



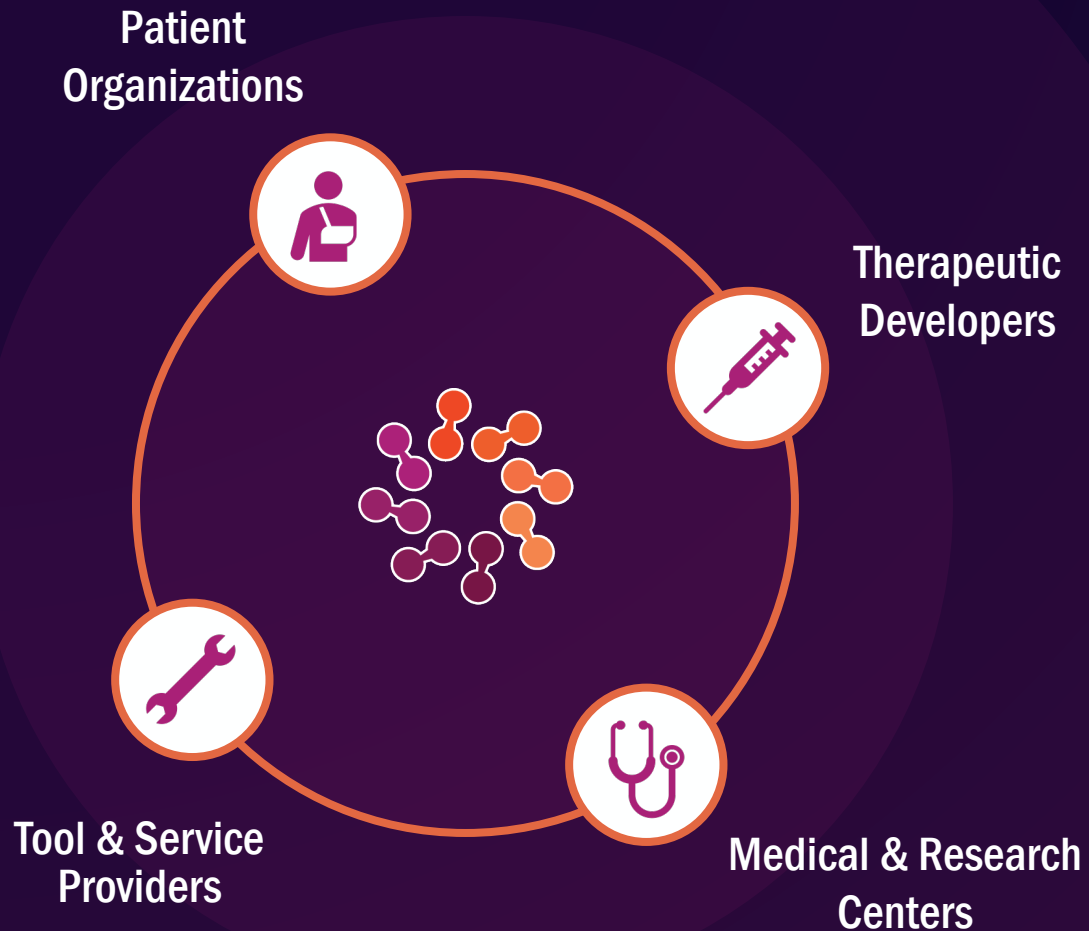
The 2021 Gene Therapy Landscape

Janet Lambert, CEO

Gene Therapy for Rare Disorders
February 24, 2020

ARM is the Global Voice of the Sector

Representing 380+ members worldwide



Promote Clear Regulation
Enable Innovative Reimbursement
Address Manufacturing Barriers
Educate Stakeholders



A world map with a dark blue background. Overlaid on the map are five semi-transparent red circles of varying sizes, each containing a white number representing the count of developers in a specific region. The circles are positioned over North America, South America, Europe, Africa, and Asia. The numbers are 543, 13, 209, 25, and 295 respectively.

1,085

TOTAL GENE, CELL & TISSUE-
BASED THERAPEUTIC
DEVELOPERS WORLDWIDE

543

13

209

25

295



A world map with a dark blue background. Overlaid on the map are five semi-transparent circles of varying shades of blue and green, each containing a white number representing the count of developers in a specific region. The circles are positioned over North America, Europe, Asia, South America, and Africa. A large red circle is in the bottom left corner, containing the total count.

