



Alliance *for*
**Regenerative
Medicine**

**2020: Growth & Resilience
in Regenerative Medicine
ANNUAL REPORT**

2020



Alliance *for* Regenerative Medicine

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 and reimbursement 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 11-year history, ARM has become the global voice of the sector, representing the interests of 380+ 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.

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Letter from the CEO: Growth & Resilience

When we issued the Alliance for Regenerative Medicine (ARM) 2019 Annual Report a year ago, we never imagined what 2020 would throw at the world, the regenerative medicine sector, and ARM itself.

The sector's — and ARM's — accomplishments in 2020 are all-the-more remarkable amid the backdrop of COVID-19.

As the pandemic took hold, we planned for retrenchment. Instead, ARM's membership grew by 81 organizations in 2020 to exceed 380, cementing our role as the global voice of the sector. We hired five staff members to serve this growing membership, with a focus on operations and advocacy.

ARM adapted to the virtual world by converting October's Cell & Gene Meeting on the Mesa to an online format. Instead of convening in Carlsbad, California, nearly 1,200 participants joined from their homes. We also launched the Cell & Gene Webinar Series and introduced ARM Connect to replace canceled in-person meetings.

We responded to the powerful movement for racial equality by recognizing that we needed to address the underrepresentation of Black employees in the regenerative medicine sector. ARM stepped into action, forming the Action for Equality Task Force which created the GROW RegenMed Internship Program. At least sixteen ARM member organizations will welcome the first class of GROW interns this summer.

This was all possible because the regenerative medicine sector in 2020 raised a record amount of funding, achieved significant new scientific and clinical milestones, and brought new groundbreaking therapies to patients.

At the end of 2020, there were nearly 1,100 gene, cell, and tissue-based therapeutic developers worldwide, an increase of about 100 developers from 2019. These developers raised \$19.9 billion in financing, shattering the previous record of \$13.5 billion set in 2018.

Two new therapies received approval in 2020: Orchard Therapeutics' Libmeldy, a gene therapy to treat metachromatic leukodystrophy, or MLD, was approved by the European Medicines Agency, while Tecartus from Kite, a Gilead Company, was the first CAR-T treatment approved by the Food & Drug Administration (FDA) for relapsed or refractory mantle cell lymphoma. In February 2021, the FDA approved Breyanzi, a CAR-T therapy from Bristol Myers Squibb, to treat adults with relapsed or refractory large B-Cell lymphoma.

A growing pipeline of therapies are nearing regulatory decisions, with at least three additional decisions in the U.S. and five in Europe expected in 2021. There were 1,220 clinical trials ongoing at the end of 2020, with 152 of those trials in Phase 3 — supporting the FDA's and EMA's predictions they will approve 10-20 cell and gene therapies each year by 2025.

Beyond the new approvals, there were extraordinary scientific accomplishments and recognitions. Jennifer Doudna and Emmanuelle Charpentier won the 2020 Nobel Prize in Chemistry for their discovery of the CRISPR approach to genome editing, a technique that shows clinical promise to durably treat or cure sickle-cell disease, among other applications. We saw an increased focus on, and promising clinical data for, allogeneic cell therapies with growing applications of gene-editing approaches. Induced pluripotent stem cells (iPSCs) demonstrated positive results in the clinic, including from a first-ever application for Parkinson's disease.

ARM and the sector also achieved notable policy milestones. Our advocacy efforts led the Centers for Medicare & Medicaid Services (CMS) to create a DRG for CAR-T therapies that will ensure proper reimbursement for providers. In December, CMS also finalized a rule removing barriers to innovative payment models in Medicaid programs, a significant area of focus for ARM and its members. And the EU Pharmaceutical Strategy, a blueprint for a years-long legislative push, recognized cell and gene therapies as "milestones of major progress" in healthcare. As we expand our advocacy work through new hires and resources, ARM will devote even more attention to advancing this maturing sector's policy goals.

The year was not without its setbacks or growing pains, partly a result of fast-moving science and constrained regulator bandwidth due to COVID-19. We saw at least six therapeutic programs delayed because of CMC and manufacturing-related reasons, and another four delayed due to FDA requests for additional data. There are also significant market access challenges to overcome as we work with policymakers to accommodate the paradigm shift that durable, and possibly curative, therapies represent for healthcare systems.

These challenges, however, also signify progress. For a once aspirational sector, the future is now. We are grateful for the support of our members — therapeutic developers, patient advocates, and medical and research institutions — and will continue to work tirelessly to ensure that patients can access transformative, life-changing therapies.



Janet Lambert
Chief Executive Officer
Alliance for Regenerative Medicine

Investor Perspective

2020 was a pivotal year for regenerative medicine financing and progress, with the pandemic revealing the importance of cell and gene therapies and genetic medicines more broadly. Whether the momentum is sustained in 2021 depends on whether the interest rate environment supports capital flows. Our expectation is that even if the capital markets financing environment diminishes, large biopharma will continue to invest in or acquire disruptive technologies and support valuation. In 2021 and beyond, we will continue to see investor interest in *in vivo* gene therapy, gene editing, and cell-based therapies. Manufacturing and associated regulatory delays will continue as a gating factor due to the inevitable “growing pains” as academic work is translated to production at scale.

For *in vivo* gene therapy, we expect progress to be driven by success in new target tissues like the heart, immune regimens, gene regulation, non-viral delivery, and synthetic biology. For example, Rocket Pharma showed one of the first gene therapy successes in the heart with positive data in Danon disease, likely driving investment interest in private companies like Tenaya Therapeutics. On gene regulation, we note MeiraGTx may in 2021 release *in vivo* preclinical data for its gene regulation platform, a technology that could be used to enable the body as a factory for a range of proven biologics. With non-viral gene therapy, Generation Bio’s move to \$2B in valuation is occurring with private companies like enGene still poised to go public. Longer-term, we expect to see investments in synthetic biology, such as transgene engineering.

For gene editing in 2020, our positive thesis was confirmed, as (1) an *ex vivo* proof-of-concept emerged from CRISPR Therapeutics/Vertex Pharmaceuticals, and (2) *in vivo* CRISPR dosing began in Editas Medicine’s and Intellia Therapeutics’ respective trials. For 2021, we anticipate initial *in vivo* safety and activity data, as well as longer-term follow-up on various *ex vivo* and CAR-T programs, to continue to drive investor and large biopharma interest. Over the longer-term, we expect active investor interest in *in vivo* delivery of various cargoes, for example, players like Sana and Ensoma.

For cell-based therapy, we see more investment opportunities for solid tumors and for off-the-shelf pluripotent stem cell technology. For liquid tumors, there is some potential for oversaturation due to the variety of cell sources and companies targeting limited commercial opportunities. In solid tumors, clinical competition is less concentrated and response rates leave more room for improvement, e.g. by incorporating novel technologies to address challenges such as antigen heterogeneity or an immune-suppressive tumor microenvironment. Outside of IO, off-the-shelf pluripotent stem cell technology is promising, e.g. as 2 phase I trials are set to initiate in type 1 diabetes in 2021 from Vertex Pharmaceuticals and from CRISPR Therapeutics/ViaCyte. Positive results would ignite investor interest.



Gbola Amusa, MD, CFA
Partner, Director of Research,
& Head of Healthcare Research
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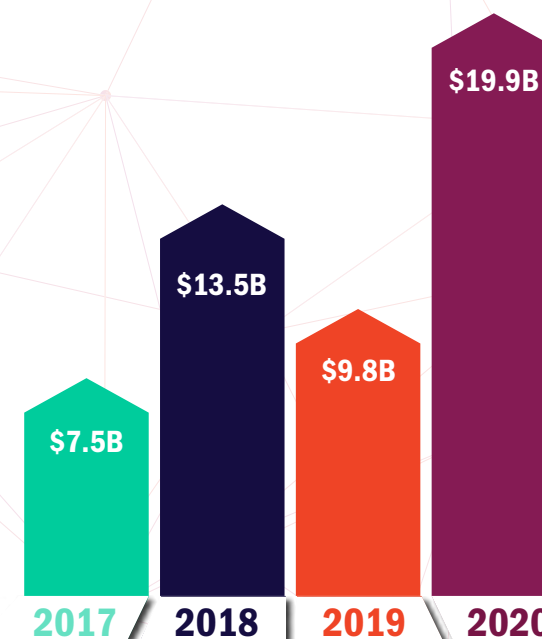
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Record Financings Bolster Maturing Sector

2020 was a watershed year for the rapidly evolving regenerative medicine and advanced therapy sector. These innovative therapies are demonstrating profound, durable, and potentially curative therapeutic benefits for patients with a wide array of disorders — and investors have taken note.

