Mark Trusheim

Strategic Director & Visiting Scientist, Sloan School of Management, Massachusetts Institute of Technology

Focus
Financing and Reimbursement of Cures in the US

Financing Solutions for Durable cures

28 October 2021
Mark Trusheim
Strategic Director, MIT NEWDIGS
Trusheim@MIT.edu

MIT CENTER FOR BIOMEDICAL INNOVATION
FoCUS: Sustainable Access for Durable Therapies via Downstream Innovation
>90 organizations & 350 individuals engaged
Focus of FoCUS: An MIT NEWDIGS Consortium

On—
Creating *precision financing solutions*

Not on—
Setting value or price
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
Patient Impact of Regenerative Medicine

40% of patients with R/R DLBCL treated with **CAR-T Therapy** experienced a complete response

60% of patients with R/R B-Cell ALL treated with **CAR-T Therapy** experienced a complete response

58% of patients with R/R B-Cell NHL treated with **CAR-T Therapy** experienced a complete response

55% of patients treated with **Gene Therapy** showed an improvement of 2+ light levels darker after treatment
Precision Financing Solutions To Meet The Challenges

- Short-term milestone-based contracts
- Multi-year performance-based annuities
- Orphan Reinsurer and Benefit Manager (ORBM) and Risk Pools
- Subscription Model
- Warranty Model

NETFLIX
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

![Diagram showing treatment and outcome assessment milestones]

1. Initial Upfront Payment
2. Assess Outcome Metric
3. Payer milestone payment if outcome met
Performance-Based Annuities Address More than Outcomes Uncertainty

The three financial challenges

- **Payment timing**
  - One-time high cost
  - Surges Smoothed

- **Performance risk:**
  - Effectiveness & durability
  - Pay for value actually received not *a priori* estimated value

- **Actuarial risk:**
  - Likelihood of receiving a case
  - Volatility Reduced
US Insurer Cigna Offering ORBM-Lite: Embarc

BEGINNING WITH 2 GENE THERAPIES

**Physicians**
submit a prior authorization

**Patients**
get the medication with no out-of-pocket expenses for the drug

**Plans**
pay per-member-per-month for gene therapy network

**Pharmacies & Sites of Care**
are paid for the drugs through the Embarc solution

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Source: http://lab.express-scripts.com/lab/insights/specialty-medications/delivering-on-the-promise-of-modern-medicine
All Approaches Must Address US Issues

• **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* need adapting to multi-year performance structures
  – 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
### No Perfect Precision Financing Designs Yet Created

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<tr>
<th>Short-term milestone contracts</th>
<th>Payment Timing</th>
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<th>US Status</th>
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FoCUS Open Resources

Go to: [https://payingforcures.MIT.edu](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**

- Preclinical
- Phase 1
- Phase 2
- Phase 3
- In Review/Approved

- Dec 31st, 2019
- Pipeline of Durable Gene and Cell Therapies
- Approved by end of 2030
- 64 (90% Forecast Range 52-74)

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Public Speaking Engagements

- **Jan 27**: Merck & Co. (West Orange, NJ), Truven Health
- **Feb 27**: Blue Cross Blue Shield Association, Aspen Institute (CO, Aspen, CO)
- **Mar 1** - **Mar 3**: Microsoft (Redmond, WA, Redmond, WA)
- **Mar 8-10**: CHOP (Philadelphia, PA), Children's Hospital
- **Mar 10-19**: Kennesaw State University (Kennesaw, GA, Kennesaw, GA)
- **Apr 7-9**: AstraZeneca (Cambridge, MA, Cambridge, MA)
- **Apr 16**: Boston Scientific (Marlborough, MA, Marlborough, MA)
- **May 1**: National Biotechnology and Biopharmaceuticals (Natick, MA, Natick, MA)
- **May 11**: Trinity Health (Chicago, IL, Chicago, IL)
- **May 16**: Janssen Biotech (Horsham, PA, Horsham, PA)

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Educational Events

- **March 16-17**: University of Pennsylvania (Philadelphia, PA), Philadelphia, PA
- **March 20-21**: Massachusetts Institute of Technology (Cambridge, MA), Cambridge, MA

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Design Labs

- **April 10**: MIT (Cambridge, MA, Cambridge, MA)

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Mark Trusheim
Strategic Director, MIT NEWDIGS
Trusheim@MIT.edu
MIT CENTER FOR BIOMEDICAL INNOVATION
Dino Sepulveda

Head of Department of HTA and Evidence-Based Medicine, Ministry of Health, Chile;

Executive Secretary, Ministerial Advisory Commission on Rare Diseases

• Risk-sharing agreements:
  • The experience of the 4th decree of the Ricarte Soto Law
• Content

• Introduction
• Taxonomy
• RSA in the evaluation process in 4th Decree of Ricarte Soto Law
• RSA in technologies prioritised in 4th Decree of Ricarte Soto Law
• Barriers to implementation
• Endpoints
• **Introduction**

• **What is a risk-sharing agreement (RSA)?**

• An agreement between a provider [pharmaceutical company] and a payer/provider that allows access (coverage and reimbursement) to a health technology under certain conditions.

