

NEW DIGS

FoCUS

Financing and Reimbursement
of Cures in the US

Paying for Cures: Self-Insured Employers Workshop

June 17, 2021

Framing the Issues



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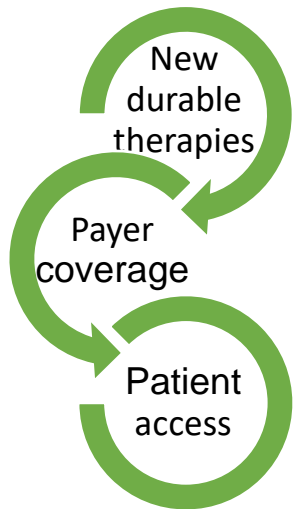
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Real Endpoints

MIT FoCUS: Multi-stakeholder “think and do tank” dedicated to making innovative cures accessible and sustainable

Opportunity

- Growing clinical pipeline on innovative durable therapies
- High one-time cost will likely create financial challenges
- Sustainability for all stakeholders is at risk



➤ **Need** to address stakeholder concerns, flesh out hurdles and foster new tools

>90 organizations,
>350 individuals engaged



Patients



Payers



Hospitals



Developers

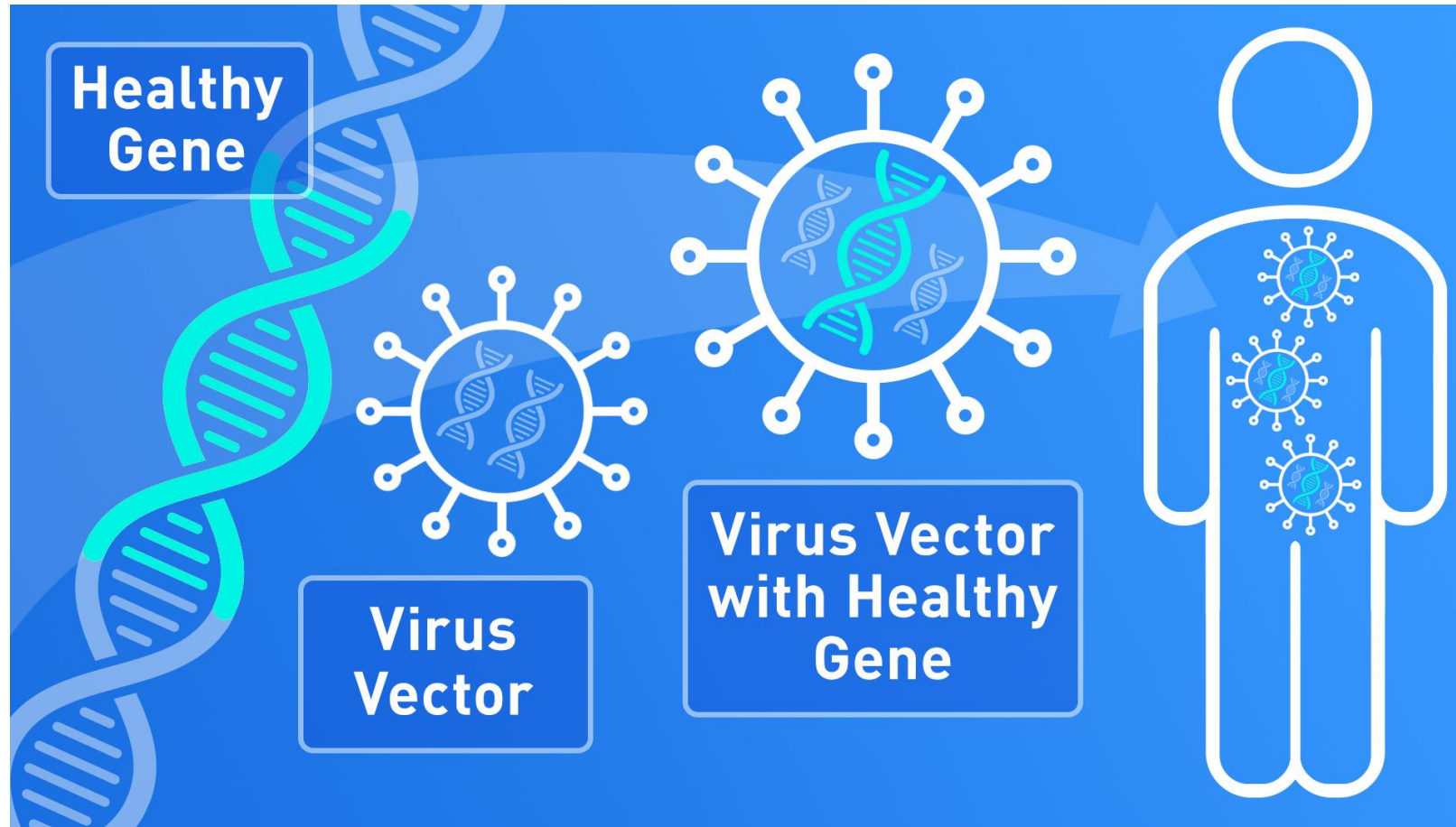


Academics



Policymakers

What is gene therapy?