The sector raised nearly \$20B in 2020, shattering previous records despite the challenges posed by the COVID-19 pandemic. The sector even exceeded the previous record of \$13.5B, set in 2018, by the close of the third quarter, when it had raised \$15.9B. Individually, many companies were able to secure massive financing rounds, with 50 deals worth more than \$100M upfront.

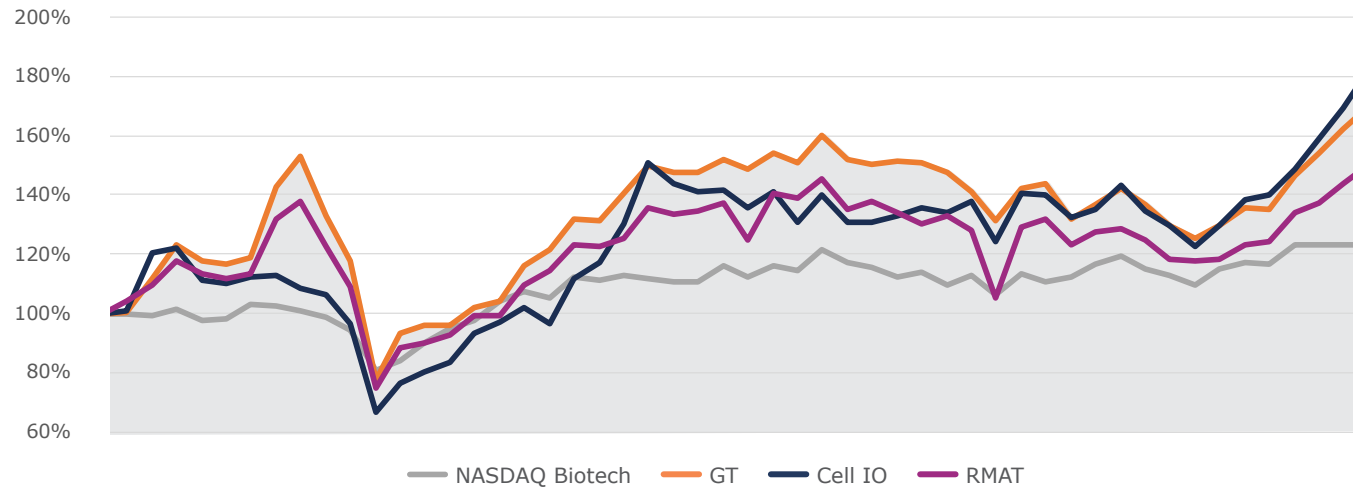
Total Global Regenerative Medicine Financing



**\$19.9B raised
in 2020**

*A 50% increase over
the previous record*

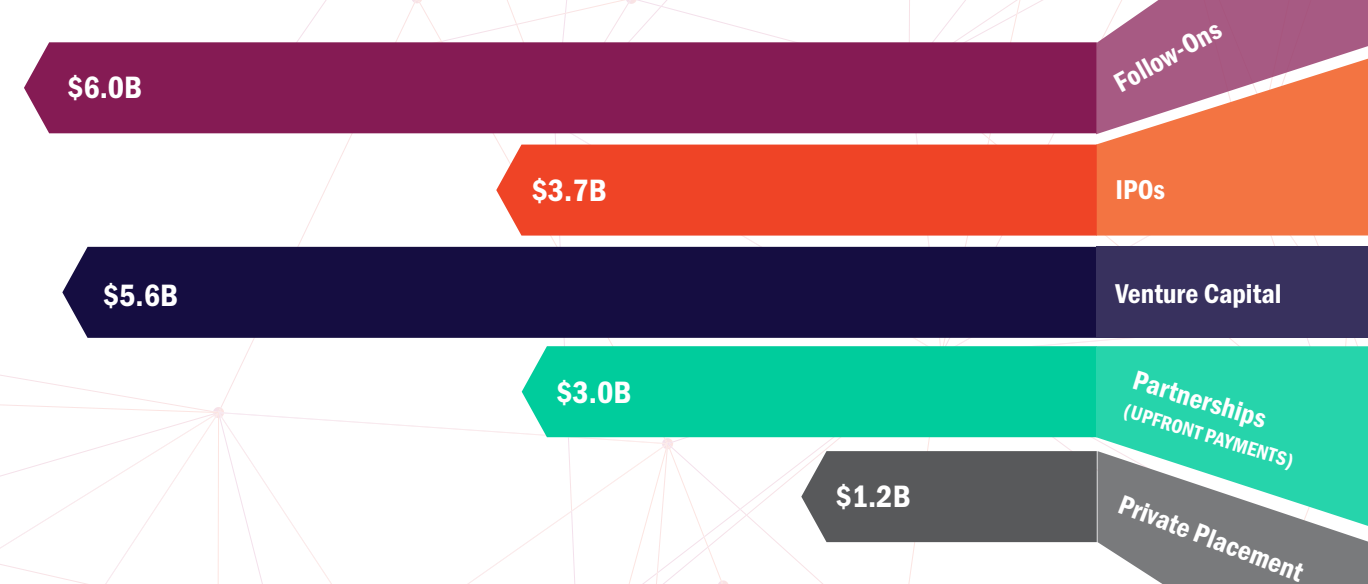
Public performance for biotech was strong in 2020 — but even stronger in the regenerative medicine sector. Initial losses in March due to the pandemic quickly reversed, and stock performance for regenerative medicine companies eclipsed that of the overall NASDAQ Biotech Index.



Cell-based IO: 82% increase **Gene therapy: 70% increase**
All regenerative medicine: 44% increase **NASDAQ Biotech: 23% increase**

Publicly traded regenerative medicine companies saw about a 44% increase in performance in 2020. While it was a strong year for the biotech industry as a whole, the performance for regenerative medicine companies eclipsed the NASDAQ Biotech Index, which increased by 23%. Given that strong track record, it's not surprising that both initial and follow-on public offerings — totaling \$3.7B and \$6.0B, respectively — surpassed those in previous years. In total, 14 developers went public in 2020, compared to 6 in 2019 and 12 in 2018.

Financings by Deal Type



Private financing was also abundant. Venture financing has been strong in recent years, and 2020 was no exception, with investors raising \$5.6B, surpassing the \$4.1B record set in 2019. Venture financing benefited both new and established companies, with \$2B raised in Series A financings; \$864M in Series B; \$642M in Series C; and \$684M in Series D and later rounds. These companies are now among the 1,085 developers worldwide looking to advance this unique field of medicine.

Investors were not the only group drawn to this innovative sector. Large pharmaceutical companies, recognizing the potential of these cutting-edge technologies, sought partnership opportunities with smaller, more nimble biotech startups. Key partnerships from this year include Biogen and Sangamo's \$350M upfront agreement to develop gene regulation therapies utilizing zinc finger proteins for neurodegenerative disorders and Janssen (Johnson & Johnson) and Fate Therapeutics' \$100M upfront deal to develop cell-based immunotherapies for hematologic and solid tumors. Those deals contributed to a whopping \$3B raised in upfront payments from corporate partnerships — with additional clinical and regulatory milestones potentially worth billions more.

\$100M+ Financings from 2020

IPOs

- Legend Bio — \$487M
- AlloVir — \$317M
- Nkarta — \$290M
- Passage Bio — \$248M
- Akouos — \$244M
- Generation Bio — \$230M
- Poseida Tx — \$224M
- Beam Tx — \$207M
- Taysha Gene Tx — \$181M
- Freeline Tx — \$159M
- Immunotech — \$142M

Follow-Ons

- Allogene — \$632M
- Iovance Bio — \$604M
- bluebird bio — \$575M
- CRISPR Tx — \$426M
- Adaptimmune Tx — \$259M
- Adverum Bio — \$217M
- Editas Medicine — \$216M
- Atara Bio — \$202M
- Fate Tx — \$201M
- Adverum Bio — \$150M
- TCR2 Tx — \$143M
- Beam Tx — \$135M
- Krystal Bio — \$125M
- IVERIC — \$117M
- Intellia Tx — \$115M
- Replimmune — \$115M
- AVROBIO — \$100M

Venture Capital

- Sana Bio — \$700M
- Orca Bio — \$192M
- ElevateBio — \$170M
- Legend Bio — \$150M
- Encoded Tx — \$135M
- Freeline Tx — \$120M
- Imvax — \$112M
- Generation Bio — \$110M
- Neogene Tx — \$110M
- Vor Bio — \$110M
- Poseida Tx — \$110M
- Akouos — \$105M
- JW Tx — \$100M

