

The Regenerative Medicine Landscape

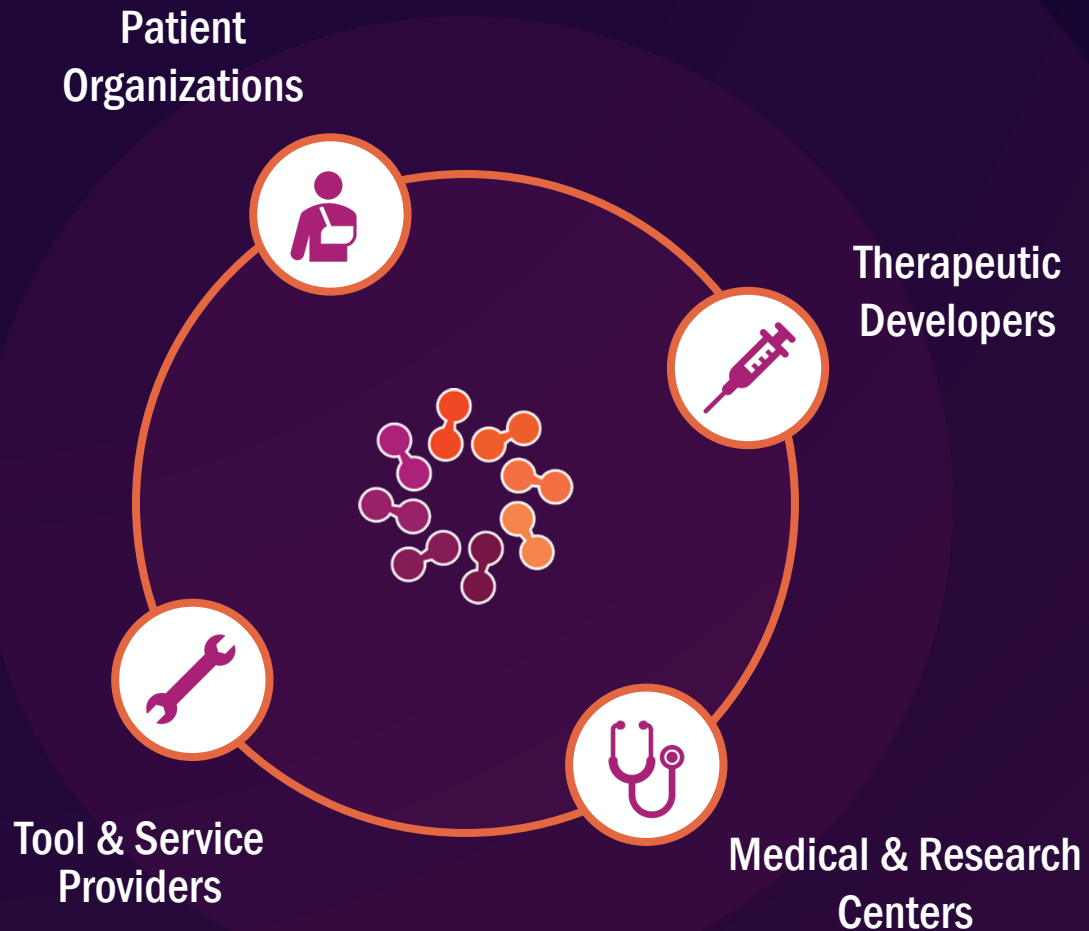
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Academy of Managed Care Pharmacy
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ARM is the Global Voice of the Sector

Representing 400+ members worldwide



Promote Clear Regulation
Enable Innovative Reimbursement
Address Manufacturing Barriers
Educate Stakeholders

