Regenerative Medicine in 2021:
A Year of Firsts and Records
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 voice of the sector, representing the interests of 400+ 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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2021 has already been a year of ‘firsts’ for the regenerative medicine sector, with major clinical milestones, strong commercial progress, and record breaking investment. And more records may be broken before the year is over.

For the first time ever, CRISPR gene-editing technology was deployed *in vivo* in human patients — to treat ATTR amyloidosis — with extremely positive interim Phase 1 results. An *ex vivo* approach has shown compelling early-stage results in sickle cell and beta thalassemia patients.

We are on track to have the highest annual number of regulatory approvals of new gene therapy and gene-modified cell therapy products, with three already approved and an additional four to receive regulatory decisions across the US and Europe in the remainder of 2021. The approvals so far include two CAR-T treatments, one for a new indication (multiple myeloma), and a gene therapy for cerebral adrenoleukodystrophy, a deadly inherited disease. The previous record number of approvals for this product category was 3 in 2017. For all regenerative medicine products, we could exceed the previous record of nine approvals in 2016.

And we’re on the cusp of an even larger breakout. There are more than 2,600 trials ongoing worldwide — including 1,320 industry-sponsored trials by nearly 1,200 companies — with 243 of those in Phase 3. The breadth of medical applications spans from rare and devastating diseases like ALS that have few, or no, treatment options, to more prevalent conditions like heart failure. CAR-T therapies, conventionally used as treatments of last resort for certain blood cancers, are comparing favorably to earlier line treatments.

The sector raised more than $14B in the first half, the strongest half on record and already reaching 71% of the record $19.9B raised in 2020. Twenty companies have issued initial public offerings (IPOs) so far this year, exceeding the record number of IPOs raised in all of 2020.
Global dynamics continue to shift, with China approving its first CAR-T therapy. Europe is at risk of falling behind, with the number of developers and new clinical trials lagging far behind the pace set by the US and Asia over the last three years. That’s why ARM is heavily engaged in the EU’s Pharmaceutical Strategy and associated policy and regulatory reviews. ARM’s advocacy achieved an important outcome for patients and the sector in Germany by reducing a bureaucratic reimbursement hurdle for hospitals that provide advanced therapies. We are also shaping US policy for the sector by working with congressional sponsors on ‘Cures 2.0’ legislation and advocating for increased funding for FDA’s Center for Biologics Evaluation and Research (CBER).

We are returning to in-person events with our hybrid Meeting on the Mesa in October. While the ongoing pandemic will shape our experience, we are thrilled to welcome 1,200 sector leaders for the serendipitous interaction that only occurs face-to-face. We are also excited to give the 17 Black summer interns who comprise our inaugural GROW RegenMed Internship class the full Mesa experience. These impressive graduate and undergraduate students are already making an impact this summer at ARM and 13 of our member organizations.

We’re grateful for the opportunity to represent our now 400+ members and will work tirelessly to ensure that 2021 ends as strongly as it has begun.

Janet Lambert
Chief Executive Officer
Alliance for Regenerative Medicine
Regenerative medicine and advanced therapies financing has soared to new heights so far this year, raising $14.1B in the first half — already 71% of what was raised in full-year 2020. The surge of investment in the sector has made H1 2021 the strongest half on record and put the sector on a path to outperform 2020, which broke financing records with nearly $20B raised despite the challenges of the COVID-19 pandemic.

Total Global Regenerative Medicine Financing

- 2018: $13.5B
- 2019: $9.8B
- 2020: $19.9B
- H1 2021: $14.1B

$14.1B raised in H1 2021
A 35% increase from H1 2020
While the market has cooled from its highs in Q1, performance in Q2 still outstripped that from any point prior to December 2020. Though follow-on public offerings raised $2.5B in the first half — a 38% decrease compared to H1 2020 — initial public offerings have been star performers. In H1, 14 cell and gene therapy companies went public — and with six additional IPOs already in Q3, the total number of IPOs YTD has risen to 20, surpassing the full-year record number of 14 IPOs set in 2020. And with the market remaining strong, it seems likely that additional companies will follow in the second half.

