Affording Hope: Paying for Multimillion-Dollar Cell and Gene Therapies

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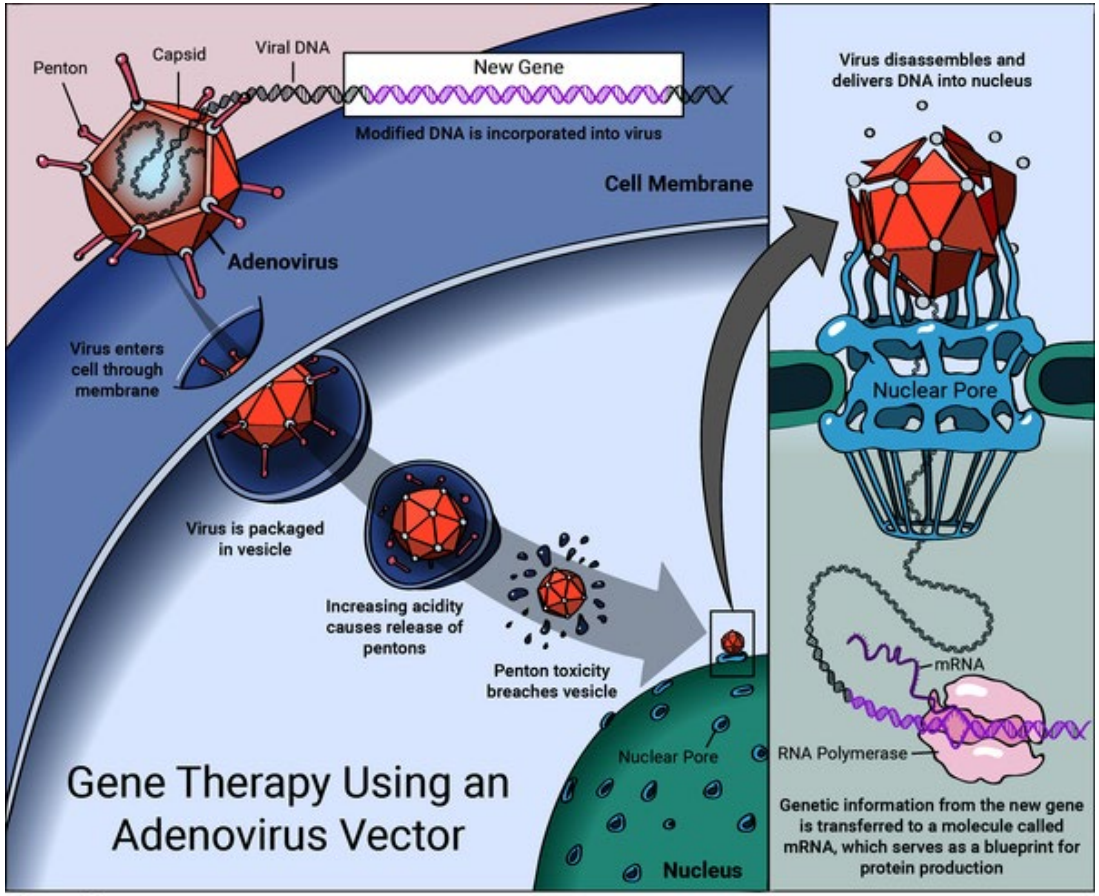
Agenda

- What are Cell & Gene Therapies?
- Why are they important?
- Currently available and pipeline therapies
- The role of the Actuary in assessing the risk
- Payer concerns and potential solutions