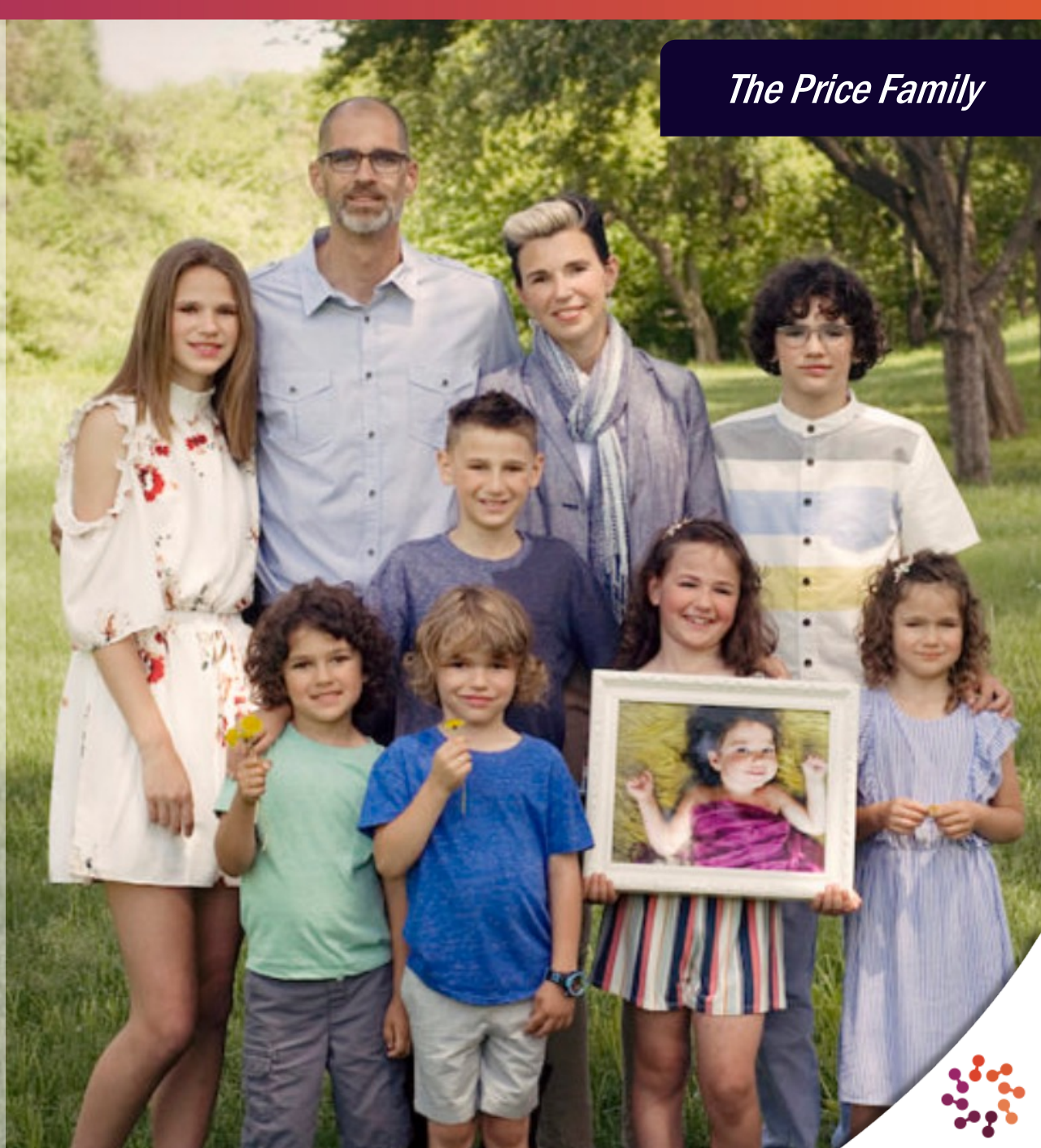


Patients continue to benefit from innovative therapies.

“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





The Scientific Fundamentals

Examples of Regenerative Medicine



Gene Therapy

Introduces a functioning gene into a patient's cells

One approved durable therapy for 'SMA1', a devastating genetic disorder that kills 90% of children who have it before their second birthday



Gene Editing

Inserts, replaces, removes or modifies DNA

Shows promise in clinical trials to durably treat or cure diseases including sickle-cell, which affects 1 in every 365 Black babies born in the US



Cell Therapy

Transfers cells into a patient to treat disease

5 approved CAR-T therapies to treat leukemia, lymphoma & multiple myeloma that are unresponsive to other treatments



Tissue-Based Therapy

Replaces damaged tissue with natural or synthetic tissue

FDA approved Stratagraft earlier this year to treat severe burns; products may also be used to repair or replace damaged organs



Key Concepts



In Vivo

A therapy that is delivered directly to the patient's body



Ex Vivo

Cells are taken from a patient and modified externally before being re-administered



Autologous

A therapy that is manufactured using a patient's own cells



Allogeneic

A universal or “off-the-shelf” therapy created with donor cells





Gene Delivery

Viral Vectors

- Deactivated virus -- adeno-associated viruses (AAV), lentiviruses

Non-Viral Vectors

- Nanoparticles, nanospheres, transposons, electroporation, and others



Gene Editing Technologies

- ZFNs and TALENs
- CRISPR/Cas9
- Base editing & prime editing
- Germline versus somatic cell gene editing





The Scientific and Clinical Landscape

CAR-T therapies as an earlier line of treatment

New BCMA-targeting therapies

Significant milestones for in vivo gene editing

iPSCs offer new promise for large scale therapies

Automation gaining traction





Sector Overview

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 gene, cell, and tissue-based therapeutic developers in that region. The circles are positioned over North America, Europe, Asia, South America, and Africa. The number 1,195 is shown in a large orange circle on the left side of the map.

