

# The State of the Industry

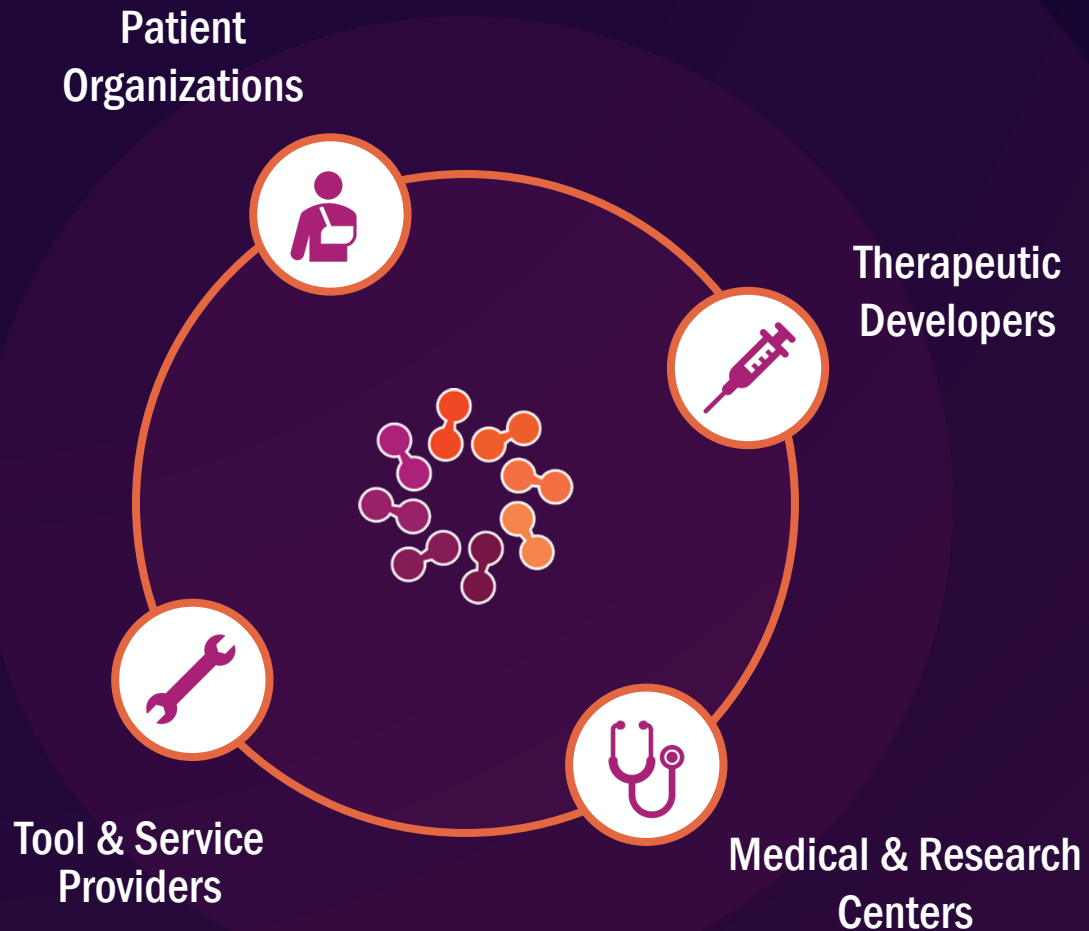
Janet Lambert, CEO

Phacilitate Advanced Therapies Week  
January 26, 2021



# ARM is the Global Voice of the Sector

*Representing 400+ members worldwide*



**Promote Clear Regulation**  
**Enable Innovative Reimbursement**  
**Address Manufacturing Barriers**  
**Educate Stakeholders**





# New paradigms in regenerative medicine





# Investment Landscape



# Long-term growth and short-term volatility

- Record breaking investment
- Gene editing rising in prominence
- Venture capital ascendant
- Down year for public equities





A hand is shown reaching out from the left, with the index finger pointing towards a glowing, digital globe. The globe features a world map overlay and is surrounded by a network of lines and dots, suggesting a global or digital theme. The background is dark with a subtle pattern of light streaks.

