



ARM: The Global Voice of the ATMP Sector

Stephen Majors, Director of Public Affairs

**ATMP World Tour
26 April 2021**

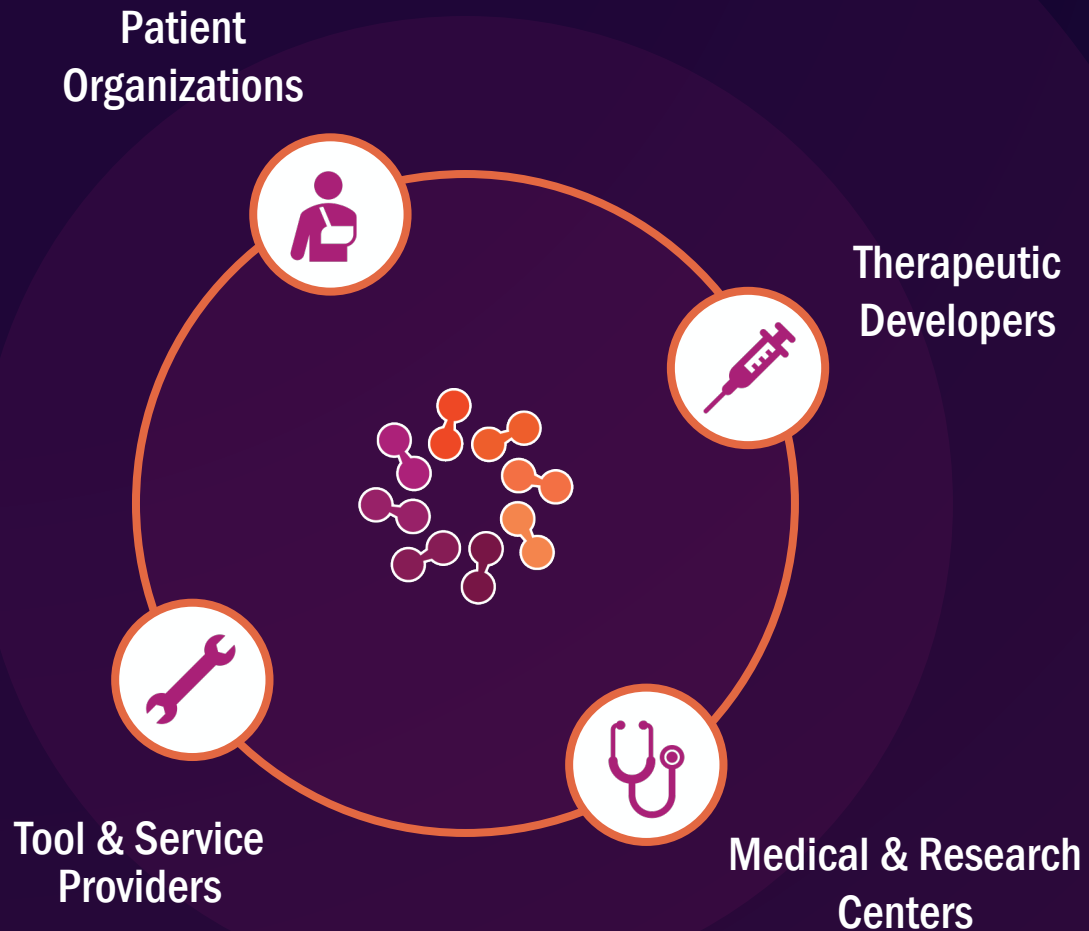




About ARM

ARM is the Global Voice of the Sector

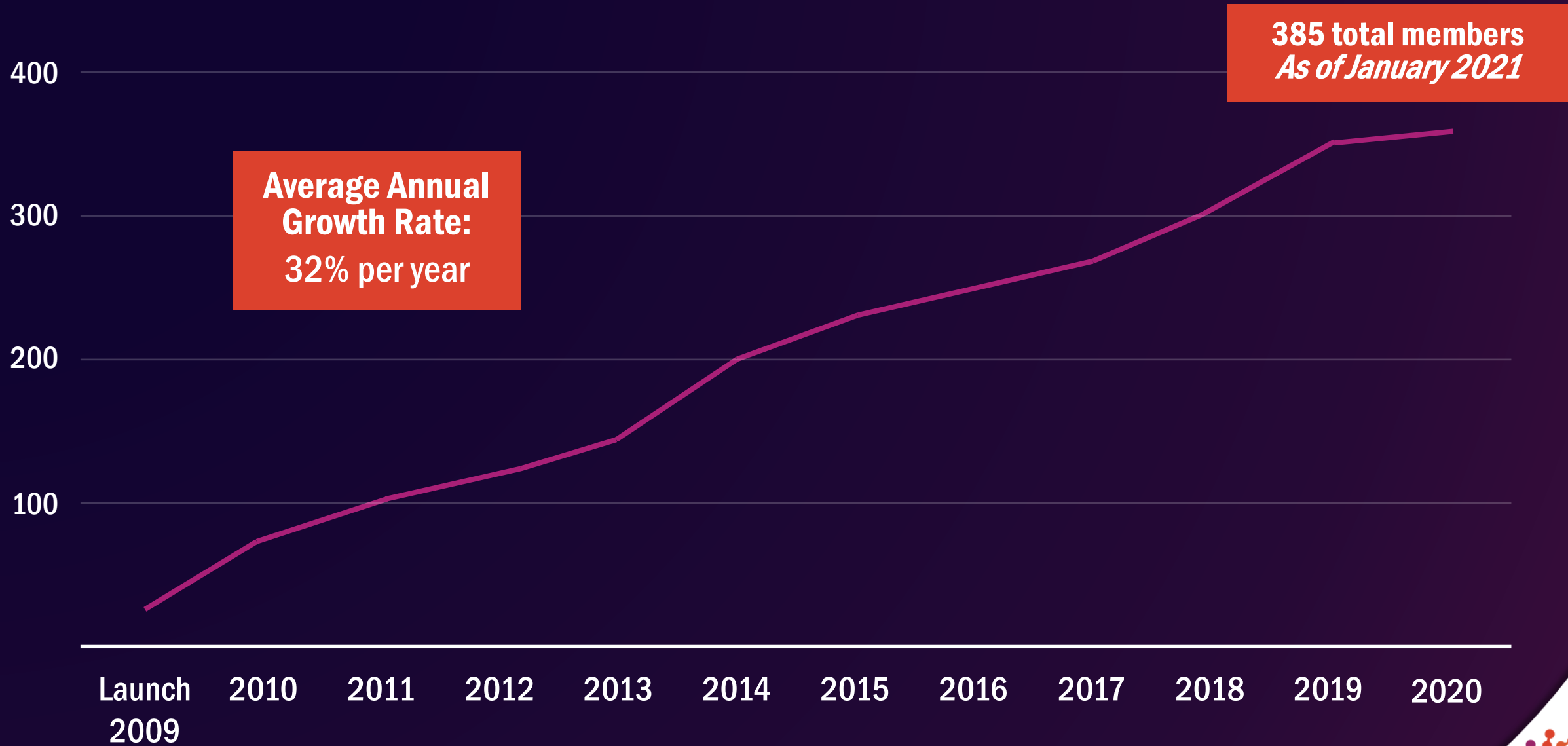
Representing 380+ members worldwide



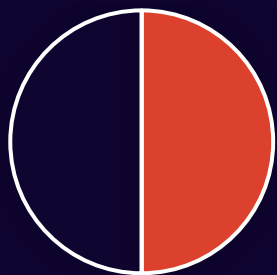
Promote Clear Regulation
Enable Innovative Reimbursement
Address Manufacturing Barriers
Educate Stakeholders



ARM Membership Growth

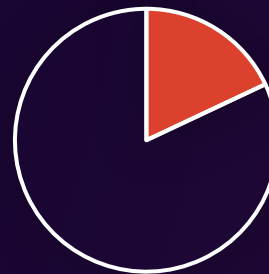


Diversity of Membership



50%

Corporations:
Rev < \$50 Mil, < 100 FTEs



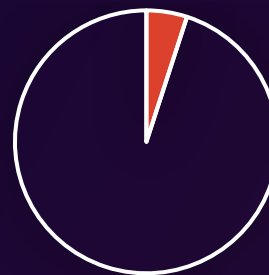
18%

Non-profit organizations



14%

Corporations:
Rev < \$50 Mil, > 100 FTEs



5%

Academic organizations



9%

Corporations:
Rev > \$50 Mil



5%

Financial & affiliate
organizations

Examples of ARM Members

Gene Therapy

- AGTC
- Amicus Tx
- AskBio
- Astellas Gene Therapies (Audentes)
- BioMarin
- bluebird bio
- Celgene
- Caribou Biosciences
- CRISPR Tx
- Editas Medicine
- Intellia Tx
- Orchard Tx
- Novartis
- Pfizer
- PTC
- Oxford BioMedica
- REGENXBIO
- Sangamo Tx
- Sarepta
- Spark Tx

