



# Alliance *for* Regenerative Medicine

**2018**

**Annual Regenerative Medicine Data Report**



The Alliance for Regenerative Medicine (ARM) is the preeminent global advocate for regenerative and advanced therapies. ARM fosters research, development, investment and commercialization of transformational treatments and cures for patients worldwide.

By leveraging the expertise of its membership, ARM empowers multiple stakeholders to promote legislative, regulatory and public understanding of, and support for, this expanding field.

[www.alliancerm.org](http://www.alliancerm.org)

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# Letter from ARM CEO Janet Lambert

2018 was a great year for our sector and for the Alliance for Regenerative Medicine (ARM).

Regenerative medicines and advanced therapies continued to make their way to market, and to the patients counting on them. 2018 saw the first CAR-T therapies approved in Europe, Canada, and Australia, and already-approved products were cleared for additional indications in the United States. The EMA also approved Spark's LUXTURNa, a gene therapy for certain types of inherited retinal blindness. In Japan, the MHLW granted conditional approval to two stem cell therapies for Alzheimer's and spinal cord injuries respectively, and the U.S. FDA approved Avita Medical's RECELL cell therapy for the treatment of severe burns. These approvals follow the U.S. FDA's groundbreaking 2017 approvals of Novartis's Kymriah, Kite/Gilead's Yescarta, and Spark's LUXTURNa.

Throughout 2018, greater numbers of patients felt the transformative power of cell and gene therapies. Novartis's Kymriah Juliet and Eliana trial data showed a 40 percent complete response rate in patients with r/r DLBCL and a 60 percent complete response rate in patients with r/r B-Cell ALL respectively. Kite/Gilead's Yescarta ZUMA-1 trial produced a 58 percent response rate in patients with r/r B-cell NHL, and 72% (21 of 29) of clinical trial participants who received Spark's LUXTURNa were able to navigate through a mobility test course equivalent to a moonless summer night.

Investment grew in the sector, with more than \$13 billion in global financings, across technology focus areas. The number of clinical trials worldwide reached an all-time high of 1,028, with a target enrollment of more than 59,000 patients globally.

Here at ARM, we are inspired by the progress in the sector and its impact on patients and are dedicated to driving the development and implementation of a legislative, regulatory, and financial infrastructure that supports the clinical, scientific, and commercial progress in the field.

In 2018, ARM grew significantly, mirroring the growth we've seen in the sector. We now have more than 300 members, representing a diverse set of companies, research organizations, major medical institutions, and patient advocacy groups. These groups represent the range of technologies utilized in the sector and the spectrum of stakeholders dedicated to bringing these products to patients.

ARM built upon our strong existing relationships with policymakers, engaging in formal and informal dialogues on both sides of the Atlantic. On behalf of the sector, we submitted more than 20 official comments to international regulatory bodies to advance a positive environment for regenerative medicines and advanced therapies. We published our third white paper on innovative payment models, identifying barriers to these models in the United States. We also began work on a comprehensive report detailing the market access environment for ATMPs in key European markets, which we expect will inform our work in Europe in the following years.

In addition to our policy work, ARM furthered our role as the premier source of sector data and expertise, highlighting the clinical and financial progress of 900+ therapeutic developers worldwide. We initiated the A-Gene and A-Cell projects, designed to provide a case study-based book of knowledge for the development of gene and cell therapies respectively and engaging the support of stakeholders across the sector, including the U.S. FDA. And in June, ARM announced the launch of the ARM Foundation for Cell and Gene Medicine, an affiliate organization intended to educate, engage, and empower patients, caregivers, industry leaders, and other regenerative medicine stakeholders on a number of sector-specific topics and initiatives.

In 2019, ARM will continue to advance legislative and regulatory progress around market access, educating stakeholders about the immense value these therapies represent to ensure safe and effective products are made available to patients as quickly as possible. Our membership will continue to grow as we convene additional stakeholders across the sector, including patient advocacy groups, research institutions, and non-profit organizations.

There is a lot of hard work ahead of us as the immense potential of this sector is realized, and I am honored to be leading the charge. Thank you for your support, and we look forward to working with you in 2019.



All the best,

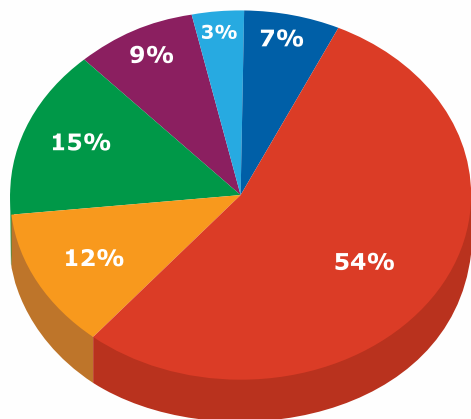
Janet Lambert  
CEO, Alliance for Regenerative Medicine



