

ARM EU Advanced Therapies Investor Day

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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.

ARM Strategic Priorities



- *Advocate for clear, predictable and harmonized regulatory and review pathways*
- *Enable market access and value-based, favorable reimbursement policies*
- *Facilitate sustainable access to capital and identify sources of potential public funding*
- *Address industrialization and manufacturing hurdles*
- *Conduct key stakeholder outreach, communication and education*

Regenerative Medicine Technologies



Regenerative medicine translates fundamental knowledge in biology, chemistry, and physics into materials, devices and systems that augment, repairs, replace, or regenerative organs and tissues.

Gene therapy seeks to modify, replace, inactivate or introduce genes into a patient's body with the goal of durably treating, preventing or even curing disease. These techniques can also be used to genetically modify a patient's cells outside of the body, which are then re-introduced to deliver modified or corrected cells into the body, an approach known as **gene-modified cell therapy**.

Genome editing is a practice in which DNA is inserted, replaced or removed using "molecular scissors," or artificially engineered gene constructs, in order to potentially cure genetic diseases and/or disorders.

Cell therapy is the administration of viable, non-genetically modified cells into a patient's body to grow, replace or repair damaged tissue for the treatment of a disease.

Tissue engineering combines scaffolds, cells and biologically active molecules into functional tissues to restore, maintain or improve damaged tissues.