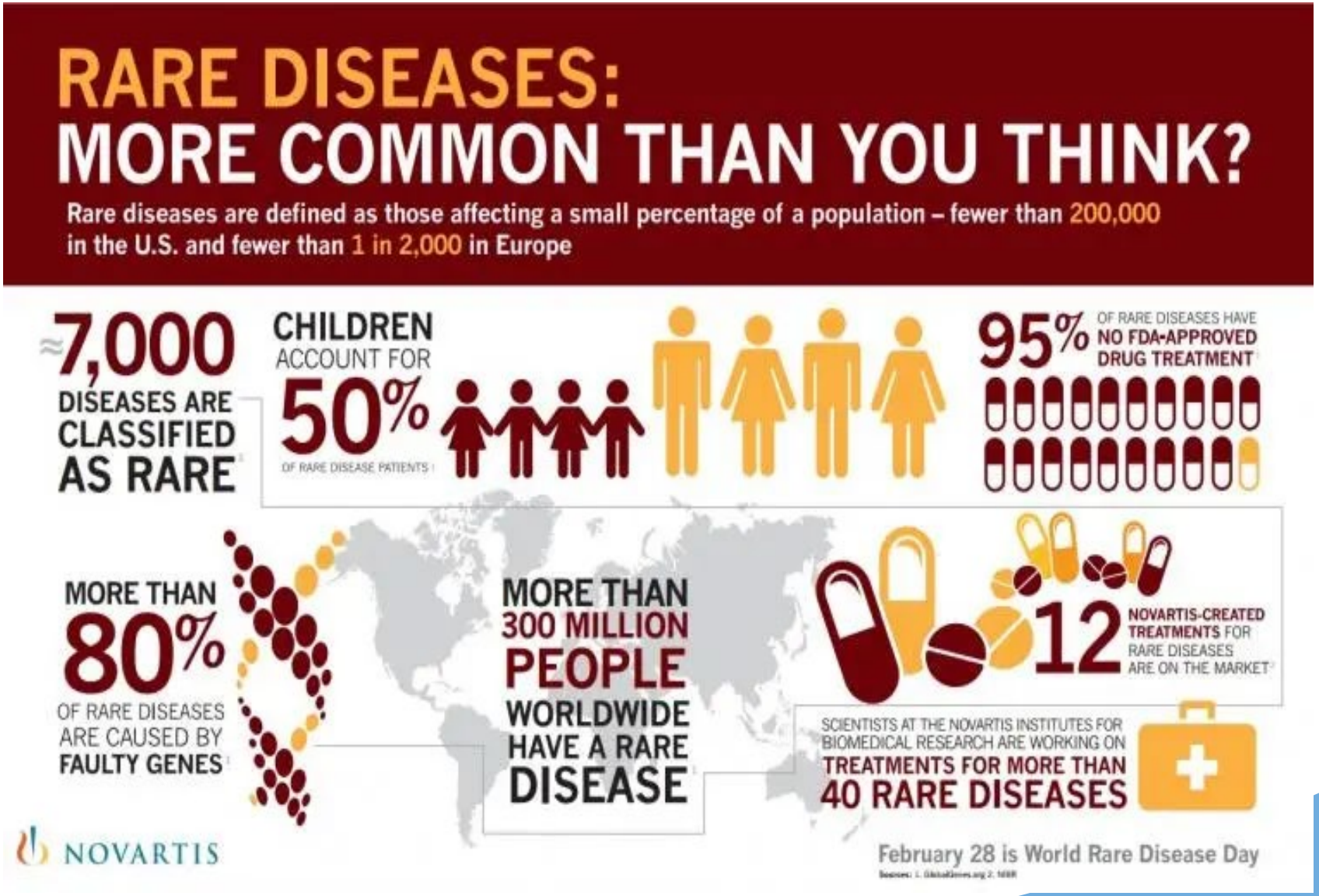
What are Cell & Gene Therapies?



- **Gene therapy** involves altering one's genes in effort to treat or stop disease either inside the body (in vivo) or outside of the body (ex vivo).
- **Cell therapy** is the injecting, engrafting, or implanted use of viable cells into a patient to receive a medicinal effect

Sources: https://medlineplus.gov/genetics/understanding/therapy/procedures/
Gene therapy - Mayo Clinic

