



# Alliance *for* **Regenerative Medicine**



Quarterly Global Regenerative Medicine Sector 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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# Global Landscape

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



## European/Israeli Companies by Technology:

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



Gene Therapy: 440



Cell Therapy: 587



Tissue Engineering/  
Biomaterials: 125

# Industry Overview

## Q2 2019 Summary Overview

This past quarter boasted two gene therapy approvals, as the regenerative medicine sector continues to advance medicines that meet unmet needs with the potential for durable effects. The U.S. FDA approved Novartis' Zolgensma (onasemnogene abeparvovec-xioi) for spinal muscular atrophy, and the European Commission granted conditional marketing authorization to bluebird bio's Zynteglo (autologous CD34+ cells encoding  $\beta^{A-T87Q}$ -globin gene) for transfusion-dependent beta thalassemia.

Novartis and bluebird bio have each introduced outcomes-based pricing over up to a five-year timeframe for their respective products, exemplifying the movement toward alternative payment models. Legislation is currently pending that would enable such payment-over-time/outcomes models to be used in Medicaid.

Several therapeutic developers have also recently shared exciting clinical data. On the later-stage side, BioMarin disclosed preliminary Phase III data that is expected to support the U.S. and EU regulatory filings for their hemophilia A gene therapy valoctocogene roxaparvovec in the fourth quarter of this year. ReNeuron demonstrated its human retinal progenitor cell therapy led to additional improvements in visual acuity in retinitis pigmentosa patients, while Krystal Biotech's KB103 closed the majority of wounds based on a Phase II study in severe generalized recessive dystrophic epidermolysis bullosa (RDEB), and the therapy received RMAT designation. ExCellThera's ECT-001 for hematological malignancies, Fibrocell's FCX-007 for RDEB, and AlloVir's Viralm-M for hemorrhagic cystitis caused by BK also received RMAT, while Janssen's JNJ-68284528 CAR-T therapy received the EMA's PRIME designation and SanBip's SB623 received Japan's SAKIGAKE designation for the treatment of traumatic brain injury.

On the regulatory and policy front, FDA launched the Tissue Reference Group Rapid Inquiry Program, a temporary initiative that will assist product manufacturers with informal FDA assessments of how these products are regulated. Gene therapy clinical trials will now avoid duplicative oversight, as the National Institutes of Health finalized guidelines that no longer require a sponsor to register or report human gene therapy protocols to the NIH.

The gene editing space is rapidly advancing, drawing investor attention and driving dealmaking. Vertex expanded its alliance with CRISPR Therapeutics and acquired Exonics, both deals focusing on DMD, while Editas Medicine and Blue Rock Therapeutics cross-licensed their genome editing technologies. Significant financings by gene-editing start-ups included Poseida's \$142M Series C round and Homology Medicine's \$125M follow-on public offering. Ethical considerations of gene editing also continue to be a point of discussion for international regulators.

There is certainly a wealth of clinical, regulatory, and commercial milestones to look out for in the second half of 2019 as the regenerative medicine sector continues to mature.



Amanda Micklus  
Senior Consultant  
Informa Pharma Consulting  
Pharma intelligence | informa



# Global Financings

## Total Q2 2019 Global Financings



### TOTAL GLOBAL FINANCINGS

**\$2.6 Billion raised in Q2 2019**  
38% decrease YoY from Q2 2018

**\$4.8 Billion raised in H1 2019**  
40% decrease YoY from H1 2018



### GENE & GENE-MODIFIED CELL THERAPY

**\$2.2 Billion raised in Q2 2019**  
18% decrease YoY from Q2 2018

**\$4.3 Billion raised in H1 2019**  
26% decrease YoY from H1 2018



### CELL THERAPY

**\$691 Million raised in Q2 2019**  
69% decrease YOY from Q2 2018

**\$1.5 Billion raised in H1 2019**  
64% decrease YoY from H1 2018



### TISSUE ENGINEERING

**\$53 Million raised in Q2 2019**  
88% decrease YOY from Q2 2018

**\$67 Million raised in H1 2019**  
91% decrease YoY from H1 2018

\*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.

