The Alliance for Regenerative Medicine (ARM) is the leading international advocacy organization dedicated to realizing the promise of regenerative medicines and advanced therapies. ARM promotes legislative, regulatory, reimbursement, and manufacturing initiatives to advance this innovative and transformative sector, which includes cell therapies, gene therapies and tissue-based therapies.

Early products to market have demonstrated profound, durable and potentially curative benefits that are already helping thousands of patients worldwide, many of whom have no other viable treatment options. Hundreds of additional product candidates contribute to a robust pipeline of potentially life-changing regenerative medicines and advanced therapies.

In its 12-year history, ARM has become the global voice of the sector, representing the interests of 425+ members worldwide, including small and large companies, academic research institutions, major medical centers and patient groups.

To learn more about ARM or to become a member, visit www.alliancerm.org.
ARM Annual Report

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The regenerative medicine sector challenged conventional thinking in 2021, redefining our expectations for what we can accomplish. Clinical advancements included the first proof-of-concept for an in vivo gene editing therapy, evidence that CAR-T therapies compare favorably with earlier-line treatments, and compelling early results that cell and gene therapies could durably treat complex, polygenic diseases.

Nearly 60% of the 2,400 ongoing regenerative medicine trials at the end of 2021 targeted prevalent diseases, a clear indication of the sector’s evolution. This category is diverse and expanding, from common cancers to diabetes to Parkinson’s.

Cell and gene therapy continues, however, to hold great promise for treating rare diseases with high unmet medical needs. 2022 is likely to be a record year for the approval of new gene therapies to treat rare diseases, with five such therapies up for approval in the US and Europe. Two therapies to treat sickle cell disease could be available in the US as soon as 2023 — one a gene therapy and one that would be the first-ever CRISPR therapy. There are now more regenerative medicine trials ongoing for sickle cell disease than for any other rare disease.

These scientific advancements are possible because of the robust investment that has poured into the sector. In 2021, investment reached $22.7B on the strength of venture capital, easily surpassing the 2020 record of $19.9B. And the steady growth of investment in recent years is bearing fruit for patients. 2021 was the second best year for new product approvals, with six new therapies globally, and the best year for CAR-T products, with three new approvals. The public markets were challenging in 2021 for gene therapy and other small biotech companies, and headwinds are continuing in 2022. But the scientific foundation upon which our sector rests remains strong.

ARM itself grew substantially in 2021, hiring 13 new team members to serve our now 425-strong membership and to help the sector challenge the status quo. We hired our first ever full-time government relations lead in Brussels to ensure that Europe remains globally competitive in cell and gene therapy and can deliver transformative treatments to European patients. We raised the sector’s profile at a crucial time, meeting with key European decision-makers on topics including GMO, BTC and the Pharmaceutical Strategy, while also securing a legislative change in Germany that makes it easier for hospitals to get reimbursed when providing ATMPs.

In the US, ARM’s advocacy helped to secure increased staffing for cell and gene therapy reviewers, language promoting CMC clarity and flexibility, and more use of real-world evidence in the PDUFA VII agreement. Our advocacy secured some of these same priorities in ‘CURES 2.0’. We will focus heavily
on the US in 2022, advocating for innovative payment models, the passage of PDUFA and our CURES priorities, and for the value of cell and gene therapy for patients, healthcare systems, society writ-large. In January, we hired our first lead of US Government Relations to help achieve these goals.

Our Science and Industry team, in partnership with our members, completed Project A-Gene, a case study applying quality-by-design standards to the manufacture of an AAV vector. Companies in the sector are already using the project as a how-to manual, while university programs are using it to train the future cell and gene therapy workforce.

For too long, a lack of diversity has been the status quo in the cell and gene therapy sector and in the biotech industry overall. In the first year of ARM GROW RegenMed Internship Program, ARM and of our members hosted Black students for summer internships across a range of functions. The number of students in the GROW program is on track to increase by around 50%, with many companies offering in-person opportunities.

Continuing to challenge conventional thinking means amplifying ARM’s role as the global voice of the sector. ARM plays a crucial role in providing the most relevant data and analysis about the sector, helping to set the agenda with policymakers and the media. Outlets including the Associated Press, Marketplace by APM, and Politico looked to ARM in 2021 to help tell the story of our sector. We are investing more in this capability in , and are pleased to share more granular data on rare and prevalent diseases, gene-editing, vectors, and liquid and solid tumors for the first time ever in the pages of this report.

The data reveal the incredible work of our members. We at ARM are dedicated to challenging the conventional wisdom and reorienting how the public and policymakers think about healthcare, so that all of this promise results in life-changing therapies for patients.

Janet Lambert
Chief Executive Officer
Alliance for Regenerative Medicine
## TRANSFORMATIVE THERAPIES REACH PATIENTS

### Product Approvals in 2021 & YTD 2022

<table>
<thead>
<tr>
<th>Product Name</th>
<th>Developer(s)</th>
<th>Indications</th>
<th>Regions Approved</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Breyanzi (CAR-T therapy)</strong></td>
<td>Bristol Myers Squibb</td>
<td>R/R large B-cell lymphoma</td>
<td>US (Feb 2021), Japan (March 2021)</td>
</tr>
<tr>
<td><strong>Abecma (CAR-T therapy)</strong></td>
<td>Bristol Myers Squibb &amp; bluebird bio</td>
<td>R/R multiple myeloma</td>
<td>US (March 2021), Canada (May 2021), EU (Aug 2021), Japan (Feb 2022)</td>
</tr>
<tr>
<td><strong>Yescarta (CAR-T therapy)</strong>†</td>
<td>Fosun Kite Biotechnology / Kite Pharma</td>
<td>R/R follicular lymphoma*</td>
<td>US (March 2021), China (June 2021)</td>
</tr>
<tr>
<td></td>
<td></td>
<td>R/R large B-cell lymphoma</td>
<td>China (September 2021)</td>
</tr>
<tr>
<td><strong>Zolgensma (gene therapy)</strong>†</td>
<td>Novartis Gene Therapies</td>
<td>Spinal muscular atrophy</td>
<td>Australia (March 2021), S. Korea (May 2021)</td>
</tr>
<tr>
<td><strong>Stratagraft (tissue therapy)</strong></td>
<td>Mallinckrodt</td>
<td>Severe burns</td>
<td>US (June 2021)</td>
</tr>
<tr>
<td><strong>Skysona (gene therapy)</strong>**</td>
<td>bluebird bio</td>
<td>Cerebral adrenoleukodystrophy</td>
<td>EU (July 2021)</td>
</tr>
<tr>
<td><strong>Carteyva (CAR-T therapy)</strong></td>
<td>JW Therapeutics</td>
<td>R/R large B-cell lymphoma</td>
<td>China (September 2021)</td>
</tr>
<tr>
<td><strong>Rethymic (tissue therapy)</strong></td>
<td>Enzyvant</td>
<td>Congenital athymia</td>
<td>US (Oct 2021)</td>
</tr>
<tr>
<td><strong>Carvykti (CAR-T therapy)</strong>*</td>
<td>Legend Biotech &amp; Janssen</td>
<td>R/R multiple myeloma</td>
<td>US (Feb 2022)</td>
</tr>
</tbody>
</table>

