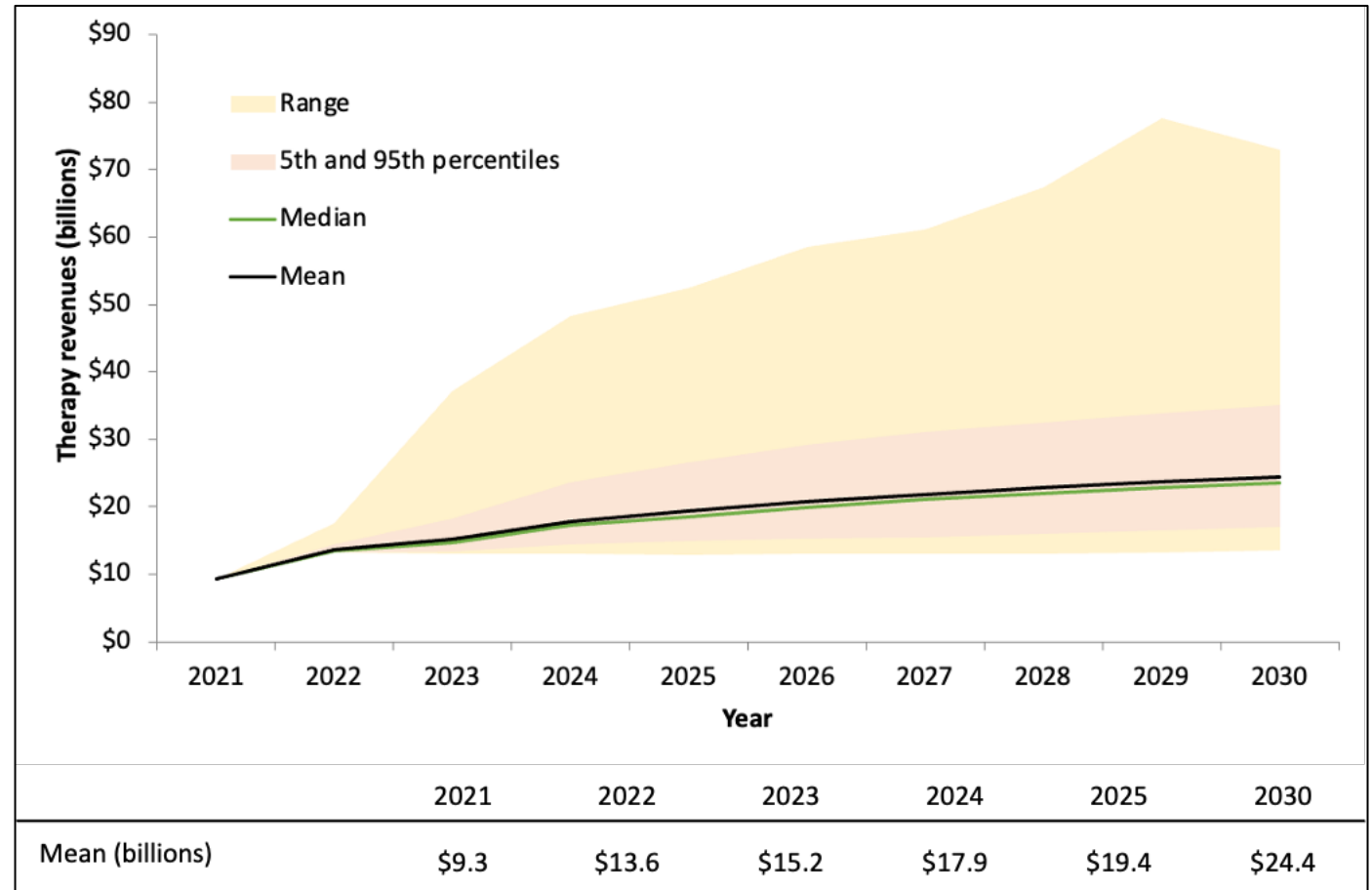
Payment timing
One-time high cost

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



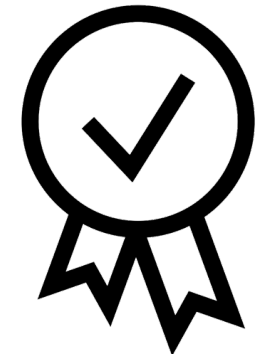
Orphan Reinsurer and
Benefit Manager (ORBM)
and Risk Pools