• These contracts have the potential benefits of allowing early access to technologies for patients and reducing uncertainty about their use's effectiveness, cost-effectiveness, and financial impact under real-world conditions.

• They are also referred to as Entry Management Agreements, Patient Access Schemes, Evidence-Based Coverage, etc.
Taxonomy of risk-sharing agreements

Financial type
Population:
- Price discount
- Price associated with volume
- Price associated with participation
- Portfolio agreement
- Capping

By Individuals:
- Discount on start of treatment
- Limit of use per patient
- Fixed cost per patient

Clinical Outcomes
Assurance for results:
- Conditional continuation of treatment
- Price linked to results
- Guaranteed reimbursement

Coverage conditional on development of evidence
Patient eligibility + registration

Hybrids

Courtesy of Mr. Sergio Poblete, on the basis of Poblete (2020)
• Risk-sharing agreements - Evaluation process 4th LRS decree

• The number of drug intention-of-price letters received for the fourth LRS decree was 103 from 32 different suppliers, covering 62 drugs.
• 70 of the quotations (68%) considered price discounts or other types of RSA.
• 65 quotations were submitted with discounts.
• There were 31 RSA proposals (other than price discounts) (28%).

Courtesy of Mr. Sergio Poblete, on the basis of Poblete (2020)
Types of risk-sharing agreements
Evaluation process of the 4th LRS Decree

- Guaranteed reimbursement
- Start of treatment
- Utilisation limit
- Hybrid
- Spending limit
- Portfolio
- Bonus units

Courtesy of Mr. Sergio Poblete, on the basis of Poblete (2020)
Limitation of Risk-sharing agreements implementation

Public procurement law
Within the Public Procurement Act framework, certain agreements such as portfolio or volume-related price agreements cannot be implemented.

The inertia of the purchasing process
Implementing risk-sharing arrangements involves changing the basis on which medicines are traditionally procured.

Information systems
The public health system does not have global clinical registry systems to monitor the evolution of clinical outcomes.

Non-understanding of benefits
Most public sector actors see these initiatives as a way for the industry to hide prices. As a result, there is no appreciation of budgetary benefits or of paying for health effects in practice.

Purchasing logic
The purchasing entity (CENABAST) operates by centralising and aggregating demand. Risk-sharing agreements require modifying this modus operandi.

Lack of skills
International experience shows that risk-sharing arrangements are complex in terms of design and implementation, requiring specialised technical teams to carry them out.

Courtesy of Mr. Sergio Poblete, on the basis of Poblete (2020)
• **Key points**

- The experience of the 4th LRS decree is pioneering for the public health sector in terms of risk-sharing agreements.
- Laboratory proposals are mainly financial, conditioned by the feasibility of implementation and technologies with high budgetary uncertainty.
- International experience indicates that ARC requires a proactive role of the public funder/payer (selective).
- There is no "gold standard" risk-sharing agreement. It will depend on health technology and uncertainty.
- The main challenge is to move forward in the area of implementation.
- It requires the generation of competencies within the public health system that allow the formulation of this type of contract and the development of specific infrastructure (information systems) that will enable the monitoring and follow-up of this type of agreement.
• References


Ricarte Soto Law, Chile: [https://leyricartesoto.minsal.cl/#/articulos/informacion-por-decreto](https://leyricartesoto.minsal.cl/#/articulos/informacion-por-decreto)
Goffredo Freddi

Executive Director, Policy & Communications at MSD Italy

The Fund for Innovative Drugs: the Italian experience

Goffredo Freddi
Executive Director
Policy & Communication
MSD Italy

October 28th 2021
Overview

For more than a century, we have been inventing to solve some of the greatest challenges to people’s health and well-being around the world.

**Businesses**
Prescription medicines, vaccines, biologic therapies, animal health products

**2020 revenues**
$48 billion; 56% of sales come from outside the United States

**Headquarters**
Kenilworth, New Jersey, U.S.

**Merck & Co., Inc.**
This is our legal name and is listed on the New York stock exchange under the symbol “MRK”

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<th>16.7K</th>
<th>422M</th>
<th>$13.6B</th>
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<td>people employed in research and development</td>
<td>people reached through our major programs and partnerships</td>
<td>invested in R&amp;D in 2020</td>
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**Our giving**

$3.1B total philanthropy in 2019
How public pharmaceutical spending is currently funded in Italy?

