

NEWDIGS

FoCUS

Financing and Reimbursement
of Cures in the US

Framing the Solutions

NEWDIGS Initiative • MIT Center
for Biomedical Innovation



Framing the Solutions



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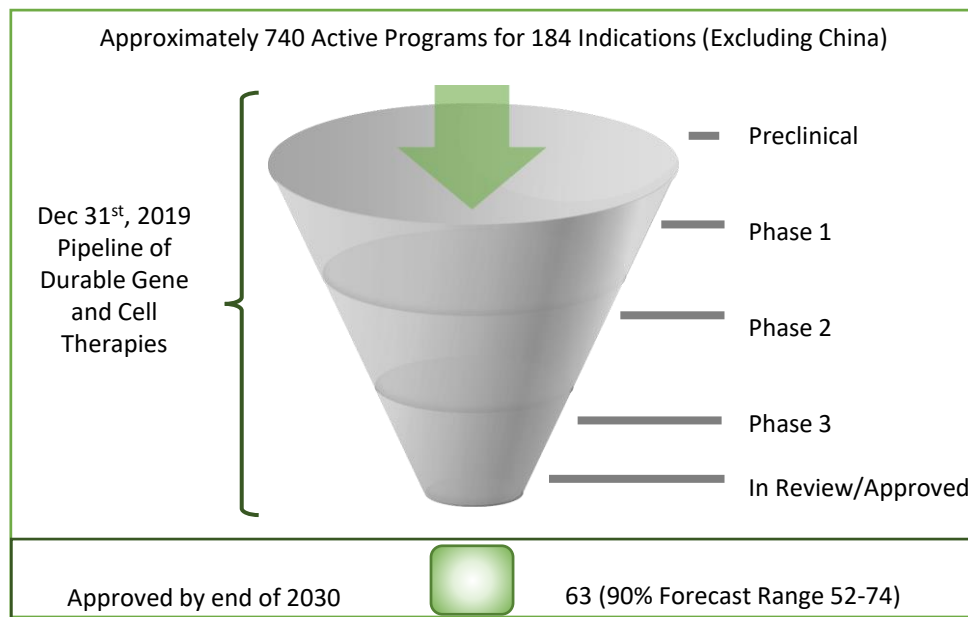
Massachusetts Institute of Technology

Outline

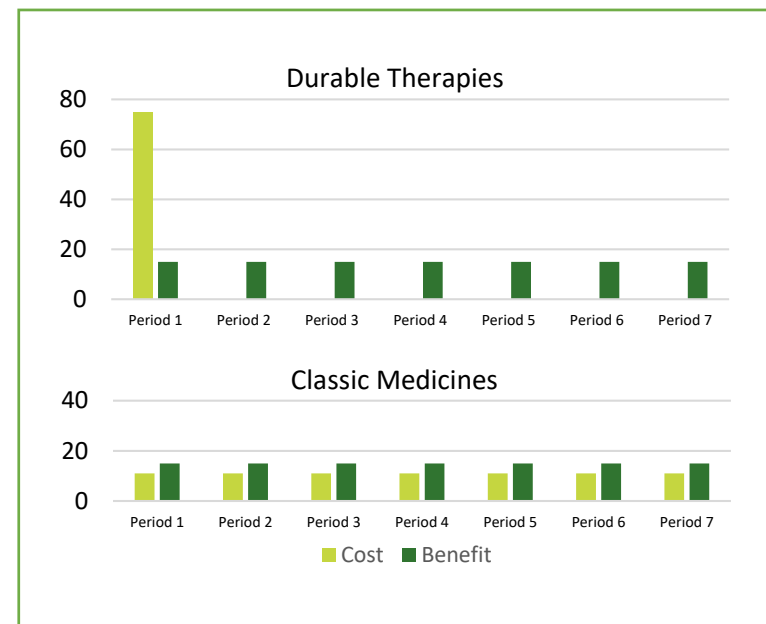
- What's coming (Pipeline)
- Possible approaches (Precision Financing Solutions)
- Overcoming barriers