## Examples of Key Financings in Q2 2019:

#### Public Offerings:

- Vertex and CRISPR Therapeutics enter into \$175M upfront partnership to develop gene-edited therapies for Duchenne muscular dystrophy and myotonic dystrophy type 1 – June 6
- Asklepios BioPharmaceutical secures \$235M in private equity – April 11
- Poseida Therapeutics secures \$142M in Series C financing – April 22
- AlloVir raises \$120M in Series B financing – May 22
- Talaris Therapeutics secures \$100M in Series A financing – April 18
- Encoded Therapeutics raises \$104M in Series C financing – June 26
- Oxford Biomedica raises \$68M in private placement – May 28

#### Corporate Partnerships/ Collaborations:

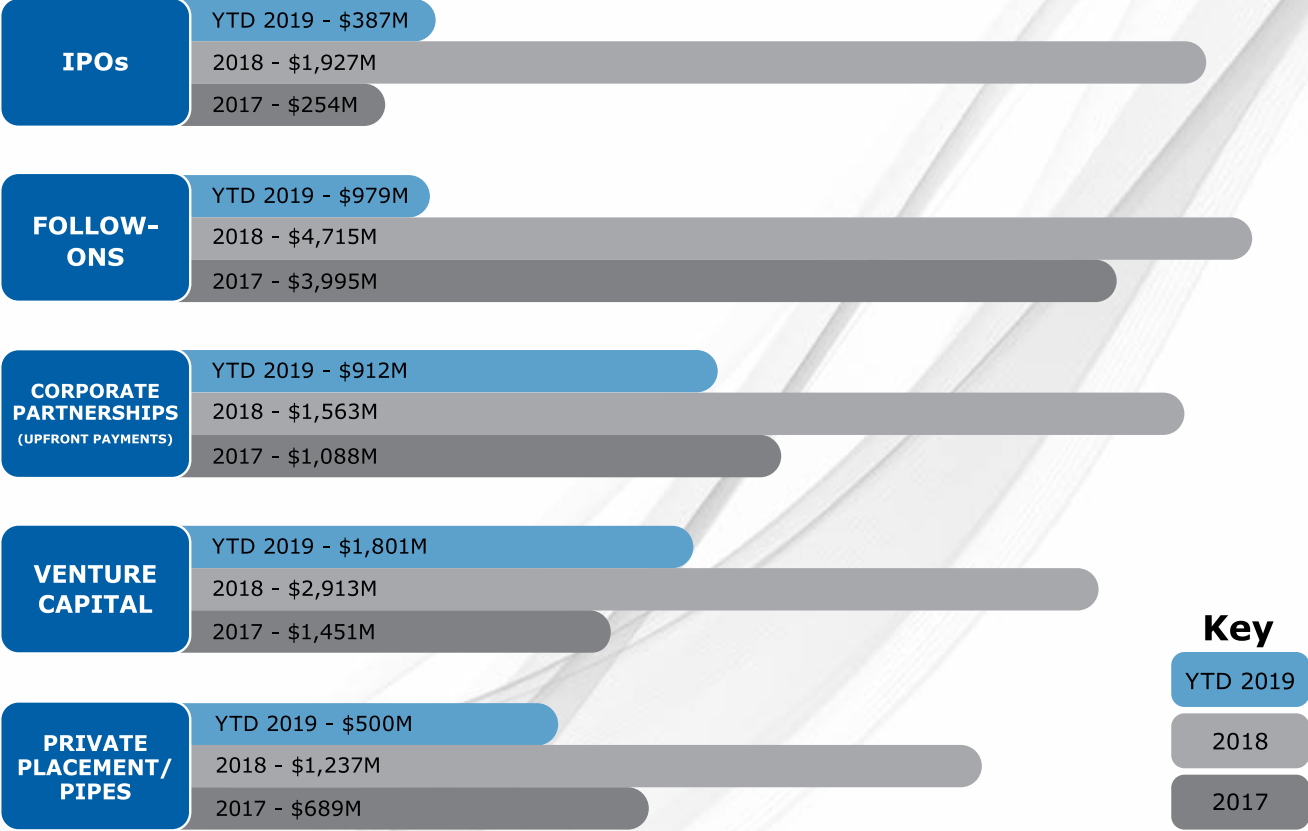
- Sangamo Therapeutics raises \$145M in follow-on public offering – April 8
- Precision Bio raises \$145M in IPO – April 1
- Homology Medicines raises \$144M in follow-on public offering – April 12
- Orchard Therapeutics raises \$128M in follow-on public offering – June 3
- Prevail Therapeutics raises \$125M in IPO – June 24
- Autolus Therapeutics raises \$115.9M in follow-on public offering – April 15
- Krystal Biotech raises \$115M in follow-on public offering – June 24