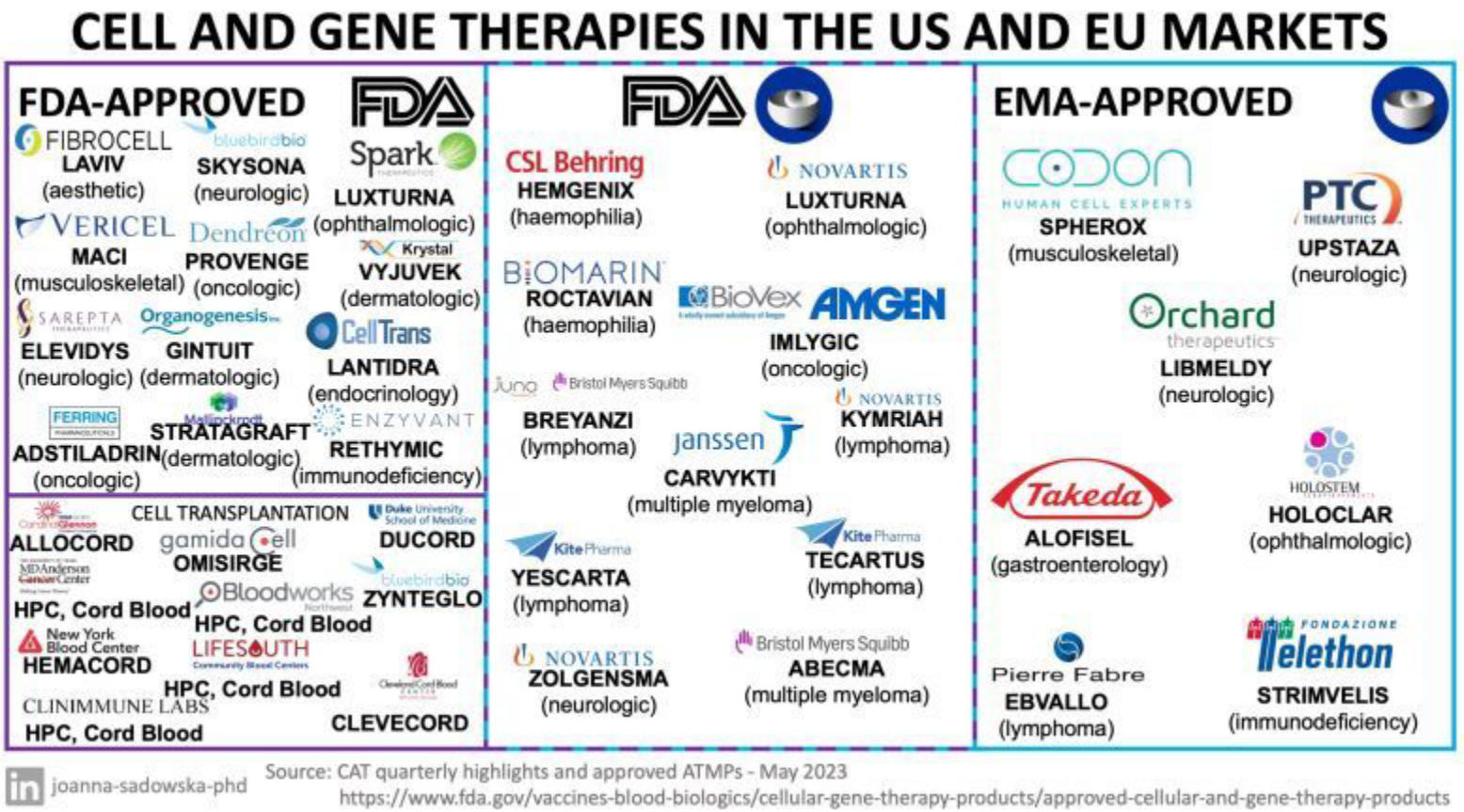
Why do we need these therapies?



Cell & Gene Therapy Implications

- **These are potentially life-saving or life-altering therapies**
- Patients with rare diseases could live without the need for ongoing treatments or the burden of daily disease management after a single course of treatment
- Primary challenges:
  - **Cost/benefit mismatch**
    - Large upfront cost with benefits (lower lifetime treatment cost, societal benefits) accruing over time
    - Patient may not stay with same payer that covered the therapy
  - Uncertainty over exposure
    - Who qualifies based on inclusion criteria and who will want the drug
  - Difficult to track outcomes across different payers/healthcare systems
  - Concerns over long-term durability and patient safety
  - Patient access (need to travel to a QTC – qualified treatment center)

What's Available Today?



Typical Cell Therapy Cost: $300-500K drug, + administration
Typical Gene Therapy Cost: $500K-**$3.5M** + administration

The Top 6 Most Expensive Drugs in the World are All Gene Therapies

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#1: Hemgenix

- Company: CSL Behring, uniQure
- Disease: Hemophilia B
- Cost per Dose: $3.5 million
- Approved: Nov 2022

#2: Elevidys

- Company: Sarepta Therapeutics
- Disease: Duchenne Muscular Dystrophy
- Cost per Dose: $3.2 million
- Approved: Jun 2023

#3: Skysona

- Company: Bluebird Bio
- Disease: Cerebral Adrenoleukodystrophy
- Cost per Dose: $3 million
- Approved: Sep 2022

#4: Roctavian

- Company: BioMarin
- Disease: Hemophilia A
- Cost per Dose: $2.9 million
- Approved: June 2022

#5: Zynteglo

- Company: Bluebird Bio
- Disease: Transfusion-dependent Thalassemia
- Cost per Dose: $2.8 million
- Approved: Sep 2022