742

TOTAL DEVELOPERS ACTIVE
IN GENE THERAPY

407

1

136

10

188





A world map with five semi-transparent blue circles overlaid on different regions. Each circle contains a white number representing the count of developers in that region. The circles are positioned over North America, Europe, Asia, South America, and Africa. The background of the map is a light beige color.

468

TOTAL DEVELOPERS ACTIVE
IN GENE THERAPY FOR
RARE DISORDERS

253

82

129

4

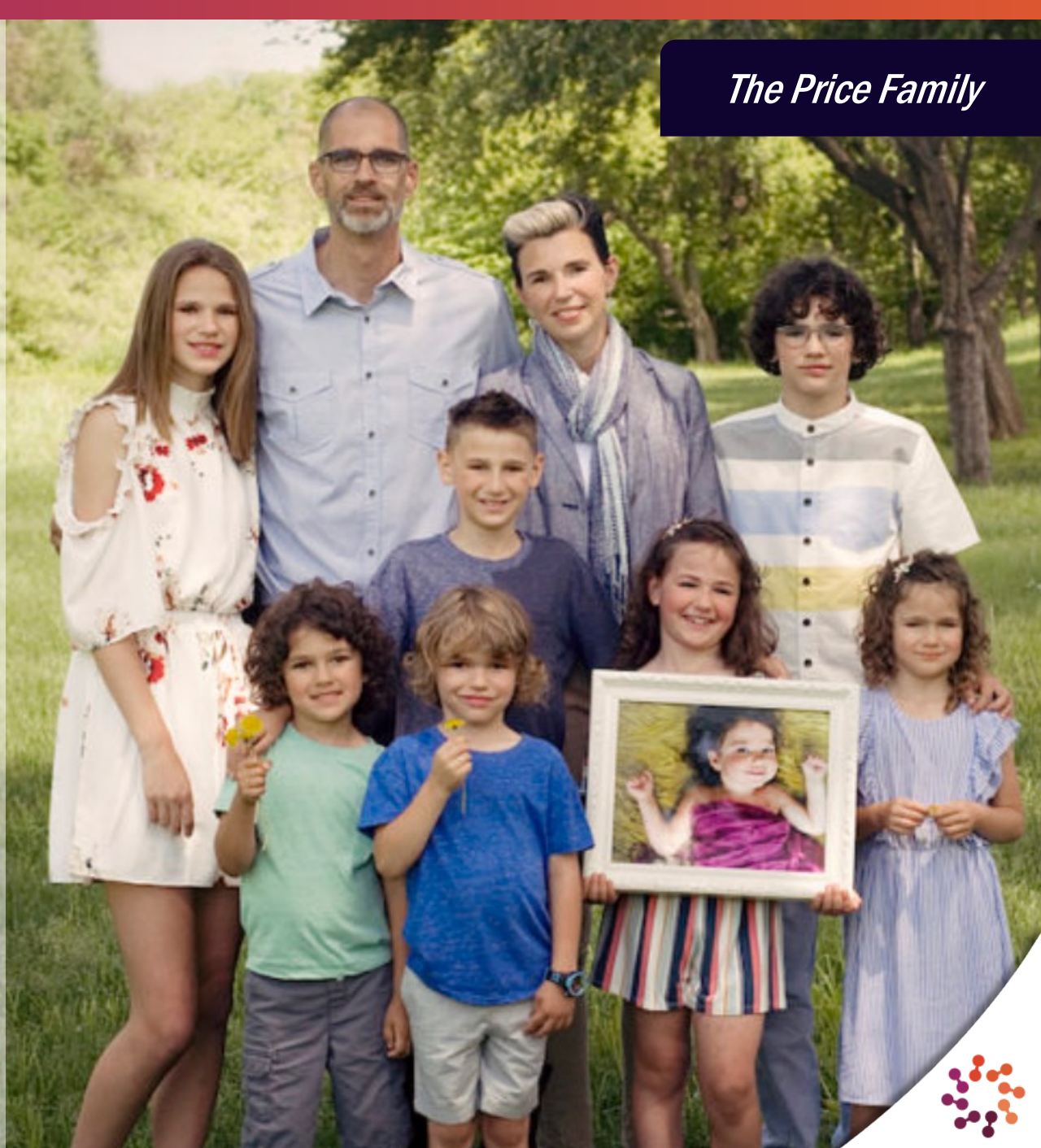
1



Patients continue to benefit from innovative therapies.