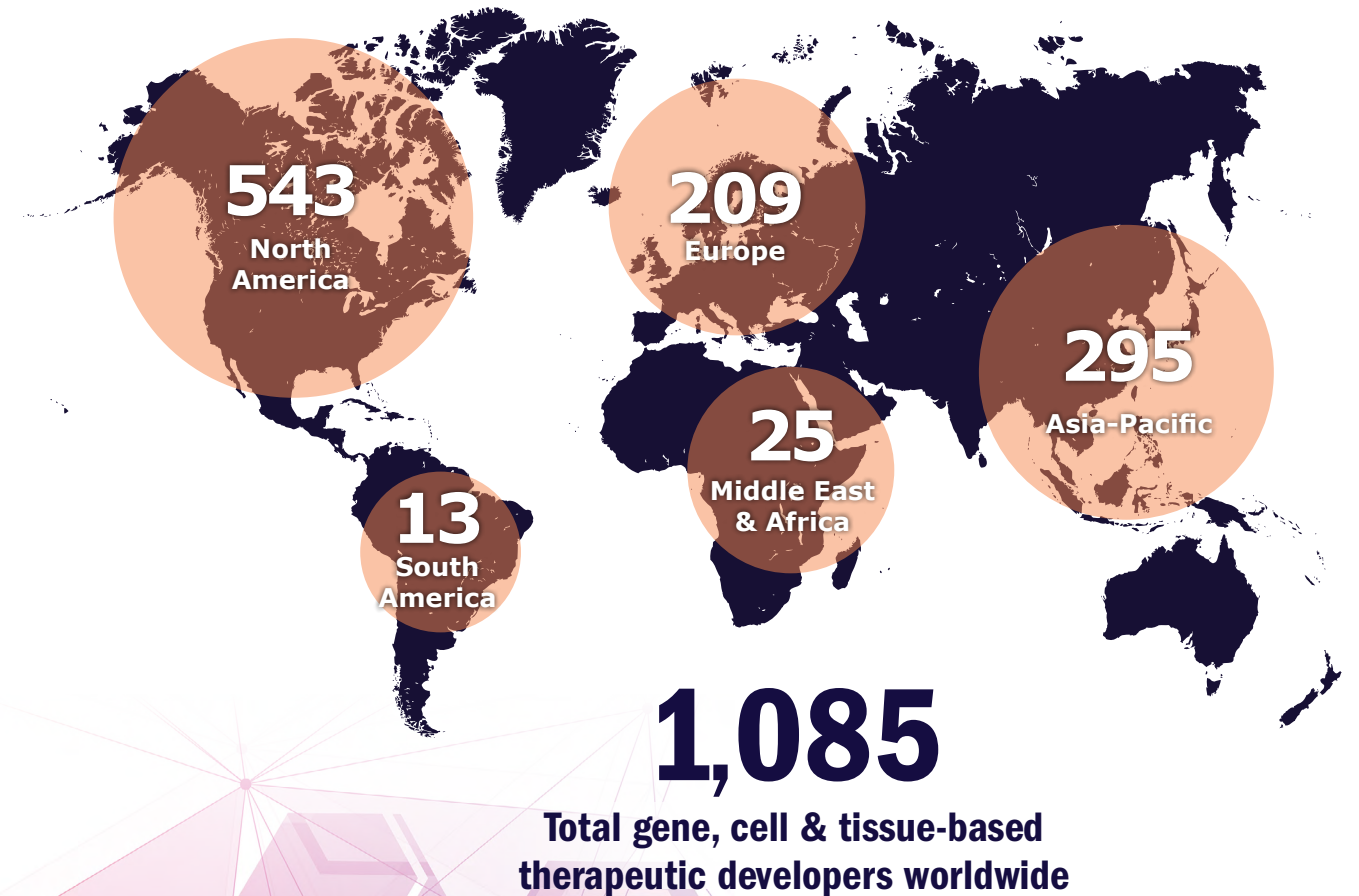
Private Placement

- Lyell Immunopharma — \$493M

Partnerships (UPFRONT PAYMENTS)

- Roche / Sarepta — \$1.6B
- CSL Behring / uniQure — \$450M
- Sangamo / Biogen — \$350M
- Ultragenyx / Daiichi Sankyo — \$200M
- Bluebird bio / BMS — \$200M
- Janssen / Fate Tx — \$100M
- Regeneron / Intellia — \$100M

ARM is excited to see the confidence that investors have in our member companies as the sector continues to grow. In 2021, we look forward to seeing how developers translate these financings into scientific advancements and — ultimately — therapeutic benefits for patients.



Real Life Miracles: The Price Family



Giovanni and Cecilia Price, holding a photo of their late sister Liviana

The gene therapy field is rapidly advancing, with the potential to durably treat and possibly cure tens of thousands of patients with rare genetic diseases, serious cancers, and other severe disorders in coming years. This innovative technology may seem like science fiction to some. But for the Price family, gene therapy has already delivered two real life miracles.

In 2010, Amy and Brad Price received devastating news. Their two-year-old daughter, Liviana, was diagnosed with metachromatic leukodystrophy (MLD), a genetic disease that leads to progressive paralysis, dementia, and — historically — death by the age of six. By the time Liviana received her diagnosis, the disease had already progressed too far to be treated. She passed away in 2013 at five years old.

Shortly following Liviana's diagnosis, Amy and Brad's son Giovanni also tested positive for the disorder, though he was not yet showing symptoms. In 2011, Amy left Liviana and Brad in Omaha, Nebraska, to take Giovanni to Italy for six months, where he was treated with an experimental gene therapy.

Ten years later, having far surpassed the life expectancy for most children with MLD, Giovanni remains healthy — even cured, by all appearances. Another of Amy and Brad's children, Cecilia, was also diagnosed with MLD and received gene therapy in 2014. Like Giovanni, she remains healthy, showing no sign of the disease that killed Liviana.

The gene therapy, Libmeldy, received approval in Europe in December 2020. Developer Orchard Therapeutics plans to submit a biologics license application (BLA) for the therapy in the U.S. later this year.

The Price Family

“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 ten years post gene therapy.”

—Amy Price, mother of two gene therapy patients

In 2021, regulatory decisions are expected on at least eight new regenerative medicines and advanced therapies worldwide. Dozens of additional product candidates are in late-stage development. More patients than ever stand to benefit from these cutting-edge therapies — but a robust infrastructure for the development, approval, and reimbursement is needed to ensure patients like Giovanni and Cecilia can access the treatments they need.

As the global voice of the sector, ARM is committed to working with industry, patients, regulators, and policymakers to realize the profound therapeutic benefits of this field.



Giovanni Price becomes the second patient to receive gene therapy for MLD



The Price family

The View from Science & Industry Affairs: Manufacturing Key to Pipeline Advancement

There was a remarkable amount of growth in the regenerative medicine and advanced therapies sector in 2020, despite the COVID-19 pandemic. The number of developers in the space is now up to 1,085. We've seen some significant scientific milestones this year in areas including iPSCs, gene editing, and allogeneic cell-based immunotherapies. And the regenerative medicine pipeline continues to expand.

While many clinical trial sites initially paused new enrollment because of the pandemic, most trials have resumed. There may be longer-term effects on the sector due to follow-up issues and missed data, but developers have largely weathered the difficulties presented by COVID-19. Looking forward, we expect the number of patients who benefit from these innovative therapies to grow. Therapies targeting indications with larger patient populations — such as diabetes, cardiovascular disease, stroke, Alzheimer's and Parkinson's disease — continue to enter the clinic.

COVID-19 created some ongoing supply chain interruptions, but manufacturing capacity fared well overall in 2020. It remains to be seen whether there will be any knock-on effects as vaccine manufacturing scales up, which will consume a lot of manufacturing capacity. Conversely, it's possible that the surge in manufacturing to produce vaccines using viral vectors may ultimately benefit the manufacturing of viral vectors to be employed in gene therapies. Contract manufacturing organizations are working to rapidly expand their capacity. Recently announced expansions include FUJIFILM's \$2B investment in a new large-scale cell culture production plant; Thermo Fisher's recent \$880M acquisition of viral vector manufacturer Honegen; and Cytivia's \$500M plans to increase global manufacturing capacity.

However, the complex manufacturing process for cell and gene therapies — and the regulatory environment surrounding it — remains a key challenge for the sector.

We saw several programs' timelines elongated in 2020 due to regulators' feedback concerning manufacturing and CMC. Given the innovative nature of this field, many existing CMC guidelines are not tailored to the complexities of cell and gene therapies. Promoting CMC regulatory clarity and flexibility remains a key priority for ARM. In 2021, ARM will roll out A-Genie, a case study of the application of Quality by Design (QbD) principles to AAV vector manufacture. ARM is also working on a similar product focusing on cell therapy manufacturing.

