



# Alliance *for* Regenerative Medicine



**H1  
2022**

**Regenerative Medicine:  
The Pipeline Momentum Builds**  
September 2022



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-engineered 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 13-year history, ARM has become the global voice of the sector, representing the interests of 450+ 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](http://www.alliancerm.org).

# Table of Contents



- 02** Foreword
  - 03** Regulatory Update
  - 07** Clinical Trials by the Numbers
  - 11** Cell and Gene Therapy Developers Worldwide
  - 13** Sector Investment
  - 17** ARM Advances the Sector
  - 25** ARM 2022 Officers & Board of Directors
  - 26** Meet the ARM Team
- 

# Foreword

Twice a year, we survey the cell and gene therapy landscape and ARM's initiatives to advance the sector and provide an update to our members, our stakeholders, and the public at large. While external conditions ebb and flow, the sector's progress — in clinical milestones, regulatory developments, and patient impact — steadily advances.

It has already been a record year for the approval of new gene therapies to treat rare diseases. Two Chimeric Antigen Receptor T-cell (CAR-T) therapies for blood cancers have now been approved by the US Food and Drug Administration (FDA) as earlier-line treatments. The approval of new CAR-Ts this year in the US and Europe now means that a total of six CAR-Ts are available to patients in both regions. Ten years after the discovery of CRISPR, we are less than a year away from possible FDA approval of the first CRISPR therapy — for sickle cell disease, a once overlooked disorder that is now the single most targeted rare disease in ongoing regenerative medicine clinical trials. In June, a new version of gene editing, base editing, entered the clinic for the first time to treat familial hypercholesterolemia, a common cause of heart disease.

The investment headwinds, however, are clear. Following two successive record years of financing flowing into the sector, 2022 is on track for a significant retrenchment. The biotechnology sector's equity performance is down, with the strongest punishment doled out to small, early-stage cell and gene therapy companies. Inflationary conditions and expectations have been particularly unkind to the sector. Initial public offerings have nearly dried up, after hitting a record in 2021. But venture capital financing for the sector, while off its 2021 peak, remains robust and is the single largest driver of sector investment. This is a clear sign of continued excitement around scientific breakthroughs and the opportunity to change the treatment paradigm across a range of rare and prevalent diseases.

Europe's standing in developers, clinical trials, and investment continues to stagnate as growth flows to other regions. ARM remains heavily engaged with European Union (EU) policymakers on joint clinical assessments, the forthcoming revision of the pharmaceuticals legislation, and other policy initiatives that are crucial for the region to remain a leader. In the US, Congress' upcoming reauthorization of the Prescription Drug User Fee Act (PDUFA) will provide vital funding for new personnel and programs to support FDA review of the coming wave of new therapies. Efforts to change the payment and access paradigm continue, with a Centers for Medicare and Medicaid Services (CMS) rule to facilitate the use of outcomes-based payment models in state Medicaid programs that took effect in July. ARM's release of *Project A-Cell* in July is part of an ongoing initiative to carry the sector forward on the core challenge of Chemistry, Manufacturing and Controls (CMC).

The following pages of this report contain an overview of the clinical milestones and trials landscape, investment picture, and key ARM initiatives. This report depicts a landscape of steady progress, notable achievements, and resilience.

# Regulatory Update



## A Year with More Firsts

It has already been a record year for the approval of new gene therapies to treat rare diseases. In July, the European Commission (EC) authorized PTC Therapeutics' Upstaza, the first-ever gene therapy approved to treat the rare genetic nervous system disorder aromatic L-amino acid decarboxylase (AADC) deficiency. In August, the EC granted marketing authorization to BioMarin for Roctavian, the first-ever gene therapy to treat Hemophilia A. There has never been more than one new gene therapy to treat a rare disease approved in a single year; one more decision on a new gene therapy to treat a rare disease — EtranaDez from uniQure and CSL Behring to treat Hemophilia B — is expected by the end of 2022, perhaps in both the US and Europe. In other notable gene therapy news, bluebird bio's Zynteglo, which earned EU approval in 2019, was approved by the FDA in August to treat beta thalassemia in adults and children. An FDA decision on another bluebird bio therapy, eli-cel, for the treatment of cerebral adrenoleukodystrophy, is expected in September.

This has also been a notable year for CAR-T therapies. With the approval of Legend Biotech and Janssen's Carvykti in the US in February and in the EU in May, and the approval of Breyanzi in the EU in April, there are now six CAR-T therapies available in the US and EU. Novartis AG's Kymriah CAR-T therapy was granted accelerated approval by the FDA in May of this year to treat adult relapsed or refractory (R/R) follicular lymphoma. In addition, the therapy is approved for adults with R/R diffuse large B-cell lymphoma and for pediatric use in children and young adults with R/R acute lymphoblastic leukemia. This makes Kymriah the only CAR-T therapy approved for both adult and pediatric settings.

CAR-T therapies are also becoming earlier treatment options. In 2022, Kite Pharma's Yescarta received market authorization as a second-line treatment by the EC for R/R follicular lymphoma and as a second line treatment by the FDA for R/R large B-cell lymphoma. The FDA granted approval to Bristol-Myers Squibb for Breyanzi as a second-line treatment for R/R large B-cell lymphoma. It is also under review in Europe as a second-line treatment.

## Approved Therapies

### New Therapies Approved in 2022

#### Carvykti (CAR-T)

*Legend Biotech & Janssen*

R/R multiple myeloma  
US (Feb 2022) EU (May 2022)

#### Roctavian (Gene Therapy)

*BioMarin Pharmaceutical*

Hemophilia A  
EU (August 2022)

#### Upstaza (Gene Therapy)

*PTC Therapeutics*

Aromatic L-amino acid  
decarboxylase (AADC) deficiency  
EU (July 2022)

### Therapies Approved in New Geographies or New Indications in 2022

#### Breyanzi (CAR-T)

*Bristol-Myers Squibb*

R/R Large B-cell lymphoma  
Primary mediastinal large  
B-cell lymphoma  
Follicular lymphoma grade 3B  
EU (April 2022)  
R/R Large B-cell lymphoma  
US (June 2022)

#### Kymriah (CAR-T)

*Novartis*

R/R follicular lymphoma  
US (May 2022)

#### Yescarta (CAR-T)

*Kite Pharma (Gilead)*

R/R large B-cell lymphoma  
US (April 2022)  
R/R follicular lymphoma  
EU (June 2022)

#### Zynteglo (Gene Therapy)

*bluebird bio inc.*

Beta-thalassemia  
US (August 2022)

## Therapies Anticipating Decisions

Several other therapies are on track for regulatory decisions in late 2022 and 2023, indicating an active late-stage pipeline.

In Europe, Atara Biotherapeutics has reported that Tab-cel, a cell therapy to treat Epstein-Barr virus-associated post-transplant lymphoproliferative disorder, is on track to receive a regulatory decision in the fourth quarter of 2022.

In the US, Gamida Cell's Omidubicel, designed for blood cancer patients who need an allogenic hematopoietic stem cell transplant, is expecting an FDA decision in January of 2023. Similarly, Stemcyte's HPC Cord Blood, which is used for patients who need hematopoietic progenitor cell transplantation, had its Biologics License Application (BLA) accepted this year and an approval decision is expected in 2023.

The FDA has also accepted a priority review of Krystal Bio's BLA for its B-VEC gene therapy, which is used to treat patients with dystrophic epidermolysis bullosa. A regulatory decision is anticipated by the FDA by February of 2023.