- *"It's always really interesting to me – in pre-pandemic days – when I would attend*
- *Zovigermas (Novartis Gene Therapies) huge*
- *Japan, Canada where they're talking about gene therapy as if it's this fantastical futuristic thing.*
- *Luxturna (Spark / Roche) – Canada*
- *I've got photos on my phone of a child who is 10*
- *years post gene therapy."*
- *Breyanzi (Bristol Myers Squibb) – US*
- *Tecartus (Kite, a Gilead company) – US*
- *– Amy Price, mother of 2 gene therapy patients*

The Price Family



1,220 ONGOING REGENERATIVE MEDICINE &
ADVANCED THERAPY TRIALS WORLDWIDE

383

PH
1

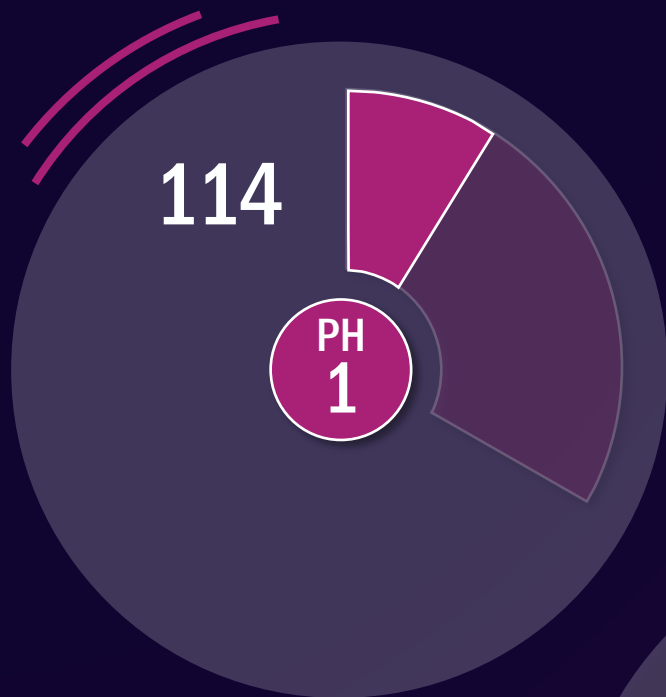
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152

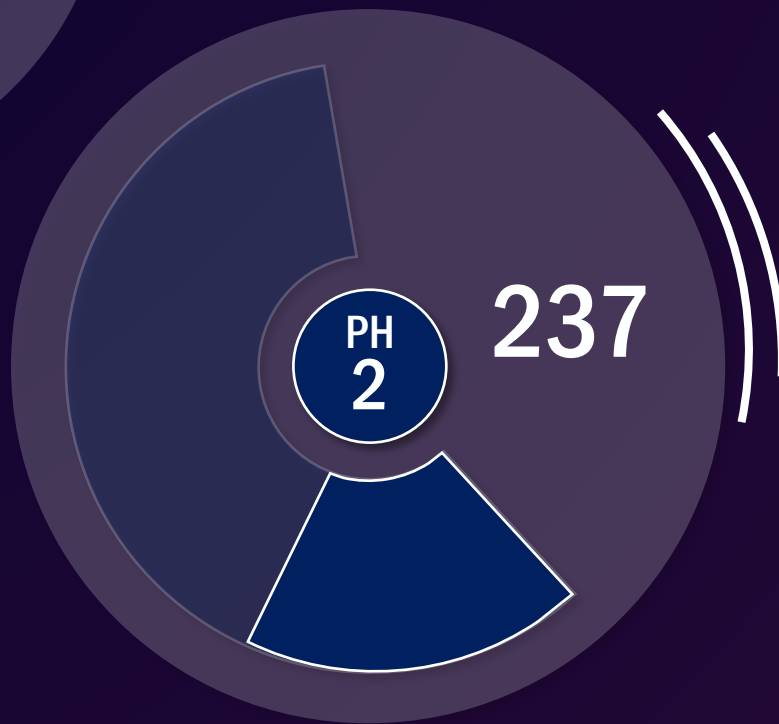
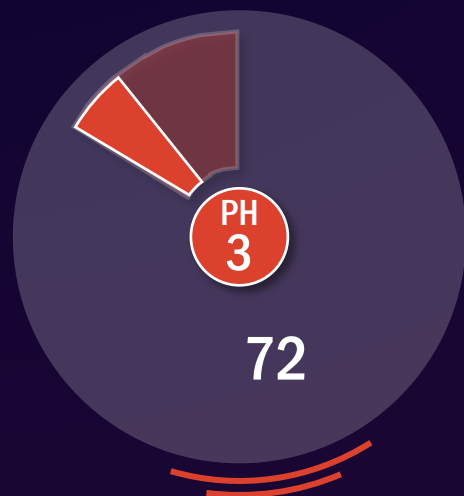
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685