- Replace a gene that causes a medical problem with one that doesn't
- Add genes to help the body to fight or treat disease
- Turn off genes that are causing problems

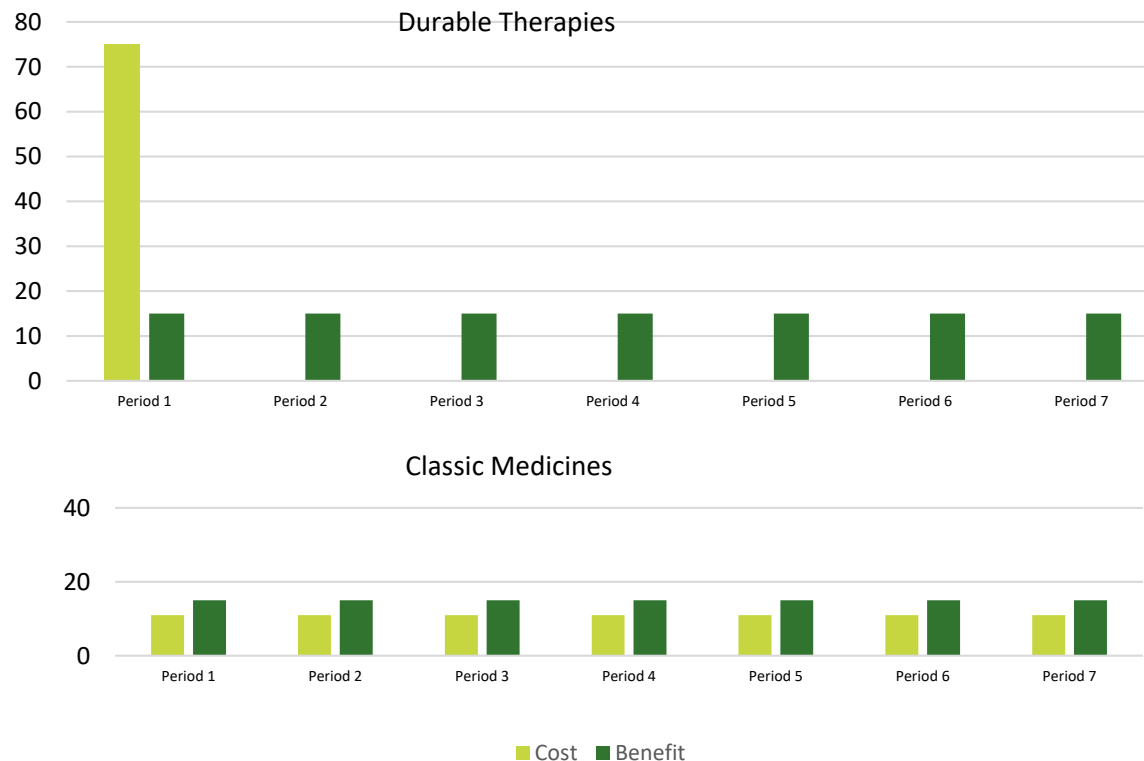
FDA: What Is Gene Therapy? How Does It Work? <https://www.fda.gov/consumers/consumer-updates/what-gene-therapy-how-does-it-work>

Multiple treatments have been approved since 2017



Durable Therapies Create Financial Challenges

Durable Therapies Distill Payments Upfront



- 1. Payment timing risk:** Cost is front-loaded, but benefits accrue over time
- 2. Actuarial risk:** Likelihood of encountering a case
- 3. Product performance risk:** Effectiveness and durability
- 4. Mobility:** Challenges as patients move in and out of plans

Methodology

- 15-question online survey
 - Launched 9/20/2018 - concluded 4/4/19
 - Email invitation sent to over 400 clinical and financial health plan leaders
 - Payer segments included commercial fully-insured, self-insured employers, Medicare advantage and Medicaid plans
- **Goal:** Assess payer perspectives regarding current and future management of high cost durable one-time treatments

MIT FoCUS Project Whitepaper 2019F206v039: Payer Survey
https://newdigs.mit.edu/sites/default/files/FoCUS%20Research%20Brief_2019F206v039.pdf

77 participants representing 153 payer segments completed the survey

Participation by Segment and Covered Lives

	Commercial Fully-Insured	Medicaid	Medicare Advantage	Self-Insured Employer	Total/Range
<i>Payer segments n; (percent of participants)</i>	55 (36%)	42 (27%)	46 (30%)	10 (7%)	n=153
<i>Range of covered lives (000's)</i>	10 – 54,000	10 – 13,200	5 – 8,000	3 – 400	3 – 54,000
<i>Lives Covered</i>	184,335,447	59,379,661	36,508,957	933,854	281,157,919

Commercial payers report the highest degree of concern; self-insured employers report less concern overall

Level of concern regarding managing the financial risk and impact of high cost durable therapies, by payer segment (n=153)