# ARM: Voice of the Sector

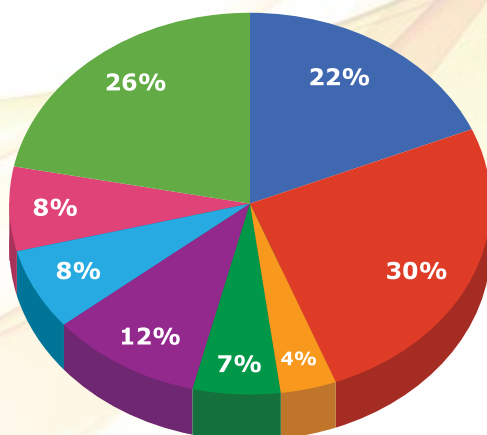
## ARM's Multi-Stakeholder Membership

ARM's Total Member Organizations = 304



### Diversity of Organizations

- Corporations (Revenue > \$50M)
- Corporations (Revenue < \$50M, < 100 Full Time Employees)
- Corporations (Revenue < \$50M, > 100 Full Time Employees)
- Non-Profit Institutions
- Academic Institutions
- Affiliates & Financial Institutions



### Diversity of Technology Focus

- Gene Therapy
- Cell Therapy
- Gene & Cell Therapy
- Tissue Engineering
- CMOs and CROs
- Service Providers
- Advocacy Organizations
- Research Organizations

## ARM's Strategic Focus Areas

### Regulatory

- Promote clear, predictable, and efficient regulatory frameworks.
- Assess all FDA, EMA, and related guidance relevant to cell and gene therapy, including guidance related to manufacturing, CMC, and other industrialization issues.
- Promote international convergence of key regulation and guidance to promote global product development by identifying specific areas of regulatory inconsistency among jurisdictions and developing proposals for adoption by regulatory agencies.

### Reimbursement

- Develop principles of ARM-endorsed global value framework.
- Develop strategies to remove or mitigate barriers via regulatory changes or legislation for public and private payers both in the U.S. and in key EU countries.
- Secure favorable access and reimbursement for RM / AT products.

### Industrialization and Manufacturing

- Reduce standards, technical, and regulatory barriers to the scale up of RM / AT therapies.

# Letter from ARM Chairman Matt Patterson

This is an exciting time to be a part of the regenerative medicine sector, and it is my honor to serve as Chairman of the Alliance for Regenerative Medicine during this transformative period for our field. The pace of research and the forward momentum in the industry are truly remarkable. New medicines that dramatically impact the lives of patients and families are now on the market, and dozens of additional therapies are in late-stage clinical trials. The regulatory environment is evolving rapidly alongside the science, which will further enable innovation and help drive needed policy changes to improve access to new products. These events demonstrate that the sector has emerged and is poised for stronger growth at a global scale.

That being said, this is not a time to rest and simply congratulate ourselves on jobs well done. To the contrary—there are tremendous challenges ahead that we can and must solve together. On the research and development side, the progress is undeniable and thrilling. But we must appreciate that we still have much to learn about the science and application of regenerative medicine technologies. On the commercial side, we are in desperate need of innovation and collaboration between manufacturers, governments, payors, health care providers, and advocacy groups. The simple reality is that no one in the world today lives in a health care system designed to ensure patient access to products like those of the regenerative medicine space, in particular one-time potentially curative treatments. The specific challenges ahead of us include the need to establish value-based payment models that recognize and account for the potential impact of these therapies over time, the need to collaborate with treatment centers to ensure optimal patient care and follow up, and the need to solve the scientific and operational complexities of manufacturing and supply chain at a large and global scale.

These are not easy obstacles to overcome, but ARM is uniquely positioned to play a critical role in advancing this amazing field, and with your help, to ensure we solve the challenges ahead. I am confident we will succeed. We have no choice. What a shame it would be if we solved the incredible challenges of researching, developing, and licensing these medicines, only to see patients unable to access them. This isn't an acceptable scenario to any of us, so let's get to work with all the relevant constituents to ensure a brighter future for patients and their families.

Thank you for your many contributions and continued support. I look forward to working together in 2019.



Matthew Patterson  
CEO and Chairman  
Audentes Therapeutics

# Global Landscape

906+

Regenerative medicine companies worldwide, including gene therapy, cell therapy, and tissue engineering therapeutic developers





# Industry Overview

2018 goes down in the books as one of the most exciting years for the regenerative medicine and advanced therapies sector thus far. The optimistic view for cell and gene therapies at the end of 2017 was realized as these companies closed successful financings, progressed in the clinic, and received additional approvals internationally.

2018 was also the biggest year yet for IPOs in the sector. Among the largest: Allogene (\$372.6 million), Rubius Therapeutics (\$277.3 million), Orchard Therapeutics (\$225.5 million), Autolus (\$172.5 million), and Homology Medicines (\$165.6 million). Secondary financings were just as successful, with multi-million-dollar rounds from bluebird bio (\$651.3 million in January and \$632.5 million in July), AveXis (\$439.1 million), Iovance Biotherapeutics (\$252.2 Million), Audentes Therapeutics (\$231.4 million), and Sangamo Therapeutics (\$230 million).

There was significant M&A activity in 2018, including Celgene's acquisition of Juno for \$9 billion and the \$8.7 billion acquisition of AveXis by Novartis. Takeda's acquisition of TiGenix for \$626.4 million, PTC Therapeutics' acquisition of Agilis Biotherapeutics for \$200 million upfront, and Astellas' acquisitions of Quethera (\$109.4 million) and Universals Cells (\$102.5 million), among others, helped push upfront M&A values for 2018 to nearly \$19 billion.

