State of the Industry Briefing
by Tim Hunt

cell&gene day 2023

Cell and Gene Therapy: An Expert Perspective on the State of the Industry

On May 11, 2023, Precision ADVANCE, the Center for Breakthrough Medicines, and the Alliance for Regenerative Medicine co-sponsored the second annual Cell & Gene Day, hosted by Endpoint News. Throughout this event, over 25 successful innovators from across the advanced therapy sector shared fresh ideas and insights on overcoming critical challenges in bringing life-saving therapies to patients in need.

Tim Hunt, CEO of the Alliance for Regenerative Medicine (ARM), kicked off the event with a briefing on the state of the cell and gene therapy development landscape. With 475 members worldwide, ARM focuses on convening the sector, providing data and analysis, engaging with stakeholders, and enabling the development of advanced therapies. One of their key initiatives is modernizing healthcare systems to enable access to these novel therapeutics. Highlights of this briefing are provided in this summary.

Tim Hunt, CEO
Alliance for Regenerative Medicine (ARM)

2022: A seminal year for cell and gene therapy

There has been tremendous progress in the cell and gene therapy field as the science advances and the sector continues to mature. Since 2018, five gene therapies have been approved for rare disease indications. In 2019, then FDA Commissioner Scott Gottlieb, MD, predicted that the FDA would be approving 10-20 cell and gene therapy products per year.

Last year was an important one for the sector, with six new therapies approved—including the first allogeneic T-cell therapy—and five existing therapies approved in new geographies or indications (Figure 1).

In August 2022, the first gene therapy for hemophilia A was approved in the EU, with a US decision still pending.

New Therapies Approved

Legend Biotech & Janssen	Carvykti - US and EU (CAR-T)
BioMarin Pharmaceutical	Roctavian - EU (Gene Therapy)
PTC Therapeutics	Upstaza - EU (Gene Therapy)
UniQure and CSL Behring	Hemgenix - US (Gene Therapy)
Ferring Pharmaceuticals	Adstiladrin - US (Gene Therapy)
Atara Biotherapeutics	Ebvallo – EU (Cell Therapy)

Therapies Approved in New Geographies or New Indications

Bristol-Myers Squibb	Breyanzi (CAR-T)
Novartis	Kymriah (CAR-T)
Kite Pharma (Gilead)	Yescarta (CAR-T)
bluebird bio inc.	Zynteglo (Gene Therapy)
bluebird bio inc.	Skysona (Gene Therapy)

Figure 1. Cell and gene therapy milestones in 2022

2023 outlook

The first gene therapy for hemophilia B was approved by the FDA in November 2022 and by the EMA in February 2023. Two gene therapies for sickle cell disease, including the first CRISPR gene editing-based technology, are also poised for FDA approval this year. These therapeutics are significant not only due to their potential to transform the lives of patients with these hematologic conditions, but also because they usher in an era of advanced therapies for more prevalent diseases. These larger patient populations raise the important question of whether healthcare systems are ready to embrace this future.

In May 2023, the first topical gene therapy was approved for dystrophic epidermolysis bullosa. This approval was followed by the first US approval for Duchenne muscular dystrophy (DMD) in June. Other potential milestones on the horizon in 2023 are the US approvals of 5 gene therapies for rare diseases in a single year, the first approval of an adoptive cell therapy for a solid tumor indication, and the first US approval of an allogeneic cell therapy, all of which represent major breakthroughs in the sector (Figure 2).