# 1,307

Therapeutic Developers  
Active Worldwide

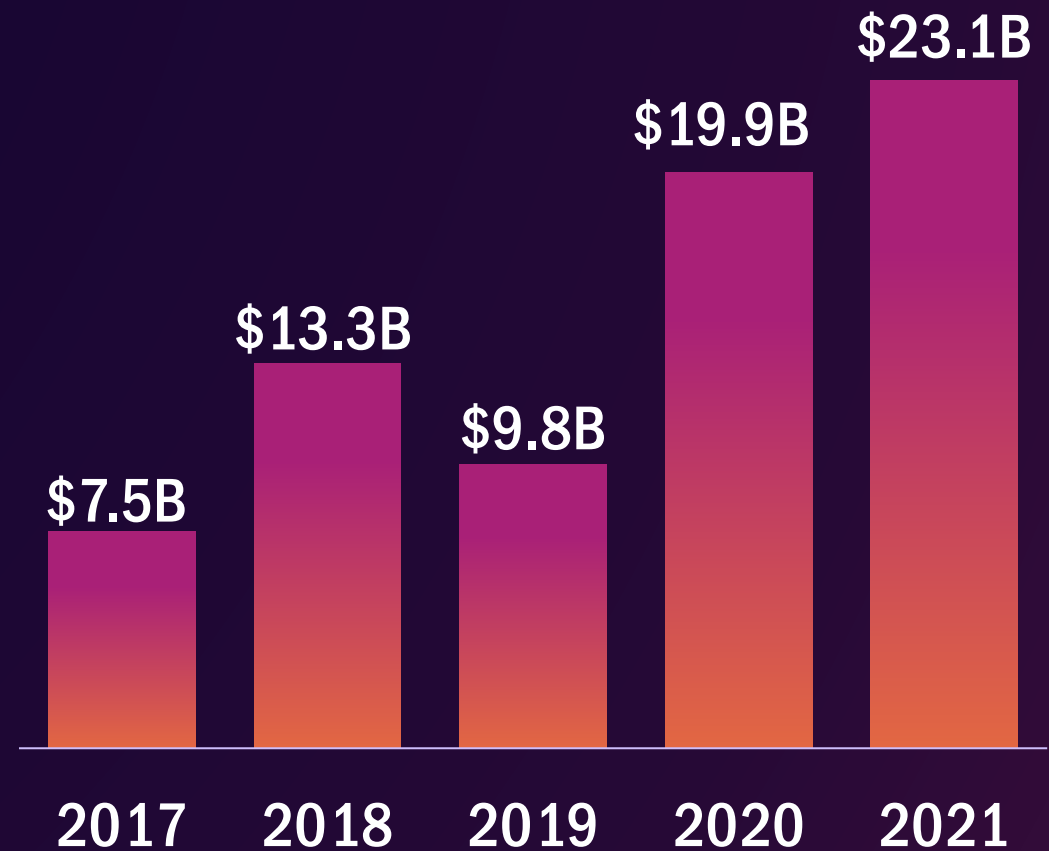






# **\$23.1B raised in 2021**

**↑ 16% from 2020**





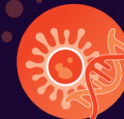
# \$23.1B raised in 2021

↑ 16% from 2020



## \$10.6B

RAISED IN  
GENE THERAPY  
*14% increase YoY*



## \$10.1B

RAISED IN  
CELL IO  
*26% increase YoY*



## \$2.0B

RAISED IN  
CELL THERAPY  