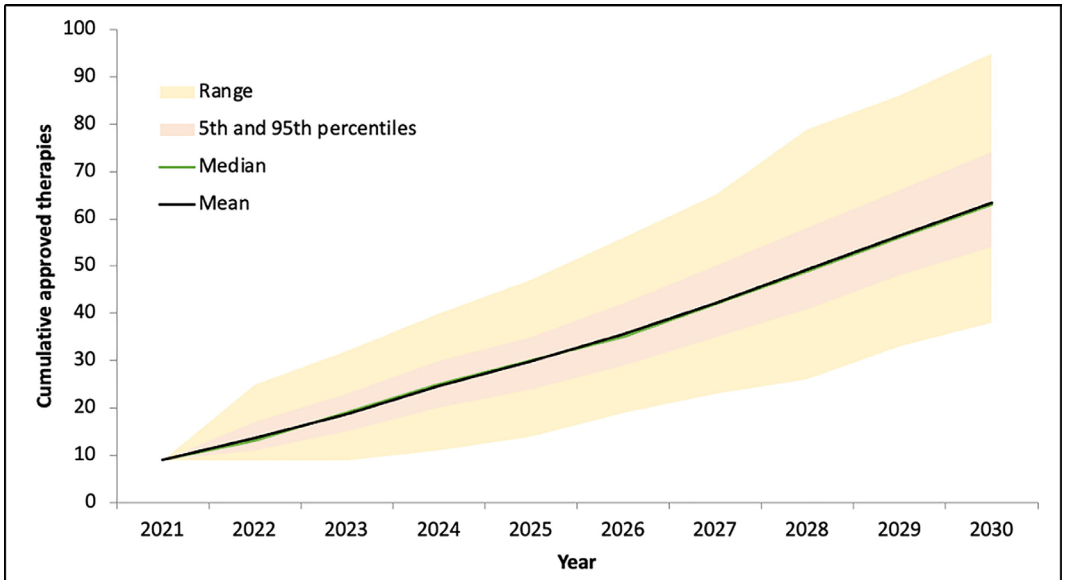
#6: Zolgensma

- Company: Novartis
- Disease: Spinal Muscular Atrophy
- Cost per Dose: $2.1 million
- Approved: May 2019

Source: 10 of the Most Expensive Drugs in the U.S.

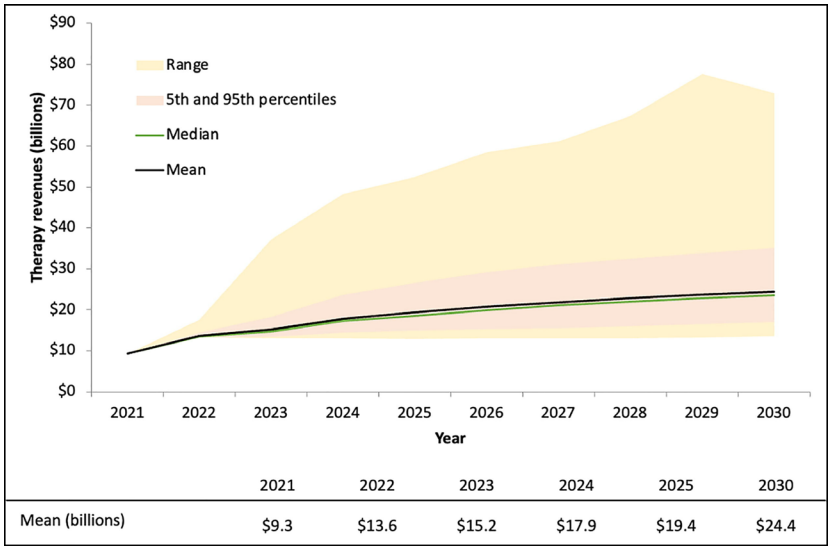
Cell & Gene Therapy Pipeline

of Approvals



	2021	2022	2023	2024	2025	2030
Cancer, hematological	6.0	6.8	8.0	9.8	11.7	31.6
Cancer, solid tumor	0.0	0.4	0.9	1.4	2.0	4.1
Cardiovascular	0.0	0.0	0.0	0.0	0.0	0.2
Hematology	0.0	1.6	2.9	4.2	5.2	9.1
Immunological	0.0	0.1	0.3	0.5	0.6	1.4
Metabolic	0.0	0.2	0.5	0.6	0.9	3.1
Musculoskeletal	0.0	0.1	0.2	0.2	0.4	1.0
Neurological	1.0	1.3	1.8	2.2	2.5	3.7
Ophthalmological	2.0	2.8	3.3	4.1	4.5	6.2
Other	0.0	0.4	0.9	1.6	1.9	3.1
Total	**9.0**	**13.6**	**18.7**	**24.7**	**29.7**	**63.5**