#### Mergers & Acquisitions:

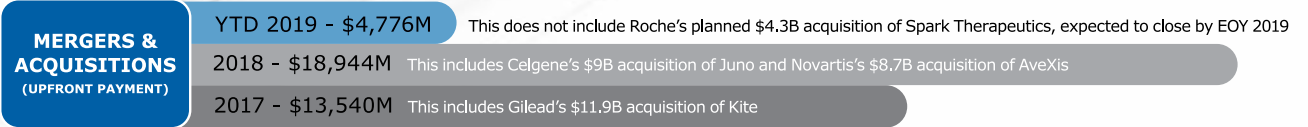
- Thermo Fisher acquires Brammer Bio for \$1.7B upfront – May 1
- Catalent acquires Paragon Bioservices for \$1.2B upfront – May 20
- Biogen acquires Nightstar Therapeutics for \$877M upfront – June 7
- Smith & Nephew acquires Osiris Therapeutics for \$661M upfront – April 17

# Global Financings

## Total Global Financings by Type, by Year



## Mergers & Acquisitions: Upfront Payments



# Clinical Trials

**1,069**

Clinical Trials Underway  
Worldwide by End of Q2 2019

**Ph. I: 358**  
**Ph. II: 617**  
**Ph. III: 94**

## Number of Clinical Trials Utilizing Specific RM/AT Technology: Q2 2019



### Gene Therapy

**Total: 366**

Ph. I: 117  
Ph. II: 219  
Ph. III: 30



### Gene-Modified Cell Therapy

**Total: 410**

Ph. I: 187  
Ph. II: 207  
Ph. III: 16



### Cell Therapy

**Total: 249**

Ph. I: 49  
Ph. II: 168  
Ph. III: 32



### Tissue Engineering

**Total: 44**

Ph. I: 5  
Ph. II: 23  
Ph. III: 16



# Clinical Trials

## Clinical Trials by Indication: Q2 2019



- 643 (60%) 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.
- 60 (6%) are in cardiovascular disorders, including congestive heart failure, myocardial infarction, critical limb ischemia, heart disease, and others.
- 57 (5%) are in diseases of the central nervous system, including multiple sclerosis, Alzheimer's disease, Parkinson's disease, traumatic brain injury, ALS, and others.

# Select Significant Clinical & Data Events: Q2 2019

## Gene Therapy & Genome Editing Programs

- Krystal Bio received RMAT Designation for its KB-103 gene therapy for the treatment of recessive dystrophic epidermolysis bullosa – June 24, 2019
- Abeona received Fast Track Designation for its ABO-202 gene therapy for the treatment of infantile Batten (CLN1) disease – June 18, 2019
- bluebird bio received EU conditional marketing authorization for its Zynteglo gene therapy for the treatment of transfusion-dependent beta-thalassemia without  $\beta^0/\beta^0$  genotype – June 3, 2019
- Fibrocell received RMAT designation for its FCX-007 gene therapy for the treatment of recessive dystrophic epidermolysis bullosa (RDEB) – May 29, 2019
- BioMarin announced positive data from its Phase III clinical trial of its valoctocogene roxaparvovec gene therapy for the treatment of hemophilia A – May 28, 2019
  - At week 26, the estimated median Annual Bleed Rate (ABR) was zero and the estimated mean ABR was 1.5, representing a reduction of 85% from baseline levels where all patients were on standard of care prophylaxis. Seven of 16 study participants reached or exceeded the pre-specified Factor VIII levels of 40 international units per deciliter using the chromogenic substrate assay.
- AveXis, a Novartis company, received FDA approval of its gene therapy Zolgensma for the treatment of spinal muscular atrophy (SMA) type 1 – May 24, 2019
- GenSight Biologics reported positive 96-week follow-up data from its Phase III trial of GS010 for the treatment of Leber hereditary optic neuropathy (LHON) – May 15, 2019
  - The results point to continued efficacy of GS010 two years past injection, with best-corrected visual acuity (BCVA) sustaining a clinically meaningful improvement over baseline. At week 96, GS010-treated eyes showed a mean improvement of -0.308 LogMAR compared to baseline, equivalent to +15.4 ETDRS letters or three lines on the ETDRS vision chart.
- Homology Medicines received Fast Track designation for its HMI-102 gene therapy for the treatment of phenylketonuria (PKU) – May 14, 2019
- MeiraGTx Announced positive six month data from their Phase I/II trial of their gene therapy AAV-RPE65 for RPE65 deficiency – May 14, 2019
  - Significant improvement in vision was demonstrated at six months after AAV-RPE65 treatment, as measured by assessments of vision-guided mobility, retinal sensitivity, visual acuity and contrast sensitivity.
- CRISPR Therapeutics and Vertex received Fast Track Designation for their CTX001 gene editing product to treat beta thalassemia – April 17, 2019
- AveXis announced positive interim data from its Phase III clinical trial of Zolgensma for the treatment of spinal muscular atrophy (SMA) type 1 – April 16, 2019
  - The data showed prolonged event-free survival, an early and rapid increase in CHOP-INTEND scores and significant milestone achievement compared to untreated natural history.
- uniQure received Fast Track Designation for its AMT-130 gene therapy for the treatment of Huntington's disease – April 8, 2019
- Abeona Therapeutics received Fast Track Designation for its ABO-101 gene therapy for the treatment of Sanfilippo syndrome type B – April 3, 2019
- Ziopharm Oncology received Fast Track Designation for Ad-RTS-hIL-12 plus Veledimex for the treatment of recurrent glioblastoma – April 1, 2019