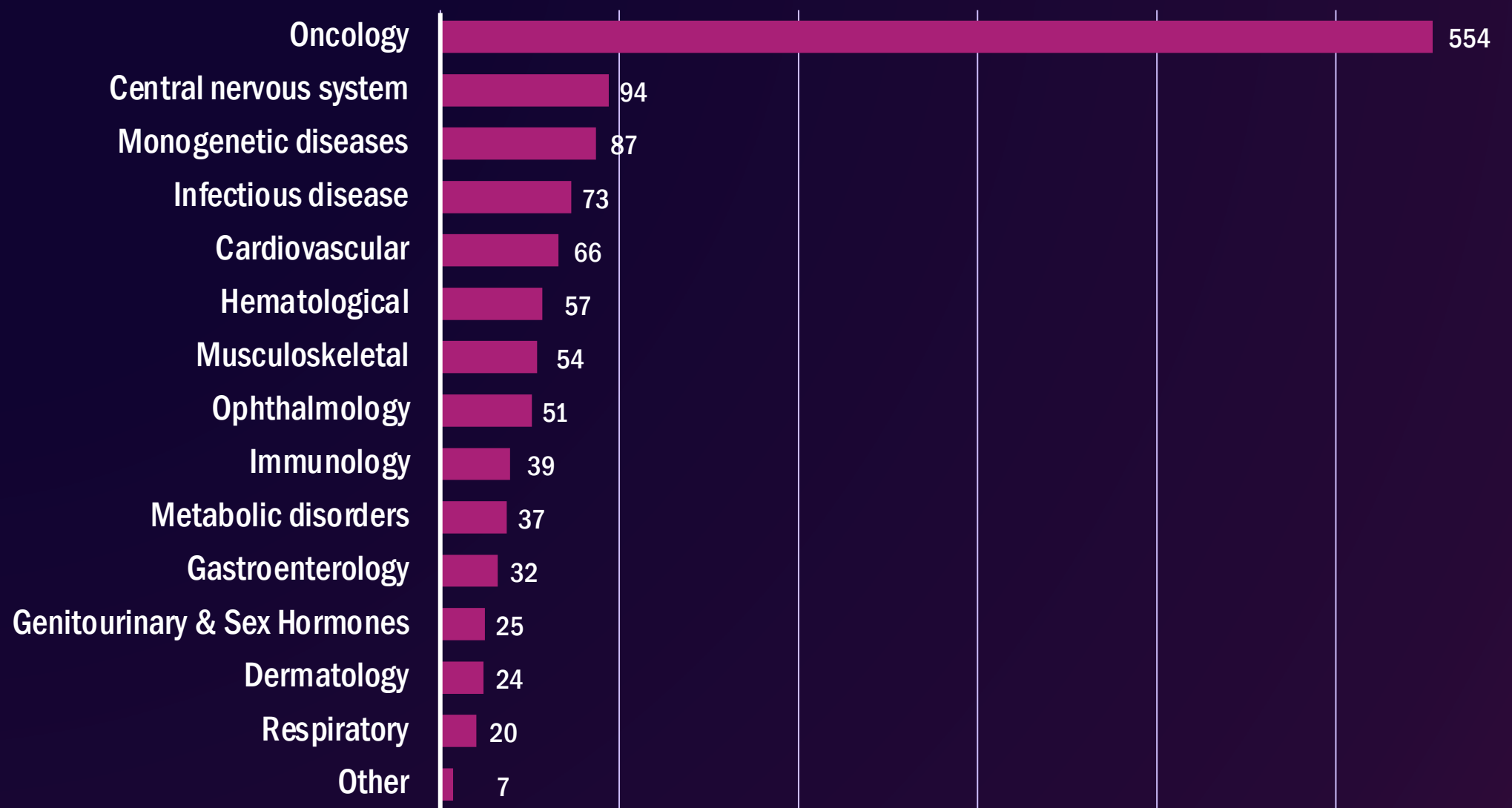
423 ONGOING GENE THERAPY
TRIALS WORLDWIDE