Private placements and venture financing are also up in the first half. Private placements totaled $1B, putting 2021 on track to be their strongest year. The current record is $1.2B raised in 2020. Developers raised $5.4B in venture financing, a 77% increase year-over-year from H1 2020. Meanwhile, upfront payments from corporate partnerships are down slightly, with $1.5B raised in the first half — a 6% decrease from H1 2020.
$100M+ Financings from H1 2021

**IPOs**
- Sana Biotechnology — $675M
- Lyell Immunopharma — $425M
- CARsgen — $400M
- Instil Bio — $368M
- Graphite Bio — $273M
- Verve Tx — $267M
- Century Tx — $243M

**Follow-Ons**
- Fate Tx — $432M
- Iovance — $350M
- Editas — $231M
- REGENXBIO — $230M
- Generation Bio — $225M
- Solid Bio — $144M
- TCR2 Tx — $140M
- Adicet Bio — $137M
- Krystal Bio — $125M
- Autolus — $100M

**Venture Capital**
- Blackstone Life Sciences — $250M
- Tessera Tx — $230M
- Umoja Bio — $210M
- Amplify Bio — $200M
- G2 Bio — $200M
- Century Tx — $160M
- Graphite Bio — $150M
- Gyroscope Tx — $148M
- Jaguar Gene Tx — $139M
- eGenesis — $125M
- Artiva Bio — $120M
- Forge Bio — $120M
- Arcellx — $115M
- Caribou Bio — $115M
- Affinia — $110M
- Tenaya Tx — $106M
- Senti Bio — $105M
- Dyno Tx — $100M
- Scribe Tx — $100M
- TScan Tx — $100M

**Private Placement**
- Beam Tx — $260M
- Orchard Tx — $150M
- Mesoblast — $110M
- Vertex & CRISPR Tx — $900M
- Eli Lilly & Precision Bio — $135M
- Ensoma & Takeda — $100M

**Partnerships (UPFRONT PAYMENTS)**
- Gracell Bio — $209M
- Vor Bio — $203M
- Achilles Tx — $175M
- Talaris Tx — $150M
- Decibel Tx — $138M
- NexImmune — $126M

**Public Performance**

![Graph showing public performance of NASDAQ Biotech, Cell-Based Immuno-Oncology, RMAT, and Gene Therapy](image-url)
Gene Therapy: $6.4B  
Cell-Based IO: $6.6B  
Cell Therapy: $1.1B

Cell-based immuno-oncology (IO) financing has for the first time surpassed gene therapy, raising $6.6B in H1 2021. Gene therapy developers raised $6.4B and cell therapy developers raised $1.1B. In terms of public performance, cell-based IO companies first surpassed gene therapy companies in mid-2020, but the gap widened significantly following their extremely strong market performance in Q1 of this year. Cell-based IO companies closed out the half up 232% from 2018 levels, well above the NASDAQ biotech index at 153% and gene therapy at 152%.

As the field progresses, the demand for internal and external manufacturing capacity continues to grow. Smaller cell and gene therapy contract development and manufacturing organizations (CDMOs) are attractive targets for acquisitions, driving more than $10.8B in mergers and acquisitions announced in the first half of this year. These include Danaher’s $9.6B acquisition of Aldevron, a manufacturer of plasmids, mRNA and proteins, as well as Charles River Laboratories’ acquisition of the CDMOs Cognate BioServices and Vigene Biosciences for $875M and $293M, respectively. Companies are also building out internal capacity and investing in new manufacturing facilities, such as BioNTech’s recent acquisition of a cell therapy facility from Kite Pharma.
The strong financing environment funds a thriving sector, with nearly 1,200 companies worldwide actively developing these therapies — nearly 200 more than this time last year. Looking to the second half of 2021, ARM is excited to see these companies accelerate scientific and clinical advances and bring life-changing therapies to patients worldwide.

1,195 Total regenerative medicine and advanced therapy developers worldwide

594 North America

209 Europe

361 Asia-Pacific

23 Middle East & Africa

8 South America
Record Product Approvals Incoming

We are on track for a watershed year for approvals of new regenerative medicine and advanced therapies globally. Decisions are expected on 18 regenerative medicine products across 6 geographies, with 10 of these on products that have never been previously approved in any geography — meaning new product approvals could exceed the record of nine set in 2016. Four of these have already been approved: Bristol Myers Squibb’s Breyanzi, bluebird bio and BMS’ Abecma, and Mallinckrodt’s Stratagraft, all approved by the FDA, and bluebird’s Skysona, approved in Europe. Three of these products, Breyanzi, Abecma, and Skysona, are gene therapy/gene-modified cell therapies, which means 2021 is likely to be a record year for new approvals of this category of products. Decisions are expected this year on four more gene therapy/gene-modified cell therapy products, with the possibility of seven total approvals — which would more than double the previous record of three in 2017.