*15% decrease YoY*



## \$341M

RAISED IN  
TISSUE ENGINEERING  
*10% increase YoY*







# \$23.1B raised in 2021

↑ 16% from 2020



## \$18.0B

RAISED BY  
US DEVELOPERS  
*53% increase YoY*



## \$3.3B

RAISED BY  
EUROPEAN DEVELOPERS  
*8% decrease YoY*

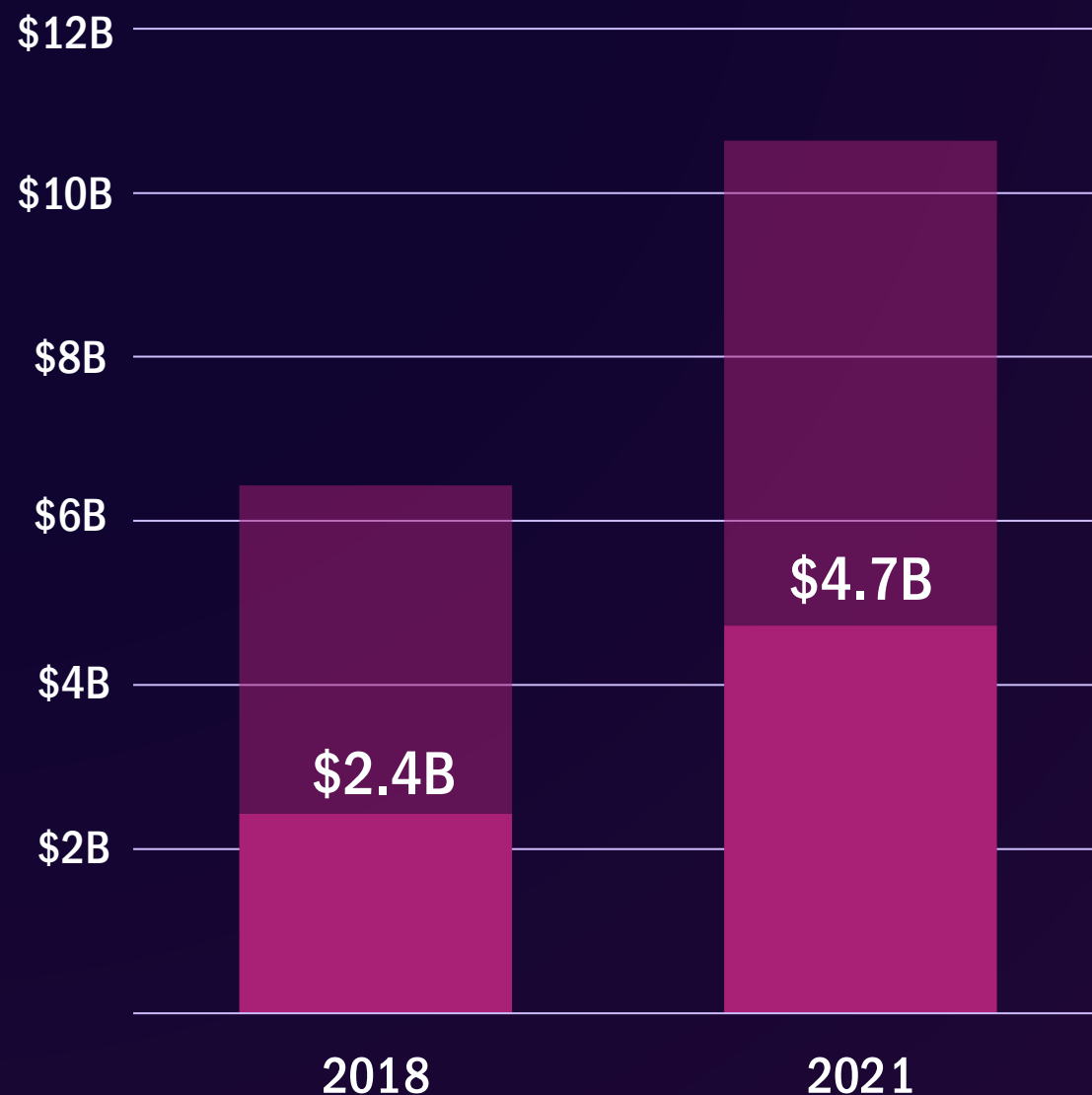


## \$2.2B

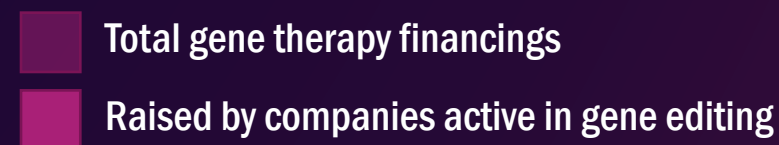
RAISED BY  
APAC DEVELOPERS  
*4% decrease YoY*