*268 gene therapy trials
in rare diseases*

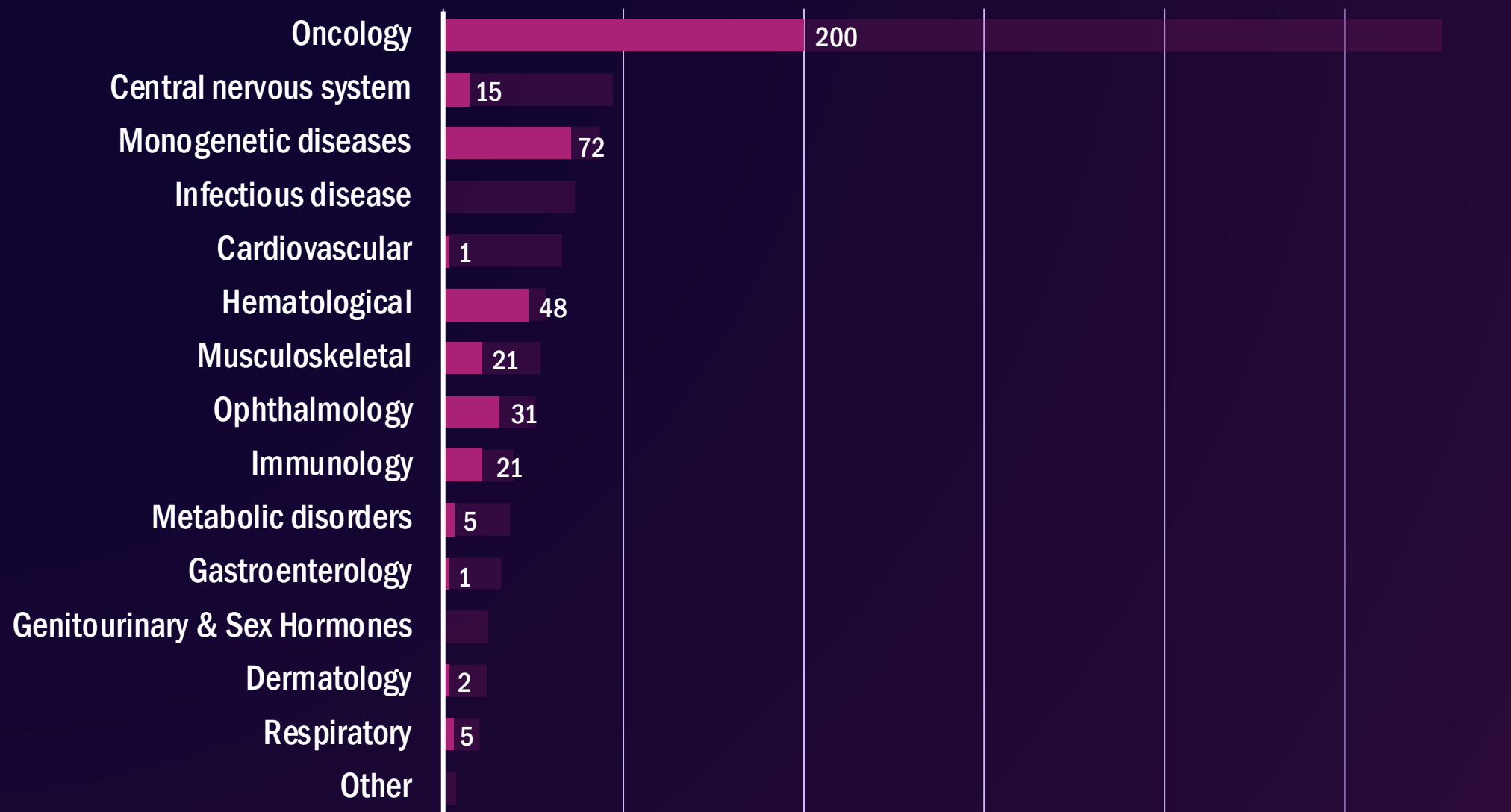


Clinical Trials by Indication



Clinical Trials by Indication

Gene therapy





The Scientific and Clinical Landscape

Gene editing advances in the clinic

Bespoke therapies for ultra-rare disease