Cell Therapy

- Akron Biotech
- AGC Biologics
- Atara Bio
- Athersys
- BlueRock Tx
- BMS
- CDI/Fujifilm
- Celyad
- Cytiva
- EMD Millipore
- GSK
- J&J
- Kiadis Pharma
- Kuur Therapeutics
- Minaris
- Novadip Bio
- ReNeuron Group
- Takeda Pharma
- Thermo Fisher
- Tmunity Tx

Tissue Engineering

- Ankasa
- Aspect Biosystems
- AxoGen
- Avery Tx
- Avita Medical
- AxoGen
- Bone Tx
- Castle Creek Bio
- DiscGenics
- Fibrocell Science
- Mesoblast
- MiMedx Group
- Miromatrix Medical
- Pluristem Tx
- STEL
- StemBioSys
- TERMIS – Americas
- Theradaptive
- Verigraft
- Videregen

Non-Profit Organizations

- CGT Catapult
- CCRM
- Chemelot Campus
- CIRM
- City of Hope
- Cleveland Clinic
- FARA
- Fondazione Telethon
- Fraunhofer Institute
- Fred Hutchinson
- GENETHON
- Global Genes
- LLS
- MJ Fox Foundation
- Missouri Cures
- MSK
- NHF
- Northwestern U.
- NYSCF
- UPENN



Member Committees

ARM members can join any/all member committees



Technology

Science & Technology
Committee

Annual Town Halls

Cell Therapy Interest Group

Tissue Engineering Interest Group

Gene & Gene-Modified Cell Therapy
Interest Group

Task Forces

Regulatory-CMC Task Force

Gene Editing Task Force



Policy

Market Access & Value Committee
– U.S. & Europe

Regulatory Affairs Committee –
U.S. & Europe

U.S. Government Affairs Committee

Meeting Schedule

Monthly

Quarterly

Annually

As Needed



Cross-Cutting

The Accelerator

Patient Engagement Committee

Public Affairs Town Halls

**By Invitation Only:
Advisory Committees**

*Gene Therapy, Cell Therapy, Tissue
Engineering, & European Affairs*





U.S. Advocacy Priorities: 2021

Drive clarity & flexibility in CMC regulation

Advance innovative payment models through removal of statutory and regulatory barriers