ARM also plays a crucial role in the global dialogue around the proper regulatory and ethical framework for genome editing. ARM will work to maintain an appropriate regulatory framework for somatic cell gene editing, and will continue to engage with the World Health Organization to influence the global gene-editing dialogue.

2021 will likely be another strong year for the sector, with increasing focus on dosing, repeat administration for gene therapies, and development and commercialization of therapies for ultra-rare disorders. We're looking forward to working with stakeholders to help clarify the regulatory environment, provide a forum for discussion between developers and regulators, and advocate for the resources regulators need to adapt to this new age of science.



Michael Lehmicke
Senior Director, Science & Industry Affairs
Alliance for Regenerative Medicine

2020 & YTD 2021 Approvals

Breyanzi (Bristol Myers Squibb)
R/R diffuse large B cell lymphoma
U.S. (Feb. 2021)

Libmeldy (Orchard Therapeutics)
Metachromatic leukodystrophy
Europe (Dec. 2020)

Luxturna (Spark Therapeutics)
Retinal dystrophy caused by biallelic RPE65 mutations
Canada (Oct. 2020)

Tecartus (Kite, a Gilead company)
R/R mantle cell lymphoma
U.S. (July 2020), Europe (Dec. 2020)

Yescarta (Kite, a Gilead company)
R/R diffuse large B-cell lymphoma
Japan (Jan. 2021)
R/R follicular lymphoma
U.S. (March 2021)

Zolgensma (Novartis Gene Therapies)
Spinal muscular atrophy
Japan (March 2020), Europe
(May 2020), Canada (Dec. 2020)

Upcoming Regulatory Decisions

Breyanzi (Bristol Myers Squibb)
R/R diffuse large B cell lymphoma
Europe, Japan

Cilta-cel (Janssen & Legend Biotech)
R/R multiple myeloma
U.S.

Eli-cel (bluebird bio)
Cerebral adrenoleukodystrophy
Europe

GT-AADC (PTC Therapeutics)
AADC deficiency
Europe

Ide-cel (Bristol Myers Squibb & bluebird)
R/R multiple myeloma
U.S., Europe

JWCAR029 (JW Therapeutics)
R/R diffuse large B-cell lymphoma
China

Lumevoq (GenSight Bio)
Leber hereditary optic neuropathy
Europe

Stratagraft (Mallinckrodt)
Severe burns
U.S.

Yescarta (Kite, a Gilead company)
Additional r/r lymphomas
U.S.

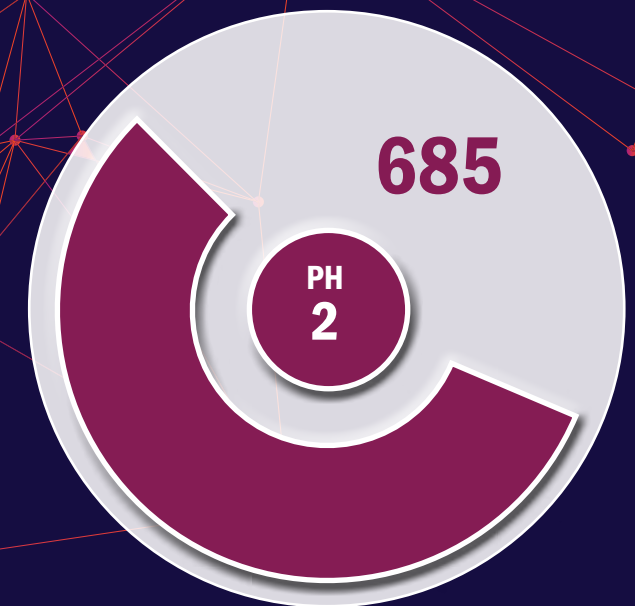
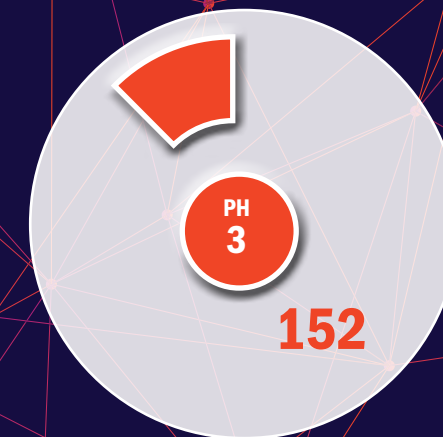
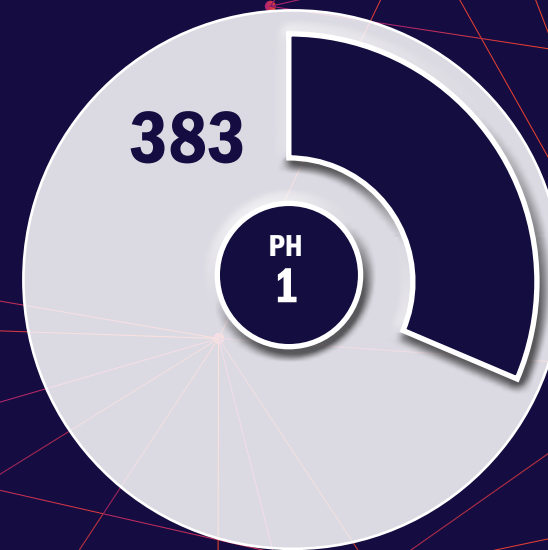
Progressing Through the Pipeline

The regenerative medicine sector is maturing rapidly — and more patients than ever stand to benefit. New therapies can provide unprecedented benefits to patients with severe disorders. Tecartus, a CAR-T therapy developed by Kite, a Gilead company, and approved in the U.S. in 2020, has a nearly 90% response rate among patients with relapsed or refractory (R/R) mantle cell lymphoma. Libmeldy, which received its first approval in Europe in 2020, is the first approved therapy to treat early-onset metachromatic leukodystrophy, a historically fatal genetic disease. The therapy, which has shown in clinical trials that it can preserve cognitive and motor function for at least eight years post-treatment, represents an immense step forward for patients with this terrible disease.

There are more ongoing clinical trials in regenerative medicine than ever before, with 1,220 trials aiming to enroll more than 90,000 patients worldwide. With 152 trials in Phase 3 — and regulatory decisions expected in the U.S., Europe, and globally on at least eight additional product candidates in 2021 — more patients will soon benefit from regenerative medicines. In February, the U.S. FDA approved Bristol Myers Squibb's CAR-T therapy Breyanzi, the first therapy with the FDA's Regenerative Medicine Advanced Therapy (RMAT) Designation, for the treatment of diffuse large B-cell lymphoma.

Representatives from the U.S. FDA and the European Medicines Agency have predicted that those agencies will each be approving 10-20 cell and gene therapies per year by 2025. And expedited approval pathways for innovative therapies — such as the U.S. FDA's RMAT Designation and Europe's PRiority Medicines (PRIME) Designation — continue to accelerate the pipeline.