Emerging Durable Therapies Driving New Payment Models

50-75 Therapies Approved for the US Market by 2030



Durable Therapies Distill Payments Upfront



Four financial challenges exacerbated



Payment timing
One-time high cost



Performance risk:
Effectiveness & durability

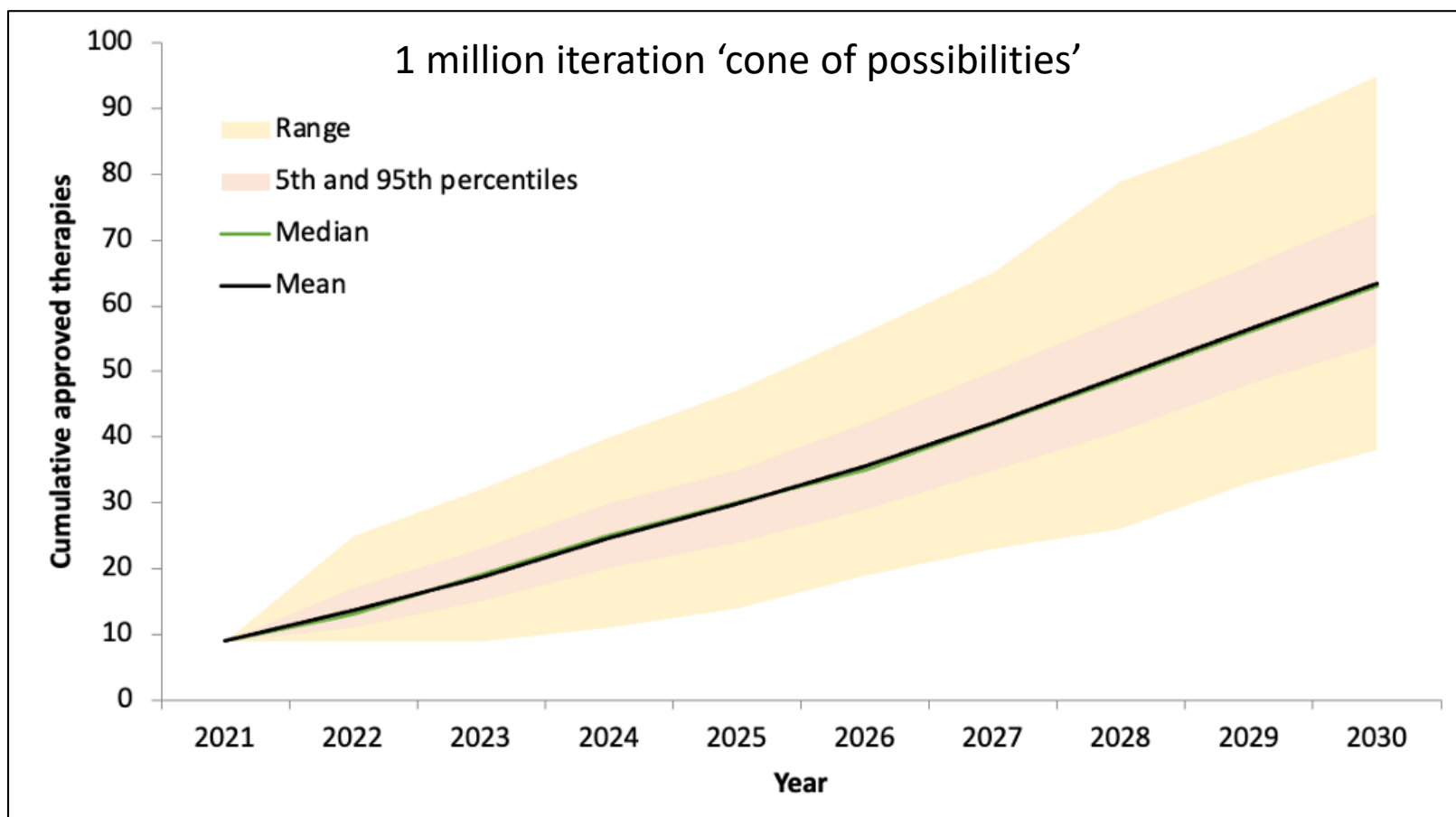


Actuarial risk:
Likelihood of encountering a case



Mobility:
patients moving to a new plan

About 19 FDA Approvals expected by 2023 Up from 7 today



Leukemias/Lymphomas: Already Here

- 5 CAR-Ts for blood cancers expressing CD 19 or BCMA (~45,000 eligible per year)
 - Acute Lymphoblastic Leukemia (ALL):
 - Diffuse Large B-Cell Lymphoma, or DLBCL
 - Large B-Cell Lymphoma (includes DLBCL)
 - Mantle Cell Lymphoma
 - Multiple Myeloma
- 2nd generation versions coming
 - Lower side effects
 - Allogeneic (common for everyone rather than individually made for each patient)
- Solid tumors likely multiple years away, with low/no success so far. But could change rapidly

Most Likely by 2023 ($\geq 50\%$ Likelihood)

