Alliance for Regenerative Medicine

SECTOR SNAPSHOT

AUGUST 2023

BUILDING A NEXT-GEN WORKFORCE

In this edition…

- **New and improved data and a pipeline outlook**
- **What's driving the sector's workforce gap?**
- **Workforce solutions industry leaders are considering**

About ARM

The Alliance for Regenerative Medicine (ARM) is the leading international advocacy organization championing the benefits of engineered cell therapies and genetic medicines for patients, healthcare systems, and society. As a community, ARM builds the future of medicine by convening the sector, facilitating influential exchanges on policies and practices, and advancing the narrative with data and analysis. We actively engage key stakeholders to enable the development of advanced therapies and to modernize healthcare systems so that patients benefit from durable, potentially curative treatments. As the global voice of the sector, we represent more than 475 members across 25 countries, including emerging and established biotechnology companies, academic and medical research institutions, and patient organizations.

New and Improved Sector Data

ARM is constantly seeking new ways to improve clarity, accuracy, and consistency in our quarterly data reports. As of Q1 2023, we have updated our approach to collecting sector data by building our data collection around the identification of relevant drug assets within ARM's technology scope. This approach has enhanced our data in multiple ways, including identifying 1,000+ developers that were undetected in our previous approach centered on identifying relevant clinical trials.

To access our full data and learn more about our new data approach and methodologies, visit www.alliancerm.org/data or scan the QR code.

Q1 2023 Sector Data

	North America	Asia Pacific	Europe	Total
Developers	1,235	888	543	2,760
Clinical Trials	917	648	329	1,687
Investment	$2.4B	$0.5B	$0.2B	$3.0B

Clinical Pipeline Outlook

There have been six regulatory approvals of a cell or gene therapy between the United States (US) and the European Union (EU) thus far in the calendar year 2023.

- **THE EMA COULD PROVIDE A RECOMMENDATION** on CTX001 (Vertex/CRISPR) this summer, with a European Commission approval in the fall, making it the first CRISPR therapy to receive approval. CTX-001 has a PDUFA date of December 8 in the US for the sickle cell indication.
- **DESPITE THIS MILESTONE,** there is a decline in regulatory decisions in the EU pipeline compared to the US.
- **THE FDA IS SET TO POTENTIALLY APPROVE** up to nine cell and gene therapies in 2023, which would be a record for the most approved in a year. This includes potential approvals of five gene therapies for rare genetic diseases – matching the number of such therapies approved by the FDA in the previous five years combined.

2023 Pipeline Outlook

	Therapy	Type	Indication	Status
Approved	**Hemgenix** *(uniQure and CSL Behring)*	Gene Therapy	Hemophilia B	Approved (February 2023)
	Omisirge *(Gamida Cell)*	Cell Therapy	Reduce time to neutrophil recovery and infection in patients with hematologic malignancies	Approved (April 2023)
	Vyjuvek *(Krystal Biotech)*	Gene Therapy	Dystrophic epidermolysis bullosa	Approved (May 2023)
	Elevidys *(Sarepta Therapeutics)*	Gene Therapy	Duchenne muscular dystrophy	Approved (June 2023)
	Lantidra *(CellTrans)*	Cell Therapy	Type 1 Diabetes	Approved (June 2023)
	Roctavian *(BioMarin Pharmaceuticals)*	Gene Therapy	Hemophilia A	Approved (June 2023)
BLA/MAA Accepted	**CTX001** *(Vertex Pharmaceuticals & CRISPR Therapeutics)*	Gene Editing Therapy	Sickle cell disease, β-thalassemia	EU decision expected in the fall of 2023 FDA decision set on sickle cell disease for December 8, 2023 FDA decision for β-thalassemia expected March 2024
	Lifileucel *(Iovance)*	Cell Therapy	Metastatic melanoma	FDA decision set for November 25, 2023
	NurOwn *(BrainStorm Therapeutics Inc.)*	Cell Therapy	Amyotrophic lateral sclerosis (ALS)	FDA decision set for December 8, 2023
	Lovo-cel *(Bluebird bio)*	Gene Therapy	Sickle cell disease	FDA decision set for December 20, 2023
BLA/MAA Submitted	**HPC Cord Blood** *(StemCyte)*	Cell Therapy	Unrelated Donor hematopoietic progenitor cell transplantation	BLA Pending

Anticipated 2024 decisions			
Afami-cell *(Adaptimmune)*	**CT-053** (CARsgen Therapeutics)	**Fidanacogene Elaparvovec** (Pfizer)	**Libmeldy** (Orchard Therapeutics)
Tab cel *(Atara Biotherapeutics)*	**Upstaza** (PTC Therapeutics)	**Vyjuvek** (Krystal Biotech)	**Elevidys** (Sarepta Therapeutics)

In Focus: The Workforce Gap

What's happening?