## Anticipated Regulatory Decisions

● United States ● Europe

2022

**eli-cel\*** (Gene Therapy)  
Bluebird bio Inc  
Early active cerebral adrenoleukodystrophy (CALD)

**EtranaDez** (Gene Therapy)  
uniQure & CSL Behring  
Hemophilia B

**Tab-cel** (Cell Therapy)  
Atara Biotherapeutics Inc  
Epstein-Barr virus-associated post-transplant lymphoproliferative disorder (EBV+PTLD)

**EtranaDez** (Gene Therapy)  
uniQure & CSL Behring  
Hemophilia B

2023

**Afami-cel** (Cell Therapy)  
Adaptimmune Therapeutics  
Advanced synovial sarcoma

**bb1111** (Gene Therapy)  
bluebird bio  
Sickle cell disease

**B-VEC** (Gene Therapy)  
Krystal Bio  
Dystrophic epidermolysis bullosa

**CTX001** (CRISPR Therapy)  
CRISPR Therapeutics &  
Vertex Pharmaceuticals  
Sickle cell disease (SCD) & beta-thalassemia

**HPC cord blood** (Cell Therapy)  
StemCyte  
Unrelated Donor hematopoietic progenitor cell transplantation (HSCT)

**Omidubicel** (Cell Therapy)  
Gamida Cell  
Hematopoietic stem cell transplant in patients with hematological malignancies (blood cancer + HSCT)

**CT-053** (CAR-T Therapy)  
CARsgen Therapeutics  
R/R multiple myeloma

**fidanacogene elaparvovec** (Gene Therapy)  
Pfizer (formerly Spark Therapeutics)  
Hemophilia B

**Lifileucel** (TIL therapy)  
Iovance  
Metastatic melanoma

**Libmeldy** (Gene Therapy)  
Orchard  
Metachromatic leukodystrophy (MLD)

**Tab-cel** (Cell Therapy)  
Atara Biotherapeutics Inc  
Epstein-Barr virus-associated post-transplant lymphoproliferative disorder (EBV+PTLD)

**Upstaza** (Gene Therapy)  
PTC Therapeutics  
Aromatic L-amino acid decarboxylase (AADC) deficiency

**Roctavian** (Gene Therapy)  
BioMarin  
Hemophilia A

**B-VEC** (Gene Therapy)  
Krystal Bio  
Dystrophic epidermolysis bullosa

**Lumevoq** (Gene Therapy)  
GenSight Biologics SA  
Leber hereditary optic neuropathy (LHON)

\*Approved as "skysona" after initial publication on 9/16/2022.

## Therapies on Fast-track

Several regenerative medicine therapies in the US and EU have been placed into designations that can “fast-track” a regulatory decision. In the US, the Regenerative Medicine Advanced Therapy (RMAT) designation is designed for cell and gene therapies, therapeutic tissue engineering products, and human cell and tissue products intended for serious or life-threatening diseases or conditions. In the EU, the PRiority MEdicines (PRIME) designation is a process that enhances support for the development of medicines that target an unmet need.



### RMAT Designation

#### **ALLO-501A (CAR-T Cell Therapy)**

*Allogene Therapeutics Inc*  
R/R non-Hodgkin lymphoma

#### **CCAR-039 (CAR-T Cell Therapy)**

*Cellular Biomedicine Group Inc*  
R/R non-Hodgkin lymphoma

#### **CT-041 (CAR-T Cell Therapy)**

*CARsgen Therapeutics Ltd*  
Claudin18.2 (CLDN18.2)  
positive advanced GC/GEJ

#### **obe-cel (CAR-T Cell Therapy)**

*Autolus Therapeutics Inc*  
R/R B-cell acute lymphoblastic leukemia

#### **ExoFlo (Cell Therapy)**

*Direct Biologics*  
Acute respiratory distress syndrome

#### **Posoleucel (Cell Therapy)**

*AlloVir Inc*  
BK virus, cytomegalovirus,  
adenovirus, Epstein-Barr virus,  
human herpesvirus 6 and JC virus

#### **REACT (Cell Therapy)**

*Prokidney Corp*  
Diabetic kidney disease

#### **SkinTE (Tissue Engineering)**

*PolarityTE*  
Chronic cutaneous ulcer

#### **AProArt (Gene Therapy)**

*UC San Francisco*  
Artemis-SCID

#### **NeoCart (Tissue Engineering)**

*Ocugen*  
Articular cartilage lesion of the knee



### PRIME Designation

#### **BNT-211 (CAR-T Cell Therapy)**

*BioNTech SE*  
Advanced solid tumors

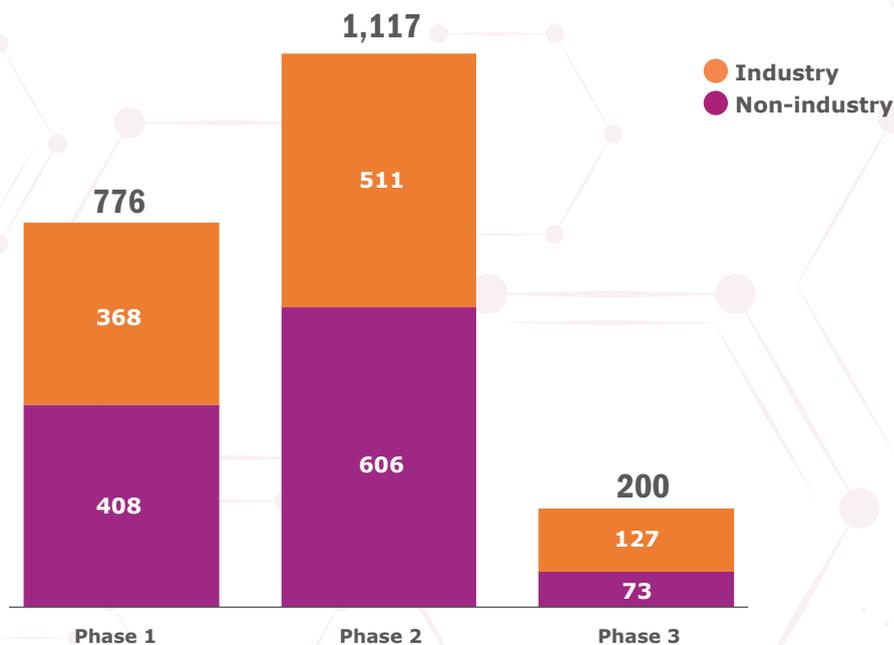
#### **ADVM-022 (Gene Therapy)**

*Adverum Biotechnologies Inc*  
Neovascular or wet age-related macular degeneration

# Clinical Trials by the Numbers

There were 2,093 trials ongoing globally at the end of June 2022, a 13% decrease from the end of 2021. Industry sponsored trials are back at the levels they were in 2019 and 2020. Non-industry trials (academic and government sponsors) have continued their decline since 2018.