# Select Significant Clinical & Data Events: Q2 2019

## Cell-Based Immuno-Oncology Programs

- Torque Therapeutics received FDA Fast Track Designation for its T cell immunotherapy TRQ-1501 for the treatment of relapsed or refractory solid tumors or lymphomas expressing five tumor-associated antigens (PRAME, WT-1, SSX2, Survivin, and NY-ESO-1) – June 18, 2019
- Poseida Therapeutics received FDA Orphan Drug Designation for its B-BCMA-101 CAR-T therapy for the treatment of multiple myeloma – May 13, 2019
- Autolus Therapeutics received FDA Orphan Drug Designation for its AUTO3 CAR-T cell therapy for the treatment of acute lymphoblastic leukemia (ALL) – April 23, 2019
- The University of Pennsylvania confirmed that they had dosed the first two U.S. patients to be treated with a CRISPR-based gene editing therapy as a part of a trial of a gene-edited cell-based therapy to treat cancer – April 16, 2019
- Janssen received PRIME designation for its CAR-T therapy JNJ-68284528 for the treatment of multiple myeloma – April 4, 2019

## Cell Therapy Programs

- Mesoblast Ltd. received FDA Orphan Drug Designation for its cell therapy Revascor for the prevention of gastrointestinal bleeding in patients with left ventricular assist devices – June 24, 2019
- Orgenesis received FDA Orphan Drug Designation for its autologous insulin-producing cell therapy product for the treatment of severe hypoglycemia-prone diabetes resulting from total pancreatectomy – June 17, 2019
- AlloVir received RMAT designation for its Viraly-m cell therapy for the treatment of cystitis caused by BK virus in adults and children following allogeneic HSCT – June 11, 2019
- Mesoblast Ltd. initiated a rolling BLA submission to the FDA for its remestemcel-L cell therapy for the treatment of acute graft-versus-host disease – May 30, 2019
- Athersys received Fast Track Designation for its MultiStem cell therapy for acute respiratory distress syndrome – May 14, 2019
- ExCellThera's ECT-001 cell therapy received RMAT designation for use in novel curative cord blood transplant therapies for patients with blood cancers – April 23, 2019
- SanBio received SAKIGAKE designation for its SB623 cell therapy for the treatment of traumatic brain injury – April 8, 2019

## Tissue Engineering Programs

- Enzyvant Therapeutics announced FDA acceptance of its BLA and priority review status for its RVT-802 tissue engineered product for the treatment of pediatric congenital athymia – June 5, 2019

# Spotlight: Gene Therapy for Rare Diseases

## Spotlight: Gene Therapy for Rare Disorders – Outlook for the Sector in the Age of Zolgensma and Zynteglo

In Q2 2019, the gene therapy sector experienced two major approvals: AveXis / Novartis's Zolgensma (onasemnogene abeparvovec-xioi), an *in vivo* AAV gene therapy for the treatment of spinal muscular atrophy (SMA) in infants under the age of two, and bluebird bio's Zynteglo (autologous CD34+ cells encoding  $\beta^{A-T87Q}$ -globin gene), an *ex vivo* lentiviral gene therapy for the treatment of transfusion-dependent beta thalassemia (TDT).

Each of these therapies is poised to provide a substantial positive impact to thousands of patients. Prior to 2016, there was no approved treatment for spinal muscular atrophy; over 90% of infants born with SMA Type 1, the most serious form of the disease, would die or need permanent breathing support before the age of two. Treatment with Spinraza, approved in 2016, requires invasive regular injections to the spinal cord and can cost millions of dollars over the lifetime of the patient.