Maturation of gene therapy



A Challenging Environment

COVID-driven operational and clinical disruptions

Pandemic limited regulators' CGT bandwidth and travel

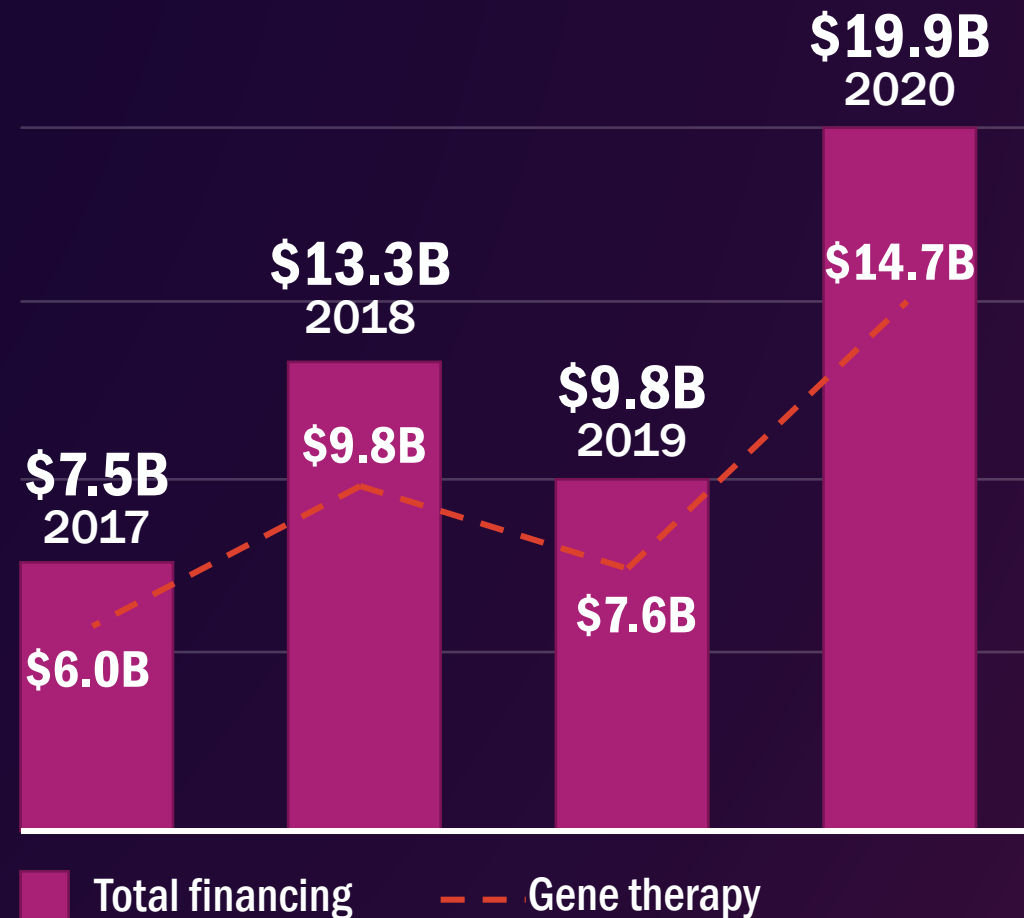
Growing industry demands and fast-moving science