Projected Revenue (billions)



	2021	2022	2023	2024	2025	2030
Mean (billions)	$9.3	$13.6	$15.2	$17.9	$19.4	$24.4

Pipeline Product Indications:

- #1: Hematological cancers
- #2: Hematology
- #3: Ophthalmological

Source: Durable cell and gene therapy potential patient and financial impact: US projections of product approvals, patients treated, and product revenues - ScienceDirect

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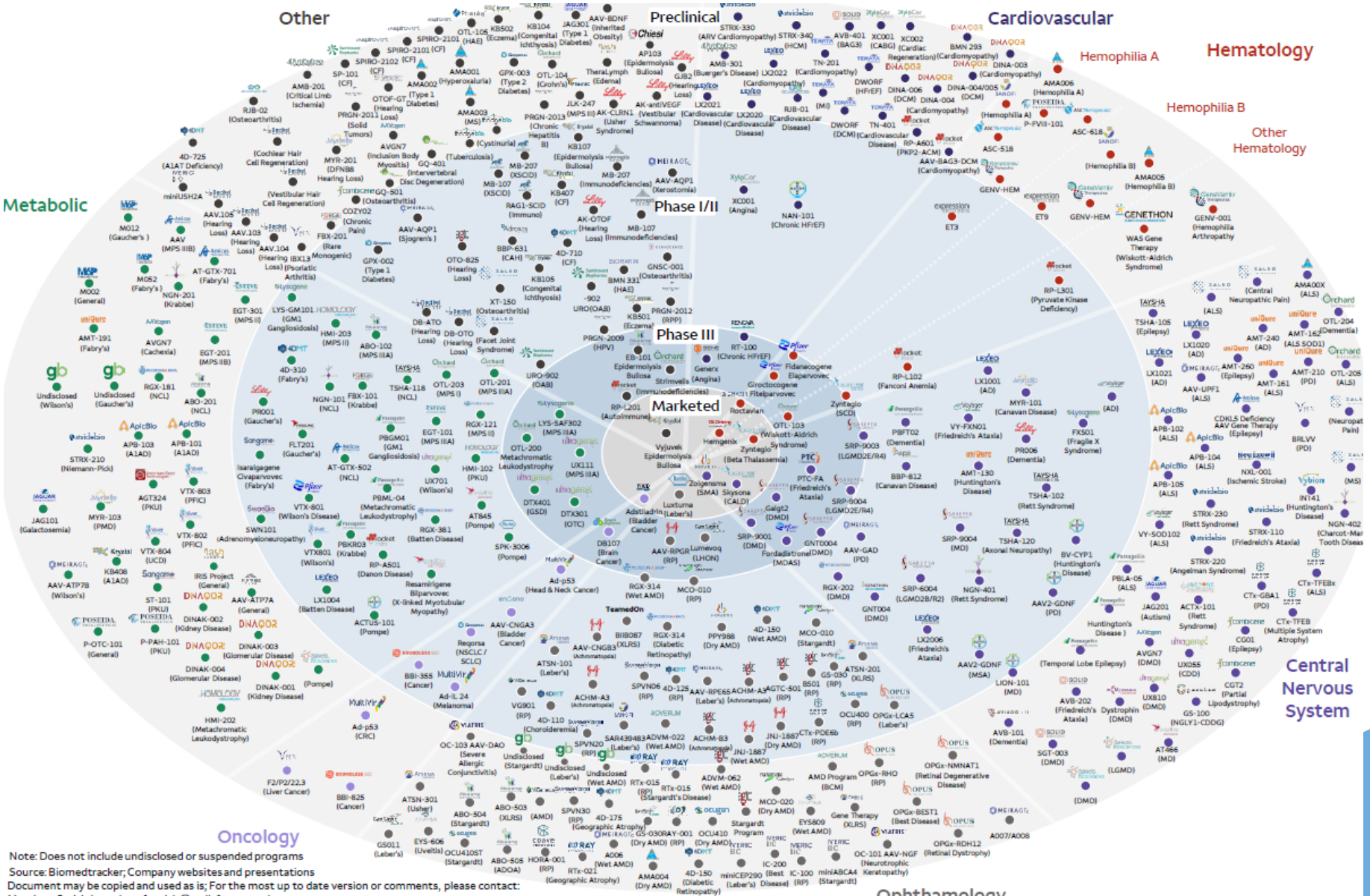
Gene Therapy Pipeline

