

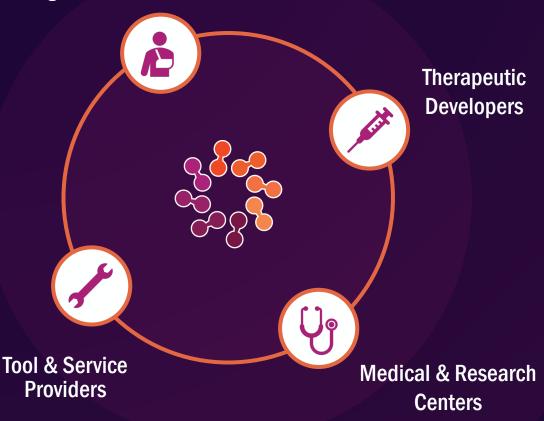
2021 CELL GENE STATE of the INDUSTRY BRIEFING

Janet Lambert, CEO January 13, 2021

ARM is the Global Voice of the Sector

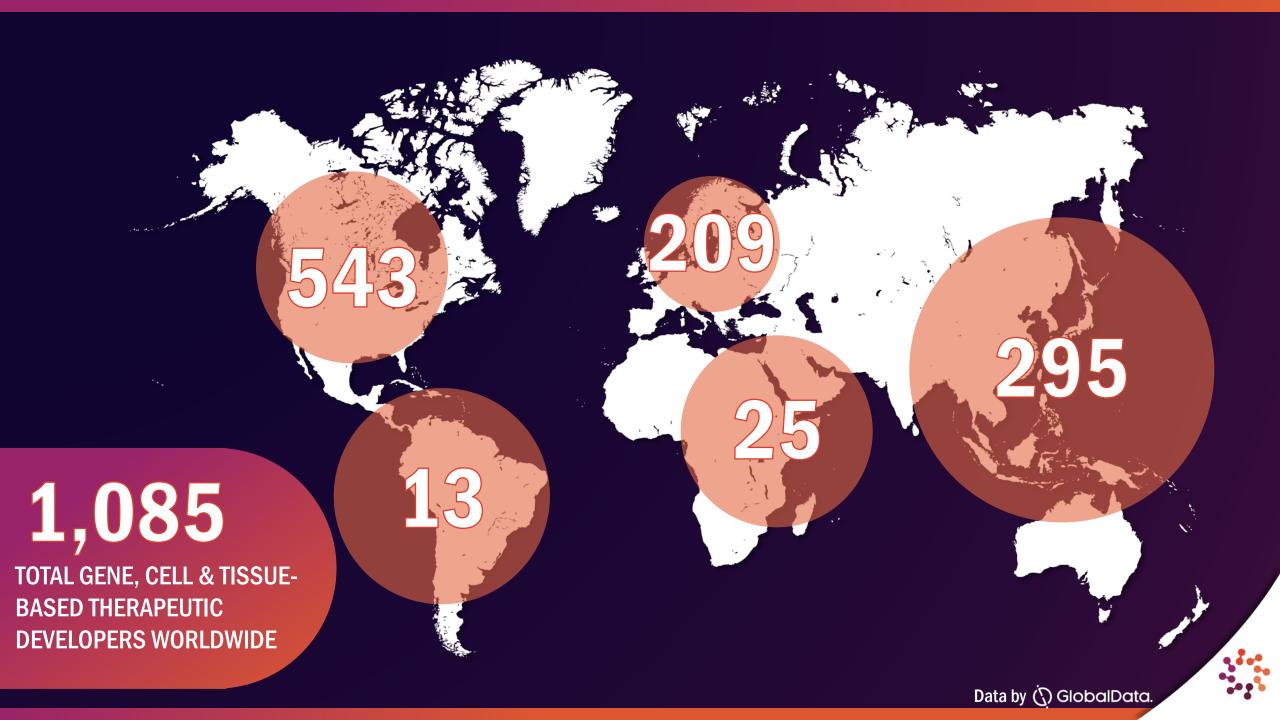
Representing 370+ members worldwide

Patient Organizations



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 prepandemic 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."