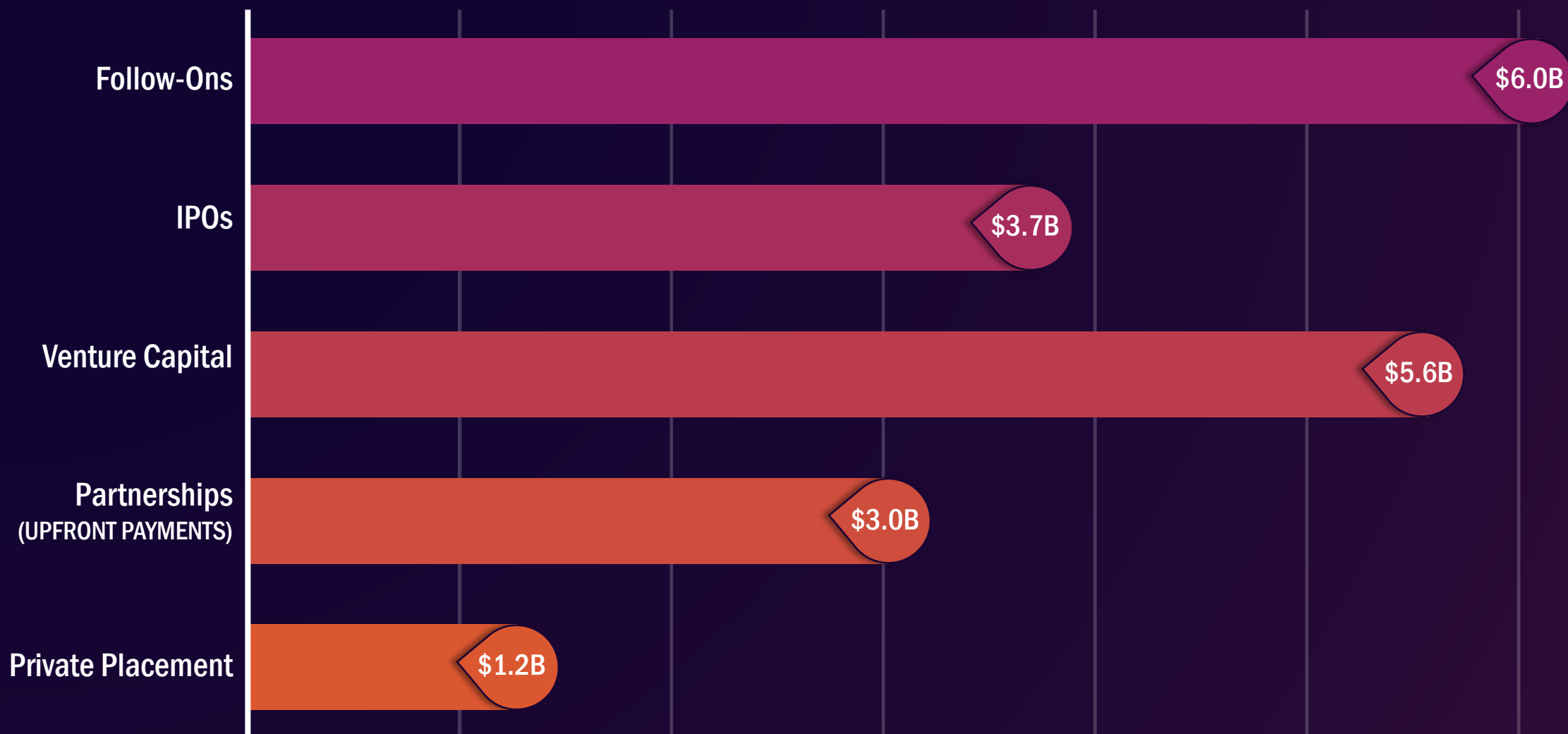


\$19.9B raised in 2020

14.7B raised by companies active in gene therapy



Financing by Type



Gene Therapy for Rare Disease Financings Flourish

PRIVATE FINANCINGS

- Encoded Tx – \$135M (Jul 22)
- Freeline Tx – \$120M (Jun 30)
- Generation Bio – \$110M (Jan 10)
- Akouos – \$105M (Mar 3)