TDT can cause life-threatening anemia if it goes untreated. Regular chronic blood transfusions, resulting in iron buildup in the heart and other organs, can contribute to serious medical complications in many patients. In both of these cases, gene therapy represents the potential to provide a significant improvement in the standard of care.

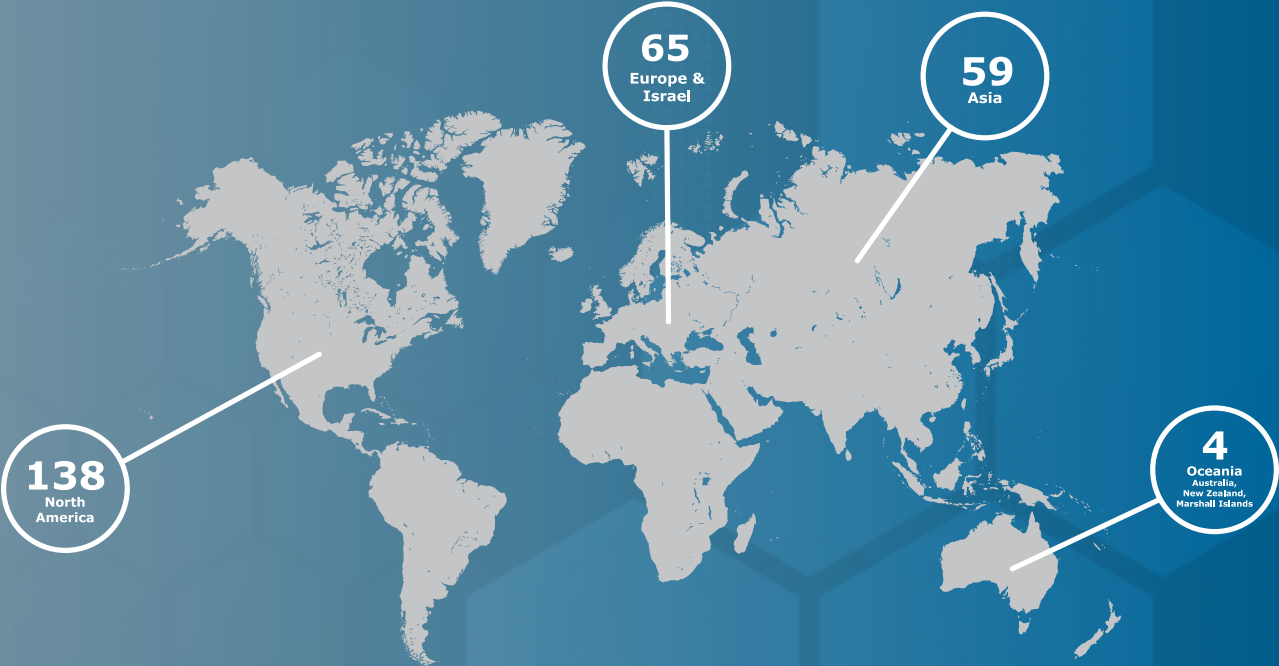
The approval of these therapies, which have a high upfront cost but represent an incredible value to patients and healthcare systems over the course of a patient's lifetime, has spurred a conversation regarding patient access. Current reimbursement systems are typically designed to provide chronic care, whereas gene therapies and other regenerative medicines have the potential to provide a durable and perhaps curative therapeutic effect following a single administration.

Both AveXis, a Novartis company, and bluebird bio are engaging with payers and other stakeholders to develop and implement innovative financing models better suited to these complex therapies. AveXis is currently working with payers to create five-year outcomes-based arrangements and pay-over-time options. bluebird bio has announced that they plan to offer five-year payment plans for Zynteglo as well, with payments benchmarked against positive health outcomes. These types of arrangements help to offset the perceived risk of these novel therapeutics and amortize the cost in accordance with their long-term value.

As the number of approved gene therapies grows, it imperative that stakeholders continue to convene to identify strategies to ensure patients can access these lifechanging therapies.

# Spotlight: Gene Therapy for Rare Diseases

There are currently 266 gene therapy and gene-modified cell therapy developers active in rare disease.