Clinical Trials by Phase: 2020



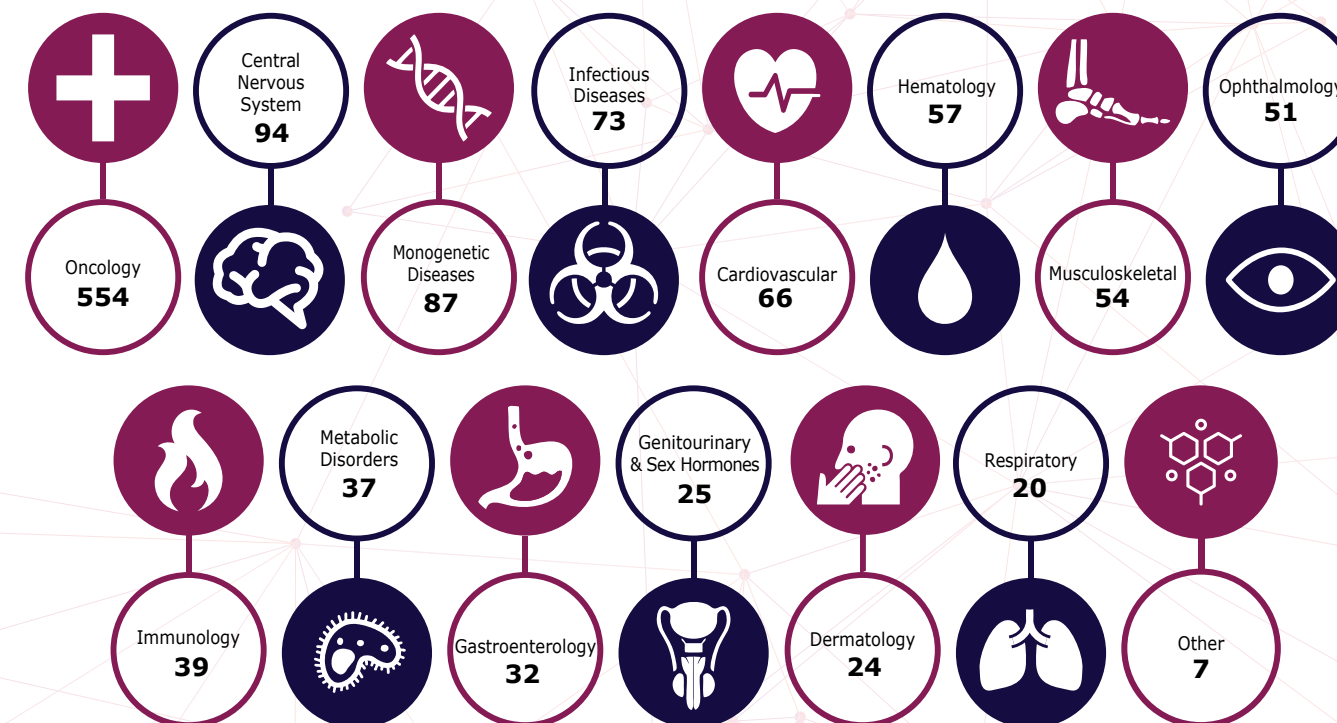
2020 RMAT Designations

- AB205 (Angiocrine Bioscience) — organ vascular niche injuries in lymphoma patients
- Multistem (Athersys) — acute respiratory distress syndrome
- AMDC-USR (Cook Myosite) — urinary sphincter repair
- CTX001 (CRISPR & Vertex) — severe hemoglobinopathies
- Ilixadencel (Immunicum) — metastatic renal cell carcinoma
- MDR-101 (Medeor Therapeutics) — prevention of kidney transplant rejection
- Kymriah (Novartis) — r/r follicular lymphoma
- Orca-T (Orca Bio) — hematopoietic stem cell transplant
- TT11 (Tessa Therapeutics) — r/r Hodgkin lymphoma
- TTAX02 (TissueTech) — spina bifida

2020 PRIME Designations

- AAV-RPGR (MeiraGTx) — retinitis pigmentosa
- ADPA-2M4 (Adaptimmune) — synovial sarcoma
- ATGTX-501 (Amicus Tx) — Batten disease
- CTX-001 (CRISPR Tx / Vertex) — sickle cell disease
- ECT-001 (ExCellThera) — hematopoietic malignancies
- OTL-203 (Orchard) — mucopolysaccharidosis type I
- Posoleucel (AlloVir) — serious infections in allogeneic hematopoietic stem cell transplantation (HSCT) recipients
- TT-11 (Tessa Tx) — r/r Hodgkin Lymphoma
- Zynteglo (bluebird bio) — sickle cell disease

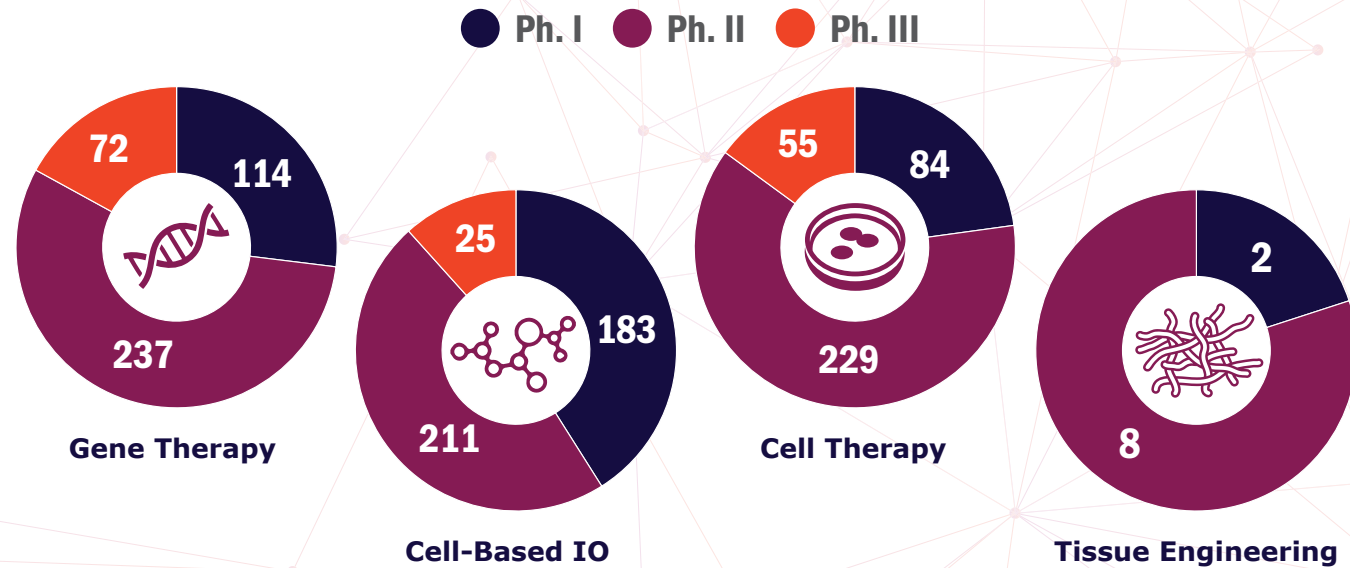
Clinical Trials by Indication: 2020



Cell-based immuno-oncology continues to make up a growing proportion of the pipeline, including about half of Phase 1 trials. We've seen promising clinical data readouts from a diverse array of approaches, including B cell maturation antigen (BCMA), as well as programs focused on other targets. In addition, we've seen data from multiple companies combining gene-editing approaches — such as ARCUS, TALENs, and, more recently, CRISPR — and allogeneic therapies. These therapies, which do not require engineering a patient's own cells, may be faster and more cost-efficient to produce. There are 290 ongoing trials at the end of 2020 using allogeneic approaches.

We've also seen considerable advancement in earlier clinical and preclinical technologies, including induced pluripotent stem cells (iPSCs). Fate Therapeutics reported positive data from ongoing trials of iPSC-derived NK cell therapies — providing compelling in-human evidence of the potential effectiveness of this therapeutic approach. Also in 2020, researchers published data from the first-ever attempt to use iPSCs to treat Parkinson's disease, showing increased dopamine and consequential functional improvements in patients out to 24 months.

Number of Clinical Trials by Technology Type: 2020



Gene editing continued to advance in the clinic. In the spring, Editas Medicine treated the first patient with a clinical trial for Leber congenital amaurosis 10, the first trial administering an *in vivo* CRISPR therapy. Later in the year, Intellia Therapeutics treated the first patient with an intravenous CRISPR therapy as a part of the company’s trial for transthyretin amyloidosis. In addition to the data presented from trials of gene edited CAR-T therapies, CRISPR Therapeutics and Vertex Pharmaceuticals shared promising preliminary data from their trial of a gene edited therapy for sickle cell disease and beta thalassemia. So far, 19 patients have been treated, with all evaluable sickle cell patients free of vaso-occlusive crises and all evaluable beta-thalassemia patients transfusion independent. The promise of cutting-edge gene-editing technologies was recognized globally in October when Emmanuelle Charpentier, co-founder of CRISPR Therapeutics, and Jennifer Doudna, co-founder of Intellia Therapeutics, received the Nobel Prize in Chemistry for their work in developing CRISPR gene editing. While first-gen editing technologies such as ZFNs have already demonstrated proof-of-concept in the clinic in recent years, we’re now beginning to see these technologies rev up to treat a range of new, more complex indications ranging from HIV to solid organ transplantation.

By now, there is no doubt that regenerative medicine is an established therapeutic modality — with commercial products, a deep pipeline, strong support from large pharma and biotech, and consistent investor enthusiasm. Nevertheless, advancing this cutting-edge science is not without its challenges. In 2020, we grappled with issues such as dosing, delivery, and CMC. With a robust pipeline, we expect to continue to mature and learn — with advances as well as setbacks — as more products move toward and into the market.

The View from Public Affairs: ARM Expands Advocacy Focus

When COVID-19 put the world on defense in 2020, ARM invested in becoming more proactive. A maturing sector needed a single global voice to ensure that policies and regulations could deliver scientific advancements that were no longer the future, but the present.