With two mutations

no β -globin

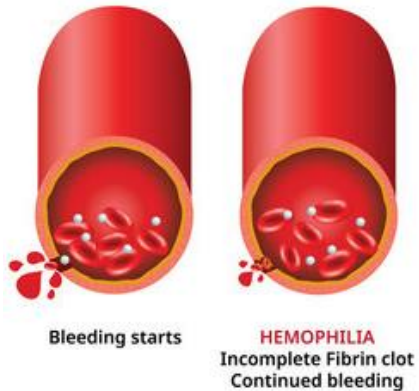


- Beta Thalassemia (3,500 eligible prevalence, 60 incidence)
 - Already approved in Europe
 - Filed with FDA

- CALD (Epidermolysis bullosa, dystrophic, recessive)

- Hemophilias A and B

- Very expensive patients today with Factor VIII and IX replacement therapy
- >21,000 US patients
- BUT only 3,000 initially expected to be eligible based on age, severity and molecular
- Potential value-based pricing suggested as high as \$4M per patient



Additional likely by 2025

- Leber's hereditary optic neuropathy
- Leukodystrophy, metachromatic
- Mucopolysaccharidosis, Type IIIA
- Retinitis pigmentosa, x-linked
- SCID, ADA

Larger Populations indications also likely within 5 years

- Wet AMD/diabetic retinopathy/diabetic macular degeneration (VEGF continuous generation)
- Sickle Cell Disease