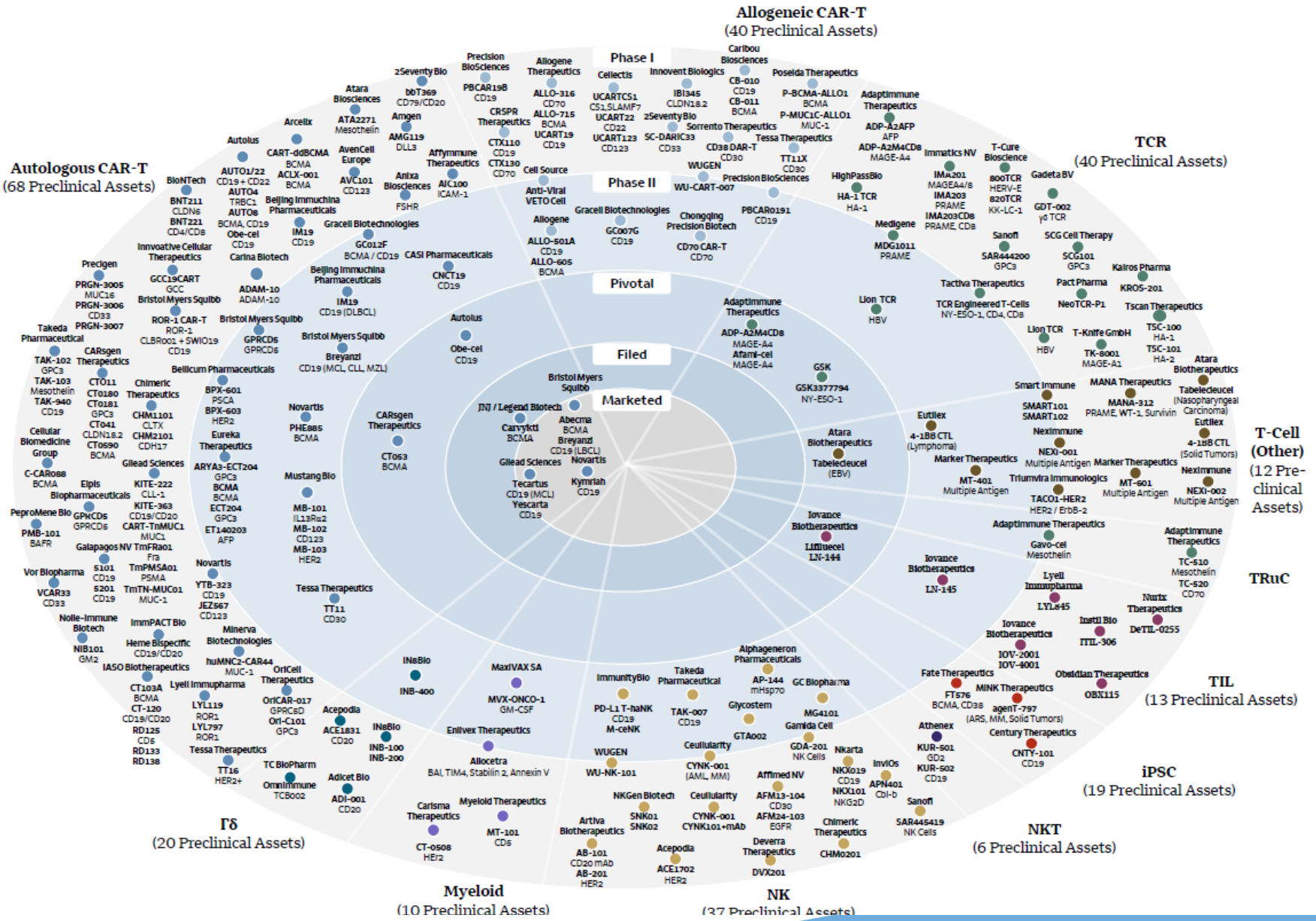


Note: Does not include undisclosed or suspended programs

Source: Biomedtracker; Company websites and presentations

Matthew Smith (matthew.f.smith@wellsfargo.com)

Cell Therapy Pipeline



A Case Study in Uncertainty: Sickle Cell

Exa-Cel

- Vertex/CRISPR
- First US marketed CRISPR therapy if/once approved
- Ex-Vivo therapy utilizing Cas9 gene editing

Lovo-Cel

- Bluebird Bio
- Ex-Vivo therapy using a lentiviral vector encoding a modified beta-globin gene into an autologous transplanted hematopoietic stem cell to produce anti-sickling hemoglobin

