

State of the Cell & Gene Therapy Industry



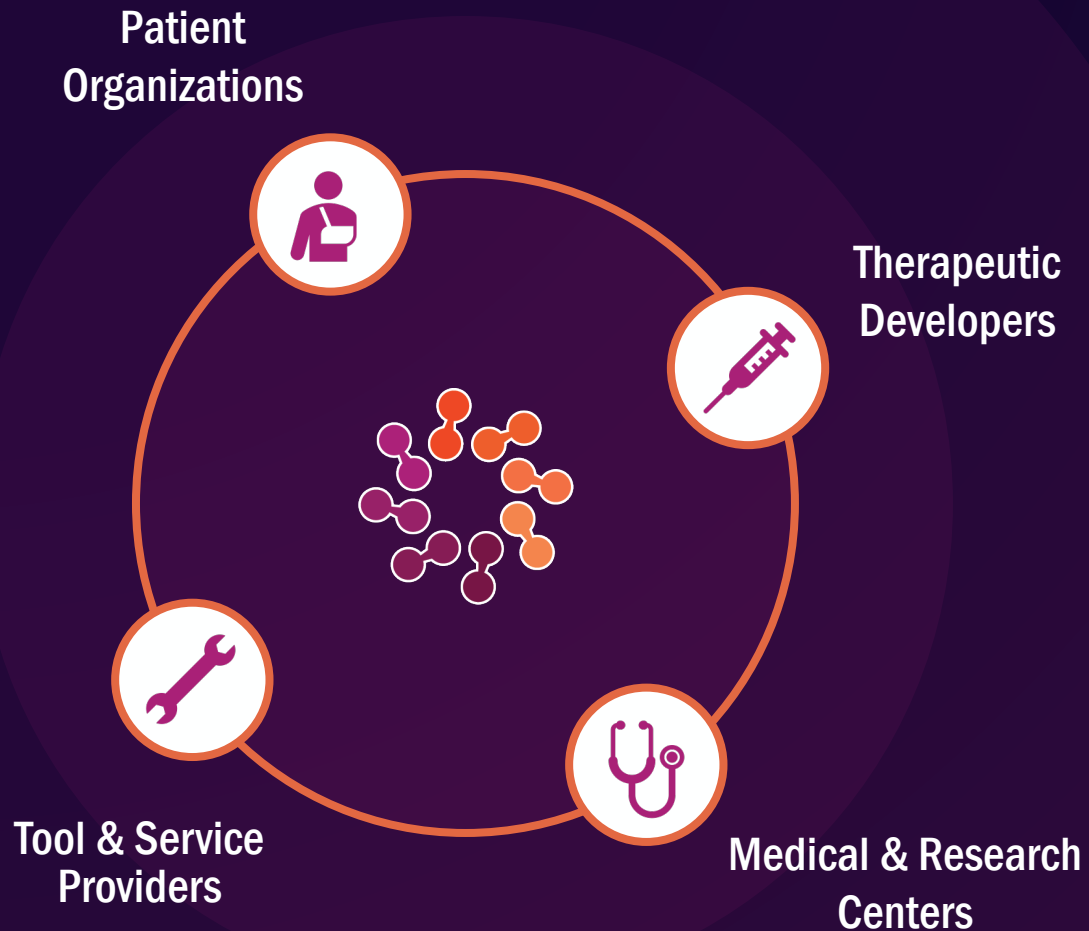
Janet Lambert, CEO

CASSS Cell & Gene Therapy Products 2021 Keynote
June 7, 2021



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 orange 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 (North America), 209 (Europe), 295 (Asia), 25 (Africa), and 13 (South America).