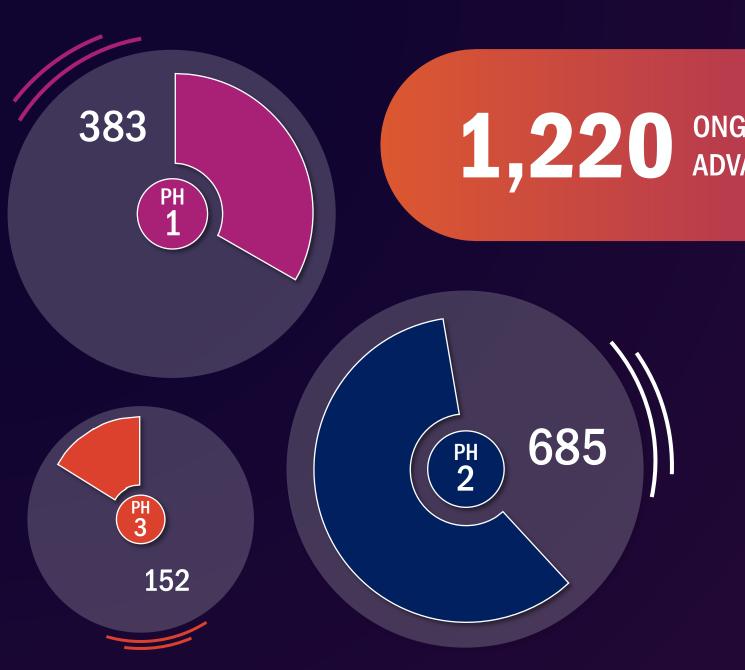
-- Amy Price, mother of 2 gene therapy patients



Patients continue to benefit from innovative therapies.

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

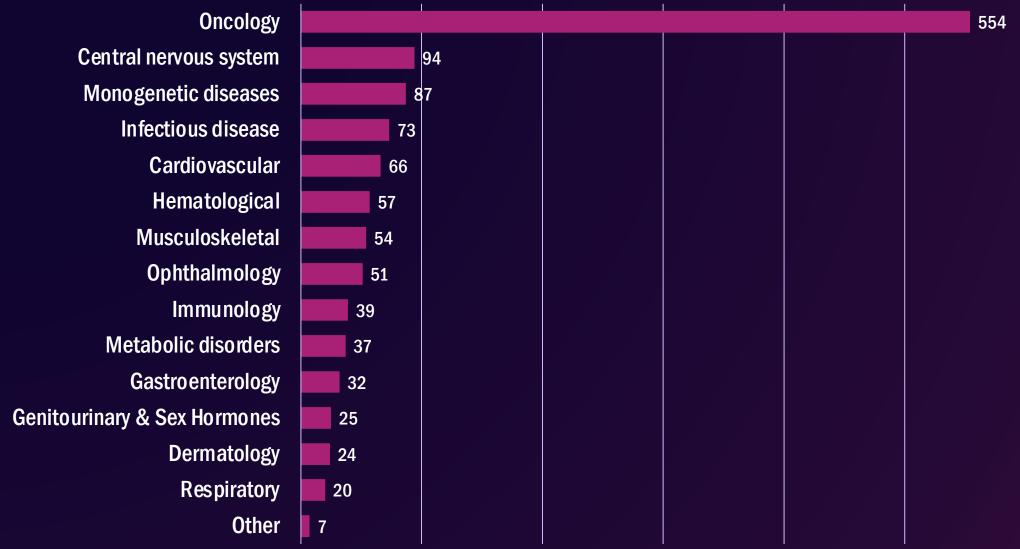




1,220 ONGOING REGENERATIVE MEDICINE & ADVANCED THERAPY TRIALS WORLDWIDE

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



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





\$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)
- bluebird \$575M (May 18)
- Allogene \$550M (Jun 1)
- Rocket \$299M (Dec 14)
- Adaptimmune \$259M (Jun 4)