1,195

TOTAL GENE, CELL & TISSUE-
BASED THERAPEUTIC
DEVELOPERS WORLDWIDE

594

8

209

23

361





\$14.1B raised in H1 2021

Already 71% of what was raised in FY 2020

\$19.9B RAISED
IN 2020

\$9.8B RAISED
IN 2019

\$13.3B RAISED
IN 2018



Cell-Based Immuno-Oncology Outraises Gene Therapy for the First Time



Cell-Based IO
\$6.6B



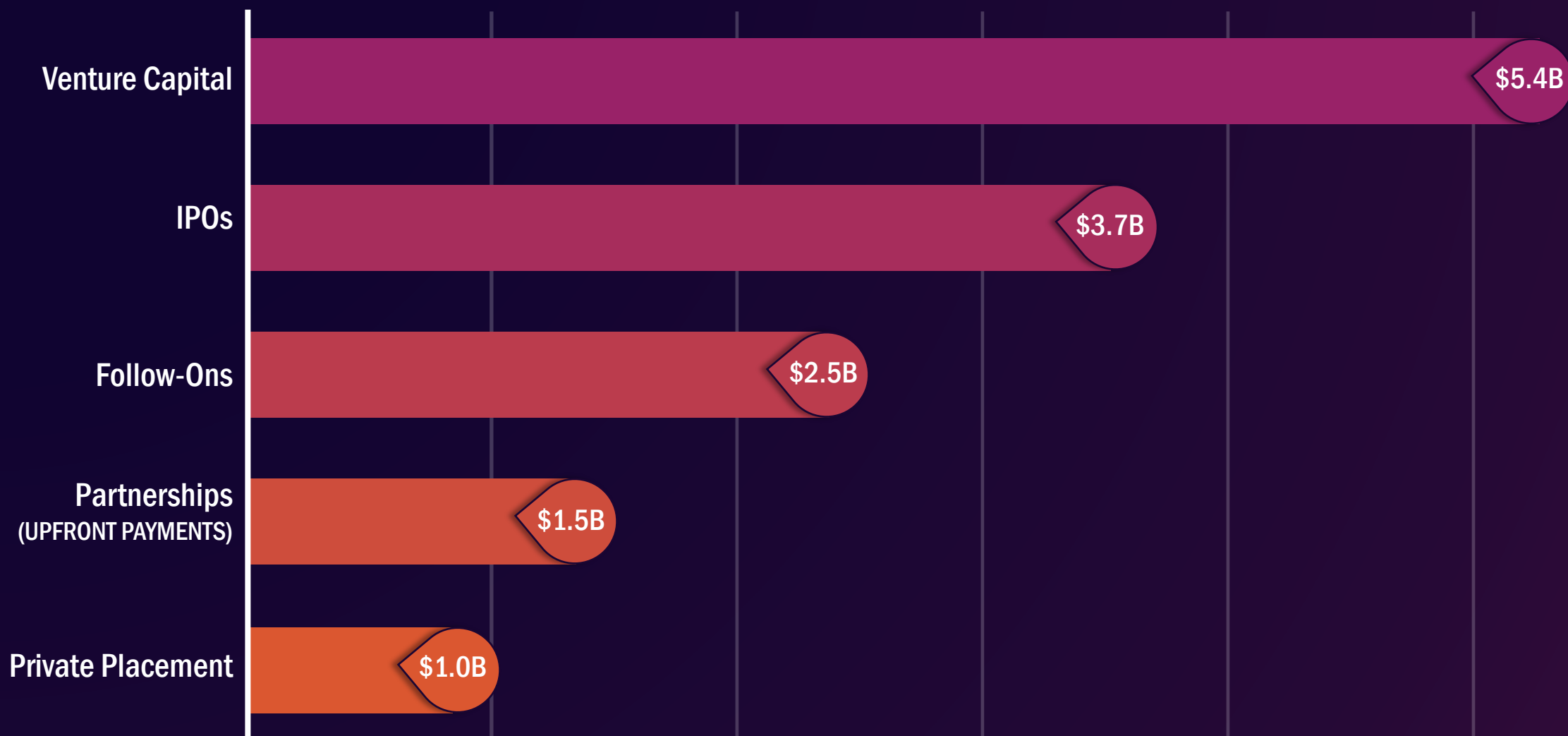
Gene Therapy
\$6.4B



Cell Therapy
\$1.1B



Financing by Type



2021 Poised to Break Financing Records

FOLLOW-ON FINANCINGS

- Intellia Tx – \$600M (July 2)
- 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)
- Blackstone LifeSci – \$250M
- Tessara – \$230M (Jan 12)
- Umoja Bio – \$210M (June 15)
- AmplifyBio – \$200M (May 3)
- G2 Bio – \$200M (May 18)
- Century Tx – \$160M (March 3)
- Graphite Bio – \$150M (March 15)
- Orchard Tx – \$150M (Feb 5)
- Gyroscope Tx – \$148M (March 26)
- eGenesis – \$125M (March 2)
- Artiva Bio – \$120M (Feb 26)
- Forge Bio – \$120M (April 29)
- Arcellx – \$115M (April 13)

INITIAL PUBLIC OFFERINGS

- Sana Biotech – \$675M (Feb 3)
- Lyell Immunopharma – \$425M (June 21)
- CARsgen – \$400M (June 18)
- Instil Bio – \$320M (March 23)
- Caribou Bio – \$304M (July 27)
- Graphite Bio – \$273M (July 2)
- Verve Tx – \$267M (June 21)
- Immunocore – \$258M (Feb 4)
- Century Tx – \$243M (June 22)
- Gracell Biotech – \$209M (Jan 7)
- Tenaya Tx – \$180M (July 30)
- Vor Biopharma – \$177M (Feb 9)
- MaxCyte – \$176M (July 30)
- Achilles – \$175M (March 30)
- Decibel – \$127M (Feb 11)
- NexImmune – \$119M (Feb 11)
- Talaris – \$100M (May 6)
- Tscan Tx – \$100M (July 20)
- Miromatrix – \$43M (June 28)



2,648 ONGOING REGENERATIVE MEDICINE &
ADVANCED THERAPY TRIALS WORLDWIDE

901

PH
1

1,504

PH
2

243

PH
3

*The late-stage clinical pipeline
continued to advance*



Ongoing Global Clinical Trials in Regenerative Medicine

1,354



CANCER

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