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Possible Approaches



Precision Financing Solutions To Meet The Challenges



Orphan Reinsurer and
Benefit Manager (ORBM)
and Risk Pools



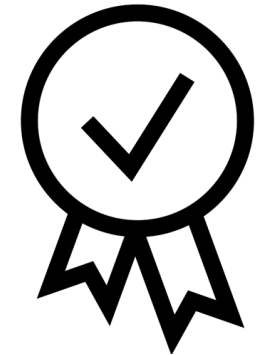
Short-term
milestone-based
contracts



Multi-year
performance-based
annuities

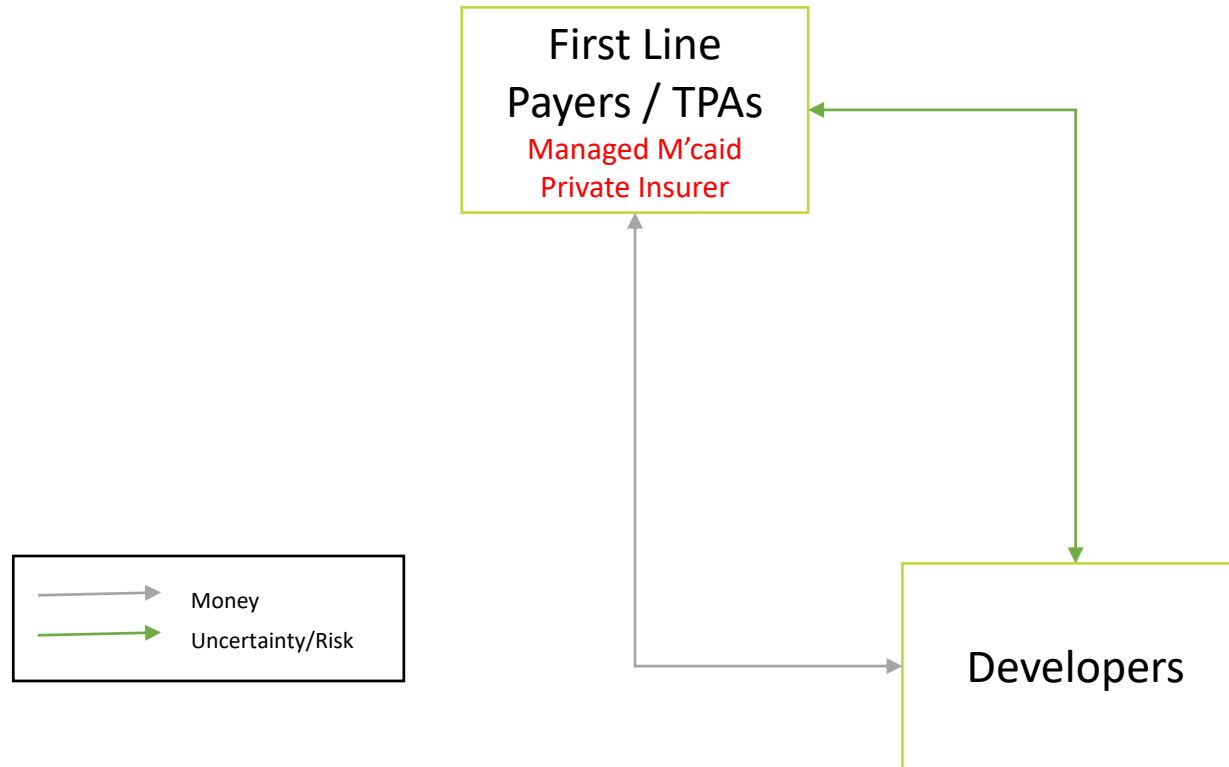
NETFLIX

Subscription Model

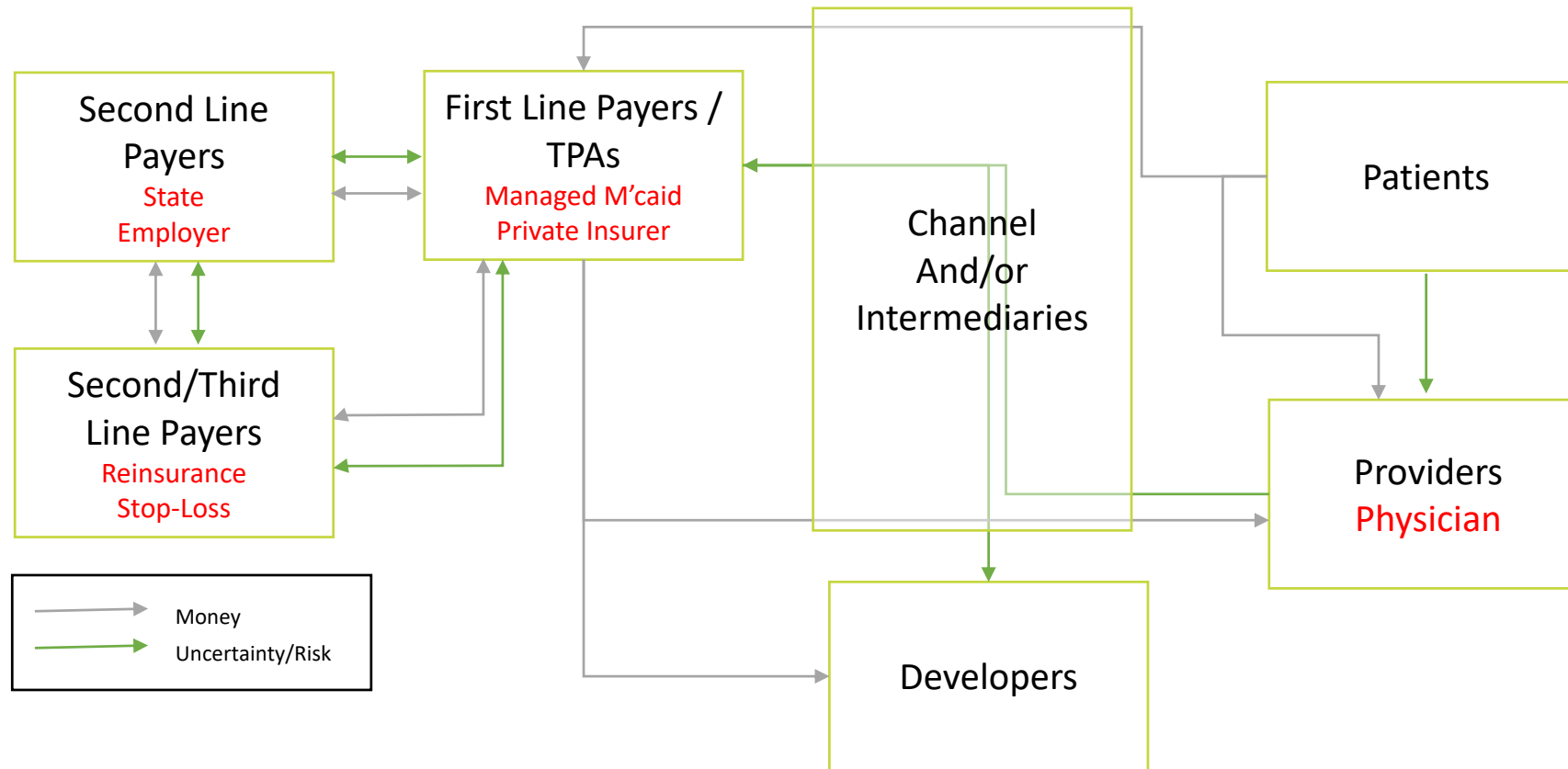


Warranty Model