Total global financings for gene therapy and gene-modified cell therapy developers active in rare disease

H1 2019: \$3.1B  
2018: \$8.8B  
2017: \$6.0B

There are 562 gene therapy and gene-modified cell therapy trials currently in the rare disease space.

| By Phase:   | By Tech Type:                   |
|-------------|---------------------------------|
| Ph. I: 212  | Gene-modified cell therapy: 321 |
| Ph. II: 320 | Gene delivery: 196              |
| Ph. III: 30 | Oncolytic virus: 22             |
|             | Gene editing: 21                |
|             | Other: 2                        |

79% of gene therapy clinical trials in rare disease are for rare cancers.  
6% are in inherited blood disorders.  
5% are in endocrine, metabolic, & genetic disorders.



# Spotlight: Gene Therapy for Rare Diseases

Spinal muscular atrophy (SMA) is a rare genetic disease caused by a mutation in the survival motor neuron 1 (SMN1) gene. SMA affects one in every 10,000 babies born each year and is the leading cause of genetic infant death in the U.S. Friday, May 24, 2019 marked a historic milestone for the SMA community when the U.S. FDA approved AveXis' transformative gene therapy, Zolgensma® (onasemnogene abeparvovec-xioi), for children less than two years of age with SMA. Zolgensma delivers a healthy copy of the SMN1 gene, targeting the underlying cause of SMA and halting disease progression.

Nearly two months later, we are highly encouraged by the response of the health care community, including physicians, parents of children with SMA, payers, and hospitals. We're pleased to share that our first patient was dosed just two weeks post-approval, and multiple patients across different SMA types, weights, and ages (up to two years old) have now been treated across several hospital and payer types.

We are proud to bring this one-time gene therapy to pediatric patients with SMA and remain committed to advancing the science behind Zolgensma to transform SMA, as well as other rare genetic diseases. We are anticipating regulatory decisions in Europe and Japan in the second half of the year for Zolgensma (AVXS-101), and we have plans to develop other novel treatments for rare neurological diseases, including Rett syndrome and a genetic form of amyotrophic lateral sclerosis caused by mutations in the superoxide dismutase 1 (SOD1) gene. For additional information, including prescribing information and boxed warning, please visit [www.zolgensma.com](http://www.zolgensma.com) or [www.avexis.com](http://www.avexis.com).



Dave Lennon  
President  
AveXis

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In June, the European Commission granted conditional marketing approval of LentiGlobin® for transfusion dependent beta thalassemia (TDT), the first gene therapy for TDT, and the first approval for a product based on the technology of lentiviral vector transduced hematopoietic stem cells. This milestone represents the dedication and commitment of scientists, clinical investigators, healthcare providers, patients and their families, and our employees, who all helped to advance this treatment from concept to approval.

Like many rare genetic diseases, the impact of TDT on the lives of patients and their families is often unappreciated beyond those living with the disease or caring for those affected.



# Spotlight: Gene Therapy for Rare Diseases

TDT is a severe, and potentially lethal disease caused by mutations in the  $\beta$ -globin gene that result in reduced or absent hemoglobin. In order to survive, people living with TDT depend on frequent, lifelong blood transfusions. These transfusions carry the risk of progressive multi-organ damage due to unavoidable iron overload.

Marketed as ZYNTEGLO® (autologous CD34+ cells encoding  $\beta^{A-T87Q}$ -globin gene)\* in the EU, LentiGlobin for TDT is a one-time gene therapy that addresses the underlying genetic cause of TDT and offers patients 12 years and older who do not have a  $\beta^0/\beta^0$  genotype the potential to become transfusion independent, which, once achieved, is expected to be lifelong – an outcome that was previously possible only with allogeneic hematopoietic stem cell (HSC) transplantation.

In clinical studies adverse events were generally consistent with the known side effects of HSC collection and bone marrow ablation with busulfan. One serious adverse event (SAE) of thrombocytopenia was considered possibly related to ZYNTEGLO.

This approval represents another significant milestone in the rapidly advancing field of gene therapy, which has moved beyond an investigational approach to emerge as an expanding pillar of modern medicine with recent approvals across numerous cell and vector platforms designed to treat a range of devastating diseases. The growing body of data and clinical experience serve as a foundation to extend existing gene therapy platforms to address new diseases, and to catalyze the emergence of new technologies with the potential to bring transformational therapies to even more patients.

bluebird bio is committed to continuing our pioneering work to bring new gene therapies that deliver value to patients, caregivers, and the health care system. We are proud of our contributions to advance the science of gene therapy, and the potential of what is possible for patients.