* Received positive CHMP opinion in the EU
** bluebird voluntarily withdrew marketing authorization for Skysona in December 2021
† Already approved in another geography or for another indication
Six new regenerative medicines were approved across the US, Europe, and China in 2021, and an additional therapy — Legend Biotech and Janssen’s Carvykti for multiple myeloma — was approved in February 2022. This approval builds upon the momentum for CAR-T therapies in 2021: three new CAR-Ts, Abecma, Breyanzi, and Carteyva, received approval last year.

It was also a banner year for the FDA’s Regenerative Medicine Advanced Therapy (RMAT) designation. Breyanzi became the first RMAT-designated therapy to reach the market in February 2021, followed by Stratagraft in June and Rethymic in October. More therapies are likely to follow — the FDA has granted RMAT designation to a total of 68 therapies, including seven in 2021. The EMA’s PRIME designation also continues to grow, with seven cell, gene, and tissue products receiving PRIME in 2021.

### RMAT Designation

<table>
<thead>
<tr>
<th>Therapy</th>
<th>Company</th>
<th>Disease/Condition</th>
</tr>
</thead>
<tbody>
<tr>
<td>Libmeldy (gene therapy)</td>
<td>Orchard Therapeutics</td>
<td>Metachromatic leukodystrophy</td>
</tr>
<tr>
<td>Valrox (gene therapy)</td>
<td>BioMarin</td>
<td>Hemophilia A</td>
</tr>
<tr>
<td>ReNu (tissue therapy)</td>
<td>Organogenesis</td>
<td>Knee osteoarthritis</td>
</tr>
<tr>
<td>RP-L201 (gene therapy)</td>
<td>Rocket Pharmaceuticals</td>
<td></td>
</tr>
<tr>
<td>ALLO-715 (CAR-T therapy)</td>
<td>Allogene Therapeutics</td>
<td>R/R multiple myeloma</td>
</tr>
<tr>
<td>CTX110 (CAR-T therapy)</td>
<td>CRISPR Therapeutics</td>
<td>R/R B-cell malignancies</td>
</tr>
<tr>
<td>FT516 (NK cell therapy)</td>
<td>Fate Therapeutics</td>
<td>R/R large B-cell lymphoma</td>
</tr>
</tbody>
</table>

### Prime Designation

<table>
<thead>
<tr>
<th>Therapy</th>
<th>Company</th>
<th>Disease/Condition</th>
</tr>
</thead>
<tbody>
<tr>
<td>CTX-001 (CRISPR therapy)</td>
<td>CRISPR Therapeutics</td>
<td>Beta-thalassemia</td>
</tr>
<tr>
<td>ARU-1801 (gene therapy)</td>
<td>Aruvant Sciences</td>
<td>Sickle cell disease</td>
</tr>
<tr>
<td>CT-041 (CAR-T therapy)</td>
<td>CARsgen Therapeutics</td>
<td>Gastric cancer</td>
</tr>
<tr>
<td>RP-L201 (gene therapy)</td>
<td>Rocket Pharmaceuticals</td>
<td></td>
</tr>
<tr>
<td>MB-107 (gene therapy)</td>
<td>Mustang Bio</td>
<td>X-linked severe combined lymphoid leukemia</td>
</tr>
<tr>
<td>AUTO1 (CAR-T therapy)</td>
<td>Autolus Therapeutics</td>
<td>Acute lymphoblastic leukemia</td>
</tr>
<tr>
<td>ARI-0001 (CAR-T therapy)</td>
<td>Unknown academic center</td>
<td>R/R acute lymphoblastic leukemia</td>
</tr>
</tbody>
</table>
Regulatory decisions are expected on ten new therapies this year, including the approval of Carvykti in February. 2022 is expected to be a particularly strong year for gene therapy's traditional target: rare disease. Five never-before-approved therapies in this class could receive regulatory decisions, while two bluebird gene therapies that the company has removed or plans to remove from the European market could be approved in the US. And the CRISPR Therapeutics & Vertex Pharmaceuticals' therapy for sickle cell disease could receive approval in 2023. The often-quoted 2019 FDA prediction that it would approve 10–20 cell and gene therapies per year by 2025 looks possible, although perhaps at the lower end of that projection.

**UPCOMING REGULATORY DECISIONS**

in Europe and the US on New Products

### 2022

**United States**

<table>
<thead>
<tr>
<th>Therapy</th>
<th>Company</th>
<th>Disease</th>
</tr>
</thead>
<tbody>
<tr>
<td>Zynteglo (gene therapy)</td>
<td>bluebird bio</td>
<td>Transfusion-dependent beta-thalassemia</td>
</tr>
<tr>
<td>Lifileucel (TIL therapy)</td>
<td>Iovance Biotherapeutics</td>
<td>Metastatic melanoma</td>
</tr>
<tr>
<td>ValRox (gene therapy)</td>
<td>BioMarin</td>
<td>Hemophilia A</td>
</tr>
<tr>
<td>Skysona (gene therapy)</td>
<td>bluebird bio</td>
<td>Cerebral adrenoleukodystrophy</td>
</tr>
<tr>
<td>Omidubicel (cell therapy)</td>
<td>Gamida Cell</td>
<td>Hematopoietic stem cell transplant in patients with hematological malignancies</td>
</tr>
<tr>
<td>PTC-AADC (gene therapy)</td>
<td>PTC Therapeutics</td>
<td>AADC deficiency</td>
</tr>
<tr>
<td>Vyjuvek (gene therapy)</td>
<td>Krystal Biotech</td>
<td>Dystrophic epidermolysis bullosa</td>
</tr>
<tr>
<td>Etranacogene dezaparvovec (gene therapy)</td>
<td>uniQure &amp; CSL Behring</td>
<td>Hemophilia B</td>
</tr>
<tr>
<td>HPC cord blood (cell therapy)</td>
<td>StemCyte</td>
<td>Unrelated donor hematopoietic progenitor cell transplantation</td>
</tr>
</tbody>
</table>