## Active Clinical Trials by Sponsor and Phase



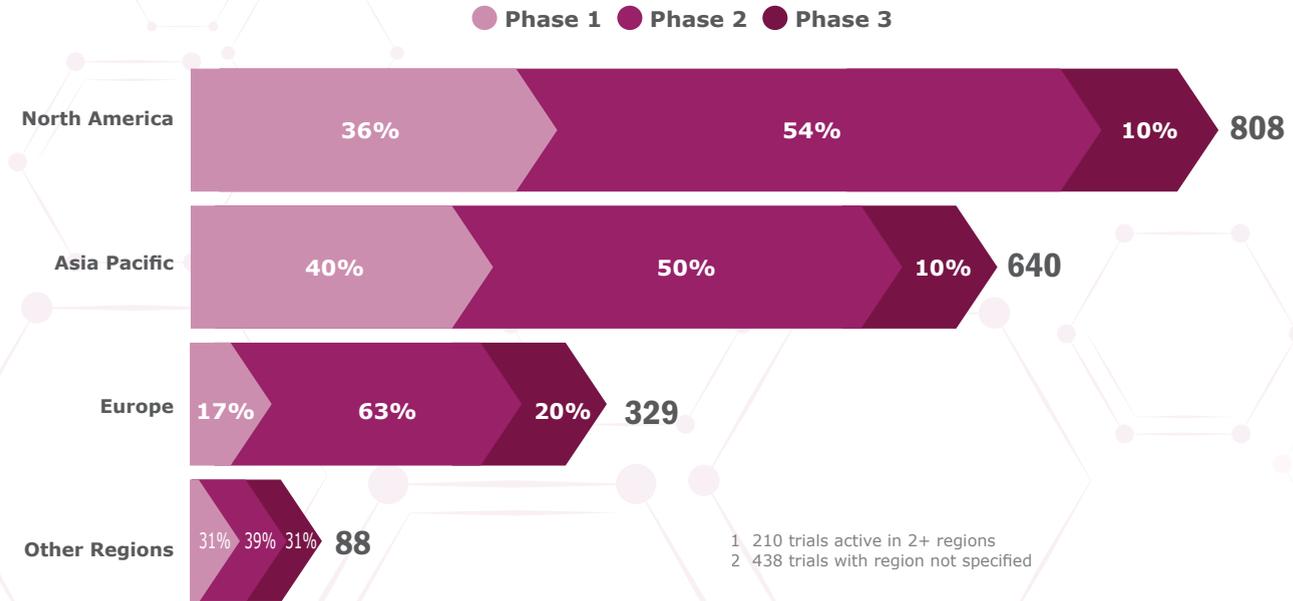
Previously, academic- and government-sponsored trials outpaced industry trials. Now, industry and non-industry are at near-parity with 1,006 (48%) and 1,087 (52%) ongoing trials, respectively.

There are 776 (37%) trials in phase 1, the smallest decrease from 2021 at 8%. Phase 2 trials are the largest bucket at 1,117 trials (53%), down 17% from 2021. Phase 3 comprises the remaining 200 trials (10%), a 10% decrease from 2021.

# Clinical Trials by Region

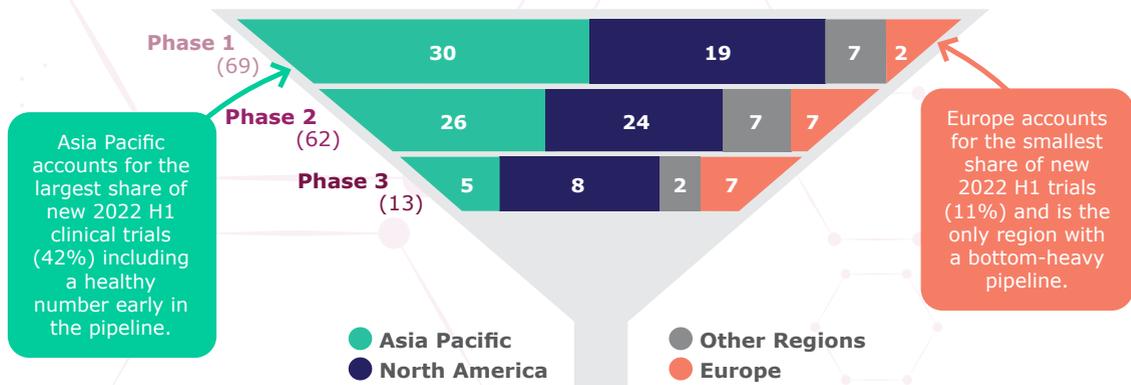
By region, North America leads with 808 active clinical trials (down 15% from 2021), followed by Asia Pacific with 640 trials (down 15%), Europe with 329 (down 14%), and 88 active trials in all other regions (down 7%).

## Active Clinical Trials by Region and Phase



## Asia Pacific takes the lead; Europe pipeline slows to a trickle

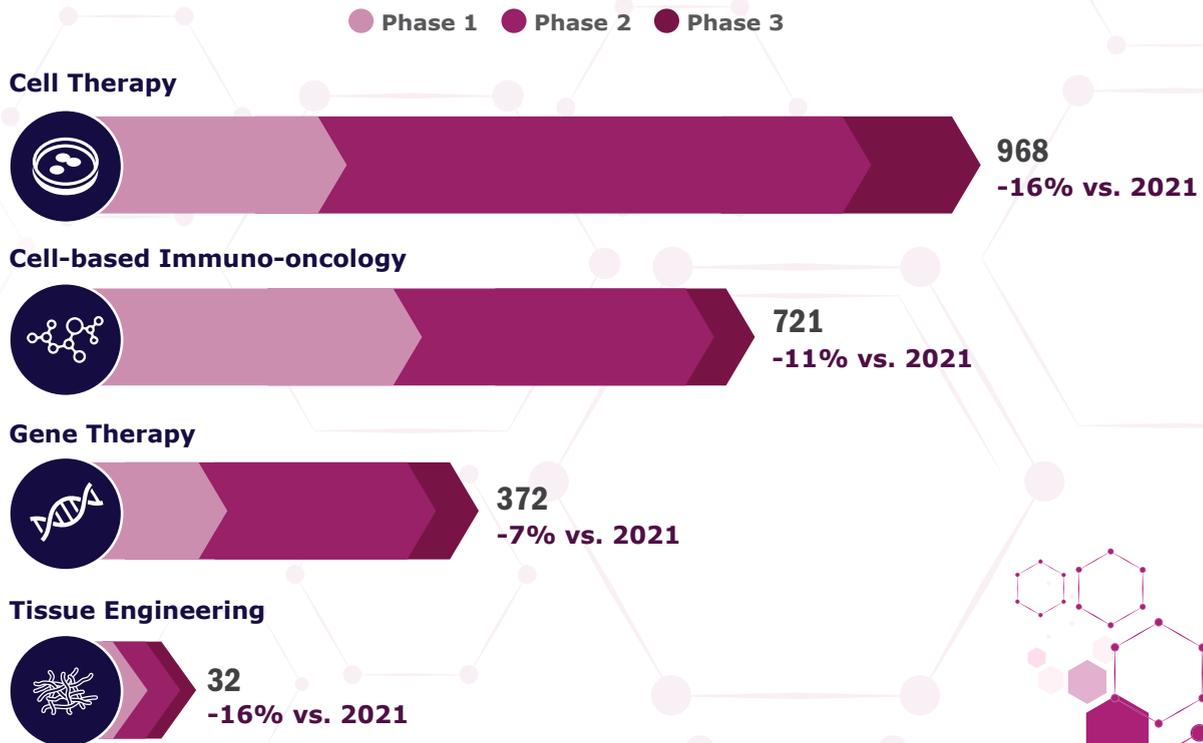
### 144 New Clinical Trials in 2022 H1



# Trials by Therapeutic Approach and Area

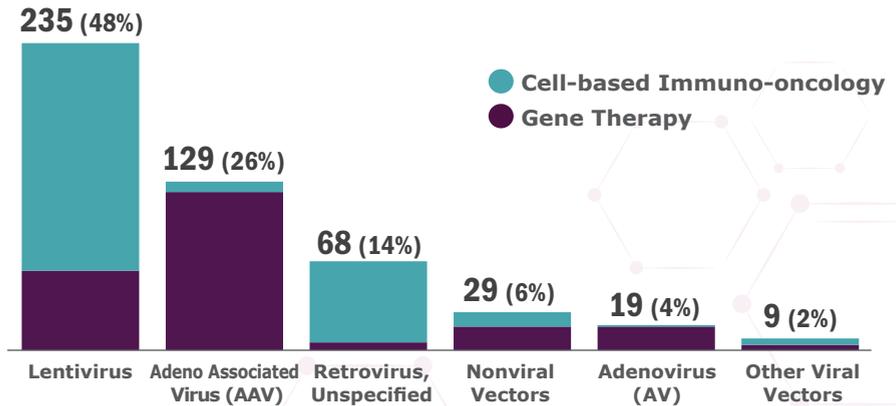
Cell therapies make up the largest category of ongoing trials (968, 46%), followed by cell-based immuno-oncology (CBIO) (721, 34%) and gene therapies (372, 18%). Tissue-engineered therapies comprise the remaining 32 (2%) of ongoing clinical trials in the cell and gene therapy sector.

