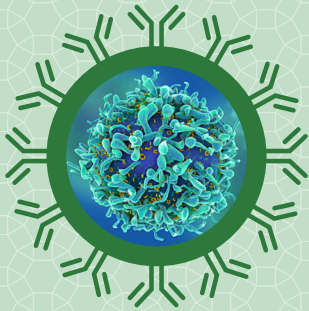


NEW DIGS

# FoCUS

Financing and Reimbursement  
of Cures in the US



## Paying for Cures: Ensuring patient access and system sustainability

National Press Club • Washington, DC  
*February 12, 2019*



**FasterCures**  
A CENTER OF THE MILKEN INSTITUTE



*Hosted by*  
MIT Center for Biomedical Innovation  
NEWDIGS Initiative

# Agenda

**Conference Facilitator:** Mark Trusheim, MSc, Strategic Director, NEWDIGS

## 9:30 Welcome

**David Goldston**, Director, MIT Washington D.C. Office

**Krystyn Van Vliet, PhD**, Associate Provost and Professor of Materials Science & Engineering & Biological Engineering, MIT

**Gigi Hirsch, MD**, Executive Director, Center for Biomedical Innovation and Executive Director, NEWDIGS

## Part 1: Understanding curative and durable therapies

### 9:40 Why Are We Here? The Science, Patients and System Readiness for Curative Therapies

This panel will bring together the science, patient and systems perspectives on the advent of durable/curative therapies. Where can the science go from here? How does it impact the hopes, dreams and daily lives of patients and what is the state of operational readiness for the system to deal with this paradigm shift in healthcare.

#### Moderator

**Janet Lynch Lambert, MBA**  
CEO, Alliance for Regenerative Medicine (ARM)

#### Panelists

*Science*  
**Usman "Oz" Azam, MD**  
President & CEO, Tmunity Therapeutics

#### Patients

**Marc Boutin, JD**  
CEO, National Health Council

#### Anish Goel, PhD

Beta-Thalassemia patient

#### System

**Murray Ross, PhD**  
Vice President, Kaiser Permanente

### 10:20 Cell & Gene Therapies Drug Development Pipeline

**Colin M. Young, PhD**, Director, Drug Development Pipeline Research, MIT FoCUS Project

## Part 2: Challenges to Accessibility

### 10:55 Financial Challenges Imposed by Curative Therapies: Real or Imagined

Panelists will react to the previous panel session on the Science, Patient and Systems dynamics as they relate to the advent of cell and gene therapy rollouts and what they meant to the future of healthcare, as well as predications of the drug development pipeline. Panelists will discuss the many challenges beyond price, presented by the durable/curative therapies from each stakeholder perspective.

#### Moderator

**Steve Usdin**,  
Senior Editor (Washington),  
Head Policy & Regulation,  
BioCentury and BioCentury  
Innovations

#### Panelists

*Provider*  
**Joseph Alvarnas, MD**  
Vice President, Government  
Affairs & Senior Medical  
Director, Employer Strategy,  
City of Hope

#### Patient Advocacy

**Tim Brent**  
Senior Director, Business  
Development, National  
Hemophilia Foundation

#### Self-Insured Employer

**Robert Galvin, MD**  
CEO, Equity Healthcare  
(former Executive Director,  
Health Services & Chief  
Medical Officer, General  
Electric)

#### Public Payer

**Darin Gordon**  
Founding Partner, Speire  
Healthcare Strategies,  
(former Director, Tennessee  
Medicaid program,  
TennCare)

#### National Payer

**Patricia Martin, RN, BSN**  
Director, Specialty  
Network Development  
Anthem, Inc.

#### Government/Regulatory

**John O'Brien, PharmD, MPH (invited)**  
Senior Advisor for Drug  
Pricing Reform, Health  
and Human Services

#### THANK YOU to the Paying for Cures Organizing Committee and management team:

Mike Ciarametaro (NPC), Ester Krofah (FasterCures), Francesca Cook (RegenXBio), Danielle Rollman, Gary Surmay (Pfizer), Rob Falb (ARM), Virginia Sweeter, Katie Torrence, Eric Small, Matt Courtney, Eric Norman, Mark Trusheim, and Deborah Young.

**12:10 Lunch****12:35 Keynote: Senator Bill Cassidy, MD, U.S. Senator for Louisiana**

How medicine is changing, what it means to improved health and why there is a need to create alternative financing mechanisms.

**Introduction Jonathan Gruber, PhD**, Ford Professor of Economics, MIT and Director, Health Care Program, National Bureau of Economic Research

**1:00 The FoCUS Project: Precision Financing Solutions**

Go behind the scenes with this panel on the creation of financing and reimbursement solutions to the myriad of challenges brought by the upfront cost of the durable/curative therapies. Stakeholder representatives will highlight and review the case studies utilized in the development of precision financing ideas.