**Europe**

<table>
<thead>
<tr>
<th>Therapy</th>
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<tbody>
<tr>
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<td>Legend Biotech &amp; Janssen</td>
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</tr>
<tr>
<td>Tab-cel (cell therapy)</td>
<td>Atara Biotherapeutics</td>
<td>Epstein-Barr virus positive post-transplant lymphoproliferative disease</td>
</tr>
<tr>
<td>Lumevoq (gene therapy)</td>
<td>GenSight Biologics</td>
<td>Leber hereditary optic neuropathy</td>
</tr>
<tr>
<td>Etranacogene dezaparvovec (gene therapy)</td>
<td>uniQure &amp; CSL Behring</td>
<td>Hemophilia B</td>
</tr>
<tr>
<td>Breyanzi (CAR-T therapy)</td>
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</table>

### 2023

**United States**

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<td>Gamida Cell</td>
<td>Hematopoietic stem cell transplant in patients with hematological malignancies</td>
</tr>
<tr>
<td>CTX001 (CRISPR therapy)</td>
<td>CRISPR Therapeutics &amp; Vertex Pharmaceuticals</td>
<td>Sickle cell disease</td>
</tr>
<tr>
<td>Afami-cel (cell therapy)</td>
<td>Adaptimmune Therapeutics</td>
<td>Advanced synovial sarcoma</td>
</tr>
<tr>
<td>Libmeldy (gene therapy)</td>
<td>Orchard Therapeutics</td>
<td>Metachromatic leukodystrophy</td>
</tr>
<tr>
<td>bb1111 (gene therapy)</td>
<td>bluebird bio</td>
<td>Sickle cell disease</td>
</tr>
<tr>
<td>CT053 (CAR-T therapy)</td>
<td>CARsgen Therapeutics</td>
<td>R/R multiple myeloma</td>
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**Europe**

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</tr>
</tbody>
</table>

*Received positive CHMP opinion*
There are 2,406 trials ongoing globally, a 15% decrease from the end of 2020. The decrease was largely due to a decline in the number of trials sponsored by academic and government institutions, which decreased by 21% over the past year; the number of industry-sponsored trials decreased by about 8%.

Cell-based immuno-oncology (cell IO) trials make up the largest proportion of industry trials (41%), outstripping the number of cell therapy trials for the first time. The number of cell IO trials is likely to continue to grow. Over half of Phase 1 industry trials (57%) are in cell IO, compared to only about a quarter (22%) of Phase 3 trials. For academic and government-sponsored trials, cell therapy remains the largest category, making up about 58% of trials. While there is also a significant portion of academic and government-sponsored cell IO trials, this group has a significantly lower proportion of gene therapy trials.
Indications With Significant Clinical Activity

**RARE DISEASES**
- Sickle cell disease (39)
- Hemophilia (28)
- Retinitis pigmentosa (26)
- Amyotrophic lateral sclerosis (ALS) (15)
- Thalassemia (15)
- Mucopolysaccharidosis (14)
- Multiple sclerosis (12)

**PREVALENT DISEASES**
- Osteoarthritis (61)
- Diabetes & related complications (54)
- Parkinson’s disease (21)
- Critical limb ischemia (18)
- Macular degeneration (18)
- Stroke (17)
- Alzheimer’s disease (10)

**CLINICAL ACTIVITY BY INDICATION**
- Oncology: 1,246 (52%)
- Infectious disease: 125 (5%)
- Immunology: 119 (5%)
- Monogenetic disease: 113 (5%)
- Hematology: 109 (5%)
- Musculoskeletal: 106 (4%)
- Cardiovascular: 105 (4%)

Industry-Sponsored
Academic & Government-Sponsored
Cancer Remains Leading Target, Representing Over Half of All Trials

Oncology remains the leading indication for the sector, making up approximately half of ongoing trials. While hematological malignancies have historically been the primary target for cell and gene therapies, the focus on solid tumors is growing. Trials in solid tumors now make up 45% of total oncology trials. The most common solid tumor type targeted is gastrointestinal cancers, which make up about 23% of trials targeting solid tumors.

Solid Tumors by Tumor Type

<table>
<thead>
<tr>
<th>Tumor Type</th>
<th>Count</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>Gastrointestinal cancer</td>
<td>130</td>
<td>23%</td>
</tr>
<tr>
<td>Brain and spinal cord cancer</td>
<td>71</td>
<td>13%</td>
</tr>
<tr>
<td>Multiple solid tumors</td>
<td>66</td>
<td>12%</td>
</tr>
<tr>
<td>2WKHURUXQVSHFLøHQ</td>
<td>52</td>
<td>9%</td>
</tr>
<tr>
<td>Respiratory cancer</td>
<td>51</td>
<td>9%</td>
</tr>
<tr>
<td>Gynecological cancer</td>
<td>48</td>
<td>9%</td>
</tr>
<tr>
<td>Skin cancer</td>
<td>41</td>
<td>7%</td>
</tr>
<tr>
<td>Breast cancer</td>
<td>36</td>
<td>6%</td>
</tr>
<tr>
<td>Head and neck cancer</td>
<td>23</td>
<td>4%</td>
</tr>
<tr>
<td>Bladder and renal cancer</td>
<td>19</td>
<td>3%</td>
</tr>
<tr>
<td>Prostate cancer</td>
<td>15</td>
<td>3%</td>
</tr>
<tr>
<td>Multiple cancer types</td>
<td>9</td>
<td>2%</td>
</tr>
</tbody>
</table>

Solid vs. Liquid Tumors

- Solid: 685 (55%)
- Liquid: 561 (45%)

There is growing attention on the development of allogeneic cell-based immunotherapies for cancer, with the emphasis on manufacturing in larger quantities and delivering to patients more quickly. Allogeneic trials make up about one-quarter (27%) of total cell-based IO trials, including 30% of trials targeting hematological malignancies. A slightly lower proportion of solid tumor trials — 22% — are using allogeneic approaches. We expect the number of allogeneic cell-IO trials to continue to grow as companies work to mitigate immune responses to these therapies. This is evidenced by a higher proportion of allogeneic therapies in Phase 1 (28% of all Phase 1 cell-based IO trials) versus Phase 3 (only 11%).
While 2022 is a big year for gene therapies targeting rare diseases, we’re also expecting clinical readouts on therapies targeting prevalent diseases to make headlines. We saw a significant milestone in 2021 when Vertex Pharmaceuticals’ cell therapy was reported to have functionally cured one patient’s type 1 diabetes. Additional data is expected in 2022. And the first approval for a gene therapy for a prevalent disease in the United States and Europe could be just a few years out, with Phase 3 programs in indications including critical limb ischemia, congestive heart failure, diabetic peripheral neuropathy, and macular degeneration.