Short-term
milestone-based
contracts



Multi-year
performance-based
annuities



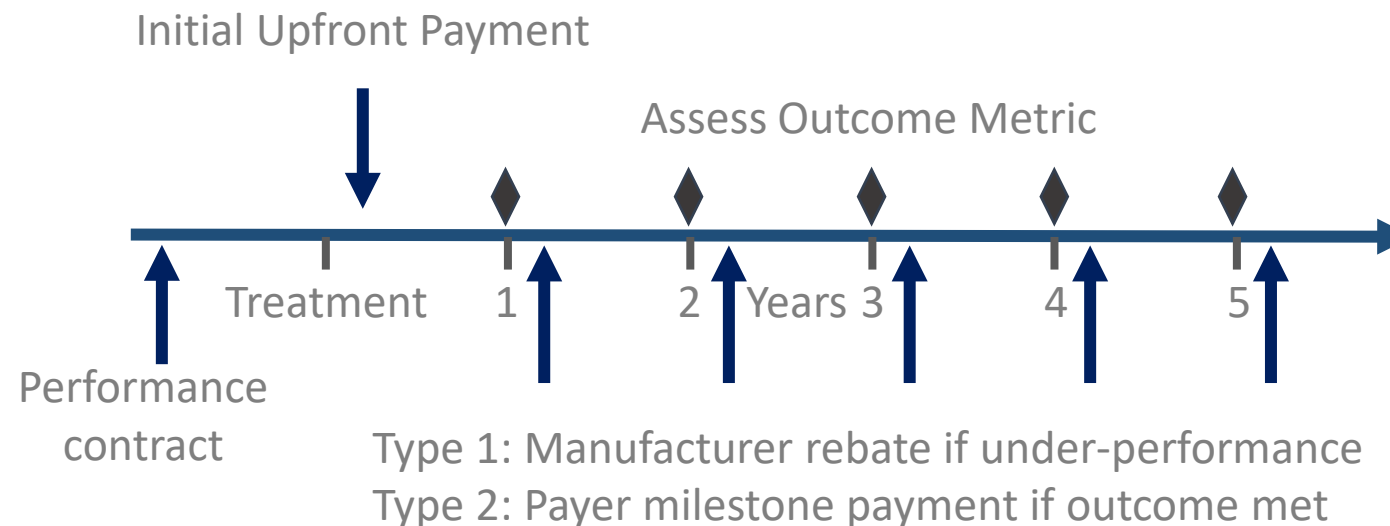
Warranty Model

NETFLIX

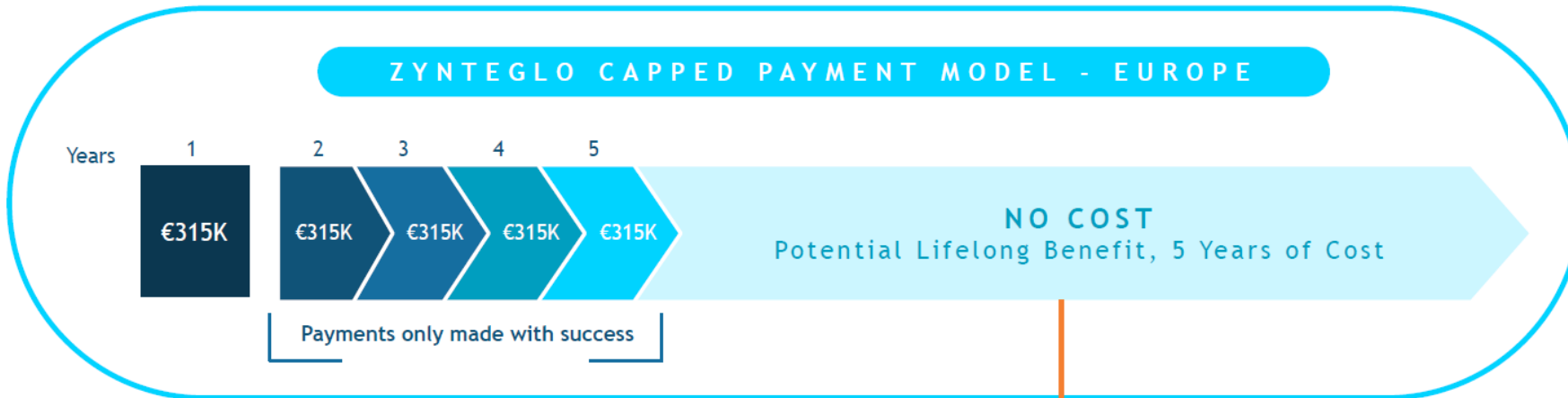
Subscription Model

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



- Spread payments over up to 5 years
- Get paid only if the treatment works

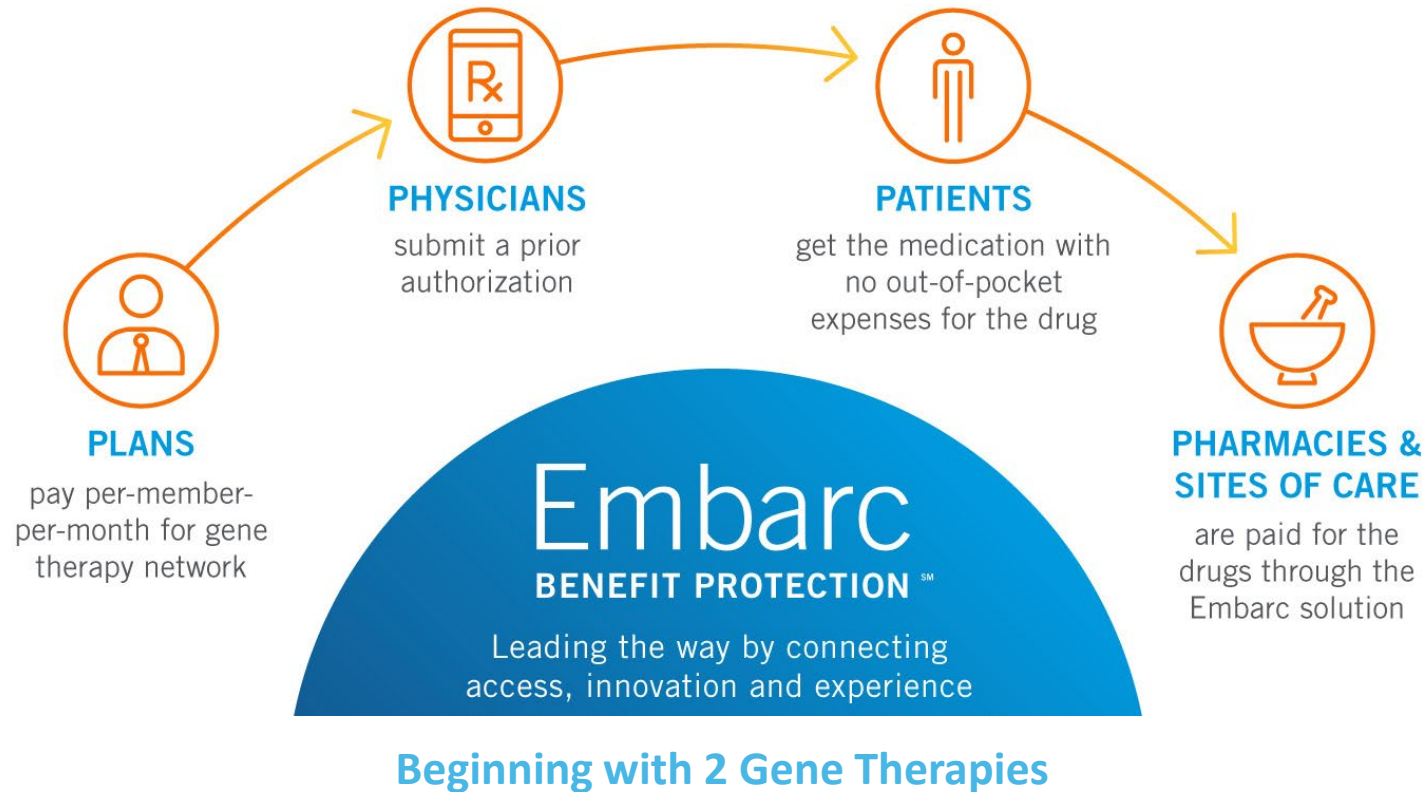
- ✓ First Year Payment: €315K*