# Gene Editing Gains Prominence

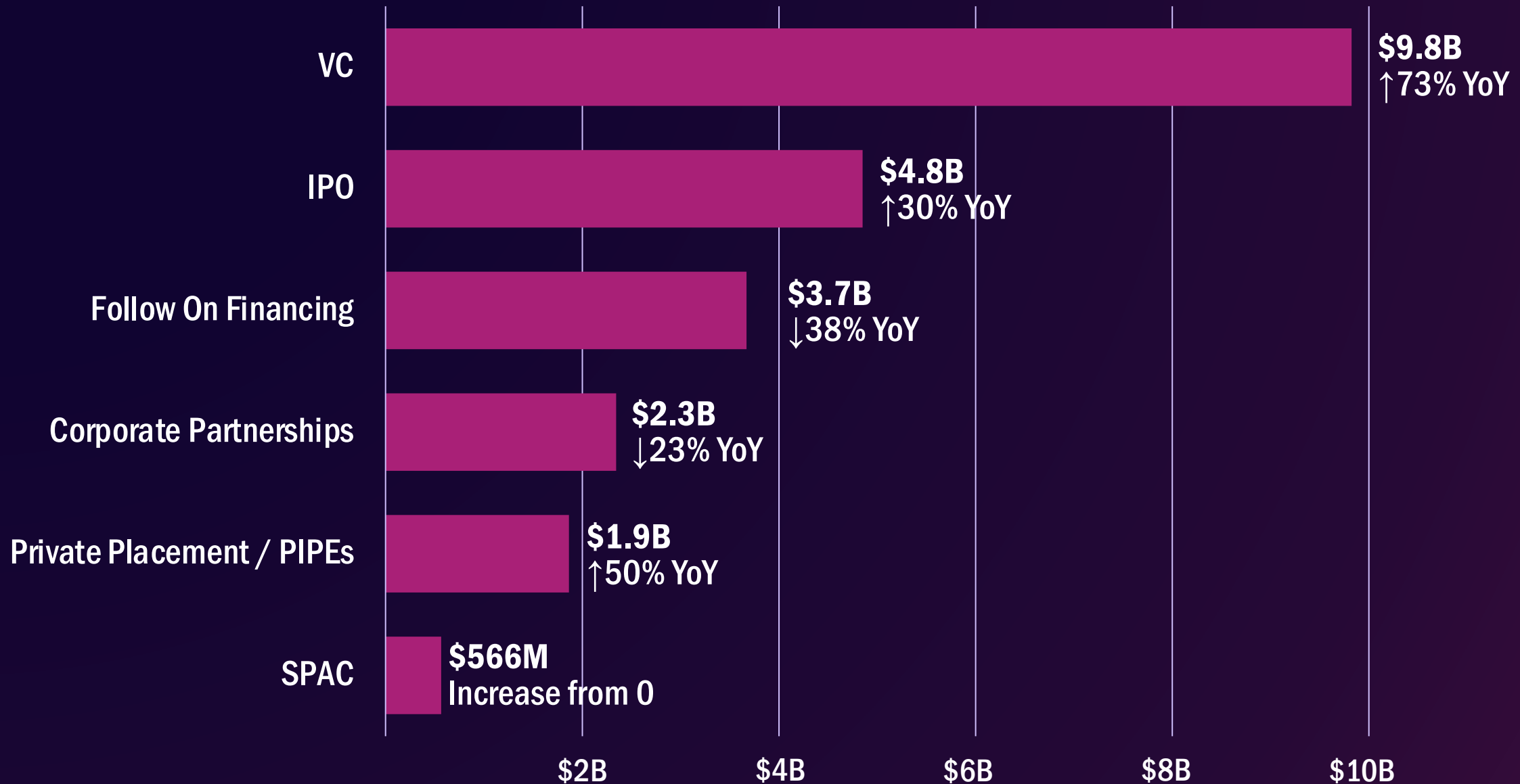


The proportion of gene therapy financings raised by companies active in gene editing increased from 38% to 45% over the past 3 years