The cell and gene therapy sector faces a workforce problem. Recent research from ARM highlights a growing gap in skills needed for employees working within the industry and regulators. The workforce gap is most pronounced within technical areas, such as development and manufacturing. This trend could have profound consequences for the sector if it persists and could eventually threaten the sector's ability to reach its full potential.

01
THE PROBLEM?
Workforce gap

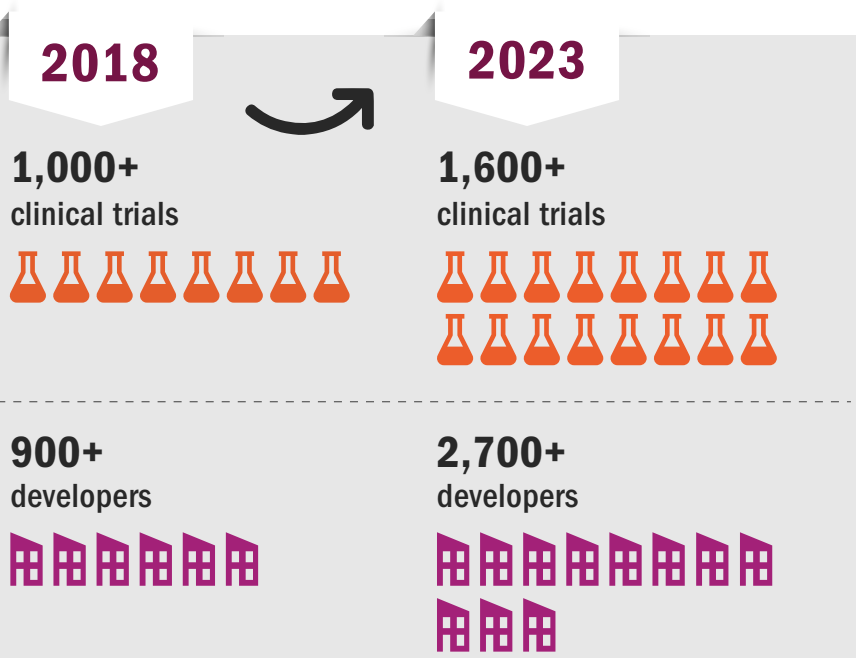
02
WHAT IS IT?
Shortage of technical skills

03
THE RISK?
Threaten our full potential as a sector

Why is this happening?

The number of companies and clinical trial programs has grown so fast that the sector needs more talent to keep pace.

- The FDA has become backlogged, not having enough capacity to review this wave of submissions
- The industry will need more skilled workers with know-how and hands-on experience to support late-stage clinical phase studies and the anticipated commercialization of products



Where are the gaps?

ARM's Workforce Gap Analysis (March 2023) identifies four categories of skills that surveyed professionals in the US consider challenging to recruit. As innovation continues to shape the workplace, industry leaders are also weighing how to find talent that can apply technological innovations, such as AI and automation, and enhance cell and gene therapy production.

01 **Critical skill gaps identified in ARM's report**

- Quality control & assurance
- Manufacturing
- Process development
- Analytical development

02 **Skills needed for the future of work**

- Information technology
- AI & automation
- Data management & analysis
- Programming

Access ARM's Workforce Development Report

What's driving the workforce gap?

At the International Society for Cell and Gene Therapy's 2023 Annual Meeting (June 2023), ARM hosted a roundtable workshop with industry experts to investigate the factors accelerating the workforce gap. The discussion highlighted two main challenges.

Volume =
Small starting talent pool

01 Awareness:
Young professionals are unaware of cell and gene therapy career opportunities.

02 Scalability:
There is little infrastructure that provides certification to aspiring talent.

Training =
Lack of technical knowledge

01 Cost:
Many training opportunities are costly and difficult to update with current innovations.

02 Opportunity:
Licensing and certification programs are limited.

Building a next-gen workforce

The landscape of regenerative medicine is evolving rapidly and is already disrupting the status quo of medicine. The sector needs a new infusion of next-gen talent to usher in the coming wave of cell and gene therapies.

There will be no one-size-fits-all solution, as local and regional factors often impact a developer's access to talent. However, our workshop at ISCT 2023 highlighted some common approaches the sector could take to address the workforce gap holistically.

Volume challenge

Training challenge

Solutions

Establish a pathway

Increase awareness

More public engagement on cell and gene therapy as a field and career option.

Create opportunities

Advocate for training centers that provide licenses and certificates.

Spotlight

White House Action Plan

In June 2023, the White House released an action plan to rapidly and equitably expand biotechnology and biomanufacturing education and job training programs in the US.

Overhaul education

Sharing is caring

Establish platforms to share best practices, especially in rapidly emerging areas such as gene editing and tissue engineering.

Train the trainer

Refresher training can help keep curriculum at pace with innovation.

Digital tools

Virtual and augmented reality can help overcome geographic and resource barriers to training.