- ✓ Five Year Total Payment With 100% Success: €1.575M

A one-time treatment expected to deliver lifelong benefit with 5 years of cost versus continual, lifelong treatment and cost

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



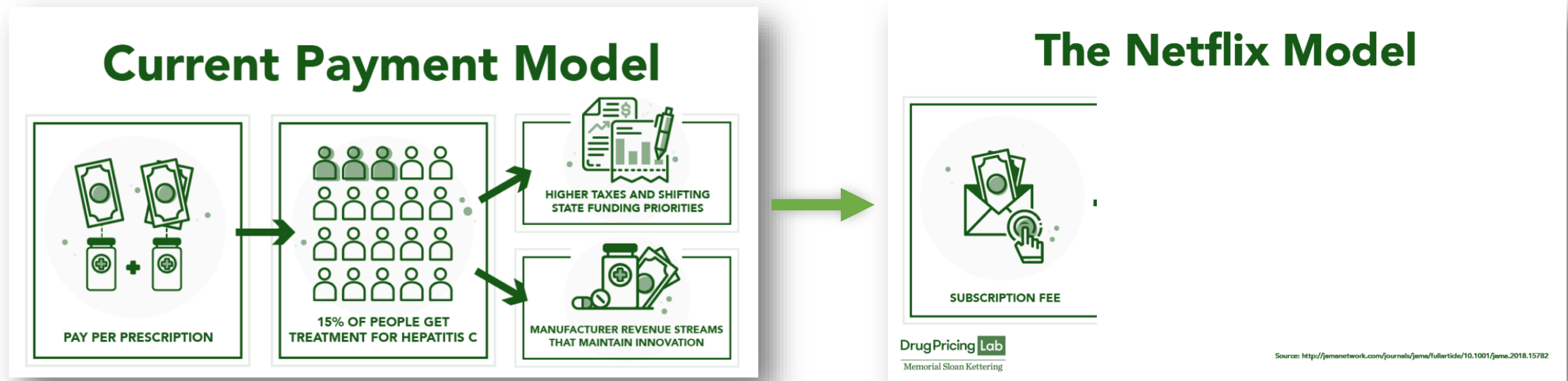
- Developer provides warranty policy
- Health plan holds warranty in the event that the therapy fails to deliver on promise