Several noteworthy partnerships propelled a 55% increase in upfront dollars from 2017 to 2018. Early in the year, Sangamo and Gilead/Kite signed a partnership potentially worth \$3.16 billion, while Voyager's deal with Abbvie could be worth \$1.05 billion. Other deals of note: Gritstone signed a \$1.23 billion agreement with bluebird for cell-based immunotherapies, SQZ Biotechnologies signed an agreement with Roche worth up to \$1.38 billion for cell-based I/O, and Eli Lilly and Sigilon agreed to develop insulin-producing beta cells from engineered iPSCs in an agreement worth up to \$410 million.

The success in this sector was substantiated by the recent announcement from FDA Commissioner Scott Gottlieb foreseeing the need for increased regulatory support for the sector to keep pace with clinical progress and an anticipated increase in product approvals. Four companies announced RMAT designation in Q4 2018: Poseida's CAR-T therapy for multiple myeloma, Iovance's TIL for metastatic melanoma, AxoGen's Avance for nerve injuries, and Rocket Pharma's RP-L102 for Fanconi Anemia.

With a jump in the Nasdaq Biotechnology Index at the start of 2019, we believe investments will continue to drive another successful year. We expect M&As to heat up as additional large pharma companies expand into cell and gene therapy and anticipate the IPO window remaining open as investors continue to gain confidence in this field. International regulatory bodies have continued to show support for these innovative therapies, while public and private payers display a willingness to engage with developers on new payment models. 2019 is setting up to be another exciting year for the sector.

-Patricia Reilly

Vice President, Intelligence Alliances and Unification

Pharma Intelligence, Informa

# Global Financings

## Total 2018 Global Financings



### TOTAL GLOBAL FINANCINGS

**\$13.3 Billion raised in 2018**

73% YoY increase from 2017



### GENE & GENE-MODIFIED CELL THERAPY

**\$9.7 Billion raised in 2018**

64% YoY increase from 2017



### CELL THERAPY

**\$7.6 Billion raised in 2018**

64% YoY increase from 2017



### TISSUE ENGINEERING

**\$936.9 Million raised in 2018**

258% YoY increase from 2017

\*Total amount raised represents sector-wide figures; please note that some companies utilize technology from more than one technology group. As a result, the total financings amount does not equal the sum of the raises of the individual technology groups.

\*\* Figures do not include M&A transaction totals.

## Select Corporate Partnerships, Financings, M&A: 2018

#### Corporate Partnerships/ Collaborations:

- Kite Pharma signs \$150M agreement with Sangamo Therapeutics for gene-modified cell therapies for the treatment of cancer – February 20, 2018
- Intrexon signs \$150M amended CAR-T agreement with Merck – December 20, 2018
- SQZ Biotechnologies signs \$125M agreement with Roche for the development of cell therapies to treat cancer – October 15, 2018
- Spark Therapeutics signs \$105M agreement with Novartis for the development & commercialization of LUXTURNA outside the United States – January 24, 2018

#### IPOs:

- Allogene \$372.6M – October 15, 2018
- Rubius Therapeutics \$277.3M – July 23, 2018
- Orchard Therapeutics \$225.5M – November 5, 2018

#### Follow-On Financings:

- bluebird bio \$651.3M – January 8, 2018
- bluebird bio \$632.5M – July 7, 2018
- AveXis \$431.9M – January 22, 2018
- Iovance Biotherapeutics \$252.2M – October 17, 2018
- Audentes Therapeutics \$231.4M – January 29, 2018
- Sangamo Therapeutics \$230M – April 30, 2018
- REGENXBIO \$201.8M – August 24, 2018
- CRISPR Therapeutics \$200M – September 19, 2018