Of the 2,406 clinical trials in the space, 59% are targeting prevalent diseases. This number is slightly higher for academic and government-sponsored trials, 62%, versus 56% for industry trials — showing that industry is more active in the rare disease space. Additionally, when looking at the types of rare diseases targeted by industry versus academic and government sponsors, industry focuses more heavily on rare hematological disorders such as hemophilia and sickle cell, as well as rare ophthalmological disorders such as retinitis pigmentosa.
Rare cancers remain the number one target, making up about two-thirds of all rare disease trials. Other rare indications that have attracted attention from cell and gene therapy developers include central nervous system disorders like ALS and Batten disease; inherited hematological disorders such as hemophilia and sickle cell disease; ophthalmological indications such as retinitis pigmentosa and choroideremia; and other rare monogenic disorders, including mucopolysaccharidosis, Duchenne muscular dystrophy, glycogen storage disorders, gangliosidosis, and Wilson disease.

While the percentage of total trials in rare versus common diseases remains fairly consistent across phases, the nature of the prevalent diseases targeted is changing. Musculoskeletal disorders such as bone fractures, osteoarthritis, and sports injuries make up 23% of Phase 3 trials targeting a prevalent disease, but only 7% of Phase 1 trials. Other prevalent disease areas targeted include central nervous system disorders such as Parkinson’s and Alzheimer’s disease and infectious GLVHDVHLQFOXGLQJ +,9

There’s also a shift towards targeting more complex, polygenic diseases within the prevalent disease category. Within central nervous system disorders, we are seeing a gradual shift from disorders such as spinal cord injury, traumatic brain injury, and neuropathic pain to more complex, polygenic disorders, including Alzheimer’s disease, autism, and even treatment-resistant bipolar disorder and depression.
While safety concerns around dosing and delivery — particularly for AAV therapies — weighed down the gene therapy sector last year, there is reason for optimism. The FDA has signaled that it doesn’t plan to apply a universal dose cap to AAV therapies. And while the regenerative medicine sector is increasingly investigating alternative delivery methods, including nonviral delivery, we’re also seeing many developers in the space looking for ways to optimize AAVs.

Among gene therapy trials, AAVs are used in at least 46% of all gene therapy trials, and 66% of trials with a known vector. After AAVs, lentiviruses are the next most common known vector, making up at least 8% of all gene therapy trials and 11% of trials with a known vector. Lentiviruses are the most common vector used in ex vivo gene therapies. Adenoviruses follow closely behind lentiviruses, making up at least 7% of all gene therapy trials and 10% of trials with a known vector.

Nonviral delivery methods for gene therapy have also been gaining interest. At least 8% of total gene therapy trials and 11% of trials with a known vector use nonviral delivery methods, including nanoparticles and plasmids. Moving forward, we expect this subcategory of gene therapy trials to continue to grow.

**Gene Therapy Vectors**

<table>
<thead>
<tr>
<th>Vector</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
<th>Total</th>
</tr>
</thead>
<tbody>
<tr>
<td>AAV</td>
<td>145 (46%)</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Lentivirus</td>
<td>24 (8%)</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Adenovirus</td>
<td>22 (7%)</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Plasmid</td>
<td>21 (7%)</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Nanoparticle</td>
<td>4 (1%)</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Other viral vector</td>
<td>4 (1%)</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Other retrovirus</td>
<td>2 (&lt;1%)</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
Gene editing continues to advance as a therapeutic modality. There are currently 41 trials ongoing in gene editing, about one-third of which are in Phase 1 with the remainder in Phase 2. The vast majority of these trials (80%) use CRISPR, demonstrating the strong foothold this technology has established since the initiation of the first CRISPR gene-editing trial in 2019.

Early data from June 2021 from a trial of Intellia Therapeutics and Regeneron’s CRISPR-based therapy for transthyretin amyloidosis, a rare disease affecting the liver, showed proof-of-concept that gene-editing therapies could be delivered in vivo. Since then, additional data released in February of this year confirmed a dose-dependent therapeutic benefit, and showed initial durability data for up to one year post-treatment.

A little over half of gene-editing trials (23 of 41) are targeting cancer. Gene editing is one tool deployed in the development of allogeneic cell-based immunotherapies; of the 23 gene editing trials targeting cancers, 13 are targeting hematological malignancies. Outside cancer, the second most frequently targeted indication area is inherited hematological disorders such as sickle cell disease and beta-thalassemia, followed by other inherited disorders including mucopolysaccharidosis, hereditary angioedema, Leber congenital amaurosis, and others.

In 2021, we saw the initiation of the first gene-editing trial for a prevalent disease: CRISPR Therapeutics and ViaCyte’s therapy targeting type 1 diabetes. In 2022, we expect to see the initiation of two trials using a novel base-editing approach: Beam Therapeutics’ therapy for sickle cell disease and Verve Therapeutics’ therapy for heterozygous familial hypercholesterolemia, a potentially fatal heart disease. And as mentioned previously, the first gene-editing therapy — Vertex Pharmaceuticals and CRISPR Therapeutics’ therapy for sickle cell disease — could receive approval in 2023.
The Global Regenerative Medicine Sector

1,308
Total gene, cell, and tissue-based therapeutic developers worldwide

Up 19% Year-Over-Year
2021 was a year of significant scientific advances for the sector. We saw clinical data supporting the *in vivo* use of gene editing, powerful early evidence that cell and gene therapies can treat complex, polygenic diseases, and even results that suggest these therapies may be able to reverse damage that has already occurred. As the science progresses, ARM’s work with industry, regulators, and other stakeholders to ensure that innovative therapies are able to reach patients in need becomes even more important.

CMC remains a key challenge, but it’s one the industry is addressing head on. We are continuing to work with FDA on this issue, which was a central topic at the FDA listening session ARM participated in last summer. We expect to increase the cadence of our interactions with the agency moving forward.