Modernize Medicare's IPPS rules

Build awareness of CGT & innovative payment models in Texas, Ohio



European Advocacy Priorities: 2021

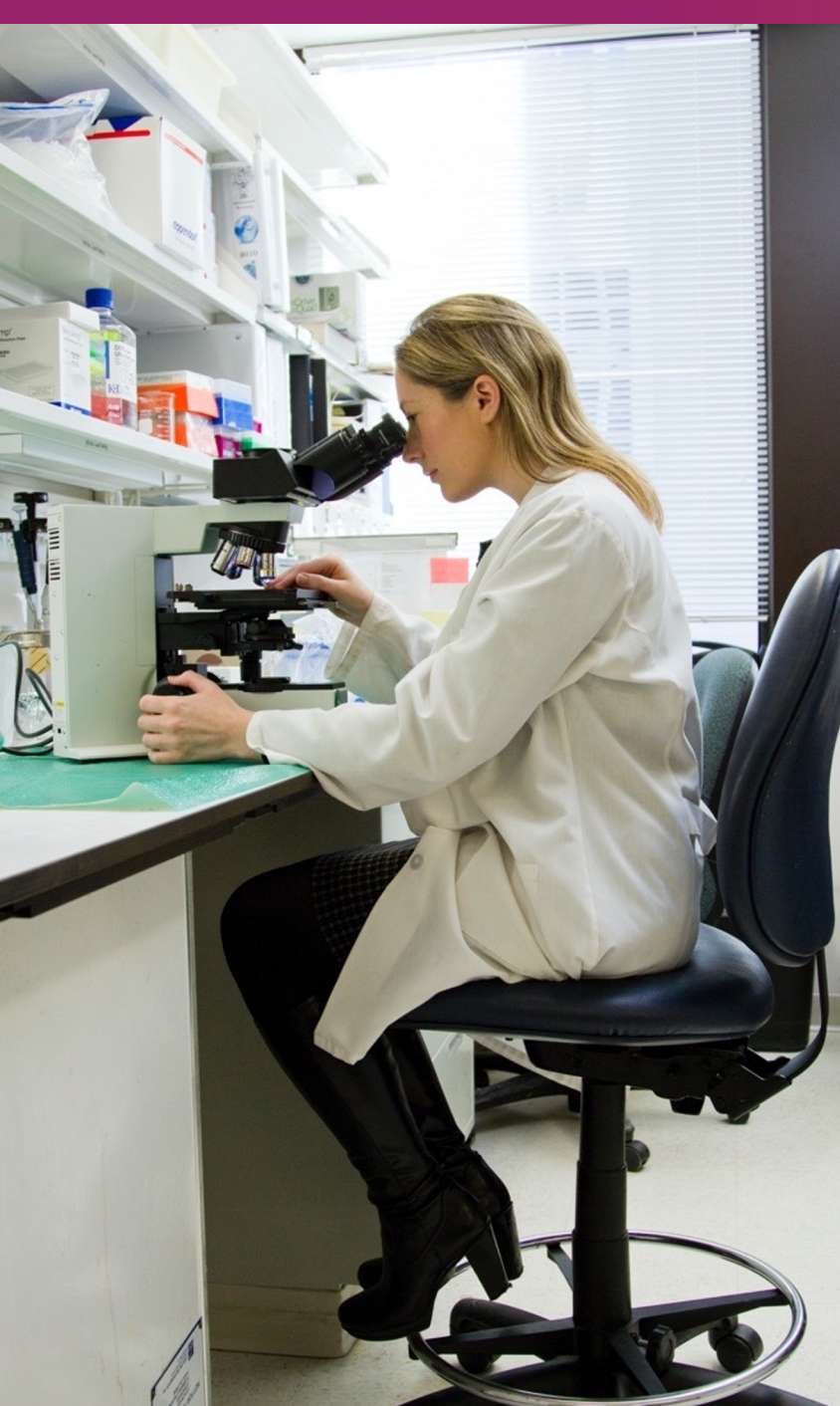
Increase ARM's political influence in Brussels

Prevent deregulation of the ATMP sector

Ensure real-world evidence (RWE) is incorporated into HTA frameworks across Europe

Advocate with payers in Germany, France, Italy and the UK to remove barriers to innovative payment models





2021 Science & Industry Affairs Priorities

Drive clarity & flexibility in CMC regulation

Develop quality by design principles for cell and gene therapy manufacturing

Influence global dialogue on gene editing

Support investor engagement



ARM Milestones



RMAT Designation

FDA pathway to bring expedite safe & effective therapies



NIH RAC Reform

Removes duplicative requirements for gene therapy trials



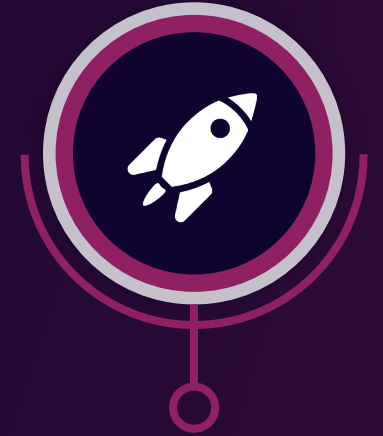
CAR-T DRG

Ensures providers are appropriately reimbursed



New Medicaid Rule

Allows state Medicaid programs to use innovative payment models



Pharma Strategy

Identified cell & gene therapies as “milestones of major progress”