Timing:	• **Dec 8, 2023 & Dec 20, 2023**
Target Population:	• Anticipating **~42,500 potential candidates** aged 12 and older, the majority of which are of African descent
Target Price:	• **$1.4M-$3.0M for drug cost alone**

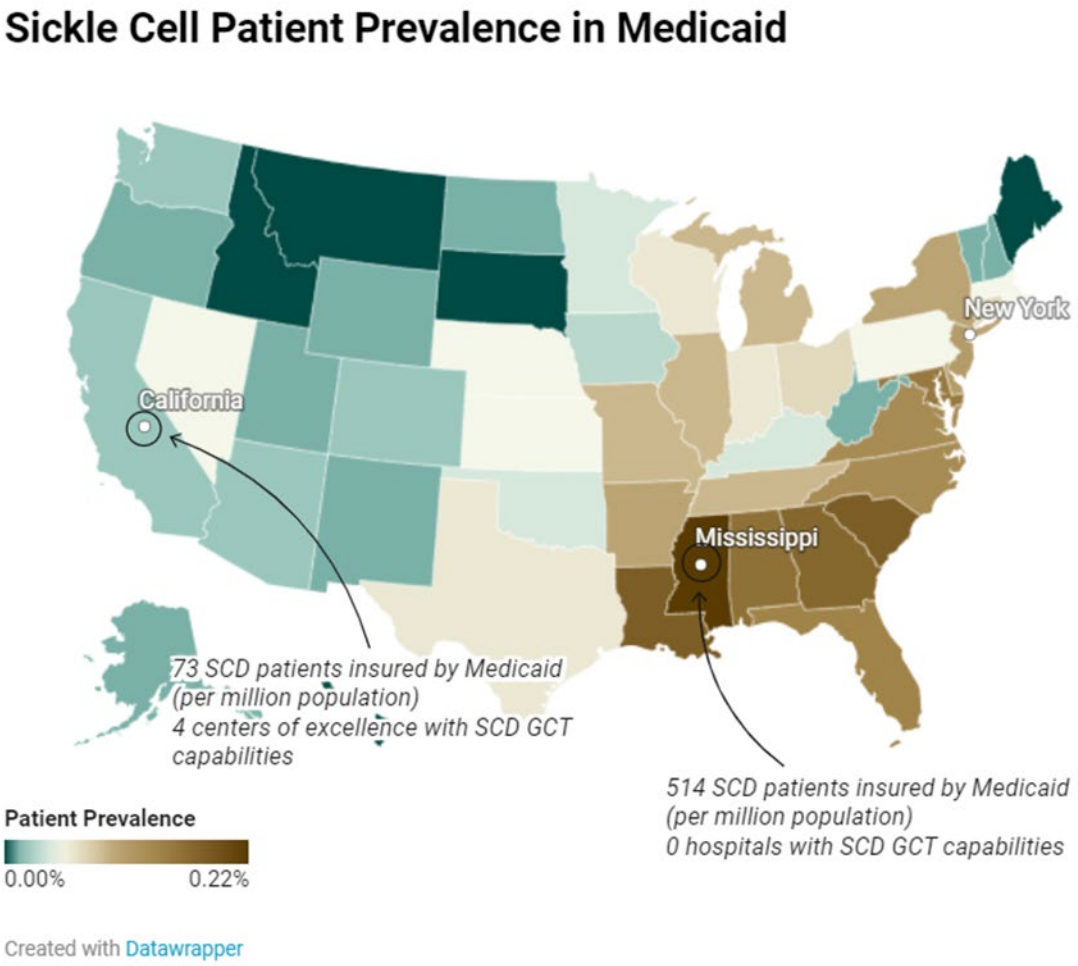
Actuarial Challenges in Quantification

Frequency

- Starting point: Use population incidence/prevalence to estimate risk
  - This could vary significantly by type of medical coverage (Commercial, Medicare, Medicaid) as well as geography
  - For example, 1/3 of sickle cell patients are in the commercial population, the other 2/3 in Medicaid programs
- ICD-10 codes for claimants (Sickle-cell disorders: D57-) can give indication within specific population of who may be eligible
- Specific clinical criteria needs to be met; having the disease is usually not enough to qualify for the therapy
- Watch pipeline closely for changes in approval dates, FDA rejections, or changes in clinical indications

The biggest unknown of all: human behavior

Geographical Considerations



Source: Quantile Health

Example Treatment Pathway

Source: AMS University (elasticvirtual.com)