An Initial View of a Two Party Transaction Challenge



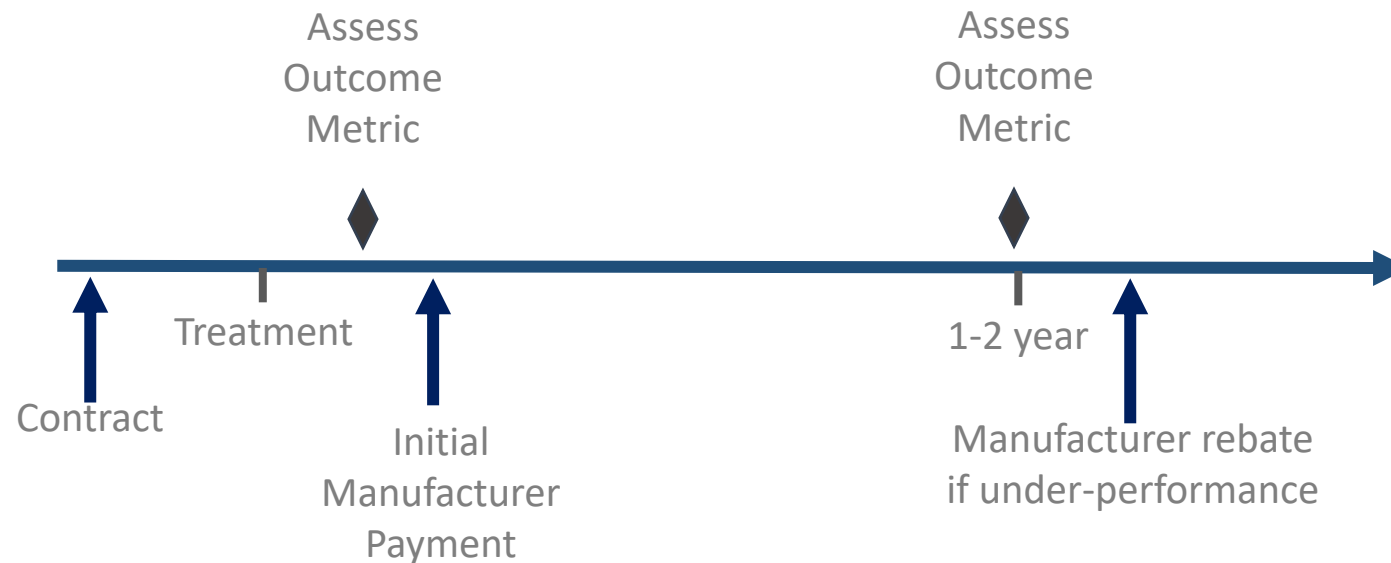
Grew Into a Financial Ecosystem Perspective



Milestone-based Contracts



- Trigger rebates to clinical milestones
- In 2016 Italy AIFA used for gene therapy for SCID provided at Vita Salute San Raffaele University in Milano



Performance-based Annuities Address the Three Challenges



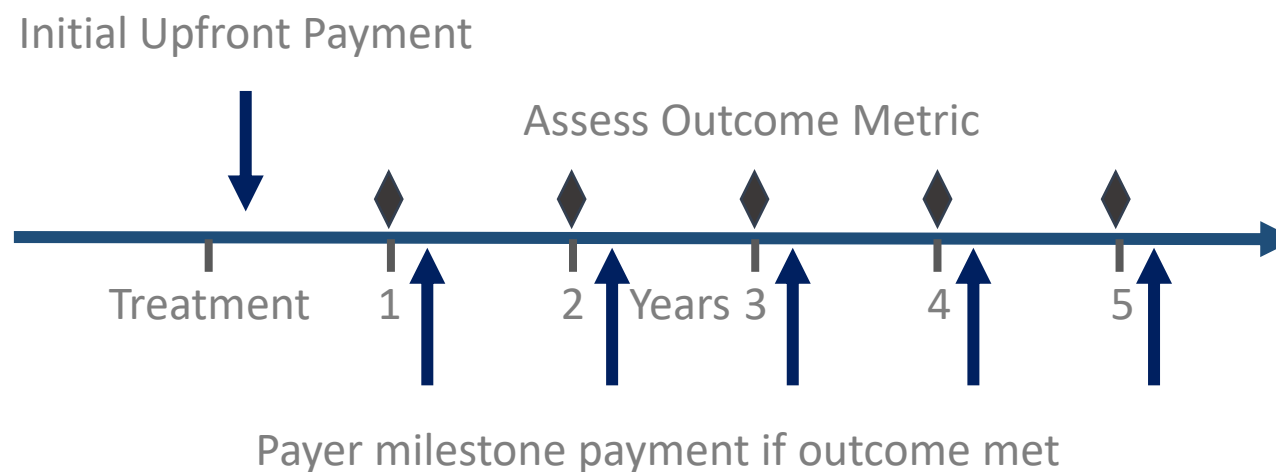
- **Payment Timing:** Match payments to cost avoidance