In 2021, we held two CMC workshops, one on broad industry issues and one focused on pluripotent stem cells, attracting 500 registrants. Additionally, we rolled out *Project A-Gene*, a multistakeholder collaboration to incorporate Quality by Design (QbD) principles into a manufacturing case study of a viral vector commonly used in gene therapies. This guide is being integrated into industry training as well as academic curricula at universities including the University College of London. *Project A-Cell*, a sister project focusing on Quality by Design principles in cell therapy CMC programs, is on track to be completed in Q2 2022.

In 2021, ARM launched a new committee designed to address the needs of early-stage member companies: The Accelerator. The committee held a webinar on manufacturing considerations for early-stage developers and assisted in the organization of a panel on investment opportunities for the 2021 *Meeting on the Mesa*. This will continue to be a priority area for ARM.

As the number of new companies grows, workforce constraints are becoming a pain point for the sector. While projects like *A-Gene* and *A-Cell*, ARM’s CMC workshop series, and our GROW RegenMed Internship Program can be important professional development opportunities for the sector, the reality is that there is a shortage of workers trained in this space. In the second half of 2022, ARM will conduct a workforce gap analysis to better understand the needs of the industry and identify solutions going forward.

At ARM, I am privileged to work with a variety of stakeholders to address the needs of this sector. We are making progress for both ARM and the sector at large, and we will work diligently to ensure that the immense promise of this field is met.

Michael Lehmicke
*Vice President, Science and Industry Affairs*
Alliance for Regenerative Medicine
Jimi Olaghere had trouble keeping up with other kids. He wasn’t as fast, and he needed to stop playing for frequent water breaks.

When he asked his mother why, she told him that he had been diagnosed at birth with sickle cell disease. For years, he had a standing appointment on Tuesdays for IV hydration. Every other Thursday, he received blood transfusions to ease his anemia and reduce the viscosity of his blood, allowing it to flow more freely. He frequently took morphine to alleviate pain crises. He was typically hospitalized every other month due to the severity of the disease. Jimi can’t watch the NBA playoffs anymore, because he too often watched them from a hospital bed.

In 2019, when he was 34, Jimi read the story of Victoria Gray, the first patient treated by Vertex Pharmaceuticals and CRISPR Therapeutics in their groundbreaking gene-editing clinical trial to address the genetic cause of sickle cell disease. He reached out to get information on participating. “I had no reservations about taking part in the trial. I was actually desperate at this point,” says Jimi. The medical team contacted him the next day.

In January of 2020, an apheresis machine collected Jimi’s bone marrow stem cells for the first time. The process was repeated three additional times over the next six months, requiring frequent travel away from his wife and newborn son at home in Atlanta to the treatment center in Nashville. A team of scientists then used CRISPR to edit the genes in these cells, engineering them to produce normally functioning hemoglobin. Finally, in September 2020 — after receiving a round of chemotherapy to destroy the bone marrow cells that were making his diseased blood cells — the edited cells were reinfused into Jimi’s body.
Jimi says it took him about two weeks to recover from the chemotherapy and procedure. He reports that the change in his health was immediate. “I remember waking up and not feeling any pain at all,” he says. Jimi says he has not returned to the emergency room since his treatment because he has not experienced any pain crises. Aside from the follow-up for the trial, Jimi says he has no additional sickle cell care ongoing.

Now, Jimi says that his goal is to “make up for lost time.” As soon as Jimi completed the treatment, he and his wife decided to grow their family. They would have had their children much earlier were it not for Jimi’s health challenges. Their twin daughters are now four months old. “Being a parent health to properly take care of my children is a really, really good feeling.”

Jimi is also able to work like he wants to and take his career where he wants it to go. He plays with his now two year-old son in the rare Georgia snow without fear of the cold triggering a crisis. He no longer needs pain medication and his mindset is clear after years of psychological strain.

Jimi emphasizes the need for mental health resources to support patients with sickle cell disease. “Even though the physical toll is there, I would argue that the mental toll it takes on a person is actually more,” he says. Even after receiving treatment, going from managing a severe and painful disease to being able to go out and live life can be daunting. “You’re not the same person that you used to be,” Jimi says.

Jimi believes that the gene editing field will advance rapidly over the next five to ten years. He’s encouraged by the cooperation between patients and cell and gene therapy developers. CRISPR therapy by the end of 2022, with a decision from the U.S. FDA expected in mid-2023. It would be

After approval, the next major milestone will be securing patient access. Jimi hopes that the therapy will be commercialized in a way that is accessible to everyone in the U.S., as well as the rest of the world. “I know there’s smart people working on this,” Jimi says. “From everything I’ve seen, I’m whole-heartedly confident that it’s going to happen.”

Disclaimer: The product discussed in this story is investigational and being studied in clinical trials. Its safety and efficacy have not yet been proven.
ARM’s growing focus on its public affairs capabilities resulted in several notable global advocacy accomplishments in 2021. And with four new key hires in place at the start of 2022, we have even more ambitious goals for this year.

In 2021, we focused heavily on Europe to establish ARM as the pre-eminent voice for the cell and gene therapy sector at a crucial time for the sector’s future. EU policymakers are revising the pharmaceuticals legislation for the first time in 20 years and addressing several other relevant concurrent policy tracks. To help seize the moment in Europe, we hired Elisabetta Zanon to be our first-ever government relations lead in Europe, based in Brussels, and Mimi Choon-Quinones as our Director of European Regulatory Affairs, based in Switzerland.

We conducted more than 75 meetings with Brussels, Berlin, and Paris-based policymakers to educate stakeholders about the sector, share concerns around Europe’s declining competitiveness, and promote our policy positions on topics including GMO and the Blood, Tissues, and Cells legislation. We also helped to shape a new EU regulation to conduct joint clinical assessments and are working to ensure that methodologies are fit-for-purpose for advanced therapy medicinal products (ATMPs).

We will leverage our new capabilities to build on 2021’s progress, which includes securing language establishing CMC regulatory clarity in both the PDUFA VII agreement and other legislative vehicles, which could include “CURES 2.0”. In 2022, we will also focus on cementing ARM as the global voice of the sector on Capitol Hill and educating policymakers about how cell and gene therapies are fundamentally different from traditional pharmaceuticals. We will continue our work to remove barriers to innovative payment models, connecting our work on Medicaid at the federal level with our expanding efforts in Texas and Ohio — two hubs for the future of our sector.