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

CARDIOVASCULAR

Including damage caused by heart attack and vascular disease

111



RARE DISEASES

Including many fatal diseases that affect infants and children

STROKE

Including stroke recovery and paralysis due to stroke

175



111



77



Examples of Approved Regenerative Medicines

2017

Kymriah
CAR-T Therapy
Novartis

Yescarta
CAR-T Therapy
Kite/Gilead

Luxturna
Gene Therapy
Spark Tx

2018

Alofisel
Cell Therapy
Takeda

2019

Zolgensma
Gene Therapy
Novartis Gene Tx

Zynteglo
Gene Therapy
bluebird bio

2020

Tecartus
CAR-T Therapy
Kite/Gilead

Libmeldy
Gene Therapy
Orchard Tx

2021

Breyanzi
CAR-T Therapy
BMS

Abecma
CAR-T Therapy
bluebird bio

Stratagraft
Tissue Therapy
Mallinckrodt

Skysona
Gene Therapy
bluebird bio

Relma-Cel
CAR-T Therapy
JW Therapeutics





Regulatory decisions expected on 10 new product candidates in 2021.

Could break the record of 9 products approved in 2016

Record 4-7 approvals expected on gene therapies & gene-modified cell therapies

Decisions expected on:

- **GT-AADC, a gene therapy to treat AADC deficiency (Europe)**
- **Lumevoq, a gene therapy to treat Leber hereditary optic neuropathy (Europe)**
- **Breyanzi, a CAR-T therapy for certain large B-cell lymphoma (Europe)**
- **Cilta-cel, an additional CAR-T targeting multiple myeloma (US & Europe)**
- **Lantidra, a cell therapy for brittle type 1 diabetes (US)**
- **RVT-802, a tissue-based therapy for pediatric congenital athymia (US)**





Access & Reimbursement

Market Access & Reimbursement

Removing barriers to innovative payment models

Using real-world evidence to guide reimbursement decisions

New DRG for innovative therapies





Innovative payment models are crucial for patient access

Examples of innovative payment models:

- **Outcomes-based models**
- **Installment or ‘annuity’ payments**
- **Subscription model**
- **Dedicated funds**