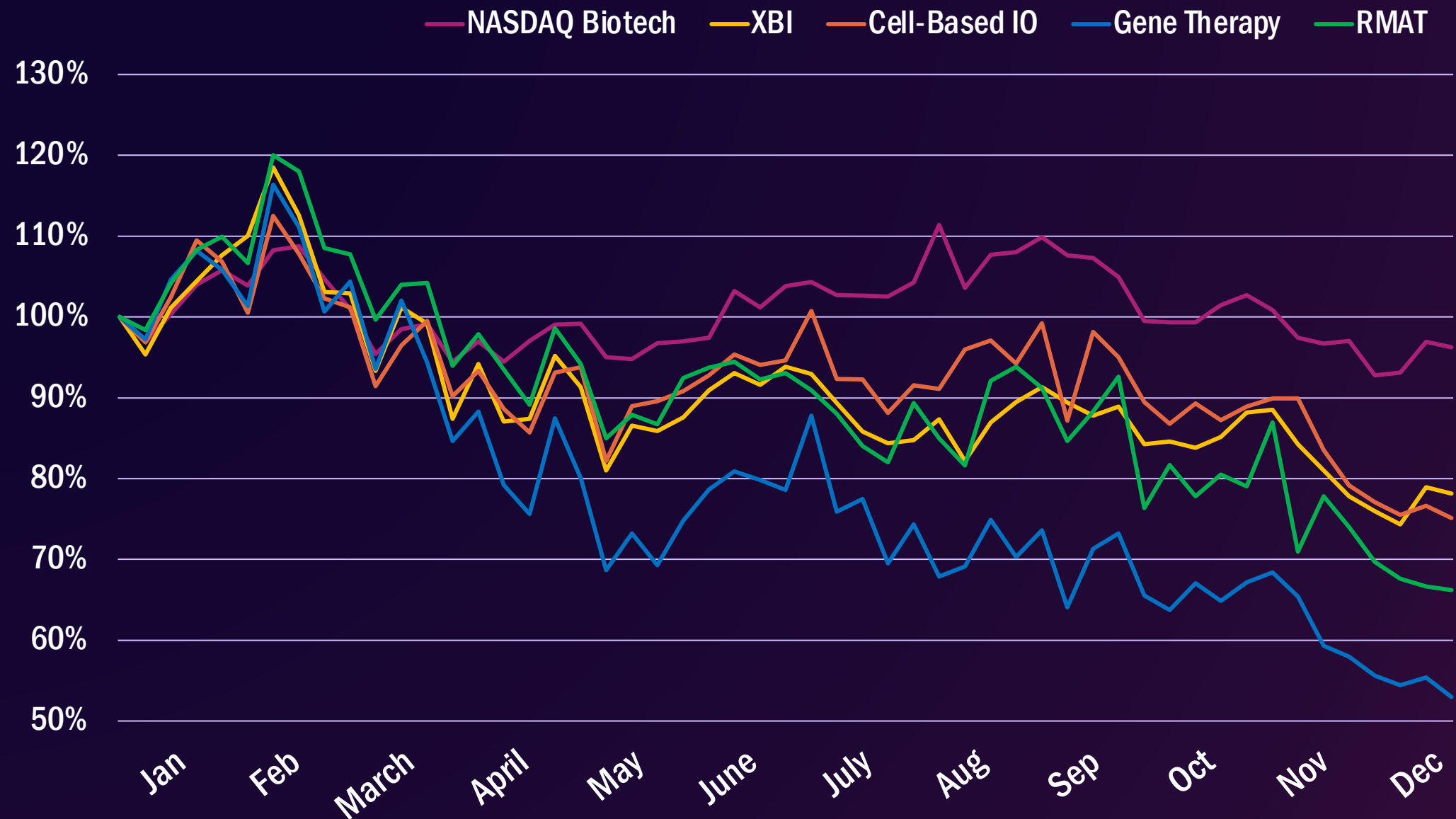




# Venture Capital Drives Record-Breaking Investment



# Market Factors Depress Public Equity Performance







# **Commercial & Clinical Landscape**



## Approvals of New Products in 2021

### **Breyanzi\* (US)**

CAR-T Therapy  
DLBCL  
BMS

### **Abecma (US & EU)**

CAR-T Therapy  
Multiple myeloma  
bluebird bio & BMS

### **Stratagraft\* (US)**

Tissue Therapy  
Severe burns  
Mallinckrodt

### **Skysona (EU)**

Gene Therapy  
Cerebral ALD  
bluebird bio

### **Carteyva (China)**

CAR-T Therapy  
LBCL  
JW Therapeutics

### **Rethymic\* (US)**

Tissue Therapy  
Congenital Athymia  
Enzyvant

\* RMAT designated product





# 2,261 Ongoing Global Clinical Trials in Regenerative Medicine

1,354



## CANCER

Including leukemias, lymphomas, breast cancer, brain cancer, lung cancer, prostate cancer, & others