In this report, and moving forward, we’re enhancing ARM’s ability to collect and analyze data and to tell the story of the sector’s progress and evolution. Combined with our enhanced presence in the US and Europe, this will only serve to bolster our role as the global voice of the sector for policymakers, the media, and other stakeholders.
THE GLOBAL COMPETITION TO DEVELOP NEXT-GEN THERAPIES

**United States**
- Therapeutic developers: 613
- Industry-sponsored trials: 566
- Academic and government-sponsored trials: 436
- Billion in investment: $17.6

**Europe**
- Therapeutic developers: 230
- Industry-sponsored trials: 200
- Academic and government-sponsored trials: 160
- Billion in investment: $3.3

**Asia-Pacific**
- Therapeutic developers: 410
- Industry-sponsored trials: 314
- Academic and government-sponsored trials: 372
- Billion in investment: $2.2
US Policy & Regulatory Update

Following significant progress in 2021 particularly around CMC challenges, ARM is leveraging an enhanced focus on the US in 2022 to advance legislative and regulatory initiatives on CMC, innovative payment models, and other priority topics for the sector.

The PDUFA VII proposal would increase FDA staffing to review cell and gene therapy applications, establish a CMC Readiness Pilot, and advance real-world evidence in regulatory reviews – all cell and gene therapy sector priorities. ARM’s advocacy also secured language promoting CMC clarity and other USRULWLVFQ&85(6WKH)ROORZQXSOHJLVDWLRQWRKHKVW&HQWUXV&XUHV$FWV$QHGLQWRODZLQ ARM will remain focused in 2022 on securing passage of the PDUFA agreement and of the sector’s CURES priorities, while also expanding dialogue with the FDA on CMC challenges.

A CMS rule that would remove barriers to innovative payment models in state Medicaid programs is VFKHGXOHGWRWDNHHIHFWLQ&X0&50DQGWKHVFWRUZLOODGYRFDWHRUUHQPHQWVWRKHPISOHHPHQWDWLRQ of this rule, and simultaneously work on legislative vehicles to remove further barriers to innovative payment models beyond Average Manufacturer Price and Best Price. Also on the CMS front, ARM has advocated — and will continue to advocate — for changes to the New Technology Add-on Payment (NTAP), including making the assignment process at least twice a year instead of once, increasing the payment amount, and extending coverage to four years.

With a new dedicated federal government relations lead, ARM will work to cement itself as the go-to resource for information and policy development on cell and gene therapy on Capitol Hill and to educate policymakers about how the sector differs from traditional pharmaceuticals in the context of drug-pricing and payment models discussions.

Building upon the launch of advocacy work in Texas and Ohio in 2021, ARM is continuing its work at the state level in 2022 and providing synergy with its work on Medicaid at the federal level. ARM supported Ohio’s application for a State Plan Amendment last year in order to enter into innovative payment arrangements with pharmaceutical manufacturers. If approved, ARM will work to ensure that this initiative will expand access to cell and gene therapy treatments for Medicaid patients in Ohio. In Texas, ARM will similarly work with policymakers to remove barriers to innovative payment models. Both states have become hubs for the cell and gene therapy sector — and ARM will help policymakers build on this foundation as global competition rises.

To help achieve the sector’s goals in 2022, ARM looks forward to returning to an in-person Congressional Fly-In in Washington on June 7–8, and to hosting sector events in Ohio (May 18) and Texas (date TBD) IRUWKHUUVWWLPH

With regulatory decisions expected on nine cell and gene therapy products in the US in 2022, ARM’s work to ensure patient access to transformative therapies has never been more important.
ARM invested heavily in 2021 in its European advocacy and is continuing to do so in 2022, in order to ensure that the region remains a competitive destination for ATMP developer headquarters, clinical trials, and commercialization. The EU Pharmaceutical Strategy and associated policy initiatives represent the first major overhaul of the policy and regulatory environment for pharmaceuticals, including ATMPs, in 20 years.

ATMPs have moved up the EU healthcare agenda, with increased policymaker focus on how the EU can remain competitive, the need to keep EU regulatory standards on par with other prominent global regulators, and the value that ATMPs can provide for patients and healthcare systems. For example, EU Commissioner for Health & Food Safety Stella Kyriakides will deliver virtual welcome remarks at ARM’s April 20–22 Meeting on the Mediterranean about the importance of cell and gene therapies for European patients.

In 2021, ARM responded to several EU policy consultations — including Blood, Tissues, and Cells (BTC) legislation, the pharmaceuticals legislation revision, and Cross-Border Healthcare — and discussed its recommendations with key European Commission officials. On BTC, ARM is calling for the distinction between ATMPs and BTCs to remain intact to avoid a two-tiered regulatory system.

ARM is also heavily engaged in the EU’s efforts to revise Cross-Border Healthcare legislation and to establish a European Health Data Space, which can aid in the generation and sharing of real-world evidence (RWE). RWE is crucial to cross-border access and more broadly for the development of, and access to, ATMPs across the EU.

ARM co-authored a paper with two other organizations calling on the Commission to exempt ATMPs from GMO requirements. In high-level policymaker meetings, the organizations noted that the EU remains an outlier in its application of GMO requirements to medicines. ARM continues to advocate for the GMO issue to be addressed in the Commission’s pharmaceuticals legislation revision expected in the fourth quarter.

ARM is also active at the country level, particularly in Germany, France, and the UK. In Germany, ARM secured changes to the NUB law that will make it easier for hospitals to be reimbursed when providing ATMPs. In France, ARM met with policymakers and the national payer to emphasize the need for innovative payment models for ATMPs. In the UK, ARM contributed to consultations on proposed new NICE methods and processes. ARM is urging a reduction in discounting percentages — from 3.5% to 1.5% — in health economic evaluations that will result in better value scores for gene therapies.

Regulatory, market access and legislative developments in Europe during the rest of 2022 will be consequential for the ATMP sector for the next decade. With a new dedicated government relations lead in Brussels, ARM will increase its advocacy work across the region to ensure patient access to transformative ATMPs.
The availability of new therapeutic modalities that in many cases address the root causes of disease drove unprecedented private investment in biotech generally and in cell and gene therapy specifically in 2021.