Annual ARM Events



State of the Industry
January 13, 2021 | Virtual



Meeting on the Med
April 6-9, 2021 | Virtual



**Washington Forum &
Legislative Fly-In**
May 19 | Virtual



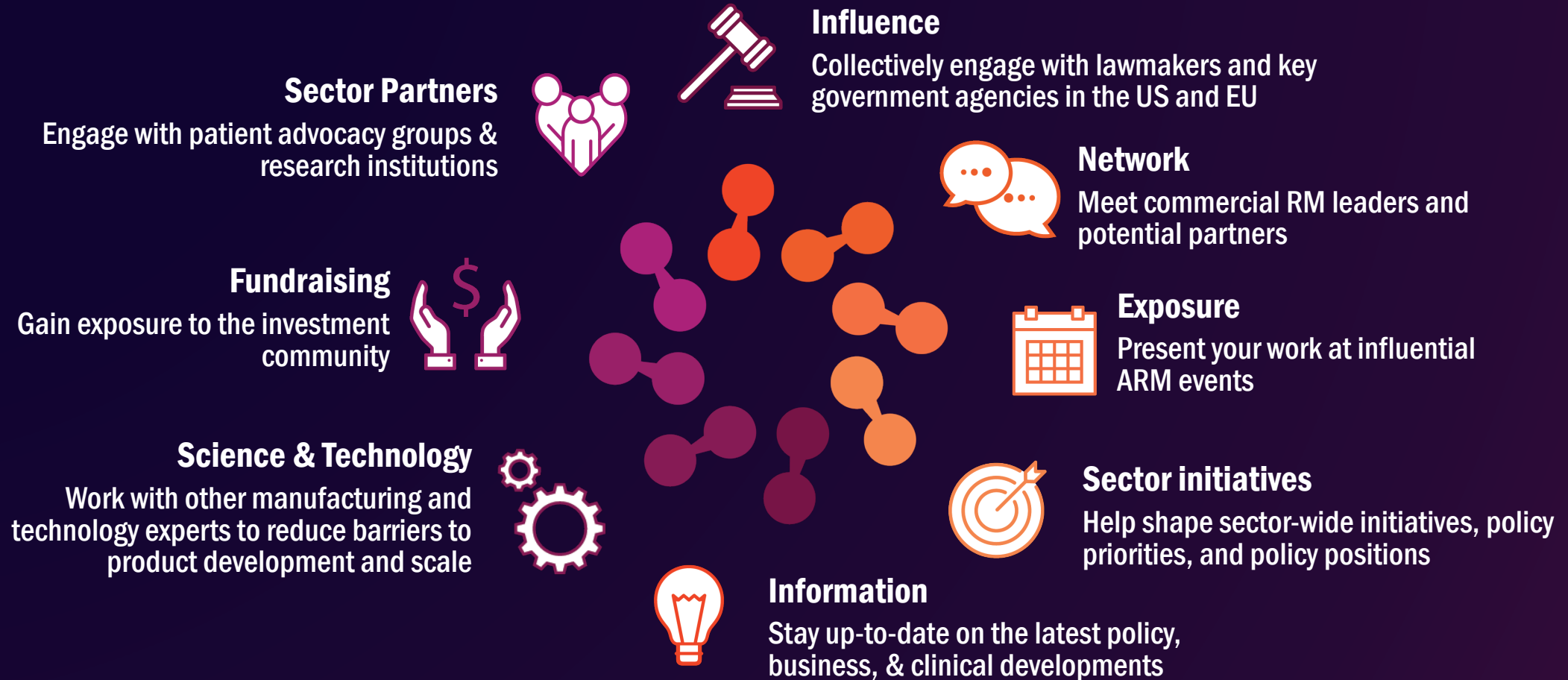
Meeting on the Mesa
Fall 2021 | Carlsbad, CA



Cell & Gene Webinars
Held Periodically



Benefits of Membership





State of the Sector

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, 13, 209, 25, and 295 respectively.