- Editas \$216M (June 23)
- Atara Bio \$202M (Jun 24)
- Fate Tx \$201M (Jun 11)
- Intellia -- \$201M (Dec 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)



Large Pharma Partners with Innovative Developers

Partnership	Upfront Payment	Milestones	Technology	Indication
Biogen & Sangamo	\$350M	\$2.7B	Gene therapy	Neurodegenerative disorders
Janssen & Fate Tx	\$100M	\$2.1B	Cell-based IO	Hematologic & solid tumors
Novartis & Sangamo	\$75M	\$795M	Gene therapy	Neurodevelopmental disorders
Merck & A2 Bio	Undisclosed	Undisclosed	Cell-based IO	Solid tumors
Bayer & Atara	\$60M	\$610M	Cell-based IO	Solid tumors
Novartis & Mesoblast	\$50M	Undisclosed	Cell therapy	ARDS caused by COVID-19
Roche & Dyno Tx	n/a	\$1.8B	Gene therapy	Central nervous system diseases



Public Performance





Policy Wins Despite a Tumultuous Year

Medicare improves reimbursement of CAR-T therapies

New rule allowing for innovative payment models in Medicaid

EU Pharma Strategy recognizes the impact of the sector

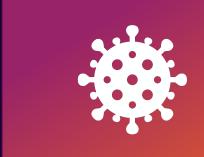




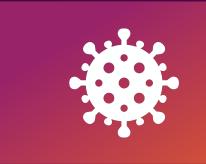
Upcoming Regulatory Decisions



Stratagraft (Mallinckrodt) Severe burns



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



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



GT-AADC (PTC Bio)AADC deficiency



Lumevoq (GenSight Bio)Leber hereditary optic neuropathy



Eli-cel (bluebird bio)Cerebral
adrenoleukodystrphy









What's Next

Science & Technology

The Social Context

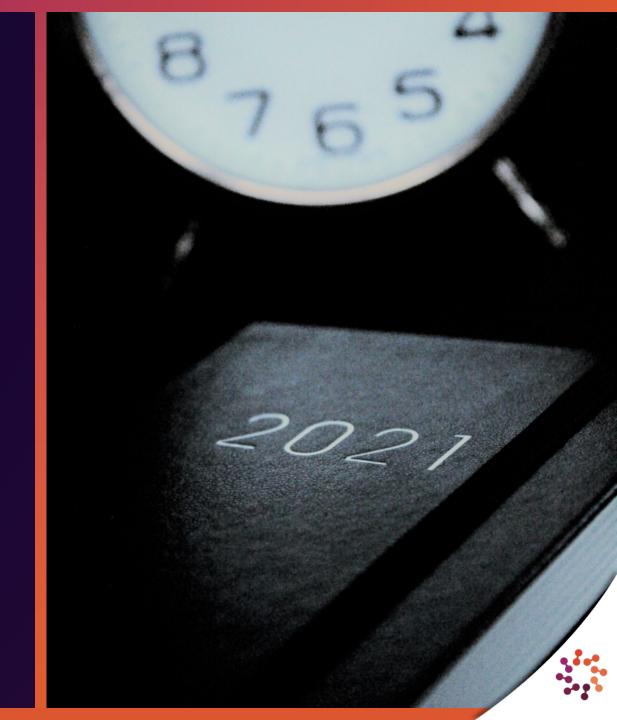
The Policy Environment





Thank you!

Slides and other resources available at www.alliancerm.org





Looking Ahead – What to Expect for the Cell & Gene Therapy Sector in 2021 and Beyond

Terry-Ann Burrell, Chief Financial Officer, Beam Therapeutics *(moderator)*

Marianne De Backer, Member of Executive Committee and EVP, Head of Strategy and Business Development & Licensing, Pharmaceuticals Division, Bayer

Sandy Macrae, President & CEO, Sangamo Therapeutics

Ken Mills, CEO, REGENXBIO

Adrian Rawcliffe, CEO, Adaptimmune







Terry-Ann Burrell

Chief Financial Officer, Beam TX