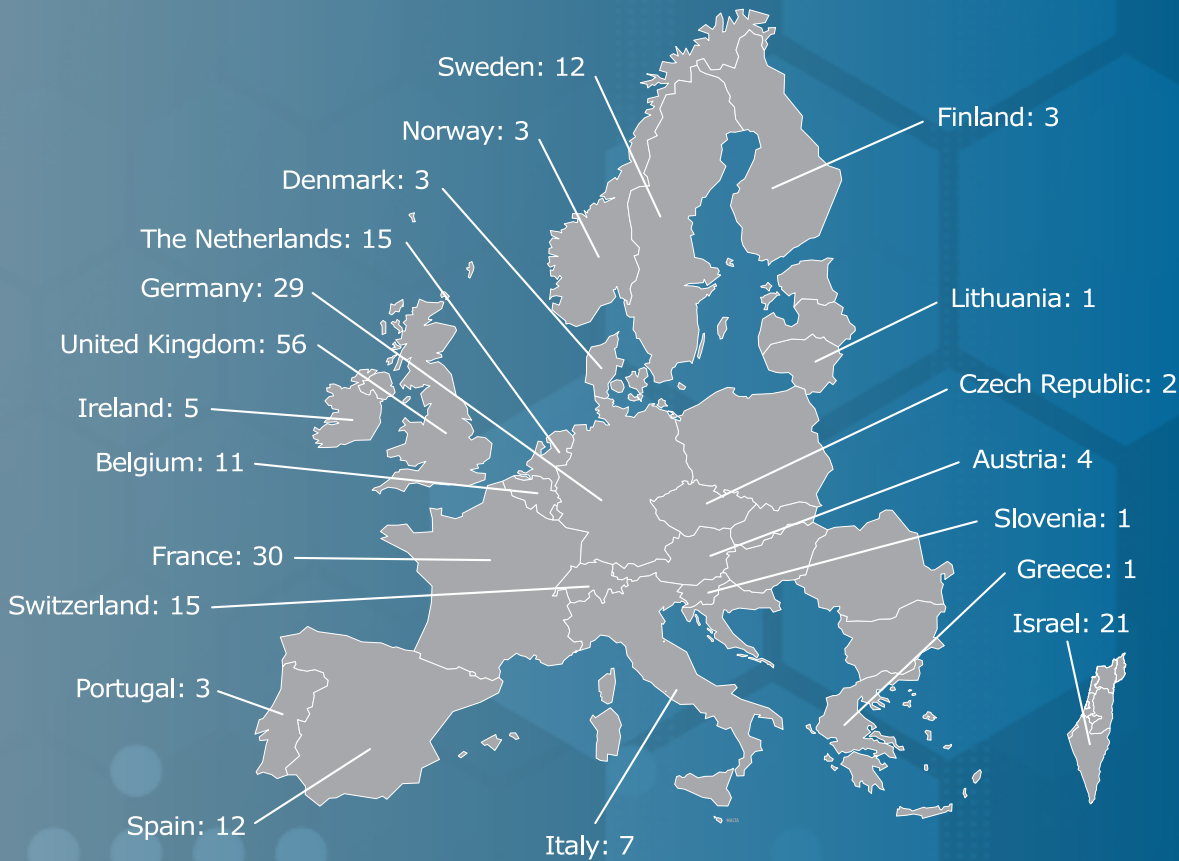
David Davidson  
Chief Medical Officer  
bluebird bio

\*LentiGlobin for TDT received conditional approval in the EU, it is not approved in other markets.

# European Sector Overview

## European Sector Landscape

**234+** 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: 107**



**Cell Therapy: 139**



**Tissue Engineering/  
Biomaterials: 37**

# European Sector Overview



## H1 2019 EUROPEAN/ ISRAELI FINANCINGS

**\$1.3 Billion**  
(appx €1.2 Billion) raised in H1 2019  
across all technology types  
17% YoY increase from H1 2018



## GENE & GENE-MODIFIED CELL THERAPY

**\$1.1 Billion**  
(appx €1.0 Billion) raised in H1 2019  
9% YoY increase from H1 2018



## CELL THERAPY

**\$631 Million**  
(appx €566 Million) raised in H1 2019  
13% YoY increase from H1 2018



## TISSUE ENGINEERING

**\$35 Million**  
(appx €31 Million) raised in H1 2019  
9% YoY decrease from H1 2018

\*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.