1,085

TOTAL GENE, CELL & TISSUE-
BASED THERAPEUTIC
DEVELOPERS WORLDWIDE

543

209

295

25

13

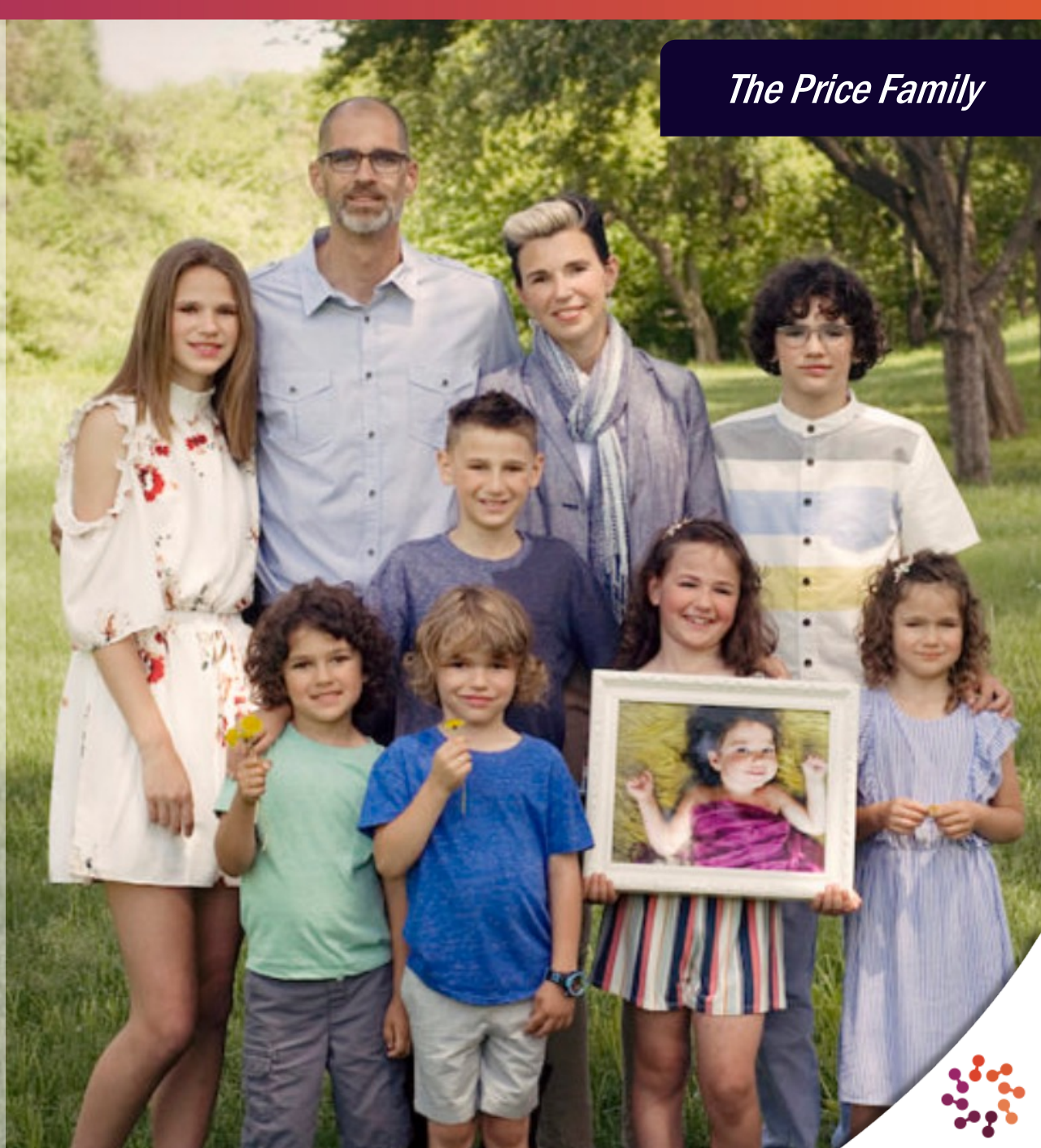


Patients continue to benefit from innovative therapies.

“It’s always really interesting to me – in pre-pandemic days – when I would attend conferences and I would sit in on the huge sessions where they’re talking about gene therapy as if it’s this fantastical futuristic thing. I’ve got photos on my phone of a child who is 10 years post gene therapy.”