**Moderator**

**Mark Trusheim, MSc**  
Strategic Director,  
NEWDIGS

**Panelists**

*FoCUS Project*

*Development & Goals*

**Edmund Pezalla, MD, MPH**

CEO Enlightenment  
Bio-consulting (former Vice  
President, National Medical  
Director, Pharmaceutical  
Policy and Strategy, Aetna)

*Blood Disorders Case*

*study / Performance-based  
Annuities*

**Chuck Bucklar**

Group Vice President,  
Commercial Operations,  
North America, BioMarin  
Pharmaceutical Inc.

*CAR-T Case Study /*

*Milestone-based Rebates*

**Krishna Komanduri, MD**

Kalish Family Chair in  
Stem Cell Transplantation;  
Professor of Medicine and  
Associate Director, Clinical  
Innovation, Sylvester Com-  
prehensive Cancer Center,  
University of Miami Miller  
School of Medicine

*Ultra-Orphans Case*

*Study / Orphan Reinsurer*

*Benefit Manager*

**Michael Ciarametaro,  
MBA**

Vice President Research,  
National Pharmaceutical  
Council

**1:50 Break****Part 3: Clearing The Way for Cures****2:00 Putting Theory into Practice**

This panel will describe the pilots FoCUS is designing, including plans for a Massachusetts pilot on performance-based annuities with commercial payers, and milestone-based contracts with public payers.

**Moderator**

**Rena Conti, PhD**

Associate Professor,  
Markets, Public Policy, and  
Law; Associate Research  
Director, Biopharma and  
Public Policy, Institute for  
Health System Innovation  
and Policy, Boston Univer-  
sity Questrom School of  
Business

**Panelists**

*Performance-based*

*Annuities with*

*Commercial Payers*

**Lovena Chaput**

Vice President, Managed  
Markets & Reimbursement,  
AveXis

**Michael Sherman, MD,  
MBA, MS**

Chief Medical Officer and  
Senior Vice President,  
Harvard Pilgrim Health Care

*Milestone-based Contracts  
with Public Payers*

**John Glasspool**

Board Member, DalCor  
Pharmaceuticals, Senior  
Advisor, MIT FoCUS Project

**Jeff Myers**

EVP Strategy and Exter-  
nal Relations, CareSource  
(former president and CEO  
of Medicaid Health Plans of  
America (MHPA)

*Continued...*

## 2:35 Identifying Opportunities for Enabling Precision Financing

All precision financing solutions, including those proposed by the FoCUS Consortium, will require improvements to existing financial systems. This panel will explore what those challenges are, how they might be overcome and what you can do to aid in this endeavor.

### Moderator

**Gregory Daniel, PhD, MPH**  
Deputy Director, Policy,  
Duke – Robert J. Margolis  
MD, Center for Health Policy  
& Clinical Professor, Fuqua  
School of Business

### Panelists

*Private Payer, PBM  
and Medicare*  
**Kim Caldwell, RPh**  
Principal, Texas Star  
Healthcare Consulting, LLC  
(former Division Director,  
Medicare Drug Benefit  
Group, CMS; VP of Phar-  
macy Professional Affairs  
& Comprehensive Health,  
Humana Inc.)

### Regulatory

**Brian Carey**  
Partner, Foley-Hoag LLP

### Stop-Loss/Reinsurance

**Mehb Khoja, FSA, MAAA**  
President, Medical Risk  
Managers Inc. (MRM)

### Patient Advocacy

**Peter Saltonstall**  
President and CEO,  
National Organization for  
Rare Disorders (NORD)

## 3:25 Keynote: Senator Sheldon Whitehouse, JD, U.S. Senator for Rhode Island

The current climate on Capitol Hill around payment reform and opportunities for innovation in pricing arrangements.

Introduction **Mark Trusheim, MSc**, Strategic Director, NEWDIGS

## 3:45 Wrap Up – Audience Engagement

**Mark Trusheim, MSc**, Strategic Director, NEWDIGS

# Paying for cures

## Challenges, solutions, and what you can do



### The growing need

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

### The challenges

- Policy, regulations, and business operations need to evolve to enable emerging solutions.
- Payers face specific and complex challenges for each of four “Cure Archetypes.”

### Precision Financing Strategies

- Milestone-based Contracts
- Performance-based Annuities
- Orphan Reinsurer and Benefit Managers

### What you can do

**21st century medicine needs a 21st century health-care financing system. To see what you can do to help, go to [payingforcures.org](http://payingforcures.org)**