# 266

Clinical Trials Underway  
by End of H1 2019

**Ph. I: 52**  
**Ph. II: 172**  
**Ph. III: 42**

- Half (50%) 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.
- 8% focus on endocrine, metabolic, and genetic disorders, including mucopolysaccharidosis types I, II, IIIA, and IIIB, Crigler Najjar syndrome, X-linked myotubular myopathy, and others.

# Commentary: The European ATMP Landscape

*Commentary from ARM's Meeting on the Mediterranean, held April 23-24, 2019*



## **The Pricing and Reimbursement Landscape: Opportunities and Challenges**

Luca Alberici, Ph.D., Chief Business Officer, MolMed:

"In every single country, you basically have two steps: one is the value definition and the second is price negotiation. I think as a matter of fact in most countries the value definition can be applied to these kinds of therapies. They will all have different models, they will generally require different data, we may be on different timelines by which they can calculate the value creation, but to a certain extent, the power of these kinds of therapies is such that defining the value is generally the easier discussion to have.

"The more problematic discussion is then how to define the cost that reflects a one-and-done treatment rather than chronic maintenance. That's where every single country has to work on their own scheme, their own budget."

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Etienne Jousseume, Head of Market Access, Cell and Gene Europe, Novartis:

"The right pricing model is a model that enables patients to have access, and that's all. So then, what does that mean for payers? We need to collaborate with them to understand what is the challenge on the price tag, on the cost of the therapy. Is it a budget impact issue, is it an issue with uncertainty, do they trust the long-term outcomes we promise? [...] In each of these cases we can propose something to address these challenges. So, if it's a matter of budget impact, we can discuss price-volume agreements. [...] If their main challenge is about long-term effects then we can go into conditional pricing depending on long-term outcomes from the pivotal trials. So, we really need to understand what is the payer's concern and then build the model with them."

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Jason Meyenburg, Chief Commercial Officer, Orchard Therapeutics:

"I think the strongest advice we would give ourselves is to be ready for feedback and embrace it. The therapy will be the same from one country to another, but whether it's in the structure of the country's budget or even a payer within the country, their own budget, or how one hospital may navigate administering the therapy differently from another – we have to be ready for the feedback, and understand how to make this precision medicine confidence-inspiring for these institutions. Hospitals, payers, physicians – [we need to] listen to how they describe their own needs and do our best to actually meet their needs."

# Commentary: The European ATMP Landscape



## Commercial Strategies From Pathfinders in the Industry

Pascal Touchon, SVP and Global Head, Cell and Gene, Novartis Oncology:

"The key lessons in terms of the most positive one has been around an anticipation and collaboration mindset. We started discussing with HTAs [Health Technology Assessment bodies] and value and pricing authorities very early on and we were pleased how welcoming these authorities were to discussing how this type of therapy could challenge their system and create new opportunities for patients. [...] We didn't find any payers or health authorities that weren't ready to discuss and find solutions for patients. We didn't see any countries that said, no, it's not the right time, let's come back in a few years. Everybody wanted to engage, wanted to find solutions adapted to each system.

"One of the challenges we all face is that one size doesn't fit all because there are so many different systems. Even in Europe, every system is different. And today, just nine months since the opening [of Kymriah], we've already obtained a type of access reimbursement for one or both indications in about 12 countries in Europe, and I think that is a great achievement."

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Claire Foreman, Head of Acute Programs, National Health Service:

"There were two key things that felt different from how we'd normally go about preparing for the introduction of an approved drug. The first was that we had to not treat it like a drug, we had to effectively treat it as if we were planning a service. When we plan a service, as the responsible commissioner in England for this list of a 150 or so services, it takes a lot of work and a lot of lead-in time. We had to work well in advance, we probably started the planning more than a year in advance of when we thought these medicines were going to be hitting the market. We worked with all of the stakeholders to ensure we understood all of their perspectives, understand in absolute minute detail the end-to-end pathway, including how the new pathway would differ from existing pathways for clinical services [...]

"The second thing that we did which was a bit different was that we had to really flex our timelines and our usual processes in order to ensure that access for patients was delivered as close to authorization and approval as possible. In England, the arrangements are that NICE undertakes the technology appraisals when a drug is approved, then there's anything up to 90 days for the whole system to prepare for that implementation. That would be really intolerable in the case of the CAR-T experience we had, so it was really necessary that we understood early on, and began to plan early on together, how we would be ready from day zero."



# Current Regulatory & Legislative Priorities

## 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 access and reimbursement for RM / AT products.

## Industrialization and Manufacturing

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



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# Alliance *for* **Regenerative Medicine**

Alliance for Regenerative Medicine

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