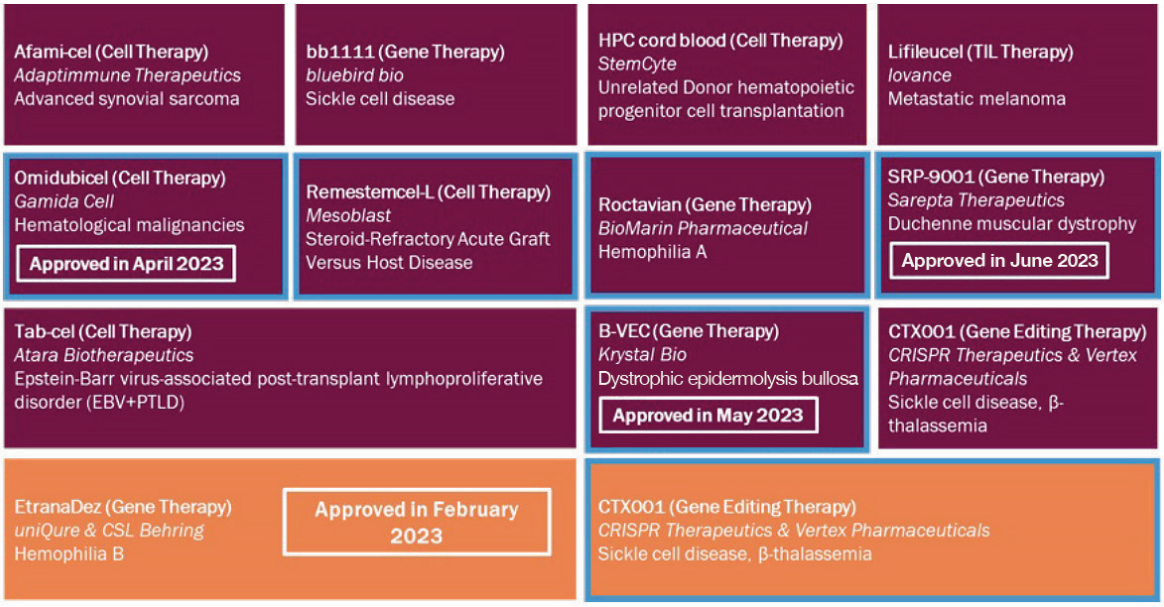

Figure 2. Potential US and EU regulatory decisions in 2023 | Published as of June 28, 2023.

Cell and gene therapy clinical trial activity

Currently, there are over 2,200 active cell and gene therapy clinical trials worldwide, with 60% focused on oncology with nearly equal representation of solid and liquid tumors. More than 250 studies were initiated in 2022, with nearly half involving sites in the Asia-Pacific region. Over 202 trials are in Phase 3 and more than 140 are investigating gene editing technologies. Importantly, 58% of active trials have potential applications in a prevalent disorder, heralding a revolution where cell and gene therapies move into the mainstream of healthcare.

Cell and gene therapy investment landscape

More than 1,450 cell and gene therapy companies, research institutes, and academic centers are focused on developing advanced therapies, representing an 11% year-over-year increase (Figure 3).