A year ago, there was no human data for in vivo gene editing — now, we have 93% reductions in TTR production in humans that Intellia Therapeutics announced on February 28. Multiple T-cell programs were approved, and a next wave of cell therapies focused on B-cells that could allow us to address chronic conditions is progressing through the clinic. We are moving from simple toxic protein knock down methods for gene therapy. Non-viral gene therapy has the potential to be re-dosable and to be more easily manufactured at scale, potentially opening the door to more prevalent conditions.

Unprecedented access to capital in the private and public markets has fueled this progress.

But some have focused on the recent downturn in public markets as a sign of the sector’s declining XBI index peaked in February 2021, declining 21% in 2021 and a further 20% through late-February 2022. We view the current, 13-month-and-counting downturn as a cyclical event, rather than as a fundamental change in the industry’s prospects. Since 2011, the biotech public markets have had six bear markets, with declines from peak to trough ranging from 27% to 49% and with trough-to-prior-peak recoveries taking from two to 20 months.

We see two main drivers for the recent declines: biotech cyclicality with an overbought public biotech space in 2020–2021 overlapping with macro cyclicality with high growth, high inflation, and expectations of tightening monetary policy, a set of conditions not seen since the 1980s. Russia’s invasion of Ukraine is a tragic human event and another exogenous shock.

While there are signs of a market bottom, including mature biotech EV/sales ratios that are approaching the lower limits typically seen in downturns, we are not in the market-prediction business. What we can say is that the essential drivers of biotech innovation and value creation remain in place: the intersection of unmet patient needs, accelerating innovation, a talented pool of biotech entrepreneurs, a committed community, and access to capital. We look forward to further advances in emerging therapeutic modalities like base editing, B-cell therapy, and non-viral gene therapy, among others.

Scientific and medical progress, of course, don’t happen on their own. Building new companies that pursue innovation requires capable management teams, an engaged regulatory environment, and funding. Working with all of you, we are committed to building innovative companies with measured and fundable plans with strong investor syndicates that can support companies in all capital market conditions. Bear markets do not last forever.

Jason Rhodes
Partner
$WODV9HQWXUH
Rapidly advancing science in the regenerative medicine field is driving continued investor interest. Developers raised a record-breaking $22.7B in 2021, surpassing the previous record of $19.9B set in 2020 by 14%. This continues a trend of upward growth in recent years, with investment more than tripling since 2017.
Companies active in gene editing have captured the interest of investors. In 2021, about one third of total regenerative medicine financing was raised by companies active in gene editing. This proportion was highest for gene therapy developers — companies active in gene editing constituted about 45% of gene therapy financings last year. That’s up from 38% just three years ago. Growing investment in gene editing likely reflects recent scientific and clinical advances, such as the announcement of the first in vivo CRISPR data in June of 2021, as well as the imminent advancement of technologies such as base editing into the clinic.

Gene therapy and cell-based IO developers raised the vast majority of investment, generating $10.2B and $10.1B, respectively. Gene therapy has historically attracted the largest amount of investment, and that trend continued in 2021. However, investment in cell-based immuno-oncology (IO) companies is growing at a faster pace than gene therapy financing. Cell therapy developers raised $2.0B and tissue-engineering developers raised $341M.
Venture capital was the main driver of investment in the sector in 2021, contributing $9.8B — a 75% increase over 2020. A record number of 26 IPOs raised a total of $4.8 billion. Private placements and private investments in public equity (PIPEs) also had a strong year, while upfront payments from corporate partnerships and follow-on financings decreased from 2020.
Despite record-breaking investment, 2021 was a tough year for public equity. Gene therapy — particularly AAV gene therapies — were the worst performers, dropping about 50% in value, compared to 4% for the NASDAQ biotech index and 22% for the XBI. Cell-based IO companies fell by about 25%, and RMAT — an index that reflects performance across all regenerative medicine technologies — closed out the year down 34%. Despite that, however, RMAT is still about 77% higher than 2018 levels.
$100M+ Financings

## IPOs
- Sana Biotechnology – $675M
- Lyell Immunopharma – $425M
- CARsgen Tx – $400M
- Instil Bio – $368M
- Caribou Bio – $304M
- Graphite Bio – $273M

## Follow-Ons
- Intellia Tx – $690M
- Fate Tx – $432M
- Iovance Bio – $350M
- Editas Medicine – $231M
- REGENXBIO – $230M

## Venture Capital
- Sonoma Bio – $265M
- Blackstone Life Sciences – $250M
- Tessera Tx – $230M
- Arbor Bio – $215M
- Umoja Bio – $210M
- Amplify Bio – $200M
- G2 Bio – $200M
- Prime Medicine – $200M
- Wugen – $172M
- Century Tx – $160M
- Gentiberio – $157M
- Quell Tx – $156M
- Graphite Bio – $150M

## Private Placements/PIPS
- Beam Tx – $260M
- Humacyte – $175M

## Corporate Partnerships (Upfront Payments)
- 9HUWH3KDUPE & 5,6357[±0
- 5*(1,%2$EE9LH±0
- Adaptimmune TX & Genentech – $150M

## Follow-Ons
- Generation Bio – $225M
- Krystal Bio – $200M
- Solid Bio – $144M
- TCR2 Tx – $140M
- Mammoth Bio – $150M
- Gyroscope Tx – $148M
- Jaguar Gene Tx – $139M
- Chroma Medicine – $125M
- eGenesis – $125M
- Artiva Bio – $120M
- CBMG – $120M
- Forge Bio – $120M
- Ring Tx – $117M
- Arcellx – $115M
- Caribou Bio – $115M
- Obsidian Tx – $115M
- Prime Medicine – $115M
- $1QLD7[±0
- T-knife Tx – $110M
- Acepodia – $109M
- IASO Bio – $108M
- Tenaya Tx – $106M
- Senti Bio – $105M
- bit.bio – $103M
- Dyno Tx – $100M
- LEXEO Tx – $100M
- Kriya Tx – $100M
- Scribe Tx – $100M
- TScan Tx – $100M

## Corporates
- Orchard Tx – $150M
- Mesoblast – $110M
- Eli Lilly & Precision Bio – $135M
- Ensoma & Takeda Pharma – $100M
ARM surpassed 400 members for the first time in 2021, closing out the year with 423 members from across the sector, including small and large therapeutic developers, contract manufacturing and development organizations, medical and academic research centers, patient organizations, and other stakeholders. While as many as ten new cell and gene therapy products could be approved in 2022, the majority of ARM’s therapeutic developer members are still pre-revenue. In 2021, ARM launched a new member committee, The Accelerator, to address the needs of these early-stage developers.