2021 has been a year of firsts for the sector. Approvals so far this year include the first two products with RMAT designation to reach the market: Breyanzi, a CAR-T therapy for relapsed or refractory large B-cell lymphoma, and Stratagraft, a tissue-based therapy for severe burns. Additionally, Abecma, for the treatment of multiple myeloma, is the first-approved BCMA-targeted CAR-T therapy. Finally, Kite Pharma’s Yescarta became the first CAR-T therapy to receive approval in China.
YTD 2021 Approvals

**Abecma (BMS & bluebird bio)**  
R/R multiple myeloma  
US (March 2021), Canada (May 2021)

**Breyanzi (Bristol Myers Squibb)**  
R/R diffuse large B cell lymphoma  
US (Feb. 2021), Japan (March 2021)

**Kymriah (Novartis)**  
R/R diffuse large B cell lymphoma & pediatric acute lymphoblastic leukemia  
Singapore (March 2021)

**Skysona (bluebird bio)**  
Cerebral adrenoleukodystrophy  
EU (July 2021)

**Stratagraft (Mallinckrodt)**  
Severe burns  
US (June 2021)

**Yescarta (Kite, a Gilead company)**  
R/R large B cell lymphoma  
Japan (Jan. 2021), China (June 2021)  
R/R follicular lymphoma  
US (March 2021)

Upcoming Regulatory Decisions

**Abecma (BMS & bluebird bio)**  
R/R multiple myeloma  
EU

**Cilta-cel (Legend Bio & Janssen)**  
R/R multiple myeloma  
EU, US

**Aloficel (Takeda)**  
Perianal fistulas due to Crohn’s disease  
Japan

**GT-AADC (PTC Bio)**  
AADC deficiency  
EU

**Lantidra (CellTrans)**  
Brittle diabetes  
US

**Lumevoq (GenSight Bio)**  
Leber hereditary optic neuropathy  
EU

**Relma-cel (JW Therapeutics)**  
R/R large B cell lymphoma  
China

**RVT-802 (Enzyvant)**  
Pediatric congenital athymia  
US

*Received positive CHMP opinion from the EMA*
There are currently 1,320 industry-sponsored regenerative medicine and advanced therapies trials ongoing worldwide. That’s an increase of 100 trials since the end of 2020 — driven primarily by the addition of new South Korean and Taiwanese trial registries to ARM’s dataset. With the Asia-Pacific region acting as a major center of growth for the sector, the addition of these registries will help to better identify geographic trends moving forward.

Additionally, while ARM has historically focused on industry-sponsored trials, there is significant clinical activity driven by academic and other research centers that we are now tracking in cooperation with our new data partner. There are an additional 1,328 trials ongoing sponsored by non-industry groups, including academic centers and government. Compared to industry-sponsored trials, this group of trials has a lower proportion of Phase 3 trials (6% versus 12%), as well as a higher proportion of cell therapy trials (59% versus 39%) and a lower proportion of gene therapy trials (6% versus 22%). Going forward, ARM will include non-industry sponsored trials in our reporting to reflect a more comprehensive picture of the sector globally.

There are 243 trials in Phase 3, including 158 industry sponsored trials and 85 trials sponsored by academic, government, and other institutions, illustrating the maturity of the pipeline. These late-stage product candidates are being tested to treat a wide range of indications, including diabetic neuropathy, heart failure, rare genetic diseases, and neuromuscular diseases such as amyotrophic lateral sclerosis (ALS).
Despite the slight decrease in the number of ongoing industry-sponsored clinical trials since the end of 2020, we’re expecting the clinical landscape to grow significantly in the coming years. There are currently 956 unique therapies in development, with 136 in Phase 3. Forecasts predict that number will grow to more than 3,100 by 2026, including 355 in Phase 3.
We’re also seeing more long-term data from CD19 CAR-T therapies. Originally approved for patients who have not responded to two or more lines of treatment, these therapies are now being tested as an earlier-stage intervention. Breyanzi and Yescarta both demonstrated statistically significant improvement in event-free survival over chemotherapy plus stem cell transplant in second line relapsed or refractory large B-cell lymphoma. These findings suggest that CAR-T therapies may be used as an earlier line of treatment moving forward.

“A scientific revolution is changing how we think about medicines. Next-generation CAR-T therapies will combine with other technologies to enhance potency and targeting. Genetic modification will allow scientists to turn therapies on or off, while gene editing platforms could help to create more potent cells. We will equip the human body to fight cancer — and the full potential of cells as medicines will bring extraordinary benefits to patients.”