1,085

TOTAL GENE, CELL & TISSUE-
BASED THERAPEUTIC
DEVELOPERS WORLDWIDE

543

13

209

25

295





\$19.9B raised in 2020

Shattering previous annual financing records

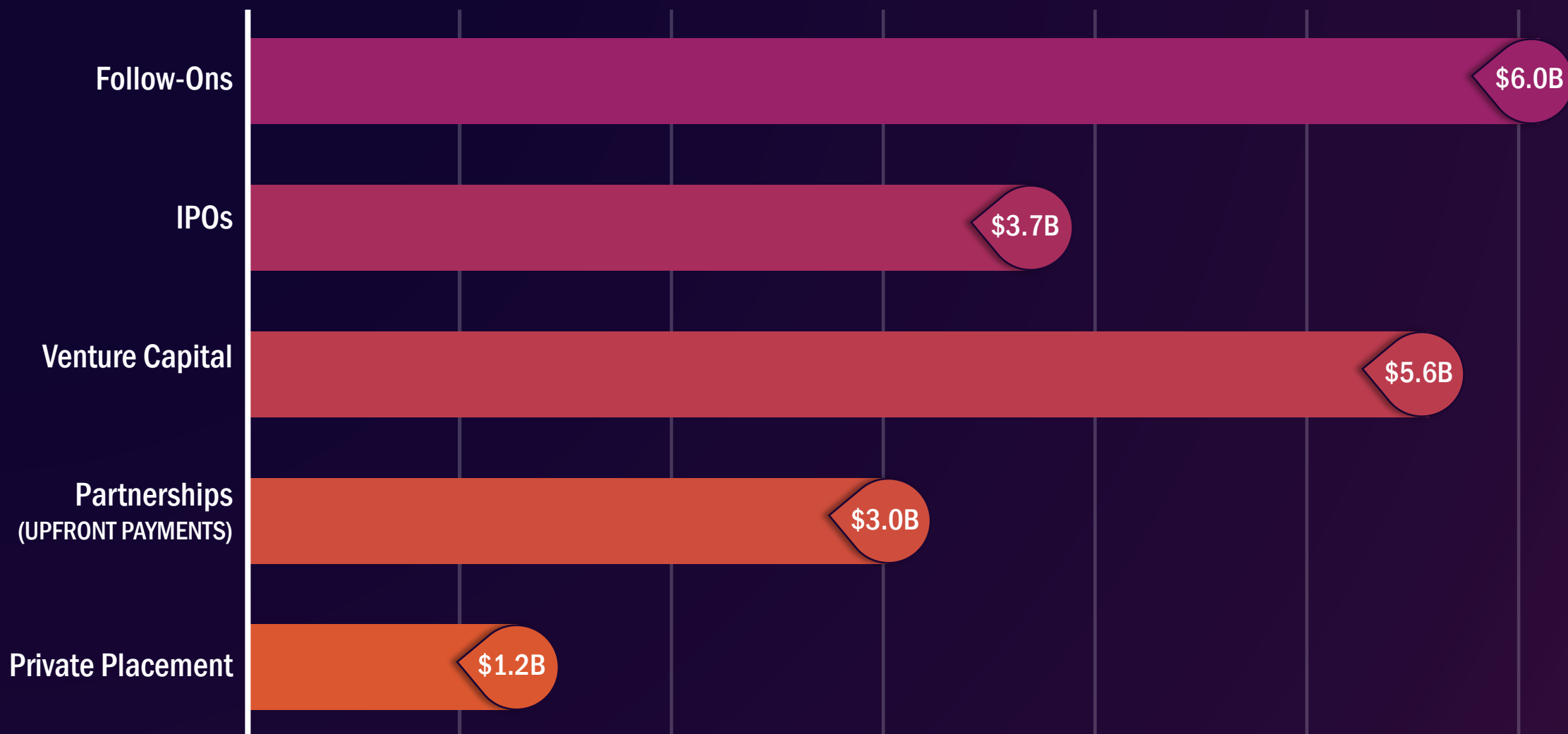
\$9.8B RAISED
IN 2019

\$13.3B RAISED
IN 2018

\$7.5B RAISED
IN 2017



Financing by Type



\$200M+ Financings Flourish

PRIVATE FINANCINGS

- Sana Bio – \$700M (Jun 23)