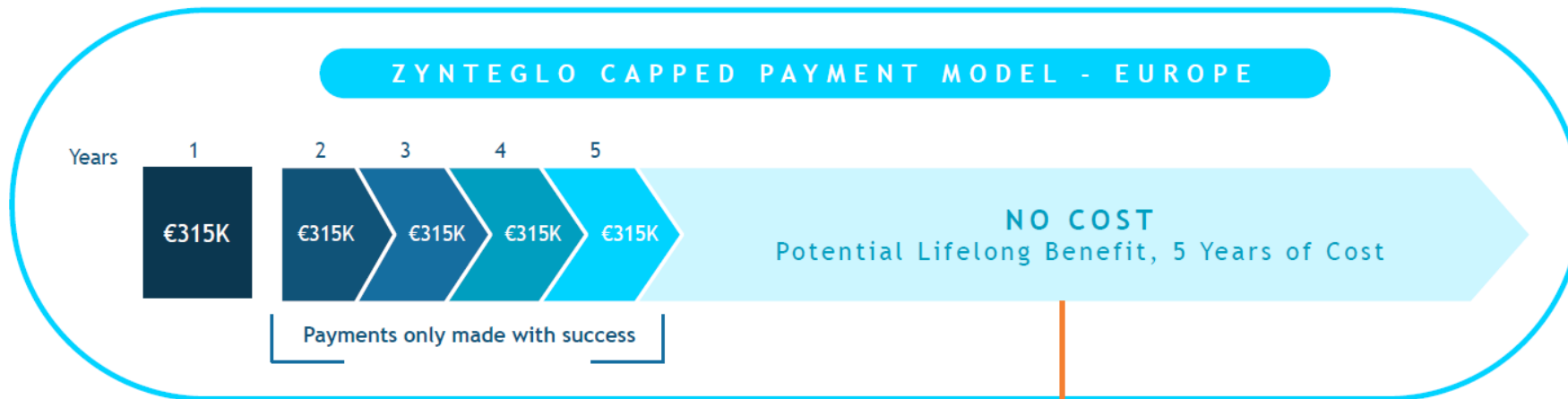
- **Performance Uncertainty:** Effectiveness & Durability



- **Actuarial Uncertainty:** 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