— Bruce Levine, Barbara and Edward Netter Professor in Cancer Gene Therapy at the University of Pennsylvania, ARM Board Director
Also entering the clinic are induced pluripotent stem cells, or iPSCs. At ARM’s Meeting on the Med in April, Dr. Gregory Block, SVP of Corporate Development at Notch Therapeutics, characterized iPSCs as the “Holy Grail” because they can be grown indefinitely and differentiated at large scale. Those factors make iPSCs a strong target for large-scale, universal or “off-the-shelf” therapies. These therapies are beginning to be tested in a variety of indications. This includes Bluerock Therapeutics’ therapy candidate for Parkinson’s, which was administered to the first patient in a clinical trial earlier this year, as well as Fate Therapeutics, which will share interim data from its PSC-derived NK cell therapy for B cell lymphoma this month. As the field continues to advance, developers seek to work with regulators to set standards for this new technology.

“Intellia’s presentation of the first clinical data in history supporting precision editing of a disease-causing gene within the body following a single, systemic dose of CRISPR/Cas9 opens a new era of medicine — one that holds the potential of curing genetic disease. We believe this is just the beginning and look forward to learning more about how to apply novel gene-editing technologies to different targets, cell types and diseases in order to improve patient care across the globe.”

— John Leonard, M.D., President and CEO, Intellia Therapeutics
Another strategy for large-scale manufacturing — automation — is also gaining traction. Fully automated manufacturing platforms can help to reduce the risk of errors that can contribute to failure in cell therapy manufacturing, as well as provide significant cost-savings. Looking forward, automation is likely to play an important role in producing therapies at scale, particularly as the number of approved therapies — as well as the number of eligible patients — continues to grow.

Manufacturing and CMC continue to be a key priority for ARM. To that end, ARM and the National Institute for Innovation in Manufacturing Biopharmaceuticals (NIIMBL) in June released Project A-Gene, a multistakeholder collaboration to incorporate Quality by Design (QbD) principles into a manufacturing case study of an AAV gene therapy. A-Gene brings best practices and a standard methodology for CMC to the burgeoning gene therapy field, where the science is rapidly advancing but manufacturing scale-up has often become an obstacle to regulatory approval and commercialization. In the future, ARM will also release A-Cell, a sister project focusing on a CAR-T case study. We look forward to collaborating with our members and sector stakeholders to advance these life-changing therapies for patients in need.

“The growing pains for our sector have often materialized in the form of CMC and manufacturing setbacks. At ARM, we’re addressing this fast-moving arena through advocacy with the FDA for regulatory clarity and with our recent release of A-Gene. Success will mean the adoption of quality by design standards by developers, and ultimately, fewer hurdles in getting these transformative therapies to patients at scale.”

— Michael Lehmicke, Sr. Director, Science & Industry Affairs, ARM
With a new administration and Congress taking office in January, ARM is focusing on educating policymakers about the unique nature of cell and gene therapies, with a particular emphasis on the need for innovative payment models and clarity around CMC requirements.

ARM provided draft legislative language to enable the use of innovative payment models in federal programs for consideration to Reps. Diana DeGette and Fred Upton for their ‘Cures 2.0’ initiative and recently responded to a discussion draft of the legislation. We hosted DeGette and Upton in a fireside chat as part of our May virtual Legislative Fly-In, in which more than 150 ARM member representatives met with 97 House and Senate offices across 21 states.

We also provided policy suggestions to modernize the New Technology Add-on Payments (NTAP) program as part of our Cures 2.0 response. While supporting the discussion draft’s efforts to address CMC bottlenecks, we provided additional suggestions to improve clarity around requirements for development and review submissions. We strongly support the legislation’s focus on the FDA’s use of real-world evidence.

The Centers for Medicare & Medicaid Services (CMS) finalized its IPPS rule expanding a DRG created last year for CAR-T therapies to other types of cellular therapies. ARM urged the agency to closely monitor and assess the impact of the new therapies in the DRG and to refine its approach in the future, as needed.

In April we presented at an FDA listening session about the end of the agency’s enforcement discretion period for certain regenerative medicine products on May 31. We support strong oversight and enforcement against bad actors and called on the FDA to dedicate increased resources to its enforcement activities as part of our recommendations for PDUFA reauthorization. We anticipate that the PDUFA agreement letter will be released soon and will recommend additional funding for the Center for Biologics Evaluation & Research (CBER) to hire reviewers for the coming wave of cell and gene therapy applications, a top ARM priority.