#### Mergers & Acquisitions:

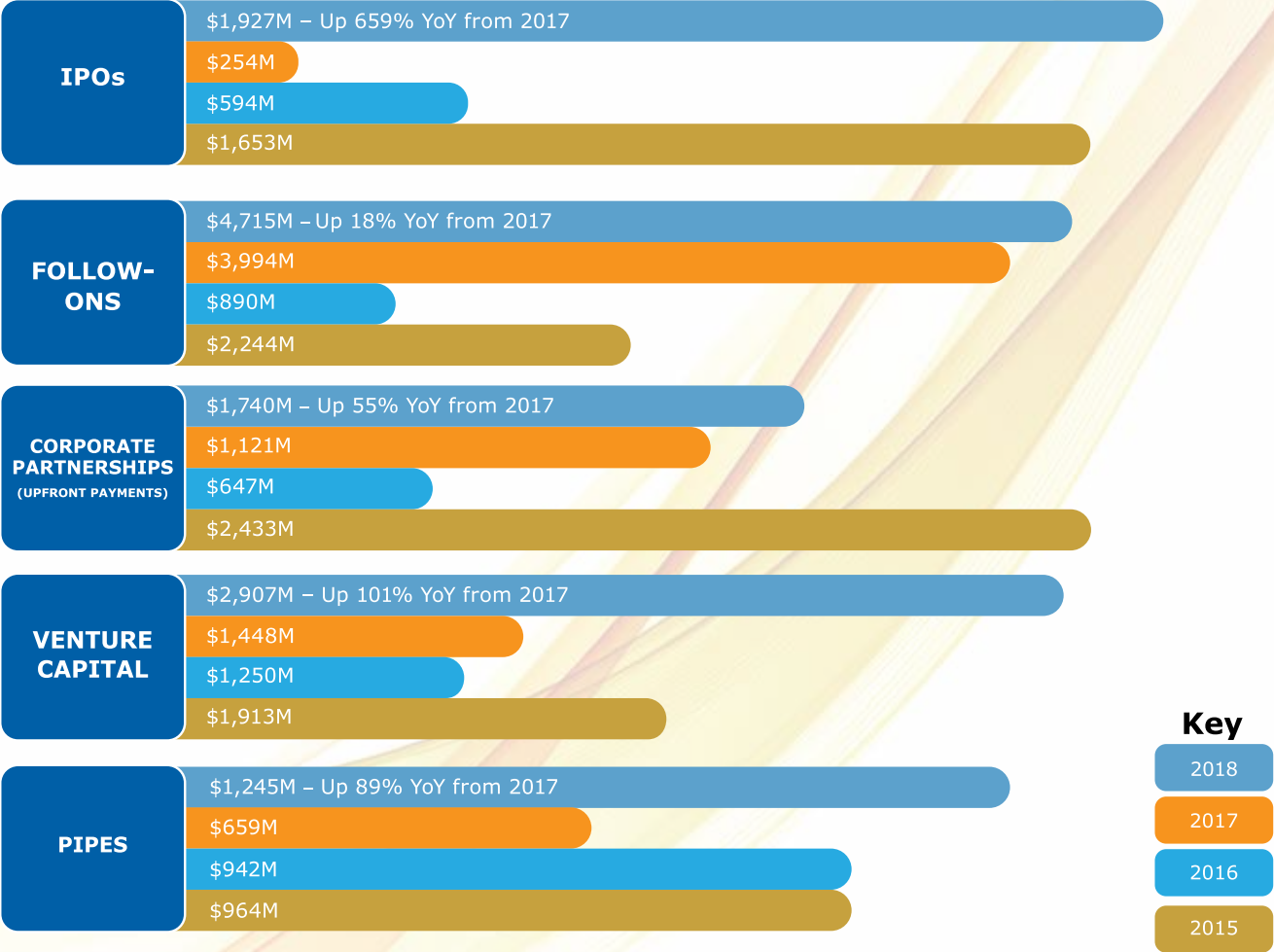
- Celgene acquired Juno Therapeutics for \$9B – March 6, 2018
- Novartis acquired AveXis for \$8.7B – May 15, 2018
- Takeda acquired TiGenix for \$626.4M – July 31, 2018
- PTC Therapeutics acquired Agilis in \$945M deal, including \$200M upfront – August 23, 2018
- Astellas acquired Quethera for \$109.4M – August 10, 2018
- Astellas acquired Universal Cells for \$102.5M – February 24, 2018
- Amicus Therapeutics acquired Celenex for \$100M – September 20, 2018

#### Venture Financings:

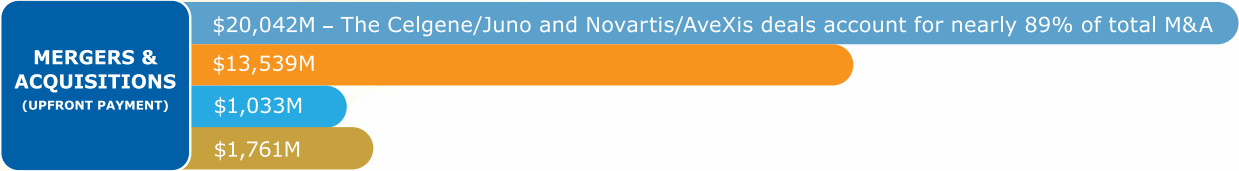
- Allogene \$300M Series A – April 3, 2018
- Orchard Therapeutics \$150M Series C – August 13, 2018
- TCR Therapeutics \$125M Series B – March 21, 2018
- Freeline \$116.6M Series B – June 19, 2018
- Generation Bio \$100M Series B – February 27, 2018
- Tmunity \$100M Series A – January 23, 2018
- 4D Molecular Therapeutics \$90M Series B – July 10, 2018
- ViaCyte \$80M Series D – November 29, 2018