Warranty Model



- Health plan approves of and pays for therapy

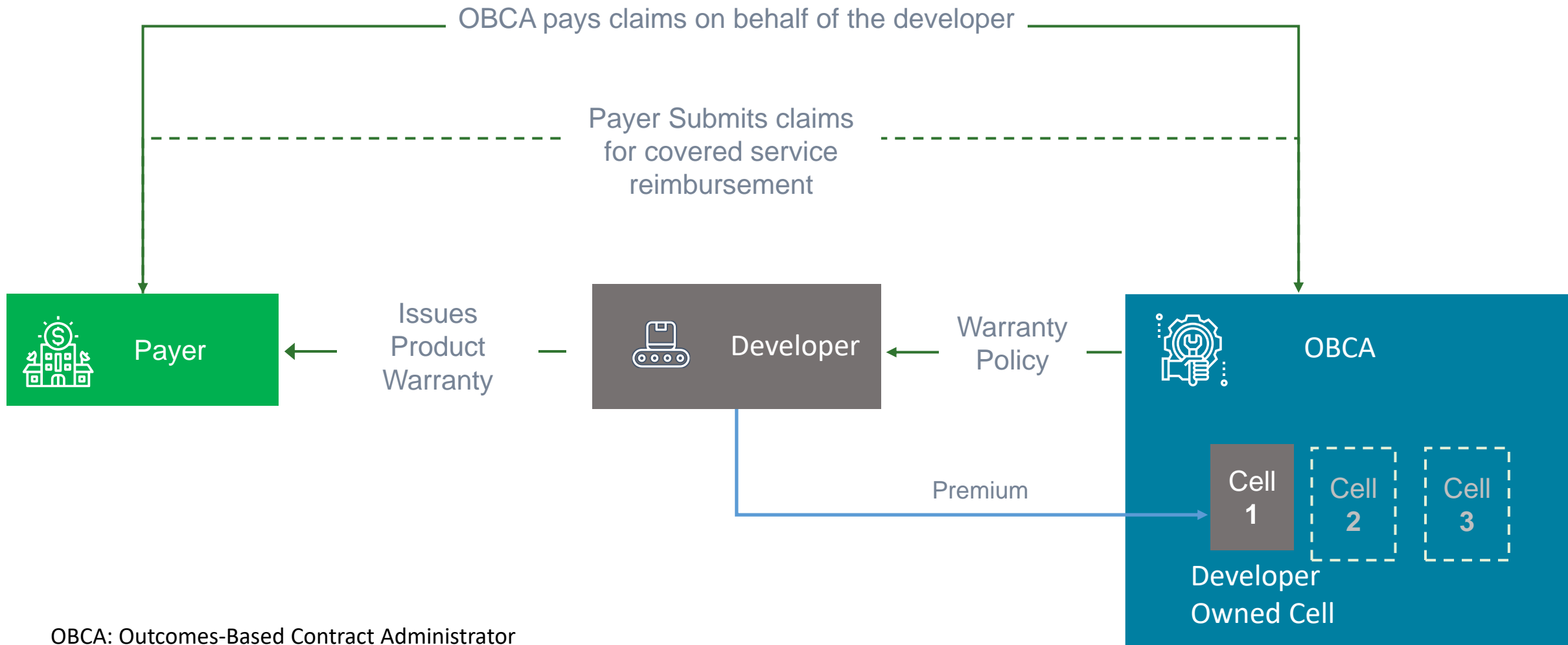


- Developer provides warranty policy
- Health plan holds warranty in the event that the therapy fails to deliver on promise



- 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

Operational Model



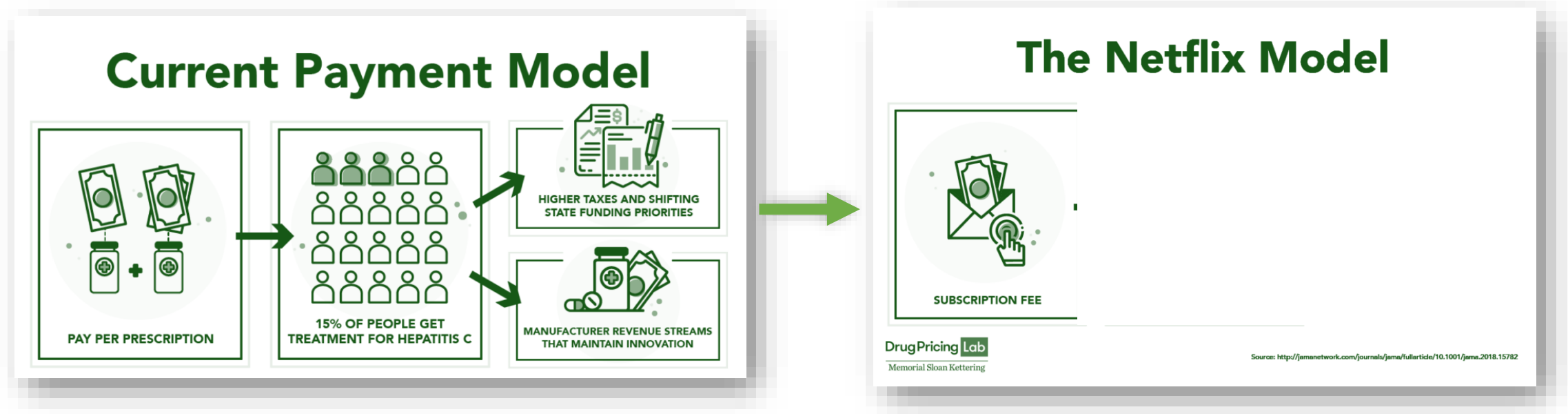
OBCA: Outcomes-Based Contract Administrator

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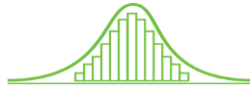

























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Netflix / Subscription Model

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



No Perfect Precision Financing Designs Yet Created

		Payment Timing	Performance	Actuary	US Status	
Short-term milestone contracts		 	 	 	 Multiple Agreements 	
Warranty Model					Agreements In Negotiation 	
Multi-year performance annuities					Pilot Stalled Medicaid Best Price 	
ORBM and Risk Pools					Proposed Private Sector 	
Subscription Model					State Demonstrations 	