A look into the FDA

A workforce gap also creates bottlenecks on the regulatory side. When a scientific field advances quickly, it can be challenging for regulators to keep pace. The recent discourse on AI illustrates this phenomenon.

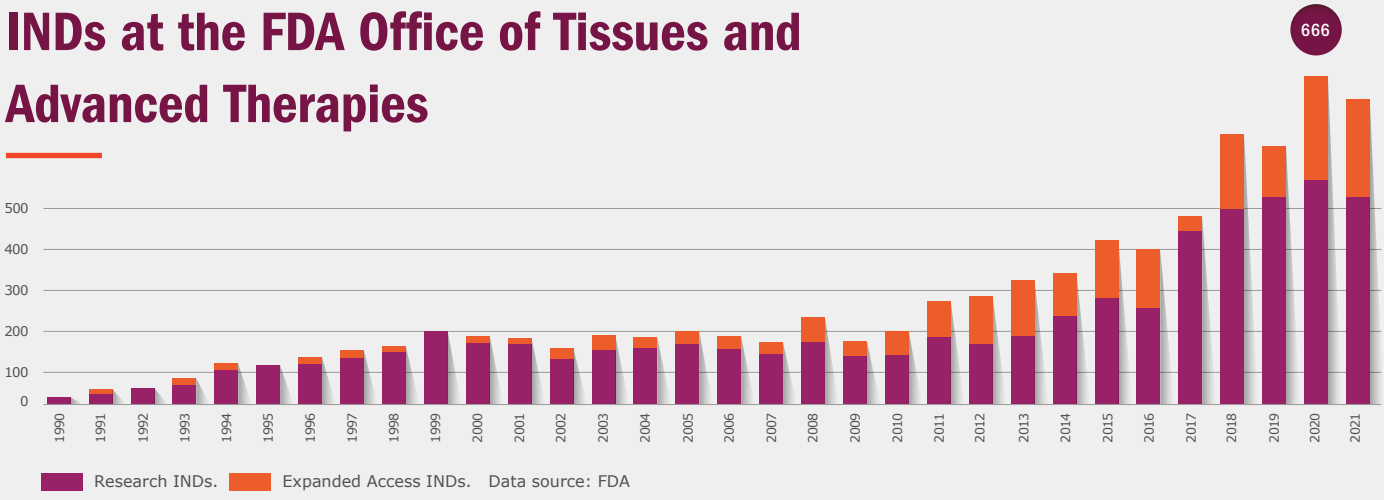
The FDA needs additional qualified people to manage the growing number of cell and gene therapy submissions. The result? Many of the 900+ cell and gene therapies in the US regulatory pipeline could reach a standstill, stifling innovation and jeopardizing access for patients who rely on them.

Adapting to innovation

To meet these growing demands, the US Congress authorized new funding for the agency when it reauthorized the Prescription Drug User Fee Act (PDUFA VII) in 2022. This has allowed the FDA to pursue the hiring of 100+ new staffers for its newly created "super office," called the Office of Therapeutic Products, which replaced the Office of Tissues and Advanced Therapies.

The move will help the agency meet its expectation of approving 10-20 cell and gene therapies annually by 2025.

INDs at the FDA Office of Tissues and Advanced Therapies



On the horizon...

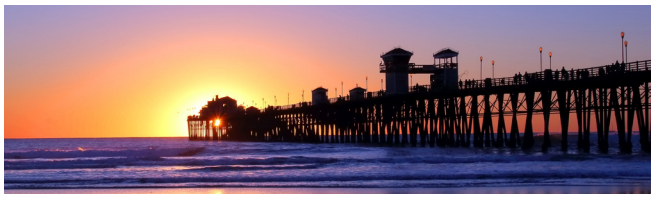
ARM will soon undertake a 'horizon scan' that will inform OTP about emerging technological trends and what capabilities its reviewers will need in the years ahead.

GlobalData.

Thanks to our data partner, GlobalData!

GlobalData is a trusted and authoritative intelligence provider to the world's largest industries. They 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.

Events



Cell and Gene Meeting on the Mesa

Carlsbad, CA October 10-12, 2023

Meetingonthemesa.com ↗

Cell and Gene Meeting on the Med

Rome, Italy April 9-11, 2024

Meetingonthemed.com ↗

Get Involved

ARM has introduced a new governance structure and decision model to support our 2023-2025 Strategic Plan. We introduced Membership Forums in June for broader member engagement, discussion, and information sharing. To get involved in a forum, please get in touch with Elaine Blauser at eblauser@alliancerm.org

Stay engaged

MEMBERSHIP AND EVENTS
Contact: Laura Stringham, lstringham@alliancerm.org ↗

MEDIA INQUIRIES
Contact: Stephen Majors, smajors@alliancerm.org ↗

Alliance *for* Regenerative Medicine

Stay connected

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