# Global Financings

## Total Global Financings by Type, by Year



## Mergers & Acquisitions: Upfront Payments

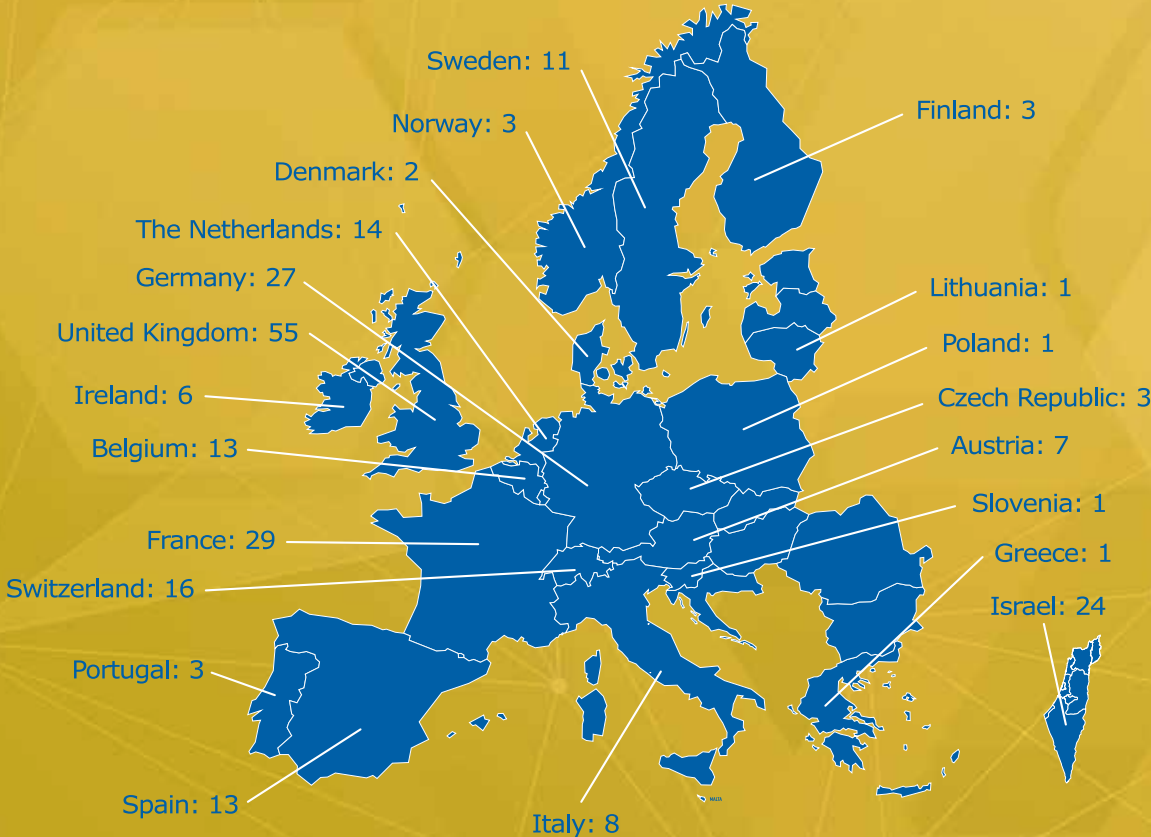


# European Sector Overview

## European Sector Landscape

241+

Regenerative Medicine / Advanced Therapies Companies  
based in Europe/Israel



### European/Israeli Companies by Technology:

\*please note, individual companies may be active in more than one technology type



Gene Therapy: 104



Cell Therapy: 134



Tissue Engineering/  
Biomaterials: 36



# European Sector Overview



## TOTAL 2018 EUROPEAN/ ISRAELI FINANCINGS

**\$2.2 Billion raised in 2018**  
(appx €1.9 Billion)  
40% YoY increase from 2017



## GENE & GENE-MODIFIED CELL THERAPY

**\$1.9 Billion raised in 2018**  
(appx €1.7 Billion)  
117% YoY increase from 2017



## CELL THERAPY

**\$884.7 Million raised in 201**  
(appx €771.2 Million)  
2% YoY decrease from 2017



## TISSUE ENGINEERING

**\$73.3 Million raised in 2018**  
(appx €63.9 Million)  
41% YoY decrease from 2017

\*Total amount raised represents sector-wide figures; please note that some companies utilize technology from more than one technology group. As a result, the total financings amount does not equal the sum of the raises of the individual technology groups.

\*\* Deals counted involve at least one European or Israeli company and include industry-funded deals only.

# 216

Clinical trials underway  
by end of 2018

**Ph. I: 42**  
**Ph. II: 139**  
**Ph. III: 35**

- Nearly half (48%) of all European or Israeli clinical trials are in oncology, including leukemia, lymphoma, glioblastoma, melanoma, myeloma, and cancers of the cervix, ovaries, prostate, and colon, among others;
- 8% focus on cardiovascular diseases and disorders, including congestive heart failure, critical limb ischemia, myocardial infarction, peripheral vascular disease, and others.

# Commentary: The Landscape for ATMPs in Europe



Christopher Vann  
Chief Operating Officer,  
Autolus

At Autolus, we are focused on making a real difference for cancer patients by developing CAR-T cell therapies which overcome the limitations of current treatment, thereby offering meaningful benefits, including the potential for cures. We're encouraged by the willingness to adopt these new technologies in Europe, as demonstrated by the relatively fast reimbursement of the first CAR-T cell therapies and the centers actively building the capabilities necessary to deliver these breakthrough products.

The two greatest challenges in Europe are the capacity to deliver ATMPs and funding to make them accessible for all qualifying patients. Broader adoption of products will come through a variety of ways: centers will build delivery capabilities and gain the necessary experience to effectively provide these therapies; companies will bring more manageable products to market; and payers will realize the potential of therapies, particularly if they are used in earlier lines of treatment and if we adopt mechanisms that better support value-based pricing. We've already seen a lot of progress. For example, in the UK, the early CAR-T cell therapies are now included in the Cancer Drugs Fund (CDF) and the NHS is establishing centers of excellence with Innovate UK funding and through this CDF access program.

ATMPs are in their naissance but have already made a significant impact on the standard of care. They are poised to have a dramatic positive effect on several types of blood cancers, with the first patients now receiving commercially available CAR-T cell therapies in several countries in Europe. As we look to the future, we anticipate a cycle of rapid cycle of innovation in this field, partnered with a maturing of the delivery systems and reimbursement environment, leading to improved products that can be used earlier in the treatment paradigm, and benefitting more patients with broader array of tumor types.