-- Amy Price, mother of 2 gene therapy patients

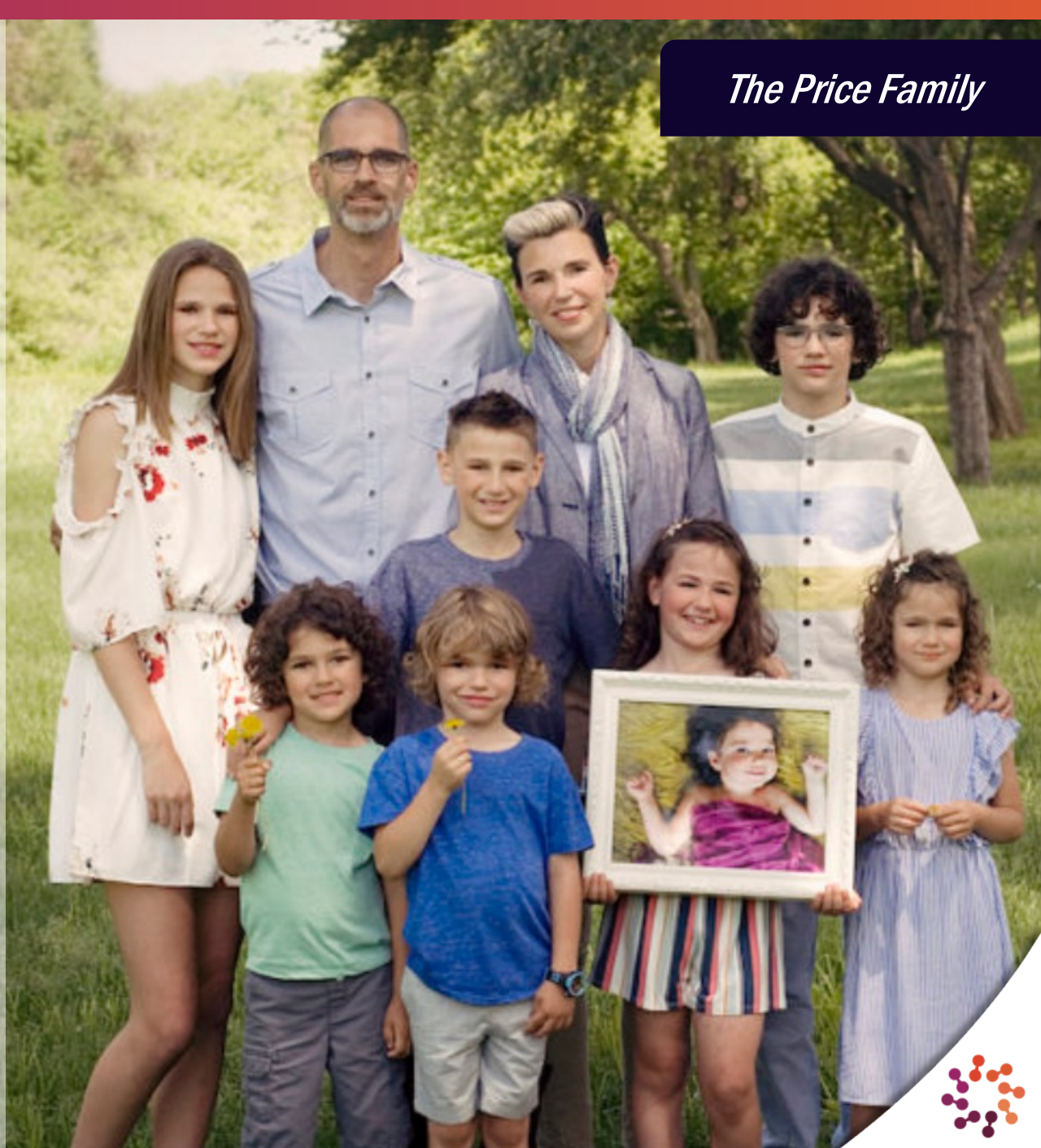
The Price Family



Patients continue to benefit from innovative therapies.

