ARM is the Global Voice of the Sector

Representing 370+ members worldwide

Promote Clear Regulation
Enable Innovative Reimbursement
Address Manufacturing Barriers
Educate Stakeholders
TOTAL GENE, CELL & TISSUE-BASED THERAPEUTIC DEVELOPERS WORLDWIDE

1,085

Data by GlobalData
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
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
The late-stage clinical pipeline continued to advance.
Clinical Trials by Indication

- Oncology: 554
- Central nervous system: 94
- Monogenetic diseases: 87
- Infectious disease: 73
- Cardiovascular: 66
- Hematological: 57
- Musculoskeletal: 54
- Ophthalmology: 51
- Immunology: 39
- Metabolic disorders: 37
- Gastroenterology: 32
- Genitourinary & Sex Hormones: 25
- Dermatology: 24
- Respiratory: 20
- Other: 7

Data by GlobalData
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

- Follow-Ons: $6.0B
- IPOs: $3.7B
- Venture Capital: $5.6B
- Partnerships (UPFRONT PAYMENTS): $3.0B
- Private Placement: $1.2B

Data by GlobalData.
$200M+ Financings Flourish

### PRIVATE FINANCINGS
- Sana Bio – $700M (Jun 23)

### 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)

### 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)
Large Pharma Partners with Innovative Developers

<table>
<thead>
<tr>
<th>Partnership</th>
<th>Upfront Payment</th>
<th>Milestones</th>
<th>Technology</th>
<th>Indication</th>
</tr>
</thead>
<tbody>
<tr>
<td>Biogen &amp; Sangamo</td>
<td>$350M</td>
<td>$2.7B</td>
<td>Gene therapy</td>
<td>Neurodegenerative disorders</td>
</tr>
<tr>
<td>Janssen &amp; Fate Tx</td>
<td>$100M</td>
<td>$2.1B</td>
<td>Cell-based IO</td>
<td>Hematologic &amp; solid tumors</td>
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<tr>
<td>Novartis &amp; Sangamo</td>
<td>$75M</td>
<td>$795M</td>
<td>Gene therapy</td>
<td>Neurodevelopmental disorders</td>
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<tr>
<td>Merck &amp; A2 Bio</td>
<td>Undisclosed</td>
<td>Undisclosed</td>
<td>Cell-based IO</td>
<td>Solid tumors</td>
</tr>
<tr>
<td>Bayer &amp; Atara</td>
<td>$60M</td>
<td>$610M</td>
<td>Cell-based IO</td>
<td>Solid tumors</td>
</tr>
<tr>
<td>Novartis &amp; Mesoblast</td>
<td>$50M</td>
<td>Undisclosed</td>
<td>Cell therapy</td>
<td>ARDS caused by COVID-19</td>
</tr>
<tr>
<td>Roche &amp; Dyno Tx</td>
<td>n/a</td>
<td>$1.8B</td>
<td>Gene therapy</td>
<td>Central nervous system diseases</td>
</tr>
</tbody>
</table>
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
Looking Ahead to 2021
Upcoming Regulatory Decisions

Stratagraft (Mallinckrodt)
Severe burns

GT-AADC (PTC Bio)
AADC deficiency

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

Lumevoq (GenSight Bio)
Leber hereditary optic neuropathy

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

Eli-cel (bluebird bio)
Cerebral adrenoleukodystrophy
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