Amy Sullivan  
Senior Vice President,  
Corporate Affairs, Kiadis

At Kiadis, we are building a biopharmaceutical company where each and every one of us can connect with and truly help patients, their families, and their care teams as they struggle with devastating diseases. Kiadis is advancing a therapy, called ATIR, that is specifically designed to help and not harm patients with acute blood cancers for whom a haploidentical hematopoietic stem cell transplant is the best option. Our deep connection to these patients and the wider community drives us to continue developing these transformative therapies.

There is groundbreaking work across the globe in the field of ATMPs with an increasing focus on treatments personalized to the individuals who need them the most. As a result, patients in the EU are poised to benefit from treatments that can truly cure life-threatening diseases.

# Commentary: The Landscape for ATMPs in Europe



Paolo Morgese  
Director,  
European Market Access  
and Member Relations, ARM

2018 was a watershed year for ATMPs in Europe. Four new marketing authorizations for ATMPs were granted in the European Union during 2018: the first two CAR-T products (Novartis's Kymriah and Kite/Gilead's Yescarta), the first allogeneic stem cell therapy (TiGenix/Takeda's Alofisel), and the first gene therapy addressing a form of blindness (Spark's LUXTURN A).

Health Technology Assessment (HTA) bodies have taken a number of positive steps for patient access to these newly-approved ATMPs, recommending the use and reimbursement of some of the therapies. With respect to CAR-Ts, the UK's NICE has introduced new Coverage with Evidence Development (CED) that will allow unrestricted access for all English patients while NICE and NHS collect data on longer term effect of Kymriah and Yescarta. The French authority HAS has also acknowledged the significant added benefit of CAR-Ts.



Annie Hubert  
Senior Director,  
European Public Policy, ARM

The European Medicines Agency (EMA) continues to be supportive for the ATMP sector. ARM contributed to the EMA's work by providing comments on the development of scientific guidelines and via participation in Interested Party meetings. Five new PRIME designations were granted to ATMPs during 2018. The PRIME scheme aims to enhance support for the development of medicines that target an unmet medical need and is based on enhanced interaction and early dialogue with developers of promising medicines, to optimize development plans and speed up evaluation so these medicines can reach patients earlier. A total of 14 ATMPs have received PRIME designation since the inception of this scheme in 2016, making up one-third of PRIME-designated product candidates.

The European Commission/EMA plan of actions on ATMPs, intended to address bottlenecks in ATMP development, continues to be rolled out in close collaboration with national competent authorities and is being updated at regular intervals. The plan of actions addresses issues such as GMO requirements for clinical trials with gene therapies or implementation of the new GMP guide for ATMPs.

In 2018, ARM has been very active in the European market access space with initiatives aiming at improving pricing and reimbursement potential in Europe. ARM has launched a research and stakeholder engagement project assessing key challenges in accessing ATMPs in key European countries and identifying consensus solutions. The project has resulted in a set of policy recommendations that were and will be used with policymakers at EU and country level to improve access to ATMPs. A report resulting from this project will be released later this year.

ATMPs hold enormous potential value for patients, healthcare systems, and society. As we go forward, ARM will continue to engage stakeholders in Europe and globally to build a supportive infrastructure for these therapies, and to ensure patients in Europe are able to access safe and effective products.

# Commentary: State of the Industry in 2019

## Cell Therapy Commercialization in Oncology



"I think the question [investors have] is, can you manufacture? Can you manufacture consistently with autologous products? What would this look like from region to region? Are you able to control that process and, really, do you think you can commercialize a product of this nature? And I think one of our key messages was, yes, absolutely. [...]"

"Investors are becoming very savvy, very data-oriented. They're like, show me the data and let me assess the data. I also think investors are becoming savvier in that they have their own analysts, they have their own scientists on staff, and they look at your data [...] Once they're convinced, they're willing to put their resources behind your company. I don't think there's any magic to this, it's just the grind. As drug developers, we have to stay focused on developing the drugs, and once you do, it's recognized."

-- Maria Fardis, President and CEO, Iovance Biotherapeutics

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"I think the realization today is that you need to fix your manufacturing process before you enter registration studies. And what we're seeing – which should be very encouraging to everyone – is a maturation of the supply network. We're seeing really revolutionary developments that are placing us in a much better place in regard to future products. It may be one of those markets that, for this reason, there's actually some advantages to being a fast follower, because many of these things are being sorted out in the market now."

-- Christopher Vann, Chief Operating Officer, Autolus Therapeutics

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"There is a lot of excitement across the globe. We're getting requests and engagements everywhere, from all kinds of countries in the world [...] Our strategy is to be thoughtful and to take into account the task at hand. It's enormously complicated to launch in different countries, especially with products as complex as this, but you have to have a systematic plan to launch globally."

-- Joanne Beck, EVP, Global Pharmaceutical Development and Operations, Celgene Corporation



# Commentary: State of the Industry in 2019

## Gene Therapy in 2019



"I think China is going to become a bigger part of our world going forward, from a clinical trial perspective but also from a research prospective. There are some fascinating things happening there, not only the move to become more regulated, but the pace of innovation there is quite remarkable [...]"