So we combined three independent areas — policy, advocacy, and communications — under the umbrella of public affairs. Moving in the same direction, each function contributed to a unified whole that was greater than the sum of its parts.

This strategy rested upon strategic hires. I joined in May to lead our global public affairs function and spearhead the integration. In August we hired Stephen Majors as Director of Public Affairs to lead our global communications strategy and to support the integration. We brought on Finsbury Glover Hering, a strategic public affairs consultancy in Brussels, to guide us as the EU Pharmaceutical Strategy ramped up. We hired political consultants in Germany and France — two of our most important European markets — to deliver country-specific strategies with local voices.

In the U.S., we launched state-level advocacy in Ohio and Texas to prepare state lawmakers for the coming wave of regenerative medicines. And we hired U.S.-based JPA Health to support our social media strategy, recognizing that this medium will be instrumental to our communications efforts.

We saw some important policy wins in 2020 that represent what we can accomplish with an enhanced focus on advocacy. The Centers for Medicare & Medicaid Services finalized a dedicated DRG for CAR-T therapies addressing liquid tumors, a long-time goal for ARM. CMS also finalized a rule in December 2020 that introduces changes allowing state Medicaid programs to enter innovative payment arrangements with regenerative medicine developers. Notably, members of Congress approached ARM for our recommendations and guidance around legislative language to address innovative payment models and CMC flexibility.

The expansion of ARM Public Affairs is not stopping in 2021.

We are deepening our expertise and specialization to serve a growing membership and address our most urgent public affairs priorities. Director Robert Falb will focus exclusively on U.S. Regulatory Affairs, and we will hire a new Director of U.S. Government Relations & Advocacy to oversee the U.S. Government Relations and Market Access Committees and lead our advocacy with Capitol Hill and the Administration. We will bring on a Senior Manager to run our state advocacy initiatives day-to-day and lead our patient advocacy efforts. We have created a new role — Director of European Public Affairs & Advocacy — to be ARM’s face in Brussels and are looking for a new consultant to lead our European Regulatory Affairs.

Our goal is simple: From Amsterdam to Zanesville, we will be the global voice of the sector for policy and legislative expertise, regulatory guidance, insights for media, and sector trends and data.



Paige Bischoff
Senior Vice President, Global Public Affairs
Alliance for Regenerative Medicine

209 Companies developing cell & gene therapies in Europe

Europe Policy & Regulatory Update

The next few years will be a critical time for patient access to Advanced Therapy Medicinal Products (ATMPs) — including cell and gene therapies — in Europe.

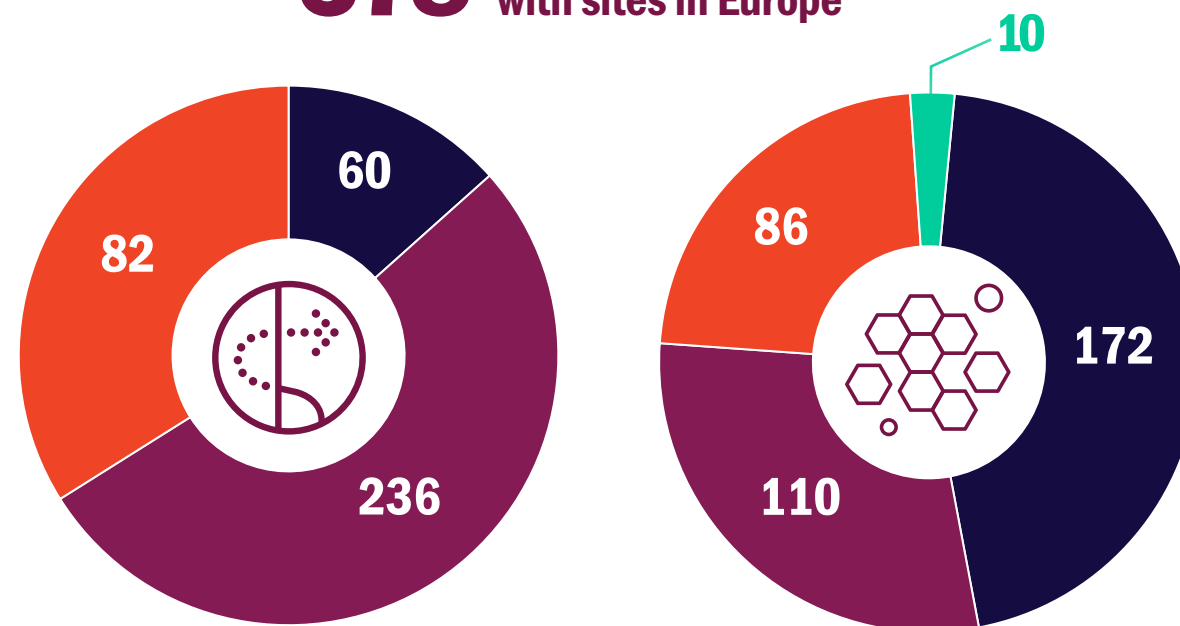
In November 2020, the European Commission released its Pharmaceutical Strategy, a high-level blueprint to address patient access, unmet medical needs, and innovation. The Pharma Strategy will serve as the basis for legislation over the next couple of years, and ARM will engage with a broad range of European stakeholders to shape a positive market access and regulatory environment for regenerative medicine. The strategy and associated legislation will shape the environment for years to come, requiring extensive engagement from ARM and our members to ensure that Europe continues to be a top destination for sector investment.

Notably, the Pharma Strategy highlighted ATMPs — and specifically cell and gene therapies — as “milestones of major progress” in healthcare and the importance of having an interoperable data access infrastructure in place by 2025, which could facilitate the use of real-world evidence. ARM and our members will advocate for innovative payment models to account for the durable, and potentially curative, nature of ATMPs.

Several other legislative and regulatory initiatives important to the sector are running in parallel with the Pharma Strategy. ARM is contributing policy recommendations to the EU Commission for its review of the Cross-Border Directive to ensure that patients can access ATMPs, which are often only available in specialized centers across national borders. ARM will also contribute recommendations for the revision of Europe’s Blood, Tissue, and Cells legislation to safeguard the classification of ATMPs and high regulatory standards for these products, and to prevent deregulation. The EU Commission has signaled its intention to ensure that rules around genetically modified organisms (GMOs) are fit-for-purpose for addressing medicines, another area where ARM will be heavily engaged in 2021.

ARM — as the global voice of the sector — stands ready to partner with a range of European stakeholders to ensure a paradigm shift in payment models and continued innovation in the ATMP sector, which is the future of medicine. We are also active at the country level in priority countries, where our advocacy includes working via legislation to change the NUB payment process in Germany and to facilitate annuity-based payments in France.

378 Ongoing clinical trials with sites in Europe



Breakdown by Phase

Breakdown by Tech Type

● Ph. I ● Ph. II ● Ph. III

● Gene therapy ● Cell-based IO
● Cell therapy ● Tissue engineering

\$3.6B raised by companies HQ'd in Europe

U.S. Policy & Regulatory Update

Following the achievement of several key policy and regulatory milestones in 2020, the sector will continue to pursue regulatory and market access priorities in 2021 amid the backdrop of a new administration and Congress.

ARM will advocate for CMC regulatory flexibility to address manufacturing hurdles for the rapidly evolving cell and gene therapy sector. ARM will engage with FDA to harmonize regulatory approaches with the European Medicines Agency and Japan's Pharmaceuticals and Medical Devices Agency where possible and to identify common issues, including potency assays, empty/full ratios and purity for AAV and LV vectors. The focus will also be on CMC requirements for platform technologies (e.g. viral vectors) and how data can be leveraged across multiple products to reduce the regulatory burden while still ensuring safety.