## Active Trials by Therapy 2022 H1



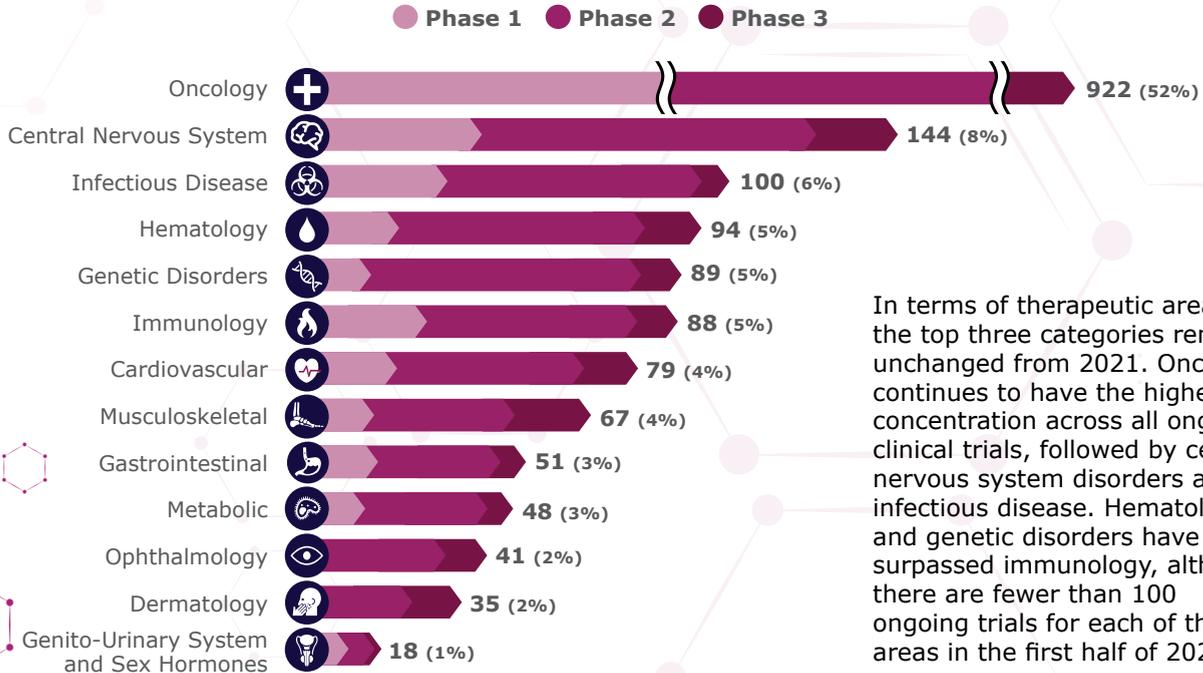
## Therapeutic Vectors

The gene modification vector is known for 489 (45%) of the 1,093 ongoing gene therapy and CBIO trials. Lentivirus is the most common vector across both gene therapy and CBIO, driven by its dominant position in CBIO trials. Adeno associated virus (AAV) ranks second among vectors used, and is the primary vector for gene therapy trials. Retrovirus follows in third place, again dominated by CBIO.



- 1 Percentages (%) based on trials with known vectors; 604 trials with vector unknown
- 2 Nonviral vectors include plasmid, transposon, and nanoparticles
- 3 Other viral vectors include herpes simplex virus (HSV)

## Therapeutic Areas

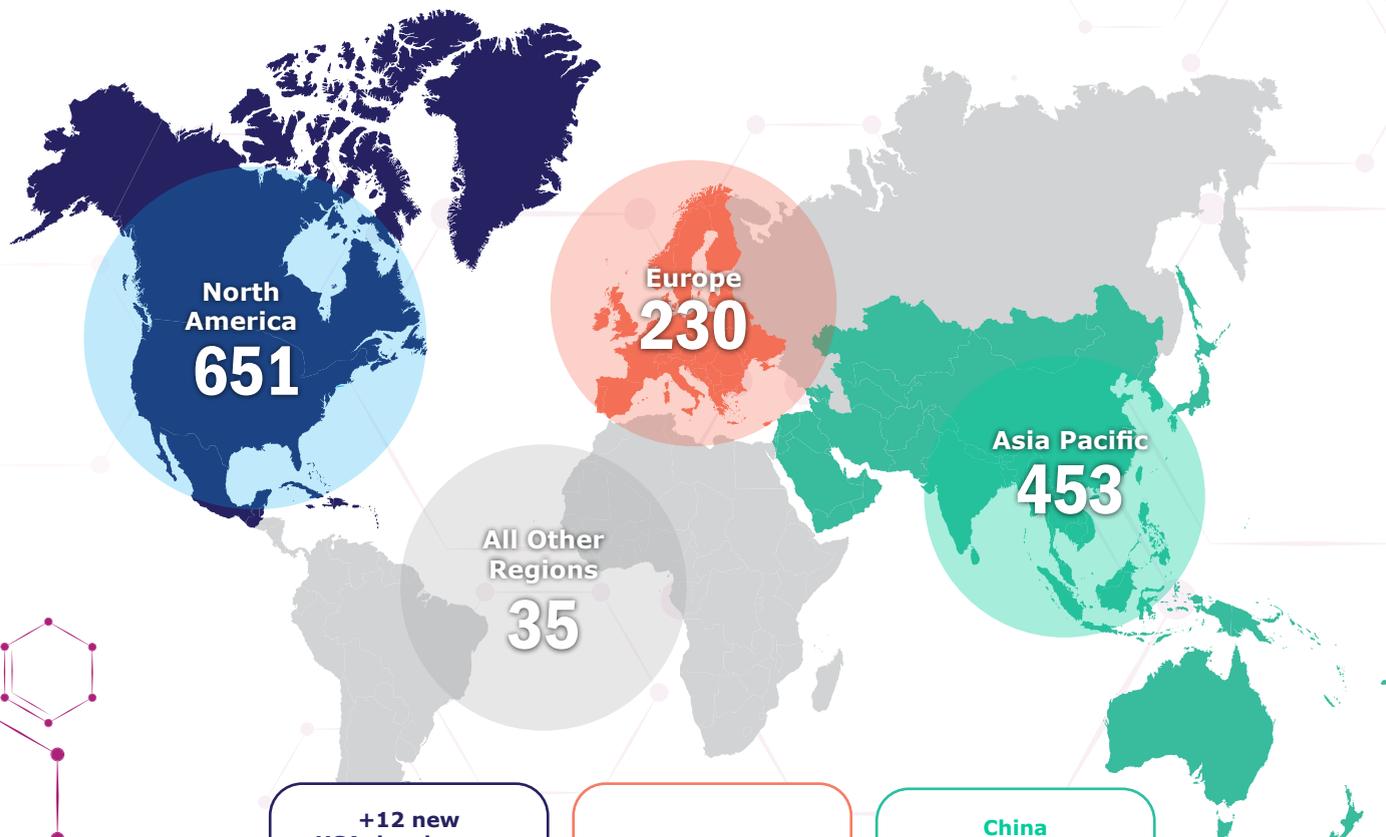


In terms of therapeutic areas, the top three categories remain unchanged from 2021. Oncology continues to have the highest concentration across all ongoing clinical trials, followed by central nervous system disorders and infectious disease. Hematology and genetic disorders have surpassed immunology, although there are fewer than 100 ongoing trials for each of those areas in the first half of 2022.