FOLLOW-ON FINANCINGS

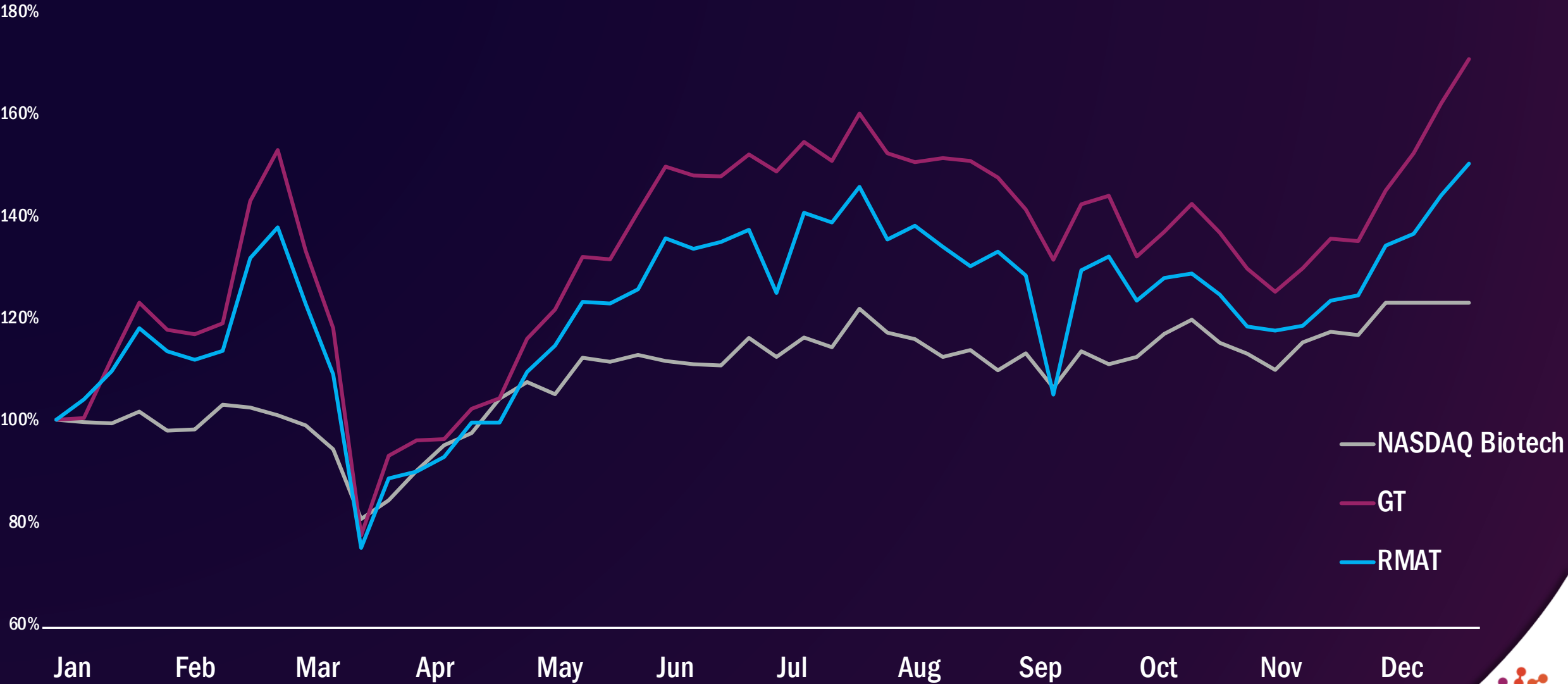
- bluebird – \$575M (May 18)
- CRISPR Tx – \$426M (July 6)
- Rocket – \$299M (Dec 14)
- Adverum Bio – \$217M (Aug 18)
- Editas – \$216M (June 23)
- Intellia -- \$201M (Dec 4)
- Krystal Bio – \$125M (Feb 5)
- IVERIC – \$117M (June 22)
- AVROBIO – \$100M (Feb 18)

INITIAL PUBLIC OFFERINGS

- Passage Bio – \$284M (Feb 3)
- Akouos – \$244M (Jun 25)
- Generation Bio – \$230M (Jun 12)
- 4D Molecular -- \$222M (Dec 15)
- Beam Tx – \$207M (Feb 11)
- Taysha Gene Tx – \$181M (Sep 29)
- Freeline Tx – \$159M (Aug 11)



Public Performance





Policy Milestones Despite a Tumultuous Year

Medicare improves reimbursement of CAR-T therapies

New rule allowing for innovative payment models in Medicaid

Draft guidance provides clarity on “sameness”

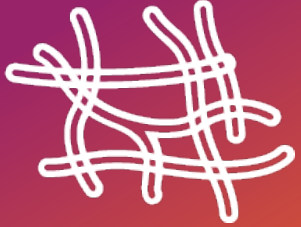
EU Pharma Strategy recognizes the impact of the sector



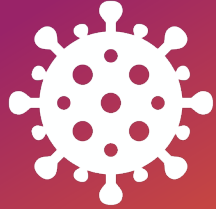


Looking Ahead to 2021

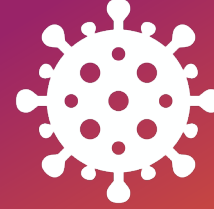
Upcoming Regulatory Decisions



Stratagraft (Mallinckrodt)
Severe burns



Ide-cel (bluebird, BMS)
Relapsed or refractory multiple myeloma



Cilta-cel (Janssen, Legend)
Relapsed or refractory multiple myeloma



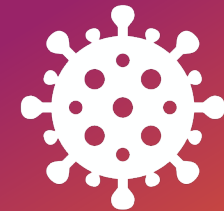
GT-AADC (PTC Bio)
AADC deficiency



Lumevoq (GenSight Bio)
Leber hereditary optic neuropathy



Eli-cel (bluebird bio)
Cerebral adrenoleukodystrophy



Breyanzi (BMS)
Relapsed or refractory large B-cell lymphoma



What's Next



Science & Technology

The Social Context



The Policy Environment



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