Membership Breakdown — Member Type & Size

- Corporate (76% of 423 members)
- Foundations/Non-Profits/Patient Advocates (15%)
- Academic Research Institutions (5%)
- Affiliate & Financial Organizations (4%)
- Corporations (Revenue > $50 Million) (13% of 322 members)
- Corporations (Revenue < $50 Million, 100+ Employees) (24%)
- Corporations (Revenue < $50 Million, 50–99 Employees) (17%)
- Corporations (Revenue < $50 Million, < 50 Employees) (46%)
Organizational Growth

To meet the needs of a growing membership, ARM hired 13 new employees in 2021 — including nine for newly created positions. The public affairs team grew significantly, with the hiring of the organization’s first Head of US Government Relations (Christina Hartman) and Director of US Market Access & State Government Relations (Brett Logan), and the first Director of European Policy and Advocacy (Elisabetta Zanon) in Europe. Mimi Choon-Quinones also joined ARM as the Director of European Regulatory Affairs. This capacity building will support ARM’s expanded public affairs work in the US and Europe in 2022 and beyond.

In 2021, ARM also hired a second member of the Science and Industry Affairs team, Josephine Lembong, to assist with projects including the release of Project A-Gene and the work on the subsequent Project A-Cell. ARM’s events team brought on Director Kelly McWhinney and Senior Manager Jancee Curl, while the membership team added Manager Kristy Rothfritz. David Townsend joined ARM as the new Vice President of Finance and Operations, Shakita Bazemore joined as the Operations Manager, and Becky Wright joined as ARM’s Staff Accountant. Following a successful inaugural year, ARM’s GROW RegenMed Internship Program brought on Rosie Walker as Program Director. Thomas Szabo came onboard as Executive Administrator.
In the spring of 2020, encouraged by member CEOs, ARM established the Action for Equality (AFE) Task Force to determine concrete steps ARM and its members could take to ally with the movement for racial equality and address the underrepresentation of Black employees within the regenerative medicine workforce. Thanks to the work of AFE, ARM launched the GROW RegenMed Internship Program to provide crucial, early-career paid opportunities in the regenerative medicine sector for Black undergraduate and graduate students.

The program hosted the first cohort of interns in Summer 2021, with 17 interns at ARM and 13 of our member organizations. The program concluded with the interns attending the 2021 Meeting on the Mesa.

The GROW Program will host its second group of interns in Summer 2022, and is on track to have approximately 25 internship opportunities at 20 participating ARM member organizations.
In the spring of 2021, ARM held its first virtual Meeting on the Med. More than 600 people participated in the meeting, exceeding in-person attendance at the first Meeting on the Med in 2019. The 2020 meeting was canceled due to the COVID-19 pandemic. The programming for the meeting featured industry leaders, investors, patient advocates, and policymakers, including EMA Executive Director Emer Cooke, Dr. Cristian Buşoi, Member of the European Parliament and Chair of the Committee on Industry, Research & Energy, and regulators from the US, Europe, and Japan.

ARM returned to in-person programming with the 2021 Meeting on the Mesa in Carlsbad, CA. The meeting, which was held in a hybrid format, was ARM’s largest yet, attracting more than 1,700 registrants — an approximately 40% increase in the number of registrants from 2019 and 2020. Nearly 1,200 of those participants attended in person, and more than 3,000 one-on-one partnering meetings were scheduled.

In January of this year, ARM held our annual State of the Industry Briefing virtually due to the rise of the Omicron variant. More than 700 people attended the virtual presentation, including the potential to reverse damage and the possibility of treating complex landscape for cell and gene therapies, ARM hosted two panels: one on gene therapy for rare diseases and another on next-generation therapies to fight cancer.
ARM looks forward to our second hybrid event this spring. The 2022 *Meeting on the Med* will be held in Barcelona, Spain on April 20–22, and will cover key topics in the sector including commercialization, manufacturing, reimbursement, and the European policy environment. Registration is available online: www.meetingonthemed.com

Additionally, ARM will host our Congressional Fly-In on June 7–8, bringing together our membership to represent the cell and gene therapy sector before policymakers on Capitol Hill. We are pleased to return to in-person meetings, and to give our members the opportunity to share the sector’s promise and priorities with the Representatives and Senators who represent their districts and states. Registration is limited to members only, and can be completed on ARM’s website.

### What’s Next?

2021 marked 12 years of ARM’s work as the global voice of the regenerative medicine and advanced therapy sector. ARM’s successes include leading the creation of the RMAT designation; driving appropriate reimbursement of CAR-T therapies through the establishment of a dedicated DRG; reducing barriers to hospital reimbursement for providing ATMPs in Germany; the publication of the

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In 2022, ARM plans to expand its advocacy work even further. The ARM team and membership are growing, and we continue to nurture key relationships with decision makers in this space. We look
UPCOMING EVENTS 2022

MEETING ON THE MEDITERRANEAN
BARCELONA, SPAIN

APRIL 20–22

CONGRESSIONAL FLY-IN
WASHINGTON, DC

JUNE 7–8

MEETING ON THE MESA
CARLSBAD, CA

OCT 11–13

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& K LHI%R%PHU%LD%0%FHU
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& K LHI6%FLHQWL%F%FHU
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R.A. Session, MBA, MSF
President, Founder & CEO,
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Curran Simpson, M.S.
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Product Development,
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REGENXBI
Bob Smith, MBA
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Devyn Smith, Ph.D.
CEO, Arbor Biotechnologies
Joe Tarnowski, Ph.D.
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Arthur Tzianabos, Ph.D.
President and CEO,
Homology Medicines
Christopher Vann
693%2%WX%RO%V
Therapeutics
Kristin Yarema, Ph.D.
CCO, Atara Bio
The Alliance for Regenerative Medicine (ARM) is the leading international advocacy organization dedicated to realizing the promise of regenerative medicines and advanced therapies.

**Influence**
Collectively engage with lawmakers and key government agencies in the U.S. and EU

**Network**
Meet commercial RM leaders and partners

**Exposure**
Present your work at LQXHQLDOS50YHQWV

**Sector Partners**
Engage with patient advocacy groups & research institutions

**Fundraising**
Gain exposure to the investment community

**Science & Technology**
Work with other manufacturing and technology experts to reduce barriers to product development and scale

**Sector Initiatives**
Help shape sector-wide initiatives, policy priorities, and policy positions

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