- 1 Percentages (%) based on trials with known therapeutic areas; 317 trials with area unknown or unclassified
- 2 Unlisted areas include known areas with small numbers (<10) of CGT trials including mouth/dental, ear-nose-throat, and unspecified male/female disorders

# Cell and Gene Therapy Developers Worldwide

**1,369** cell, gene, and tissue-engineering therapeutic developers worldwide  
*Up 5% since 2021*



**+12 new USA developers, bringing US total to 625**

*Country up 2% since 2021; region similar*



**European developer growth flat since 2021**



**China accounts for 236 developers**  
*52% of region and fastest growing country*



# Global 2022 H1 Quick Facts



North America



Asia-Pacific



Europe



Developers

**651**

+2% vs. 2021

**453**

+10% vs. 2021

**230**

0% vs. 2021



Clinical Trials

**808**

INDUSTRY 472  
ACADEMIC / GOV'T 336

-15% vs. 2021

**640**

INDUSTRY 294  
ACADEMIC / GOV'T 346

-15% vs. 2021

**329**

INDUSTRY 178  
ACADEMIC / GOV'T 151

-14% vs. 2021



Investment

**\$5.6B**

53 DEALS

-68% YoY

**\$675MM**

23 DEALS

-69% YoY

**\$398MM**

13 DEALS

-88% YoY

- 1 188 industry trials, 22 non-industry trials active in multiple regions
- 2 214 industry trials, 224 non-industry trials with region unknown/unspecified

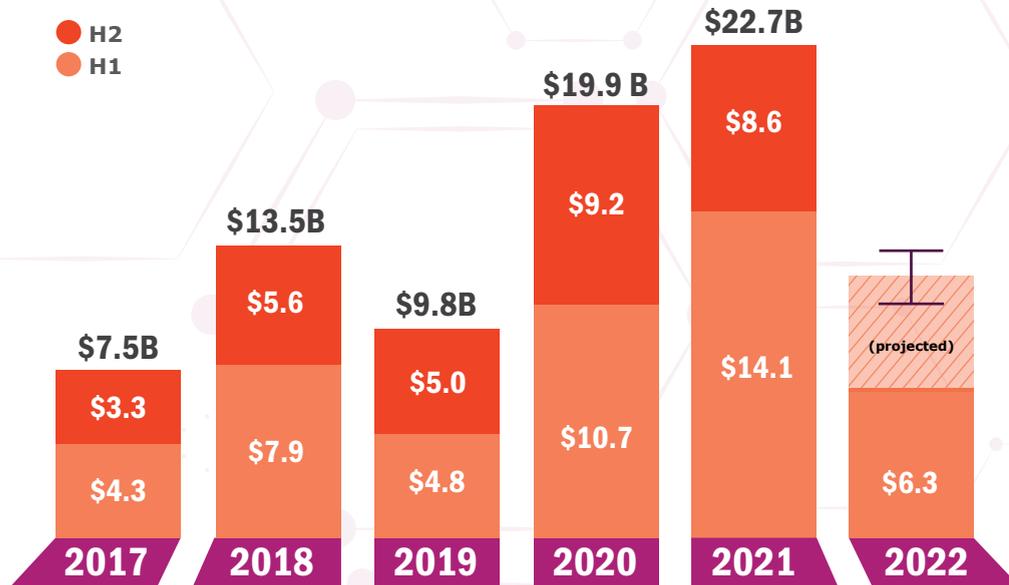
# Sector Investment



After two record-breaking years in investment for regenerative medicine and advanced therapies, the sector's 2022 performance is trending back to the levels of 2018 and 2019. During the pandemic in 2020 and 2021, the biotech sector saw an unprecedented uptick in investment due to strong public attention on healthcare innovation and the low cost of capital. Based on current patterns, 2022 is likely to land between \$9.8B and \$13.5B in total annual investment, the sector performance from 2019 and 2018, respectively.

What is the culprit of this downturn? External factors including inflation and a retrenchment in biotech financing, including cell and gene therapy, after record levels of investment have combined to produce an extremely challenging investment environment during 2022. The slowdown affects nearly all parts of the interconnected financing system, from early-stage venture capital to initial public offerings (IPOs) and the performance of publicly traded companies. Small, early-stage companies — and there are many such cell and gene therapy companies — are particularly affected by inflation expectations because they are furthest from generating profits.