Some Payers Not Positioned To Manage Gene Therapies



Small Commercial Payers
Self-Insured Employers
Medicaid
Medicare Advantage



Large Commercial Payers
Medicare FFS



Gene Therapy Impact

High

Low

Financing solutions of interest for future management of high cost durable therapies

Approach	Commercial Fully-Insured (n=55)	Medicaid (n=42)	Medicare Advantage (n=46)	Self-Insured Employer (n=10)	All Payer Segments (n=153)
Population risk-pooling	69%	40%	52%	50%	55%
Short-term MBC	75%	50%	61%	20%	60%
Long-term MBC	58%	36%	43%	30%	46%
Annuity: Spread payments over >2 years	33%	14%	24%	20%	24%
Performance-based annuity: spread payment over >2 years/tied to performance	47%	21%	35%	30%	35%

Milestone-Based Contract (MBC): Pay for therapy upfront and receive refunds tied to performance over the short-term (<2 years) or the long-term (≥ 2 years)

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Overcoming Barriers



Key Precision Financing Implementation Challenges

- **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**
 - **Anti-Kickback Statute to define explicit safe harbor**
 - **And more**

Precision Financing Data Tracking Needs



DATA TYPES



DATA QUALITY



EFFECTIVENESS METRIC:

USUALLY BILLING OR CLINICAL/EHR INFORMATION. IN THE FUTURE MAY INCLUDE PATIENT REPORTED OUTCOMES OR OTHER METRICS.

CLINICAL OUTCOMES, BIOMARKER OBJECTIVE OUTCOME, COST OFFSETS



PATIENT MOBILITY :

THE PATIENT MAY SWITCH PRIMARY PAYERS OVER TIME WHICH MAY TRIGGER TERMINATION OR TRANSFER CLAUSES.



RESPONSIBLE ENTITY :

META DATA DELINEATING WHO IS RESPONSIBLE FOR OBTAINING, SHARING AND ADJUDICATING DATA



FIT FOR PURPOSE DATA:

DATA MUST BE SUFFICIENTLY CREDIBLE, TIMELY, FEASIBLE, ACCEPTABLE, AND OPERABLE FOR ADJUDICATING PAYMENT DECISIONS.

Six Data Tracking Models Identified

Like our Precision Financing Solutions, we expect that more than one approach may work for a situation

1. Single system or identical multiple systems
2. Cloud-based dossier
3. Expanding existing product like CIBMTR or USRDS
4. Individual and separate disease registries
5. Individual and separate product databases
6. Patient's cloud-based health record

Emerging commercial solutions embed one or more of these models in their offerings

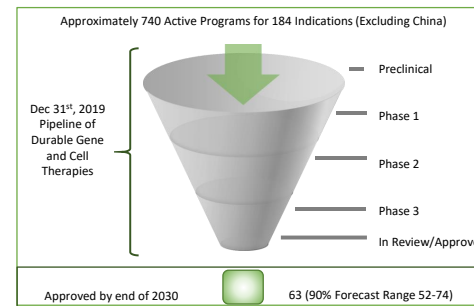
Go to <https://payingforcures.mit.edu>

Research Briefs and
Peer-Reviewed Publications

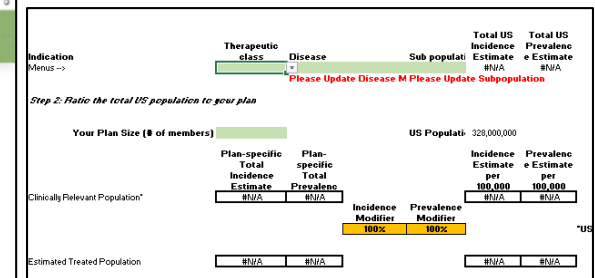
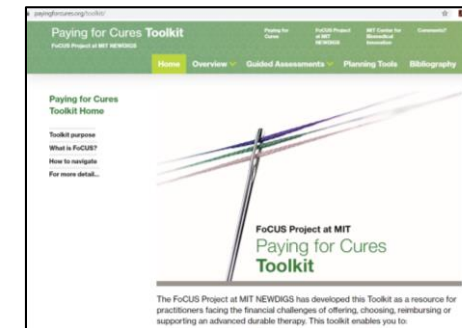


Unique Gene and Cell Therapy
Pipeline Impact Modeling

50-75 Therapies Approved for the US Market by 2030



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



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)

Educational Events



Design Labs