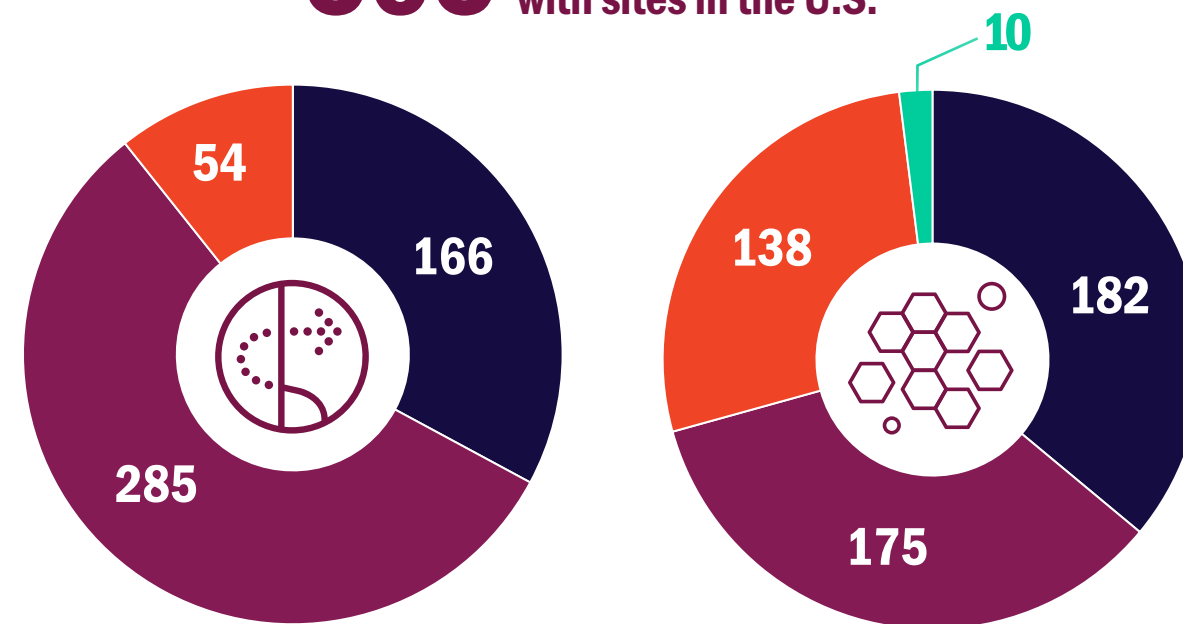
Another of ARM's top priorities is to advance innovative payment models to improve patient access to regenerative medicines. There are multiple potential legislative vehicles for this effort, including a bill being drafted to remove the major legislative and regulatory hurdles to the establishment of "outcomes-based agreements" and a "CURES 2.0" bill to update the 21st Century Cures Act, which was signed into law in December 2016 and, with ARM's support, created the RMAT designation. These initiatives follow a major sector achievement in December 2020, when the Centers for Medicare & Medicaid Services (CMS) finalized a rule generally supported by ARM that addresses barriers to the adoption of innovative payment models in state Medicaid programs.

ARM and other stakeholders achieved another notable CMS milestone last year when the agency finalized a rule to establish a dedicated DRG for the reimbursement of CAR-T therapies for liquid tumors. Building on the lessons learned from this multi-year effort, ARM in 2021 established a working group to focus on the creation of a similar DRG for solid tumors and to modernize the New Technology Add-on Payment.

In late 2020, ARM for the first time launched sector advocacy activities at the state level, in Ohio and Texas. In 2021, ARM has begun introducing the sector to state lawmakers and key agency officials in both states, and providing guidance around how states can lead on the adoption of innovative payment models in state Medicaid programs.

512 Companies developing cell & gene therapies in the U.S.

505 Ongoing clinical trials with sites in the U.S.



Breakdown by Phase

Breakdown by Tech Type

● Ph. I ● Ph. II ● Ph. III

● Gene therapy ● Cell-based IO
● Cell therapy ● Tissue engineering

\$11.8B raised by companies HQ'd in the U.S.

ARM Advances Alongside

Maturing Sector

As we've seen throughout this report, the regenerative medicine and advanced therapy sector is growing rapidly — and ARM is growing with it.

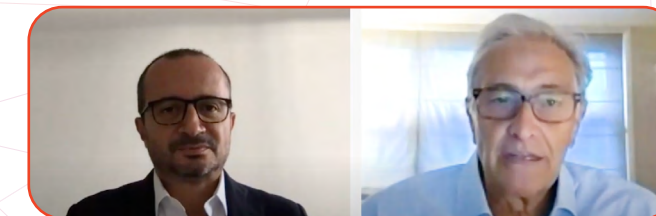
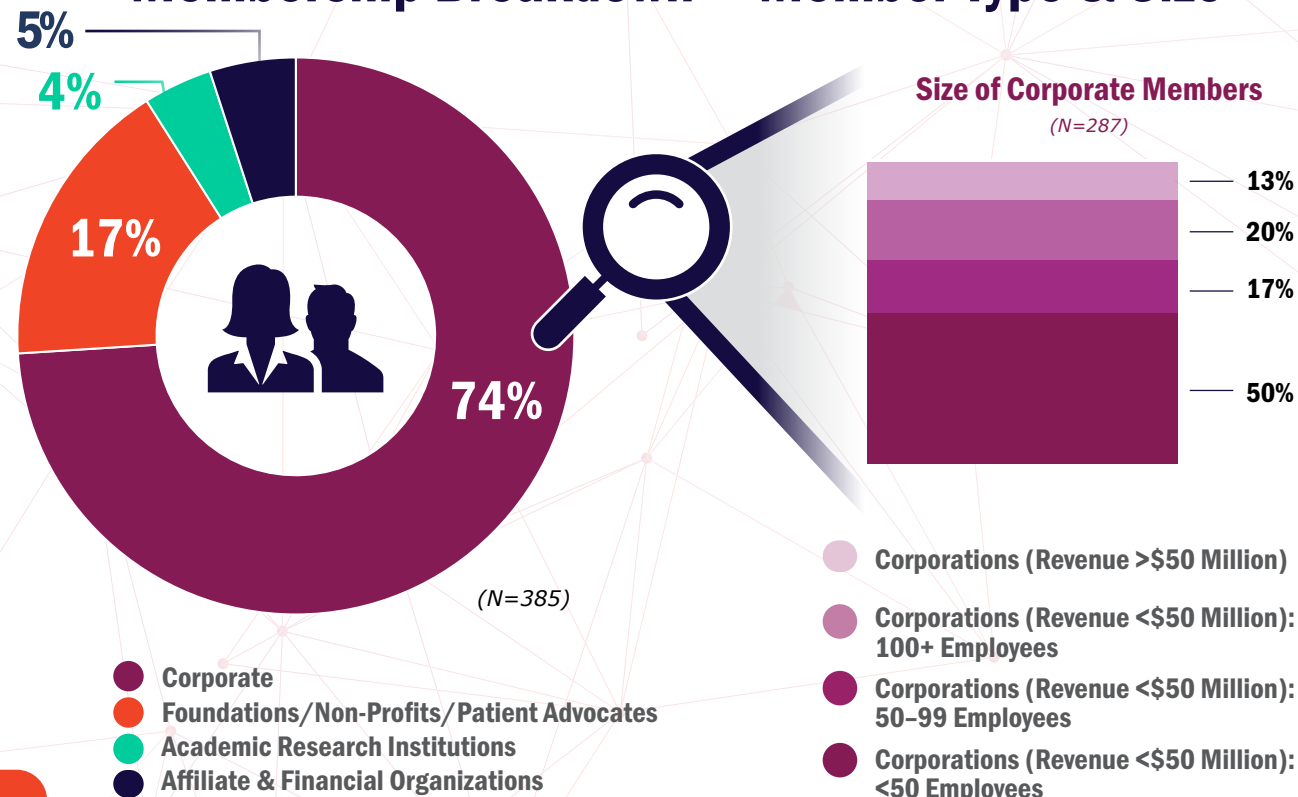
Despite the challenges of the pandemic, ARM surpassed our membership goals in 2020, closing out the year with more than 380 members from across the sector, including therapeutic developers, large pharmaceutical companies, contract manufacturing organizations, medical and academic research centers, patient organizations, and other stakeholders in this promising field. While we're seeing a number of new products come to market, the majority of ARM members are still pre-revenue, pursuing cutting-edge technological approaches to bring new therapies to a wide variety of indications with small and large patient populations. In 2021, ARM will launch a new member committee, The Accelerator, to address the needs of these early-stage developers.

Virtual Programming

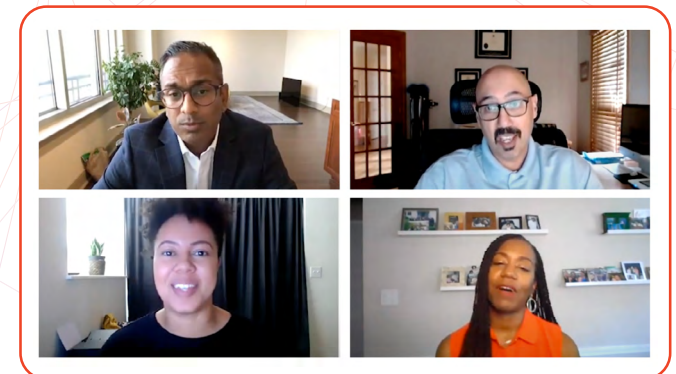
In addition to ARM's policy and advocacy work detailed earlier in the report, the organization brings considerable value to members through networking opportunities with other sector stakeholders. 2020 required all of us to adapt to working remotely, and the ARM team provided innovative ways for the sector to connect. This past spring, ARM hosted a popular webinar series looking at the potential effects of COVID-19 on sector financing, the clinical landscape, and market access. ARM also launched ARM Connect, a virtual messaging platform providing new networking opportunities in place of in-person events.