175



## NEUROLOGICAL

Including disorders such as Alzheimer's, Parkinson's, ALS, multiple sclerosis, cerebral palsy & others

172



## DIABETES

Including Type 1 & Type 2, as well as related conditions such as diabetic kidney failure

111



## CARDIOVASCULAR

Including damage caused by heart attack and vascular disease

111



## RARE DISEASES

Including many fatal diseases that affect infants and children

77



## STROKE

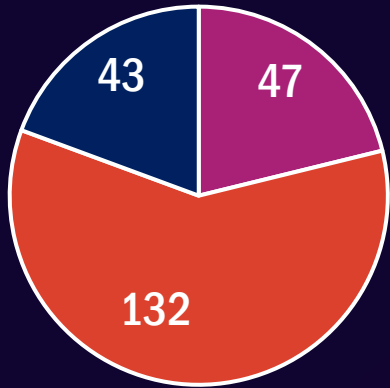
Including stroke recovery and paralysis due to stroke



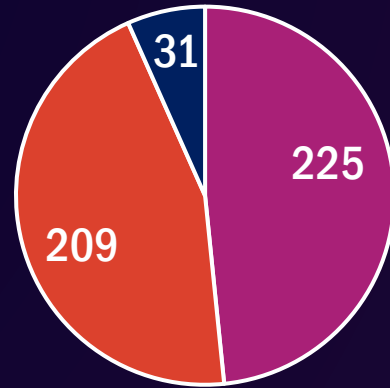
# 1,129 Industry-Sponsored Clinical Trials