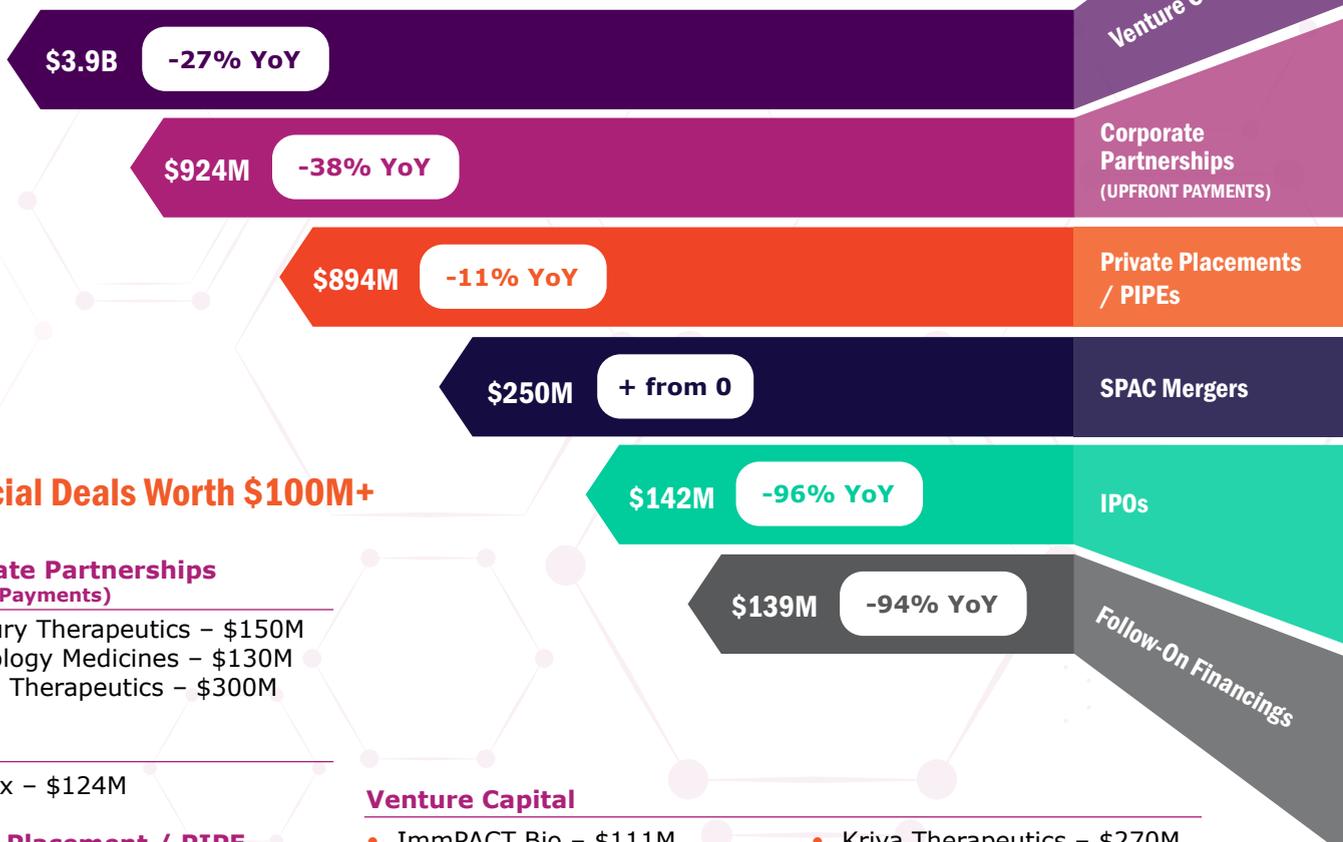
### Investment by Year



Despite a 27% decrease in venture capital from H1 2021, it remains a bedrock of funding for the sector and is pacing at 40% of 2021 FY — the smallest decline of the financing categories along with private placements (which increased in number from 3 to 11 YoY). This indicates continued investor excitement about scientific breakthroughs and new treatment possibilities.

IPOs for the first half of 2022 saw a significant decline from prior years with only 2 completed. However, 2 additional IPOs have been filed and are pending.

## Investment Breakdown



## Financial Deals Worth \$100M+

### Corporate Partnerships (Upfront Payments)

- Century Therapeutics – \$150M
- Homology Medicines – \$130M
- Beam Therapeutics – \$300M

### IPO

- Arcellx – \$124M

### Private Placement / PIPE

- ProKidney Corp – \$575M
- 2seventy bio – \$170M
- Castle Creek Biosciences – \$112.8M

### SPAC

- ProKidney Corp – \$250M

### Venture Capital

- |  |  |
|--|--|
| <ul style="list-style-type: none"> <li>• ImmPACT Bio – \$111M</li> <li>• Amplify Bio LLC – \$100M</li> <li>• Aspen Neuroscience – \$147.5M</li> <li>• Aurion Biotech – \$120M</li> <li>• Be Biopharma – \$130M</li> <li>• Frontera Therapeutics – \$160M</li> <li>• SalioGen Therapeutics – \$115M</li> <li>• Metagenomi – \$175M</li> </ul> | <ul style="list-style-type: none"> <li>• Kriya Therapeutics – \$270M</li> <li>• Maze Therapeutics – \$190M</li> <li>• ReCode Therapeutics – \$200M</li> <li>• National Resilience – \$625M</li> <li>• Satellite Bio – \$110M</li> <li>• Affini-T Therapeutics – \$175M</li> <li>• Tessa Therapeutics – \$126M</li> </ul> |
|--|--|

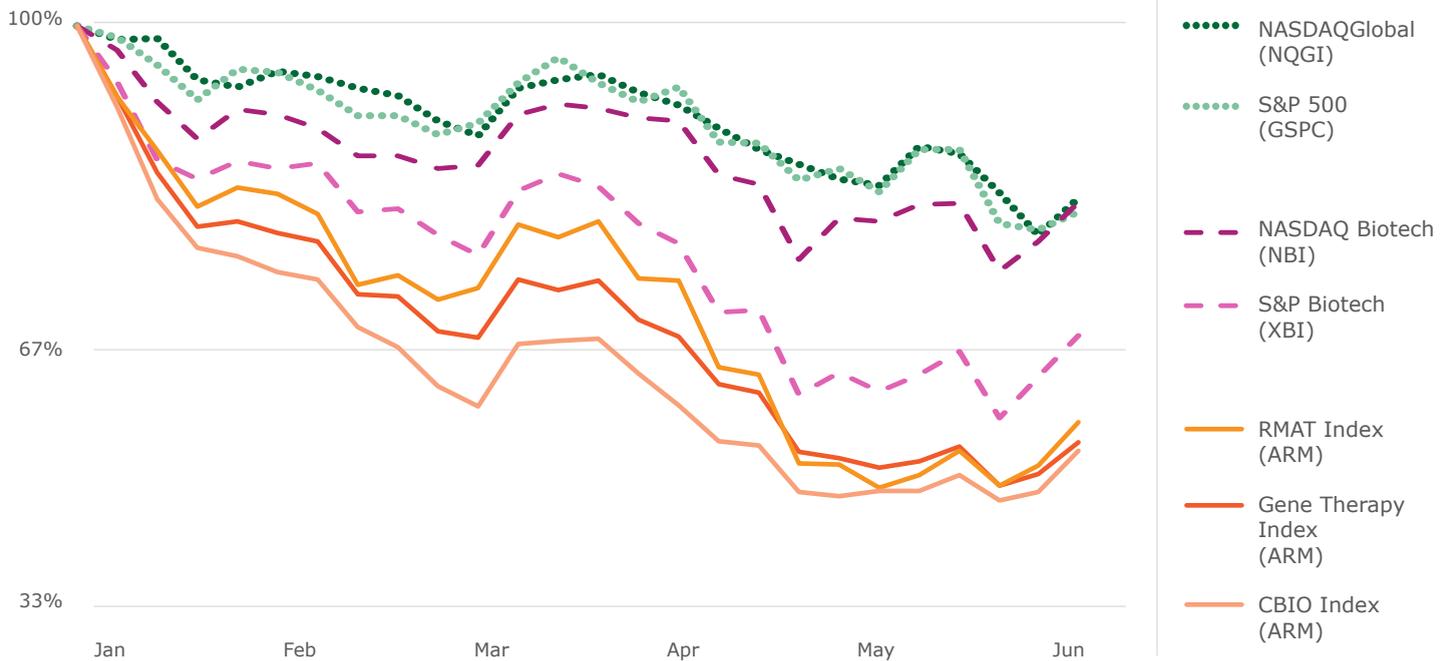
# Public Equity Performance in 2022 H1

Public equity and capital markets overall have trended down in the first half of 2022; the biotechnology sector initially performed worse than general indices but more recently has outpaced the general recovery. Indices that give higher weight to companies with larger market capitalization, such as NASDAQ's NBI, have followed the broad indices more closely while indices that provide proportionally more weight to smaller companies, such as the S&P Biotech ETF, experienced larger decreases.

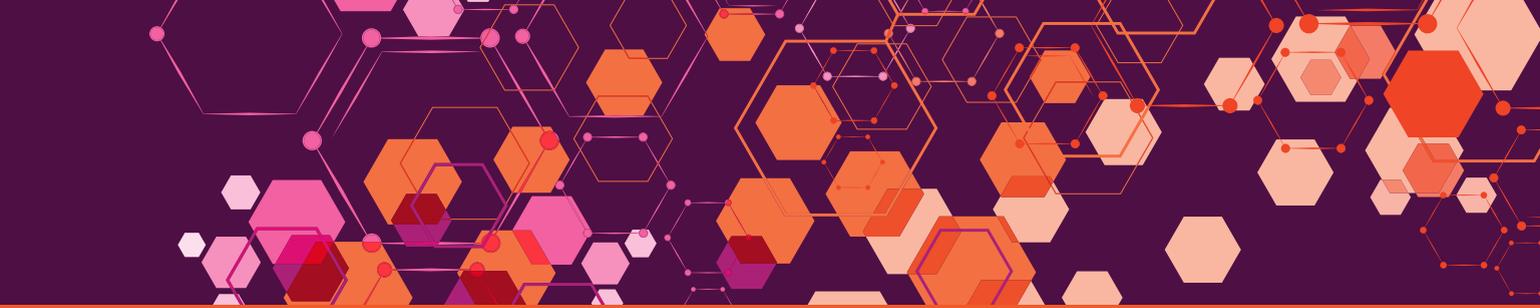
S&P Global's<sup>1</sup> research shows that inflationary fears and potential interest rate hikes have adversely impacted the valuation of publicly traded companies that are smaller and in earlier stages of development. This includes cell and gene therapy developers, which have closely followed the trend lines of broader biotech indices but at lower valuations.