- Abecma (BMS & bluebird) – US, Canada
- Breyanzi (BMS) – US, Japan
- Libmeldy (Orchard Tx) – Europe
- Tecartus (Kite, a Gilead company) – US
- Zolgensma (Novartis Gene Therapies) – Europe, Japan, Canada
- Luxturna (Spark / Roche) – Canada

The Price Family



1,220 ONGOING REGENERATIVE MEDICINE &
ADVANCED THERAPY TRIALS WORLDWIDE

383

PH
1

152

PH
3

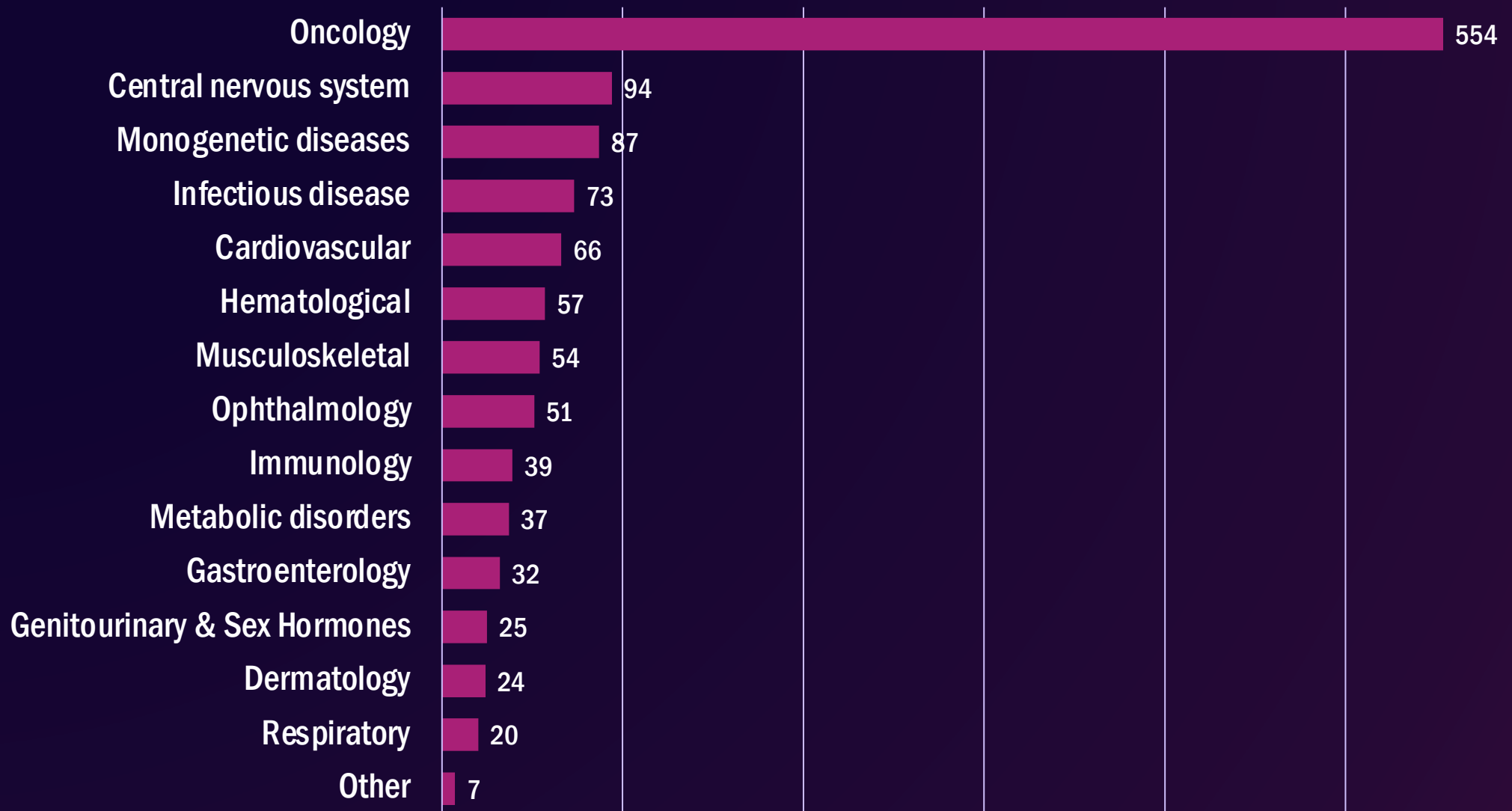
685

PH
2

*The late-stage clinical pipeline
continued to advance*



Clinical Trials by Indication





The Scientific and Clinical Landscape

Cell-based IO dominates cell therapy

New milestones for iPSCs

Gene editing advances in the clinic

Maturation of gene therapy





\$19.9B raised in 2020

Shattering previous annual financing records

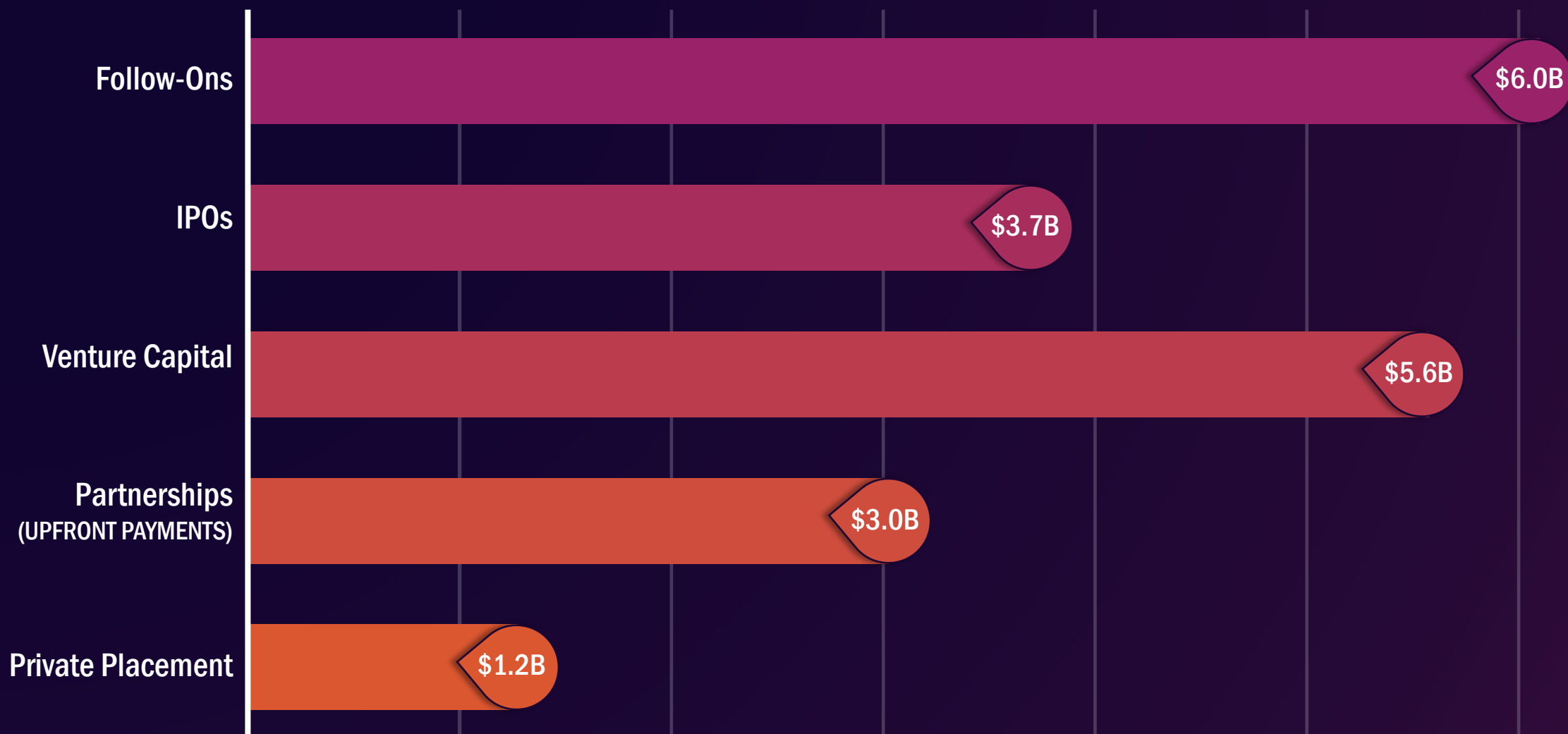
\$9.8B RAISED
IN 2019

\$13.3B RAISED
IN 2018

\$7.5B RAISED
IN 2017



Financing by Type



\$9B+ Raised in the First 5 Months of 2021

FOLLOW-ON FINANCINGS

- Fate Tx – \$432M (Jan 8)
- Iovance – \$350M (Feb 8)
- Beam Tx – \$300M (April 1)