FOLLOW-ON FINANCINGS

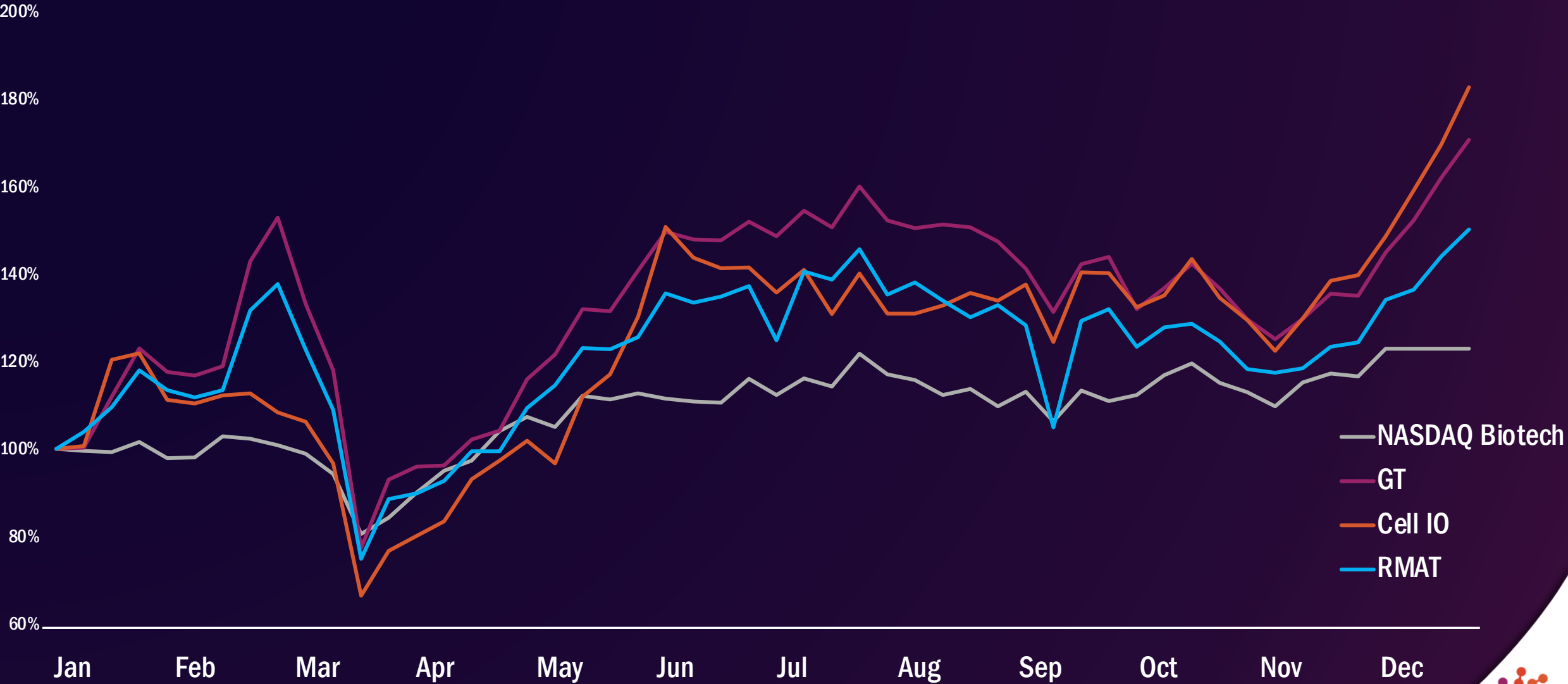
- | | |
|-------------------------------|-------------------------------|
| • Iovance – \$604M (Jun 2) | • Editas – \$216M (June 23) |
| • bluebird– \$575M (May 18) | • Atara Bio – \$202M (Jun 24) |
| • Allogene – \$550M (Jun 1) | • Fate Tx – \$201M (Jun 11) |
| • Rocket – \$299M (Dec 14) | • Intellia -- \$201M (Dec 4) |
| • Adaptimmune– \$259M (Jun 4) | |

INITIAL PUBLIC OFFERINGS

- Legend Bio – \$487M (Jun 9)
- Passage Bio – \$284M (Feb 3)
- Akouos – \$244M (Jun 25)
- Generation Bio – \$230M (Jun 12)
- 4D Molecular -- \$222M (Dec 15)
- Beam Tx – \$207M (Feb 11)
- JW Therapeutics (Nov 3)



Public Performance



1,220 ONGOING REGENERATIVE MEDICINE &
ADVANCED THERAPY TRIALS WORLDWIDE

383

PH
1

PH
3

152

PH
2

685

*The late-stage clinical pipeline
continued to advance*



Ongoing Clinical Trials in Regenerative Medicine



CANCER

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

RARE DISEASES

Including many fatal diseases that affect infants and children



CARDIOVASCULAR

Including damage caused by heart attack and vascular disease

NEUROLOGICAL

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



DIABETES

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

STROKE
Including stroke recovery and paralysis due to stroke





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)
- Stratagraft, a tissue engineered product to treat severe burns (US)
- Donislecel, a cell therapy for brittle type 1 diabetes (US)



Thank you! To learn more, visit:
www.alliancerm.org



Voice of the Sector
Monthly Member Update

4,000+ readers



The Sector Vector
Weekly ATMP Newsletter

15,000+ readers



Global Sector Reports
Policy, Financial, & Clinical Landscape

5,000+ readers

Plus access ARM publications, presentations, & more!