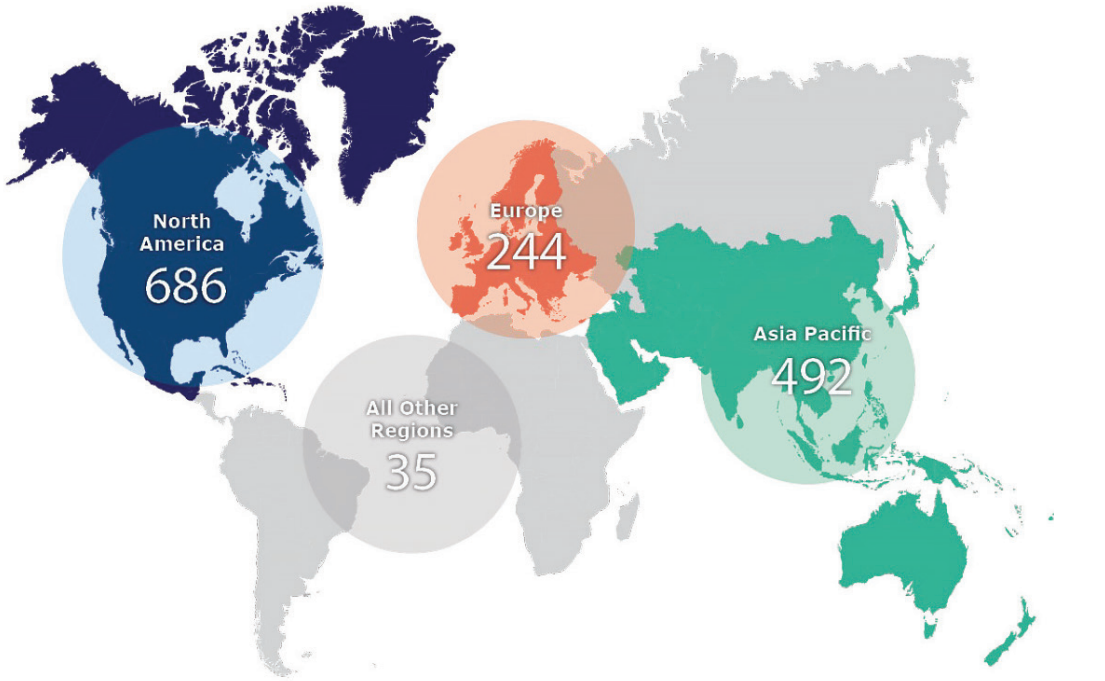

Figure 3. Cell and gene therapy developers worldwide

While investment in cell and gene therapies peaked during the COVID-19 pandemic, it has begun to normalize to pre-pandemic levels, with $12.6 billion in capital raised by advanced therapy developers in 2022 (Figure 4).

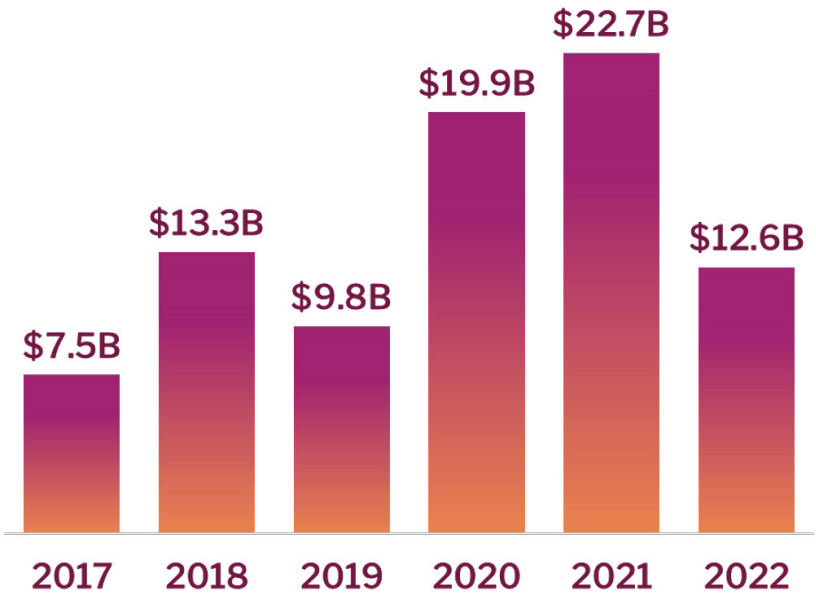

Figure 4. Investment in cell and gene therapies: 2017 to 2022

FDA & payors: Ensuring patient access

Rapid progress in the science behind cell and gene therapies has created a highly dynamic environment for healthcare systems. The advent of larger patient populations is becoming a forcing function for stakeholders—including regulators and payors—to modernize their policies. The regulatory and payor frameworks of the past are poorly-equipped for handling the medicines of the future, and change will be needed.

In March 2023, the FDA officially transitioned from its Office of Tissues and Advanced Therapies (OTAT) to the Office of Therapeutic Products (OTP) as part of its effort to keep up with growth in the cell and gene therapy space. The agency is also adding 100 new reviewers in the next five years and advocating for the appropriate use of accelerated approval pathways for gene therapies.

Progress

IS BEING MADE TO HELP EXPEDITE ACCESS TO CELL AND GENE THERAPIES

In the US, there has been a push among commercial payors to increase readiness as current systems cannot accommodate durable and potentially curative, but expensive, treatments. Progress is being made in the development of value-based frameworks to help expedite access to cell and gene therapies. At the federal government level, the reintroduction of the Medicaid VBPs for Patients (MVP) Act and the testing of a Cell and Gene Therapy Access Model to administer multi-state outcomes-based agreements in Medicaid programs offer promising opportunities to enable access to advanced therapies for patients who need them.

Conclusion

As the pace of cell and gene therapy approvals accelerates, new questions will arise regarding the ability of healthcare systems to adapt to the rate of change and the willingness of patients to embrace these technologies. The sector has reached an important turning point, but obstacles still exist, and the next five years will be telling for the future of cell and gene therapies.

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