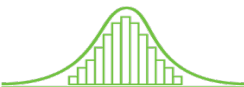





















- Warranty pays for supplemental factor needed to get the patient to proper circulating factor level
- Health plan submits claim to Outcome Based Contract Administrator (OBCA)
- Value delivered based on covered healthcare costs not a rebate

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 				Multiple Agreements 	
Multi-year performance annuities 				Pilot Stalled For Price Reporting Issue 	
ORBM and Risk Pools 				Proposed Private Sector 	
Subscription Model NETFLIX				State Demonstrations 	

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

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

Research Briefs and Peer-Reviewed Publications

ScienceDirect
Content lists available at sciencedirect.com
Journal homepage: www.elsevier.com/locate/jval

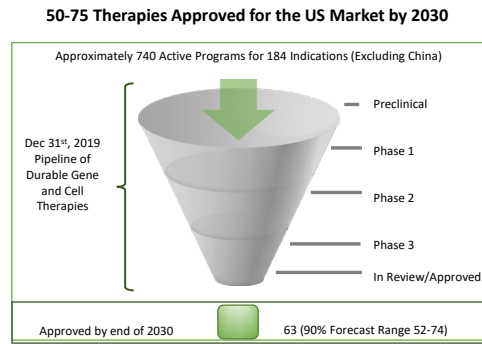
Themed Section: Curative Therapies

Are Payers Ready, Willing, and Able to Provide Access to New Durable Gene Therapies?
Jane F. Barlow, MD, MSP, MBA*, Mo Yang, PhD, J. Russell Teagarden, MA, DMI
Center for Intellectual Property, Massachusetts Institute of Technology, Cambridge, MA, USA

ABSTRACT
Objective: To explore reasons why payers are not providing access to new durable gene therapies.
Study Design: Qualitative study.
Methods: Sixty-nine payers were interviewed and analyzed using grounded theory.
Results: One-third of payers cited cost as the primary barrier to access. Other barriers included uncertainty about clinical outcomes, limited payer expertise, and limited payer engagement.
Conclusions: Access to new durable gene therapies is limited by payer-related barriers. Payers' perceptions of value and willingness to pay are key factors in determining payer performance.

ABSTRACT
Objective: To estimate, at the indication level, durable gene and cellular therapy new product launches in the United States through 2030, and the number of treated patients.
Methods: A statistical analysis of clinical trial pipeline data and disease incidence and prevalence was employed to estimate the impact of new durable gene and cellular therapies on the US market. The analysis used the National Cancer Institute's (NCI) Therapeutic Goods Database (TGD) 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 used to estimate the number of patients who will be treated by each product.

Unique Gene and Cell Therapy Pipeline Impact Modeling



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

Step 1: Select a Therapeutic class and Disease

Step 2: Ratio the total US population to your plan

Therapeutic class	Disease	Sub population	Total US Incidence Estimate #/UA	Total US Prevalence Estimate #/UA

Your Plan Size (# of members)

Plan-specific Total Incidence Estimate #/UA	Plan-specific Total Prevalence Estimate #/UA	US Population 328,000,000	Incidence Estimate per 100,000 #/UA	Prevalence Estimate per 100,000 #/UA

Clinically Relevant Population*

Incidence Modifier %	Prevalence Modifier %	Incidence Estimate per 100,000 #/UA	Prevalence Estimate per 100,000 #/UA

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:

Public Speaking Engagements

- 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 Faster Cures 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)

Educational Events

MIT CBI Paying for Cures Events Research and tools Media MIT

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

Learn Precision Financing solutions with innovation leaders and national policymakers at this widely-

Design Labs



NEW DIGS

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of Cures in the US

THANK YOU

July 26, 2021

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