Ph 1 Ph 2 Ph 3

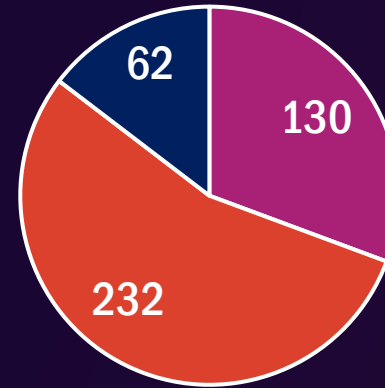
Gene Therapy: 222



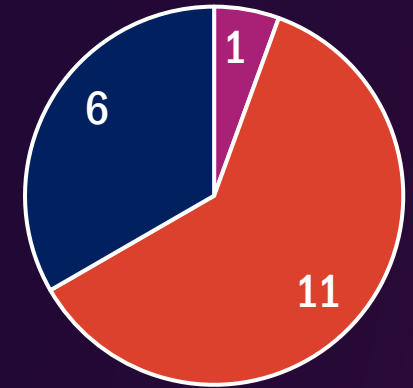
Cell-Based IO: 465



Cell Therapy: 424

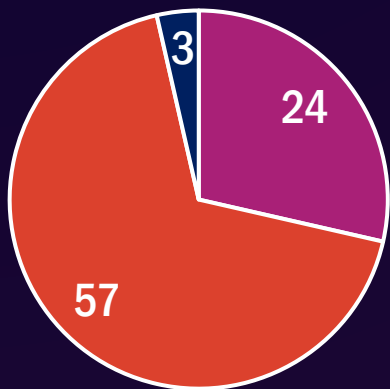


Tissue Engineering: 18

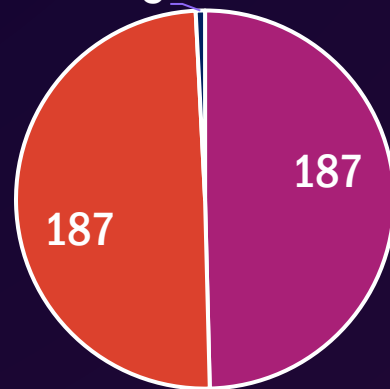


# 1,132 Academic & Government Sponsored Trials

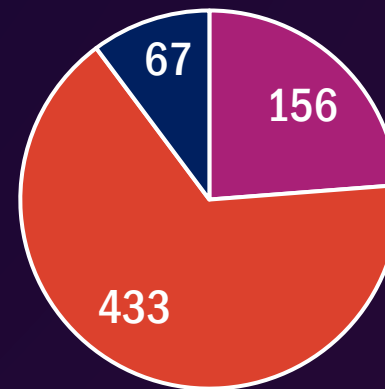
Gene Therapy: 84



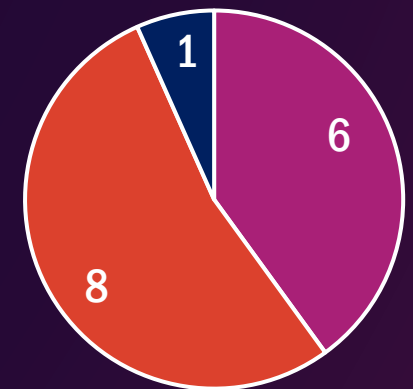
Cell-Based IO: 377



Cell Therapy: 656



Tissue Engineering: 15





# Looking Ahead: 2022+

- 2022 is a big year for rare disease approvals...
- ... amid an evolving shift in focus to more prevalent diseases in coming years



# Upcoming CGT Regulatory Decisions

United States	Iovance Melanoma				
	BioMarin Hemophilia A	Gamida Cell Blood Cancer + HSCT			
	bluebird bio Beta thalassemia	Krystal Bio Epidermolysis Bullosa	Orchard MLD	Atara Bio EBV+ PTLD	
	bluebird bio Cerebral ALD	uniQure/CSL Hemophilia B	Adaptimmune Synovial Sarcoma	CRISPR / Vertex Sickle Cell & Beta Thal	
	Janssen/Legend Multiple Myeloma	PTC Therapeutics AADC Deficiency	bluebird bio Sickle Cell Disease	CARsgen Multiple Myeloma	
	2022		2023		
Europe	Janssen/Legend Multiple Myeloma	Bristol Myers Squibb B cell Lymphoma	Orchard Wiskott-Aldrich		
	PTC Therapeutics AADC Deficiency	Atara Bio EBV+ PTLD	Krystal Bio Epidermolysis Bullosa		
	GenSight Bio LHON	uniQure/CSL Hemophilia B			
	BioMarin Hemophilia A				

■ BLA/MAA submitted  
□ BLA/MAA planned





## Additional 2022 Milestones

### Late-stage data readouts

- Pfizer, BioMarin, Solid Bio

### Early data in prevalent disorders

- Vertex

### Gene editing continues march to the clinic

- ViaCyte/CRISPR, Beam, Verve begin trials
- Data from Caribou, Intellia, & others





# Beyond 2022

**Evolution from rare monogenetic  
diseases and cancers to more prevalent  
10-20 approvals a year by 2025?**

## Examples of Ph 3 Industry Trial Indications:

Bladder Cancer	Head & Neck Cancer
Breast Cancer	Leukemia & Lymphoma
Cartilage Defects	Lung Cancer
Cirrhosis	Macular Degeneration
Congestive Heart Failure	Myocardial Infarction
Critical Limb Ischemia	Osteoarthritis
Diabetic Foot Ulcers	Ovarian Cancer
Diabetic Neuropathy	Prostate Cancer
Esophageal Cancer	Refractory Angina
Glioblastoma	





## Key Sector Challenges

Dosing & Delivery

CMC

Policy & Reimbursement



## Jayla Turner

Jayla was born in 2007 and diagnosed with complete DiGeorge syndrome

Without treatment, life expectancy is 2-3 years

She received the therapy now known as Rethymic in 2008

Jayla is now 14 years old in the 9<sup>th</sup> grade

She enjoys gymnastics, cheering, & volleyball and is an honor roll student





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Slides & a recording of this presentation will be made available on the ARM website



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