Actuarial Challenges in Quantification

Severity

- Two components: the drug cost itself and the administrative costs
  - In vivo: “go in, get a shot.”
  - Ex vivo: “cells are extracted, modified, and reinserted.” Much more expensive due to higher admin costs/inpatient stays
- Other considerations: discounts (340B rebates), hospital billing practices, complications, cost offsets (i.e., a hemophiliac no longer needing expensive factor drug), patient travel, time off from work
- Cost of drug is often not known until day of approval, sometimes even later

Current Solutions in the Market

Payer reinsurance/stop-loss/carve-out

- Carve out of financial responsibility or risk for specified cell and/or gene therapies
- Available as stand-alone products or incorporated into existing specific/aggregate/ASD coverage
- No performance guarantees

Contract negotiation & data management services

- Act as third parties to negotiate contracts for cell and gene therapies, and provide data/outcomes tracking services
- Contracts may include performance guarantees, but the service company themselves do not assume financial risk

Provider contract negotiation

- Oriented towards providers and patient care pathways
- Multiple services offered, including COE network creation and contracting, data analytics and cost containment
- Performance guarantees are oriented towards clinical services and outcomes rather than financial guarantees

Financial and pharma company warranty services

- Include payment plans, with or without performance guarantees, and warranties for purchase by pharmaceutical companies that can provide protection to payers for suboptimal product performance

Sources: https://newdigs.mit.edu/sites/default/files/FoCUS-WP-CGT-Market-Solutions.pdf;

Concerns for Carriers/Health Plans

Company / Product	RISK			COST		ADMINISTRATION		PATIENT/PROVIDER		
	Actuarial/ financial	Performance	Payment timing	Product	Ancillary	Data tracking	Contracts	COE access	Patient access	Patient care
Payer reinsurance/stop-loss/carve-out										
BCS Financial *Stop-Loss Gene Therapy*	●		●	●	●					
CVS Health *Gene Therapy Stop-Loss*	●			●	●				●	
Evernorth *Embarc Benefit Protection Program*	●	●	●	●	●	●	●	●	●	●
MedImpact Healthcare Systems *MedShield*	●			●					●	
OptumRx *Optum Gene Therapy Risk Protection*	●	●	●	●		●	●		●	●
OutcomeRx *Patient Access to Costly and Curative Therapies*	●		●	●		●			●	
PayRx *PayRx Benefit Protection*	●	●	●	●	●	●				
Contract negotiation & data management services for payers and pharma companies										
Audaire Health *Gene & Cell Therapy Outcomes Management Service*		●	●	●		●	●			

Sources: https://newdigs.mit.edu/sites/default/files/FoCUS-WP-CGT-Market-Solutions.pdf; https://www.medimpact.com/resources/client-news/surprising-impact-gene-therapies

Concerns for Carriers/Health Plans

Company *Product*	RISK			COST		ADMINISTRATION		PATIENT/PROVIDER		
	Actuarial/ financial	Performance	Payment timing	Product	Ancillary	Data tracking	Contracts	COE access	Patient access	Patient care
Real Endpoints *RE Marketplace*	●	●		●		●	●		●	
Provider contract negotiation										
BlueCross Blue Shield Association *Blue Distinction Center for Cellular Immunotherapy*		●	●	●	●	●	●	●	●	●
Emerging Therapy Solutions *ETS Programs of Excellence, ETS Analytics & ETS Buyer's Group*	●		●	●	●	●	●	●		
Financial and pharma company warranty services										
August Care *Outcomes-based Financial Solutions*	●	●	●	●		●	●	●	●	●
CVS Health *Gene Therapy Payment Plan*			●	●					●	
OutcomeRx *Specialty Therapy Warranty*		●				●	●		●	

Sources: https://newdigs.mit.edu/sites/default/files/FoCUS-WP-CGT-Market-Solutions.pdf; https://www.medimpact.com/resources/client-news/surprising-impact-gene-therapies

What Comes Next?

Figure 1. FoCUS precision financing solutions and their ability to address key challenges associated with cell and gene therapies

Blue circles represent the proportion of the associated challenge (payment timing, performance, actuarial risk) addressed by the precision financing solution. A full blue circle indicates the challenge is fully addressed; an empty circle indicates the solution has not addressed the challenge.

No single solution checks all the boxes

Source: https://newdigs.tuftsmedicalcenter.org/wp-content/uploads/2022/06/FoCUS-WP-CGT-Market-Solutions.pdf

Q&A

Thank you!

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