"There are some risks obviously associated with the Chinese market in terms of the regulatory environment changing, and in not being similar to U.S. and European market historically, but that is changing pretty rapidly. I think you're going to start seeing more and more innovation coming out of China, as well as opportunities to leverage China for its clinical capacity. [...] Whether it's in 2019 or beyond, we're going to start seeing that type of internalization of gene and cell therapy and it may be at a more rapid pace than we've seen it even in the U.S. and Europe."

-- Jeffrey Walsh, Chief Financial and Strategy Officer, bluebird bio

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"The regulatory environment in this space has just been tremendous. You hear it from everyone. We've had terrific interactions with the FDA, they've been very helpful, setting guidelines on CMC for gene therapy manufacturing, giving you a road map to go ahead. It's different than it was ten years ago, back in the day [...] I think we're going to see more of that innovative perspective that [FDA Commissioner] Dr. Gottlieb has bestowed on the agency this year. In my mind, that has really changed the paradigm in 2018."

-- Arthur Tzianabos, President and CEO, Homology Medicines

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"As an industry, we often just focus on solving the challenges of research and development, and innovation is very difficult. That's always been our focus historically, but now we live in this world where we need to do not just that but also innovate on the commercial side."

"We have to show creativity and thoughtfulness, and it requires discussion with all the different stakeholders. It's incredibly important because what a shame it would be if we lived in a world where we solve the scientific and drug development challenges but patients can't get access to the products because we haven't put in the reimbursement structures or addressed the basic challenges within the medical system for a patient to access treatment."

-- Matthew Patterson, Chairman and CEO, Audentes Therapeutics; Chairman, Alliance for Regenerative Medicine

# Clinical Trials

1,028

Clinical trials underway  
worldwide by end of 2018

Ph. I: 341  
Ph. II: 595  
Ph. III: 92

## Number of Clinical Trials Utilizing Specific RM/AT Technology: 2018



### Gene Therapy

**Total: 362**

Ph. I: 120  
Ph. II: 210  
Ph. III: 32



### Gene-Modified Cell Therapy

**Total: 362**

Ph. I: 158  
Ph. II: 188  
Ph. III: 16



### Cell Therapy

**Total: 263**

Ph. I: 53  
Ph. II: 177  
Ph. III: 33

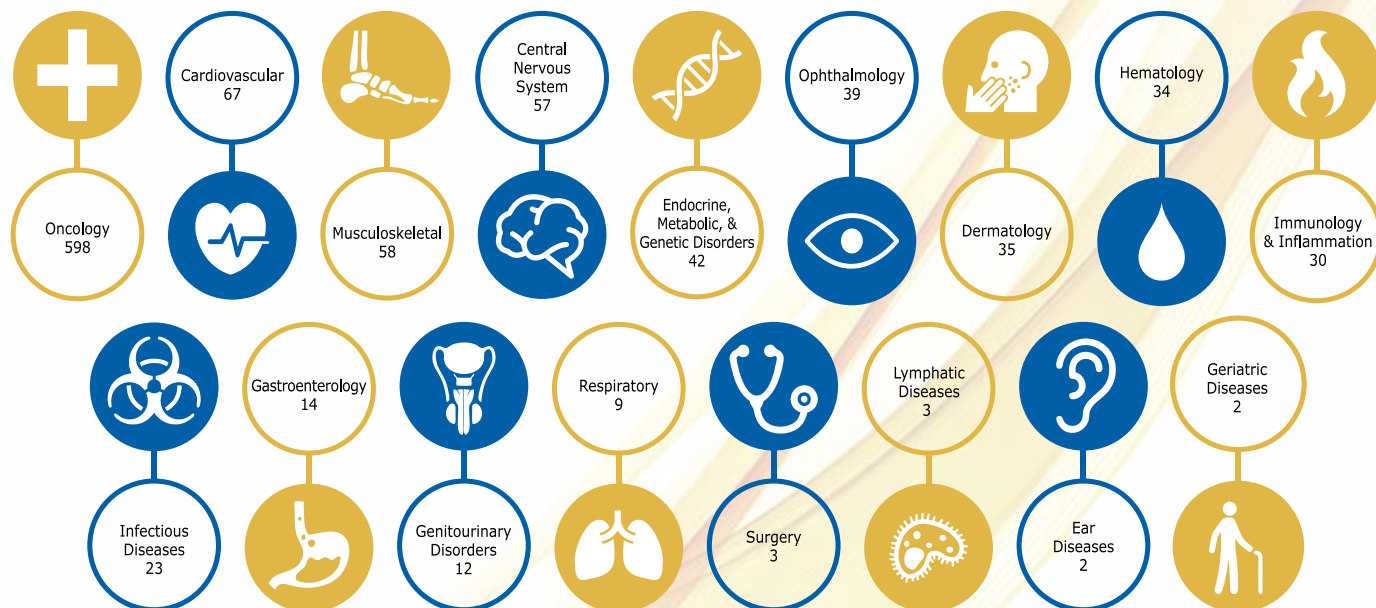


### Tissue Engineering

**Total: 41**

Ph. I: 10  
Ph. II: 20  
Ph. III: 11

## Clinical Trials by Indication: 2018



### Of 1,028 total current clinical trials worldwide:

- 58% of all current clinical trials are in oncology, including leukemia, lymphoma, and cancers of the brain, breast, bladder, cervix, colon, esophagus, ovaries, pancreas, and others.
- Nearly 6% are in cardiovascular disorders, including congestive heart failure, myocardial infarction, critical limb ischemia, heart disease, and others.