Despite this downward trend, some analysts<sup>2</sup> suggest this decline is not related to biotech's poor performance but an overcorrection by investors because of external factors like inflation and supply chain constraints.

## Public Equity Performance in 2022 H1



1 S&P Global, "Interest rate hikes likely to sting already suffering small-cap stocks," 2022.  
2 For example, Christina Bardon of BioImpact Capital



# ARM Advances the Sector





# Shaping the Policy and Regulatory Landscape in the US and Europe

## UNITED STATES

ARM continues to focus on the removal of barriers to the use of innovative payment models, improving regulatory clarity on CMC issues, and ensuring the timely passage and implementation of the Prescription Drug User Fee Act (PDUFA) VII.

During our Congressional Fly-In held June 7–8, 2022, ARM and our members asked Representatives and Senators to support and co-sponsor the Medicaid VBPs for Patients (MVP) Act and for the timely reauthorization of PDUFA. ARM will continue to advocate for the MVP Act as currently drafted and other efforts to facilitate the use of innovative payment models during this, and the next, Congress. The implementation of PDUFA VII will be a focus of ARM's upcoming FDA liaison meeting on October 28, along with leveraging platform approaches to help speed the development of gene therapies.

Also on the regulatory front, ARM has responded to a variety of FDA proposed guidances, ranging from utilization of real-world data, genome editing, clinical trial diversity plans, and CAR-T therapy development. On the state level, ARM hosted a panel and reception in Columbus in May to showcase Ohio's success in building a cell and gene therapy hub that was attended by 20 policymakers. ARM is planning an event in Austin, Texas this fall focusing on access and reimbursement challenges for cell and gene therapies.

### PDUFA VII

Contains several priorities for the cell and gene therapy sector, including an increase in resources to help the Center for Biologics Evaluation & Research (CBER) review the coming wave of transformative therapies and a pilot program to develop alternative, more efficient ways to review CMC data.

**Where it's heading:** The House and Senate HELP Committee cleared their reauthorization proposals, and parties in both chambers are negotiating to find consensus before the current authorization expires at the end of September. We expect a compromise resulting in the timely reauthorization of PDUFA.

### MVP Act

Addresses barriers to the implementation of innovative payment models in the Medicaid program. The legislation codifies a CMS rule that took effect on July 1, 2022, that addresses *Best Price* regulations, while also addressing anti-kickback regulations and modifying the definition of *Average Manufacturer Price* to accommodate value-based payment agreements.

## EUROPE

ARM continues to inform EU policymakers about the region's declining competitiveness in advanced therapy medicinal product (ATMP) developers, clinical trials, and investment. We are emphasizing the opportunity for policy changes in the upcoming pharmaceuticals legislation revision, the joint clinical assessments framework, and other initiatives to re-establish Europe as a leading destination for the sector.

ARM recently responded to the EC's proposed regulation regarding 'substances of human origin' (SoHO), advocating for the EMA to play a central role in resolving 'borderline' cases between SoHOs and ATMPs. ARM is also active in providing recommendations on how the EU's joint clinical assessment process — which will take effect in 2025 — can be fit-for-purpose for the evaluation of ATMPs. We expect the EC to release proposals for the revision of the pharmaceuticals legislation in early 2023, kicking off negotiations with the European Parliament and Council that will be central to the ATMP sector in Europe.

ARM will organize an event in the European Parliament in November focusing on the sector's core policy issues in Europe. On the country level, ARM has increased its engagement in the United Kingdom (UK), France, and Germany on access and reimbursement issues.

In Brussels, ARM is contributing expert views to EU policymakers for the shaping of the joint clinical assessment process, the revision of the pharmaceuticals legislation, and other key areas.

At the country level, ARM is advancing access and reimbursement in the UK, France, and Germany.



# Advancing A-Cell and A-Gene

## A-Cell

ARM and the National Institute for Innovation in Manufacturing BioPharmaceuticals (NIIMBL) released *Project A-Cell* on July 26 of this year. A-Cell is a multistakeholder collaboration to incorporate Quality by Design (QbD) principles into a manufacturing case study of a CAR-T therapy.

It brings best practices and a standard methodology for CMC to the cell-based therapy field, which faces challenges to manufacturing scale-up. *A-Cell* emulates previous QbD efforts that were applied to the manufacturing of monoclonal antibodies (*A-Mab*), vaccines (*A-Vax*), and more recently, gene therapies (*A-Gene*). More than 50 industry experts from over 30 leading therapeutic developers, as well as regulatory experts and the Standards Coordinating Body, contributed to *A-Cell*.



## A - CELL

A case study-based approach to integrating Quality by Design principles in cell-based therapy CMC programs



"A-Gene is already making a significant impact as an educational and workforce development tool for the gene therapy sector. We think A-Cell will similarly advance the cell therapy field and help to deliver durable and potentially curative treatments for a range of serious cancers and other diseases."

— *Michael Lehmicke, Vice President, Science & Industry Affairs, Alliance for Regenerative Medicine*

[View the Report](#) 

# Advancing A-Cell and A-Gene

## A-Gene

Released in June of 2021, Project *A-Gene* is a case study-based approach to integrating QbD principles into gene therapy CMC programs. Throughout late 2021 and 2022, ARM hosted six A-Gene webinars to cover the study in detail.

ARM and the sector will now focus on how to implement the best practices for gene therapy put forth in the case study. In addition to its use as a tool by therapeutic developers, A-Gene has received traction in university programs focusing on the sector, including the University College of London.



## A-GENE

A case study-based approach to integrating Quality by Design (QbD) principles in gene therapy

## A-Gene Webinar Series



**Six** webinars attended by more than **500** ARM members



Webinar recordings were viewed over **2,000** times



The case study document has been viewed over **5,000** times on ARM's website.

View the Series 



**A-GENE**  
WEBINAR SERIES

# Meeting on the Med



Revisit your favorite Med moments by exploring the recorded sessions on our YouTube channel.



## Shaping cell and gene therapy in Europe

ARM's 2022 *Cell & Gene Meeting on the Med* was delivered in a hybrid format on April 20–21 at the Hotel Arts Barcelona. The event featured panel sessions and fireside chats, in-person and virtual company presentations, and exclusive, one-on-one partnering meetings. More than 650 professionals from 319 organizations attended the meeting in person, making it the largest *Meeting on the Med* to date. The 2022 *Meeting on the Med* was sponsored by 55 organizations and convened more than 1,000 partnering meetings. European Commissioner for Health and Food Safety Stella Kyriakides provided a virtual welcome address for attendees.

Thank you to all those who attended, and we look forward to seeing you at the next meeting!

Join us for our next major event!

**CELL**  **GENE**  
MEETING ON THE MESA  
HYBRID

**October 11–13, 2022**  
**Carlsbad, CA**

**Register today!**



# The 2022 Congressional Fly-In

## Championing the sector on Capitol Hill

On June 7-8, ARM held its first in-person Congressional Fly-In since 2019. More than 85 representatives from ARM member organizations met with House and Senate offices from 15 states. Members engaged with leading US policymakers to advocate for important pieces of legislation, specifically the reauthorization of PDUFA and the MVP Act. At a membership dinner the night before the Fly-In, attendees heard from patient advocate Jimi Olaghere, who participated in a groundbreaking clinical trial for a gene-editing therapy to treat his sickle cell disease.