ARM's annual Meeting on the Mesa took place virtually in October and attracted more than 1,200 participants. The meeting featured 28 panels, 115 presentations from leading public and private companies in the sector, and key insights from regulators including Peter Marks, Director of the Center for Biologics Evaluation and Research at the U.S. FDA, and Guido Rasi, then Executive Director of the EMA. ARM is gearing up for our Meeting on the Med, Europe's premier gene and cell therapy conference, which will take place April 6-9.

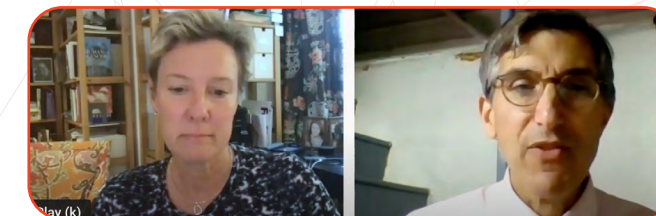
Membership Breakdown — Member Type & Size



Paolo Morgese, Director of European Market Access at ARM, discusses the European ATMP environment with former Executive Director of the EMA Dr. Guido Rasi.



ARM's Action for Equality Task Force discusses new initiatives for racial equality in the sector.



Dr. Peter Marks, head of the Center for Biologics Evaluation & Research (CBER) at FDA, tells ARM CEO Janet Lambert that gene therapy is what gets him through "COVID-19 laden days".

GROW RegenMed Internship Program

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. The AFE Task Force recognized that the representation of minority populations, in particular the Black population, is significantly below those populations' representation in broader society.

Thanks to the work of the AFE Task Force, ARM has launched the GROW RegenMed Internship Program to provide crucial, early-career paid opportunities in the regenerative medicine sector, initially to Black students and over time to a broader minority population. ARM and the AFE Task Force believe that improving the representation of Black employees at ARM member organizations and cultivating a community of these future leaders is fundamental to achieving the full promise of our work. At least sixteen ARM members will be hosting the first class of interns this summer.

Capacity Building

In 2020, ARM also built capacity to facilitate our enhanced advocacy focus and new programming and opportunities for members. We hired Paige Bischoff, our first Senior Vice President of Global Public Affairs; Rashida Dujue-Jackson, who manages the GROW Internship Program; and brought on Aishat Magbade to help manage the growing workload for the membership and public affairs teams. In addition, we hired a new Vice President, Operations and Finance, Bryan Proctor, and a new Director of Public Affairs, Stephen Majors. ARM will continue to grow in 2021, with an additional four new positions to be created.

ARM also launched four new advisory committees — three technology-focused committees working on gene, cell, and tissue-based therapies respectively, as well as a European Advisory Committee — to provide expert guidance on upcoming work. The members of these committees, alongside ARM's Board of Directors and seven member committees, are an invaluable asset to the organization as we continue to expand.

What's Next?

ARM has led the regenerative medicine sector and its advocacy efforts for more than a decade. The only global organization focused specifically on the issues facing cell, gene, and tissue therapy companies, we have successfully convened the sector to address sector-wide policy issues. ARM led the creation of the RMAT designation, drove the elimination of the duplicative NIH RAC review for gene therapies, and produced the first industry statement of gene editing principles, among other accomplishments.

We are excited to enter 2021 with significant momentum, a stronger team, a larger membership, constructive relationships with key decisionmakers, and a strong reputation as the global voice of the sector. We look forward to continuing our work with stakeholders to realize the incredible promise of the regenerative medicine and advanced therapy sector.

Years ago, when presented with the opportunity to join the ARM board, I considered it with a simple question in mind. *Will this activity be meaningful?* The answer was easy; the mission of ARM is profoundly important and meaningful. We are the convening force for the many stakeholders in our field whose voices are critical to our collective progress, and to ensuring our breakthrough medicines make it to the patients who need them. The progress ARM makes will help our entire industry to realize the promise of regenerative medicine.

Last year was a year like no other for ARM, the cell and gene therapy sector, and the biopharmaceutical industry. While there were many headwinds in 2020, ARM had a remarkable year in almost every measurable category, from policy and leadership success, to member growth and influence. Most importantly, we were swept along with the tide of excitement for the breakthrough medicines our membership is developing.

As we enter 2021, I am excited by the opportunities ahead, and motivated by the challenges of helping to navigate ARM through this energetic landscape. We need to focus on meeting our strategic objectives, while simultaneously considering the macro trends in our industry: the changing size and composition of our membership, the growing complexity and maturity of the medicines in development, the growth of other organizations in the cell and gene therapy community, and the increasingly global nature of regenerative medicine. Our goal is to ensure ARM's strategic plan considers this shifting and growing landscape, and positions ARM towards the future to expand its leadership voice and position.

I am honored to help lead ARM and look forward to sharing a great year with you in 2021.

Sincerely,



Emile Nuwaysir, Ph.D.

ARM Chair

President & CEO, BlueRock Therapeutics

ARM 2021 Board Officers



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Emile Nuwaysir

*President and Chief Executive Officer
BlueRock Therapeutics*



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Joe Tarnowski, Ph.D.
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Platform, GlaxoSmithKline

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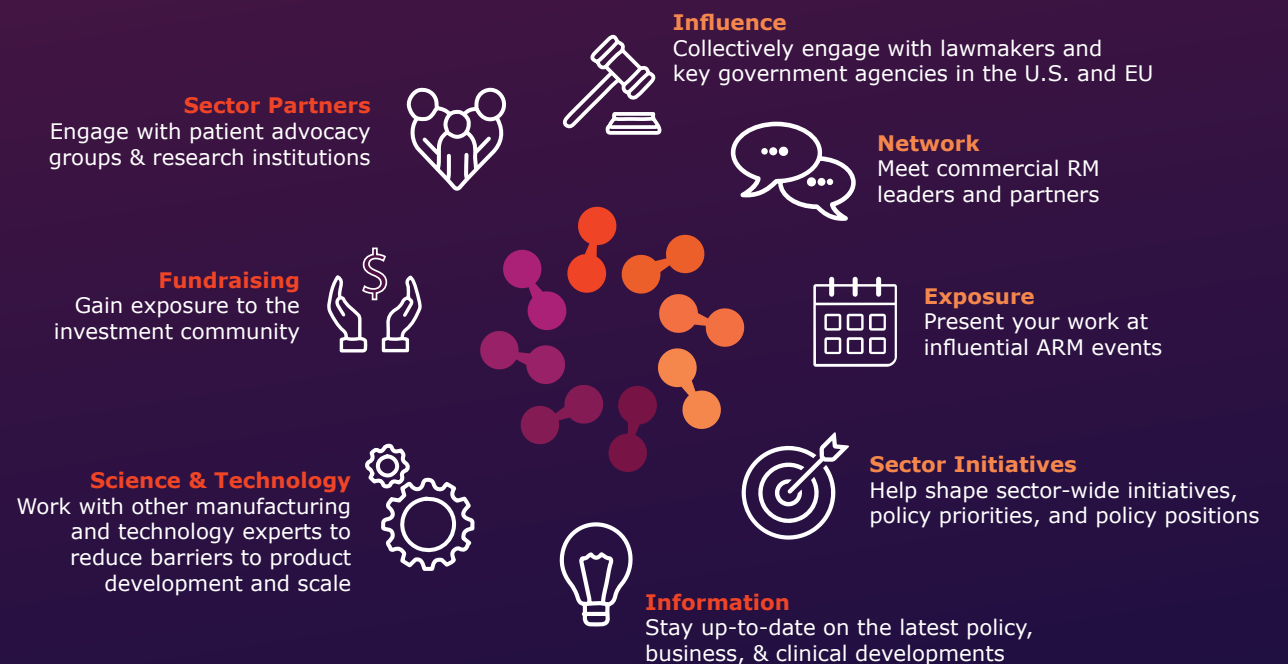
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Jason Wertheim, M.D., Ph.D.
Professor of Surgical
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University

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.



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UPCOMING EVENTS 2021

MEETING ON THE MEDITERRANEAN
VIRTUAL MEETING

6-9
APRIL



19
MAY



WASHINGTON FORUM & FLY-IN
WASHINGTON, DC

12-14
OCT



MEETING ON THE MESA
CARLSBAD, CA

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