The creation of the Funds for Innovative Drugs (2017)

Universal coverage mostly funded by general taxation

~€ 120 B National Healthcare Fund

7.65% Cap for pharma spending at hospital level

7.0% Cap for retail spending

• Chronic overshoot of the cap for pharma spending at hospital level

• Savings in the pharma spending at retail level (i.e., €1 B) not used to mitigate the overshoot in the other cap

• The overshoot of the budget for pharma spending at hospital level risks to limit patient access to innovative drugs

From 2017 onwards (on top of the two caps)

Molecules labelled as innovative by the Italian Drug Agency (36-month inclusion in the Funds)

Molecules included in the Funds are excluded from Companies’ payback + fast track inclusion in national/regional formularies

€ 500 M Fund for I/O Drugs

€ 500 M Fund for I/non O Drugs

To be labelled as innovative, the drugs must prove an important therapeutic need, therapeutic added value and quality of evidence
How does the Drug Agency evaluate whether a drug is innovative or not?

<table>
<thead>
<tr>
<th>1. Therapeutic need (maximum/Important)</th>
<th>FULL INNOVATION</th>
<th>CONDITIONAL INNOVATION</th>
<th>NO INNOVATION</th>
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<tr>
<td>- absence/limited therapeutic options</td>
<td>✅</td>
<td>✓</td>
<td>✗</td>
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<td>2. Therapeutic value added (maximum/Important)</td>
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<td>✓</td>
<td>✗</td>
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<tr>
<td>- efficacy demonstrated on clinically relevant outcomes</td>
<td>✅</td>
<td>✓</td>
<td>✗</td>
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<tr>
<td>3. Quality of evidence (High/Medium)</td>
<td>✅</td>
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<td>✗</td>
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<td>- trials and mature OS data as major drivers</td>
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<td></td>
<td>FULL INNOVATION</td>
<td>CONDITIONAL INNOVATION</td>
<td>NO INNOVATION</td>
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<tr>
<td></td>
<td>• No Payback</td>
<td>• Fast access to hospital formularies</td>
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Two major changes from the creation of the Funds:

- 3-year inclusion in the Fund granted to therapeutic indications rather than to the single molecule
- The Funds, originally foreseen for the 2017-2019 period, are now structural
Did the Funds for Innovative Drugs prove to be an effective measure?

- The evaluation on the effectiveness of the Funds for Innovative Drugs must meet two primary endpoints:
  - The rate of availability, measured by the number of medicines available to Italian patients, and the time to availability, measured by the days between EMA MA and the date of availability to Italian patients.
  - The overall affordability of the Italian Healthcare System.
Focus on Innovative Oncology Drugs

IQVIA “Patients W.A.I.T. Indicator 2020 Survey” (April 2021)

The rate of availability, measured by the number of medicines available to patients in European countries as of 2020. For most countries this is the point at which the product gains access to the reimbursement list.

In the 2016-2019 period, Italian patients had access to 33 innovative oncology drugs vs. a EU average of 24 drugs.
The *time to availability* (previously known as length of delay) is the days between EMA marketing authorisation and the date of availability to patients in European countries.

**Italy**: 373 days  
**EU Average**: 518 days
Did the Funds contribute to the affordability of the Italian NHS?


- According to the findings of the study, Italy is among the countries with the highest increase of the spending on oncology drugs (direct costs) in the 2008-2018 period.
Did the Funds contribute to the affordability of the Italian NHS?


At the same time, Italy is the only country which, while increasing the spending on oncology drugs, decreased the overall healthcare spending for oncology patients in the 2008-2018 period, thus contributing to the overall affordability of the Italian NHS.
**Latest changes in the Funds for Innovative Drugs**

**Non binding Parliament opinion** – backed by the Italian Ministry of Economy – asking the Government to *increase the financial allocation for the Fund for Innovative Drugs*

**July 2021**

**Italy to increase resources for innovative drugs**

APM - *Italy is planning to increase resources for drugs recognised as innovative in a €6 billion boost to national health funding over the next three years.*

Ministers approved the 'Draft Budgetary Document for 2022'. On healthcare, it was announced that, compared with 2021, the National Health Fund will be increased by €2 billion each year until 2024. *New resources will be allocated to the fund for innovative drugs and for spending on vaccines and drugs to contain the Covid-19 pandemic*

**October 2021**
Thank You!
Questions?