In Texas and Ohio, ARM held meetings with policymakers to introduce ARM, the sector, and its priorities around innovative payment models. In Ohio, ARM supported an expansion in newborn screening that became law as part of the state budget. In Texas, ARM submitted an amendment to carve out research and clinical trials from data privacy bills and supported a state rare disease readiness study and the creation of a Rare Disease Advisory Council. We also traveled to Austin in late July to meet with policymakers during the special legislative session and are planning a visit to Columbus in September.
566 companies developing regenerative medicines & advanced therapies in the US

588 industry-sponsored trials with trial sites in the US

 Trials by Phase

- Ph. I: 331
- Ph. II: 176
- Ph. III: 81

 Trials by Tech Type

- Gene therapy: 203
- Cell-based IO: 161
- Cell therapy: 218
- Tissue engineering: 6

$10.4B raised by developers HQ’d in the US
The European Union’s Pharmaceutical Strategy and related policy initiatives represent a crucial inflection point for a region that has been a leading destination for the Advanced Therapy Medicinal Products (ATMP) sector. From 2018 through H1 2021, 224 new industry-sponsored regenerative medicine trials started in Europe, while more than double that amount started in the US (597) and Asia-Pacific (473). The number of ATMP developers in Europe has decreased by 4% since 2018, while increasing by 23% in the US and 119% in Asia-Pacific.

“In every conversation we’re having with Brussels policymakers, we’re advising them that the EU is at an inflection point — for its ability to compete globally and to provide continued access for European patients.”

— Paige Bischoff, Senior Vice President, Global Public Affairs, ARM

In the first half of 2021, ARM educated EU and Member State policymakers about the EU’s competitive position globally and advocated for key policy and regulatory improvements to solidify Europe’s standing in the next decade. We highlighted at all levels the importance of innovative payment models, with the use of Real-World Evidence (RWE) a key enabling factor. ARM submitted consultation/impact assessments for the Pharma Legislation revision, Blood, Tissues and Cells (BTC) legislation, the EU Health Data Space, Cross-Border Healthcare, and the Orphan & Paediatric Drug Legislation. We also conducted a multi-pronged advocacy campaign seeking an exemption for ATMPs from Genetically Modified Organism (GMO) requirements.

At the country level, ARM held four multistakeholder market access workshops focusing on France, Germany, Italy, and the UK. In Germany, ARM achieved a significant policy victory for ATMP patients and the sector by advocating for a reduction in the “NUB gap” that became law in July. The law improves patient access to transformative therapies by making it easier for German hospitals to be reimbursed when administering cutting-edge ATMPs.

In the rest of 2021, ARM will focus on ensuring that new Joint Clinical Assessments (JCA) agreed to by EU stakeholders reflect the unique characteristics of ATMPs, which will be in the first category of products to undergo JCAs in three years. We will also provide another consultation in the next, more in-depth step of the Pharma Legislation revision process, and continue to advocate for innovative payment models at the EU and country level.
209 companies developing regenerative medicines & advanced therapies in Europe

238 industry-sponsored trials with trial sites in Europe

Trials by Phase
- Ph. I: 50
- Ph. II: 38
- Ph. III: 150

Trials by Tech Type
- Gene therapy: 74
- Cell-based IO: 101
- Cell therapy: 60
- Tissue engineering: 3

$1.9B raised by developers HQ’d in Europe
In 2020, following the death of George Floyd and the racial equality movement that followed, ARM established the Action for Equality (AFE) Task Force to determine concrete steps ARM and its members could take to ally with the movement and to address the underrepresentation of Black employees within the regenerative medicine workforce. The task force created the GROW RegenMed Internship Program to provide crucial, early-career paid opportunities in the regenerative medicine sector for Black students. ARM launched the internship program in June with 17 interns in the inaugural class, hosted by ARM and 13 of our member organizations. In addition to on-the-job experience, GROW interns are participating in development and mentorship opportunities and are invited to attend the Cell & Gene Meeting on the Mesa this fall. Next year, we expect the number of GROW interns hosted by ARM and member organizations to nearly double.
One of the key themes of ARM’s virtual *Meeting on the Med* in April was the impact of the COVID-19 pandemic on the regenerative medicine and advanced therapies sector. Now, with vaccination rates on the rise, ARM is looking forward to re-convening in person for our *Meeting on the Mesa* in October in Carlsbad, CA. The meeting will take place in a hybrid format, with in person programming October 12–14 and virtual partnering October 19–20. More than 1,200 people are expected to attend the conference in person, including senior executives and top decision-makers in the industry, life sciences investors, and members of the media.

Buoyed by the significant financial, clinical, and policy progress of the sector, as the global voice of the sector, ARM is energized to continue our leadership in educating and influencing stakeholders so we can collectively advance our sector during the remainder of 2021 and beyond.
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