# Select Significant Clinical & Data Events: 2018

## Approvals in 2018:

- Spark Therapeutics' LUXTURN A gene therapy for biallelic RPE65-mediated inherited retinal disease received EC approval – November 23
- Avita Medical's RECELL cell therapy for serious burns received FDA approval for the treatment of severe burns – September 20
- Gilead / Kite Pharma's Yescarta cell therapy received approval from the European Commission to treat adult patients with r/r DLBCL and PMBCL – August 27
- MiMedx's Amniofix and EpiFix tissue matrix allografts received approval from the Australian TGA for wound treatment – August 9; MidMex's EpiBurn tissue matrix allograft received approval from the Australian TGA for the treatment of burns – August 9
- Novartis's Kymriah cell therapy received FDA approval for a second indication: treatment of adult patients with r/r large B-cell lymphoma – May 1; approval from the European Commission for adult patients with r/r DLBCL and patients under the age of 25 with ALL – August 27; approval from Health Canada for pediatric and young adult patients with r/r ALL and adult patients with r/r DLBCL – September 6; approval from the Australian TGA for pediatric and young adult patients with r/r ALL and adult patients with r/r DLBCL – December 18
- TiGenix's (now Takeda's) Alofisel allogeneic stem cell therapy for treatment of perianal fistulas in Crohn's disease patients received central marketing authorization from the European Commission – March 23

## U.S. FDA RMAT Designations in 2018

*\* includes only those that had publicly announced as of December 31, 2018*

- Abeona's ABO-102 (MPS IIIA) – April 23, 2018
- Abeona's EB-101 (Epidermolysis bullosa) – January 29, 2018
- Audentes's AT132 (X linked myotubular myopathy) – August 21, 2018
- AxoGen's Avance (Nerve injuries) – October 10, 2018
- Caladrius's CD34+ cell therapy (Infractory angina) – June 19, 2018
- Capricor's CAP-1002 (Duchenne muscular dystrophy) – February 5, 2018
- Cellerant's Romylocel-L (Prevention of infections during neutropenia) – July 2, 2018
- Iovance's Lifileucel (Metastatic melanoma) – November 11, 2018
- MiMedx's AmnioFix (Osteoarthritis of the knee) – March 9, 2018
- Nightstar's NSR-REP1 (Choroideremia) – June 14, 2018
- Poseida's P-BCMA-101 (Multiple myeloma) – November 5, 2018
- Rocket Pharma's RP-L102 (Fanconi Anemia) – November 7, 2018
- Voyager's VY-AADC (Parkinson's disease) – June 21, 2018



## ARM Staff:

- Janet Lambert, Chief Executive Officer – [jlambert@alliancerm.org](mailto:jlambert@alliancerm.org)
- Morrie Ruffin, Co-Founder & Senior Advisor – [mruffin@alliancerm.org](mailto:mruffin@alliancerm.org)
- Michael Werner, Co-Founder & Senior Policy Counsel – [michael.werner@hklaw.com](mailto:michael.werner@hklaw.com)
- Bethany Kraynack, Vice President, Operations – [bkraynack@alliancerm.org](mailto:bkraynack@alliancerm.org)
- Lyndsey Scull, Vice President, Communications – [lscull@alliancerm.org](mailto:lscull@alliancerm.org)
- Annie Hubert, Senior Director, European Public Policy – [ahubert@alliancerm.org](mailto:ahubert@alliancerm.org)
- Laura Parsons, Senior Director, Marketing & Events – [lp Parsons@alliancerm.org](mailto:lp Parsons@alliancerm.org)
- Perla Corredor, Director, Business Development – [pcorredor@alliancerm.org](mailto:pcorredor@alliancerm.org)
- Robert Falb, Director, U.S. Policy & Advocacy – [rfalb@alliancerm.org](mailto:rfalb@alliancerm.org)
- Chelsey Hathaway, Director, Patient Engagement & Events – [chathaway@alliancerm.org](mailto:chathaway@alliancerm.org)
- Mike Lehmicke, Director, Science and Industry Affairs – [mlehmicke@alliancerm.org](mailto:mlehmicke@alliancerm.org)
- Paolo Morgese, Director, EU Market Access & Member Relations – [pmorgese@alliancerm.org](mailto:pmorgese@alliancerm.org)
- Kaitlyn Donaldson, Coordinator, Communications – [kdonaldson@alliancerm.org](mailto:kdonaldson@alliancerm.org)
- A-P Murphy, Coordinator, Executive – [amurphy@alliancerm.org](mailto:amurphy@alliancerm.org)
- Jeanette Lazusky, Coordinator, Events – [jlazusky@alliancerm.org](mailto:jlazusky@alliancerm.org)
- Adam Roose, Advisor – [aroose@alliancerm.org](mailto:aroose@alliancerm.org)

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# Alliance *for* Regenerative Medicine

Alliance for Regenerative Medicine  
Contact: Lyndsey Scull | Vice President, Communications  
[lscull@alliancerm.org](mailto:lscull@alliancerm.org)  
[www.alliancerm.org](http://www.alliancerm.org)