*Jimi Olaghere addressing attendees at the first night of ARM's Congressional Fly-In.*



*Representative Tom Emmer of Minnesota's 6<sup>th</sup> Congressional District speaking at the second day of ARM's Fly-In.*



**Learn more  
about our Fly-In!**



# GROW RegenMed Internship Program

## Inclusively developing the sector's next generation of leaders

In the biotech field, minority populations continue to be underrepresented among employees and leadership. In 2020, ARM formed the Action for Equality Task Force to determine concrete steps ARM and its members can take to improve equality and inclusion. As a result, the Task Force formed the GROW RegenMed Internship Program to connect Black undergraduate and graduate students with the field of regenerative medicine. This summer marks the second year of the GROW program, with 18 interns hosted by 14 ARM member organizations. Each intern gets practical experience through working in the sector in roles ranging from marketing to the bench.

[Learn more](#) about the internship program and how to host a GROW intern in 2023 and beyond.

## Summer 2022 GROW RegenMed Interns



**Benga Adegbite**

Amicus Therapeutics



**Stefan Bailey**

Dark Horse Consulting Group, Inc.



**Nathanael Brignol**

Ensoma



**Adrianna Brown**

Enzyvant Therapeutics



**Tolandra Coleman**

Alliance for Regenerative Medicine



**Rahawa Ghebreful**

Arbor Biotechnologies



**Devyn Hill**

AGTC



**Kiarra Lavache**

BlueRock Therapeutics



**Divine Ogieva**

CRISPR Therapeutics



**Zainab Olushoga**

Tenaya Therapeutics



**Salisha Peeler**

Passage Bio



**Madison Pope**

Dark Horse Consulting Group, Inc.



**Henry Renelus**

BlueRock Therapeutics



**Jazmine Richardson**

National Hemophilia Foundation



**Willie Richardson**

Castle Creek Biosciences



**Kimiyonn Sadler**

Project Farma



**Taliyah Townsend**

REGENXBIO Inc.



**Alissa Wells**

REGENXBIO Inc.

# ARM 2022 Board Officers



**CHAIR**  
**Emile Nuwaysir, Ph.D.**  
*CEO, Ensoma*  
*Chair, BlueRock Therapeutics*



**SECRETARY**  
**Amy Butler, Ph.D.**  
*President, Biosciences*  
*Thermo Fisher Scientific*



**VICE-CHAIR**  
**Devyn Smith, Ph.D.**  
*CEO*  
*Arbor Biotechnologies*



**TREASURER**  
**Dave Lennon, Ph.D.**  
*CEO*  
*Satellite Bio*



# ARM 2022 Board of Directors

**Robert Ang, MBBS, MBA**  
*CEO, Vor Biopharma*

**Bradley Campbell, MBA**  
*President and COO, Amicus Tx*

**Miguel Forte, M.D., Ph.D.**  
*CEO, Bone Therapeutics*

**Bobby Gaspar, M.D., Ph.D.**  
*CEO, Orchard Therapeutics*

**Jerry Keybl, Ph.D.**  
*Senior Director,*  
*Cell & Gene Therapy,*  
*MilliporeSigma*

**Brett Kopelan**  
*Executive Director,*  
*Debra of America*

**Bruce Levine, Ph.D.**  
*Professor in Cancer Gene Therapy,*  
*University of Pennsylvania*

**Tim Lu, M.D., Ph.D.**  
*CEO and Co-Founder,*  
*Senti Biosciences*

**John Maslowski, M.S.**  
*Chief Commercial Officer,*  
*Forge Biologics*

**Chris Mason, M.D., Ph.D.**  
*Founder & Director, Ori Biotech*

**Debra Miller**  
*Founder & CEO, CureDuchenne*

**Alison Moore, Ph.D.**  
*Chief Technology Officer, Allogene*

**Adora Ndu, PharmD, J.D.**  
*Chief Regulatory Affairs Officer,*  
*BridgeBio*

**Susan Nichols**  
*President & CEO,*  
*Propel BioSciences*

**Karah Parschauer, J.D.**  
*Chief Legal Officer, Ultragenyx*

**Louise Rodino-Klapac, Ph.D.**  
*EVP, Chief Scientific Officer, Sarepta*

**Jeff Ross, Ph.D.**  
*CEO, Miromatrix Medical*

**Laura Sepp-Lorenzino, Ph.D.**  
*EVP & Chief Scientific Officer,*  
*Intellia Therapeutics*

**R.A. Session, MBA, MSF**  
*President, Founder & CEO, Taysha Tx*

**Curran Simpson, M.S.**  
*Chief Operations & Technology*  
*Officer, REGENXBIO*

**Bob Smith, MBA**  
*SVP, Global Gene Therapy, Pfizer*

**Joe Tarnowski, Ph.D.**  
*SVP, Cell and Gene Therapy Platform*  
*GlaxoSmithKline*

**Arthur Tzianabos, Ph.D.**  
*President and CEO,*  
*Homology Medicines*

**Christopher Vann**  
*SVP & COO, Autolus Therapeutics*

**Kristin Yarema, Ph.D.**  
*CCO, Atara Bio*

# Meet the ARM Team



**Shakita Bazemore**  
Manager, Operations



**Josephine Lembong**  
Manager, Science & Industry Affairs



**Thomas Szabo**  
Executive Administrator



**Elaine Blauser**  
Coordinator, Membership and Public Affairs



**Brett Logan**  
Director, US Market Access & State Government Relations



**David Townsend**  
Vice President, Finance and Operations



**Savannah Bryant**  
Coordinator, Events



**Stephen Majors**  
Senior Director, Public Affairs



**Rosie Walker**  
Director, AFE/GROW Programs



**Jancee Curl**  
Senior Manager, Events



**Kelly McWhinney**  
Director, Events



**Erica Wilhide**  
Director, Membership & Business Development



**Mimi Choon-Quinones**  
Director, European Regulatory Affairs



**Brittany Miller**  
Manager, Events



**Michael Werner**  
Co-Founder & Senior Policy Counsel



**Robert Falb**  
Director, US Regulatory Affairs



**Paolo Morgese**  
Director, EU Market Access & Member Relations



**Adam Wolf**  
Manager, Public Affairs



**Christina Hartman**  
Head, US Government Relations



**Kristy Rothfritz**  
Manager, Membership



**Becky Wright**  
Staff Accountant



**Tim Hunt**  
Chief Executive Officer



**Aleksandra Stein**  
Manager, Data & Analytics



**Elisabetta Zanon**  
Director, European Policy & Advocacy



**Michael Lehmicke**  
Vice President, Science & Industry Affairs



**Laura Stringham**  
Senior Director, Marketing & Events



**Special thanks to our data partner**



**GlobalData.**

GlobalData is a trusted and authoritative intelligence provider to the world's largest industries. We cover a broad spectrum of the pharmaceutical, medical technology and healthcare value systems with in-depth analysis, forecasts, exclusive news, and robust databases all available through a fully integrated platform. Our Consulting team expands on the company's intelligence offering and serves our clients as strategic advisors. The team combines a global presence with the extensive industry expertise required to ask the right questions and uncover strategic insights, thereby allowing our clients to anticipate and plan for the market events likely to impact their future success.

**[globaldata.com](http://globaldata.com)**



# Alliance *for* Regenerative Medicine



Contact: Stephen Majors | Senior Director, Public Affairs  
[smajors@alliancerm.org](mailto:smajors@alliancerm.org)  
[www.alliancerm.org](http://www.alliancerm.org)