- AGTC – \$250M (April 2)
- Editas – \$231M (Jan 21)
- REGENXBIO – \$230M (Jan 12)
- Generation Bio – \$225 (Jan 11)
- Mustang Bio – \$200M (April 23)
- Rubius Tx – \$200M (March 16)
- Solid Bio – \$144 (March 23)
- Collectis – \$125M (March 29)
- Krystal Bio – \$125M (Feb 2)

PRIVATE FINANCINGS

- CRISPR & Vertex – \$900M (April 20)
- Elevate Bio – \$525M (March 15)
- Beam Tx – \$260M (Jan 19)
- Tessara – \$230M (Jan 12)
- AmplifyBio – \$200M (May 3)
- G2 Bio – \$200M (May 18)
- Esco – \$200M (May 26)
- Century Tx – \$160M (March 3)
- Graphite Bio – \$150M (March 15)
- eGenesis – \$125M (March 2)

INITIAL PUBLIC OFFERINGS

Upcoming

- Lyell – \$150M
- Verve Tx – \$100M
- Miromatrix – \$20M

Closed

- Sana Biotech – \$588M (Feb 3)
- Instil Bio – \$320M (March 23)
- Immunocore – \$258M (Feb 4)
- Gracell Biotech – \$209M (Jan 7)
- Vor Biopharma – \$177M (Feb 9)
- Achilles – \$175M (March 30)
- Decibel – \$127M (Feb 11)
- NexImmune – \$119M (Feb 11)
- Talaris – \$100M (May 6)



A Challenging Environment

COVID-driven operational and clinical disruptions

Pandemic limited regulators' CGT bandwidth and travel

The politicization of COVID vaccines

Growing industry demands and fast-moving science





Policy & Market Access

Medicare reimbursement of CAR-T therapies

New rule allowing for innovative payment models in Medicaid

CURES 2.0 & other legislative approaches





Hundreds of thousands of patients could benefit from ATMPs in coming years.

A record number of regulatory decisions are expected this year:

- GT-AADC, a gene therapy to treat AADC deficiency (Europe)
- Eli-cel, a gene therapy to treat cerebral adrenoleukodystrophy (Europe)
- Lumevoq, a gene therapy to treat Leber hereditary optic neuropathy (Europe)
- Breyanzi, a CAR-T therapy for certain large B-cell lymphoma (Europe)
- Ide-cel, a CAR-T therapy for multiple myeloma (Europe)
- Cilta-cel, an additional CAR-T targeting multiple myeloma (US & Europe)
- Stratagraft, a tissue engineered product to treat severe burns (US)
- Donislecel, a cell therapy for brittle type 1 diabetes (US)