Data provided by: **informa**

Global Sector Landscape: 2017

843+

Regenerative Medicine Companies Worldwide,
Including Gene and Cell Therapies

450
North
America

234
Europe
& Israel

122
Asia

15
South
America

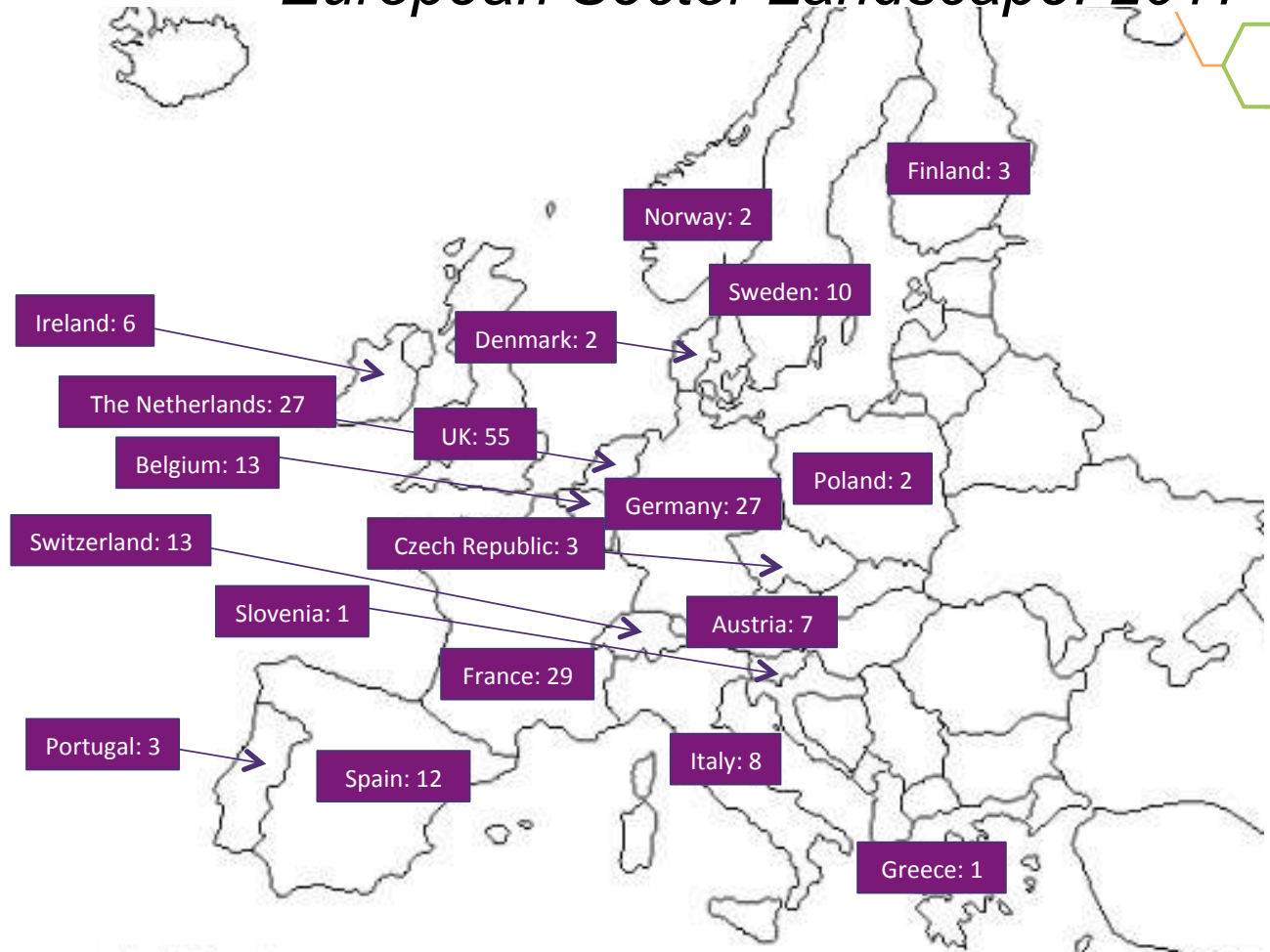
1
Africa

21
Oceania
(Australia, New Zealand,
Marshall Islands)

Data provided by: **informa**

European Sector Landscape: 2017

Total: 234



Not shown on map,
included in Europe totals:

Israel: 25

Global Financings by Technology



Total Global Financings:
\$1.85B raised in Q3 2017
\$6.12B raised YTD 2017
\$5.15B raised 2016

102% increase
from Q3 2016



Gene & Gene-Modified Cell Therapy:
\$1.62B raised in Q3 2017
\$3.68B raised YTD 2017
\$2.71B raised 2016



Tissue Engineering:
\$17M raised Q3 2017
\$313.3M raised YTD 2017
\$425.4M raised 2016



Cell Therapy:
\$1.08B raised Q3 2017
\$3.8B raised YTD 2017
\$2.9B raised 2016

*As of end Q3 2017 – these do not reflect Gilead's \$11.9B acquisition of Kite Pharma, which closed in Q4 2017

Data provided by: **informa**

Examples of Key Acquisitions, Partnerships & Financings: 2017



- *Gilead completes \$11.9B cash acquisition of Kite Pharma – October 3, 2017*
- *Sanpower Group completes Dendreon acquisition from Valeant for \$819.9M – June 29, 2017*
- *Sangamo Therapeutics signs \$545M hemophilia A gene therapy collaboration with Pfizer, \$70M upfront – May 10, 2017*
- *bluebird bio raises \$460M in public offering of common stock – July 30, 2017*
- *CSL Behring acquires Calimmune for \$416M, \$91M upfront – August 28, 2017*
- *Kite Pharma raises \$409.7M in public offering of common stock – March 18, 2017*
- *Spark Therapeutics raises \$402.5M in public offering of common stock – August 9, 2017*
- *AveXis raises \$269.8M in public offering of common stock – June 26, 2017*
- ***Oxford BioMedica signs \$100M CAR T-cell agreement with Novartis, \$10M upfront – July 6, 2017***
- ***Takeda signs \$100M agreement w/ GammaDelta Therapeutics to develop novel T cell platform – May 9, 2017***
- *Rubius Therapeutics raises \$120M in private financing – June 21, 2017*
- *Tocagen raises \$97.8M in initial public offering – April 19, 2017*
- ***Celyad signs \$96M agreement with Novartis for allogeneic TCR-deficient CAR T-cell patents – May 2, 2017***
- *Hitachi Chemical acquires the remaining 80.1% stake in PCT from Caladrius Biosciences for \$80M – May 19, 2017*
- ***Cell Medica raises \$73M in Series C financing – March 16, 2017***
- ***Adaptimmune Therapeutics exercises \$62.6M option agreement with GSK – Sept 7, 2017***

Examples of Key EU Public ATMP Companies

Company	Country	Focus Areas	Market Cap (as of 3 Nov 2017)
Adaptimmune	UK	TCR cancer immunotherapy products	\$764M
Bone Therapeutics	Belgium	Bone fracture repair and prevention	€68M
Collectis	France	CAR T-cell cancer immunotherapies	€843M
Celyad	Belgium	Cardiology, oncology	€487.8M
CombiGene	Sweden	Epilepsy; neurological disorders	41.3M SEK
CRISPR Tx	Switzerland	Hematopoietic, liver, DMD, CF	\$773M
GenSight	France	Neurodegenerative diseases of the eye and central nervous system	€124.78M
Kiadis	Netherlands	Leukemia, beta-thal, GvHD, cancer relapse, limited donor availability	€136.2M
MediGene	Germany	T-cell based immunotherapies	€280.8M
MolMed	Italy	Oncology, including blood cancers and solid tumors	€217.1M
Oxford BioMedica	UK	Ophthalmology, central nervous system disorders and oncology	£276.4m
ReNeuron	UK	Stroke disability, critical limb ischaemia, retinitis pigmentosa	£62.5m
TiGenix	Belgium	Perianal fistulas in Crohn's disease patients; AMI; rheumatoid arthritis	€262.7M
TxCell	France	Autoimmune diseases (both T-cell and B-cell-mediated)	€31.7M
uniQure	Netherlands	CNS, liver / metabolic and cardiovascular diseases	\$490.9M

934 Total Clinical Trials Worldwide

307
Phase I



548
Phase II

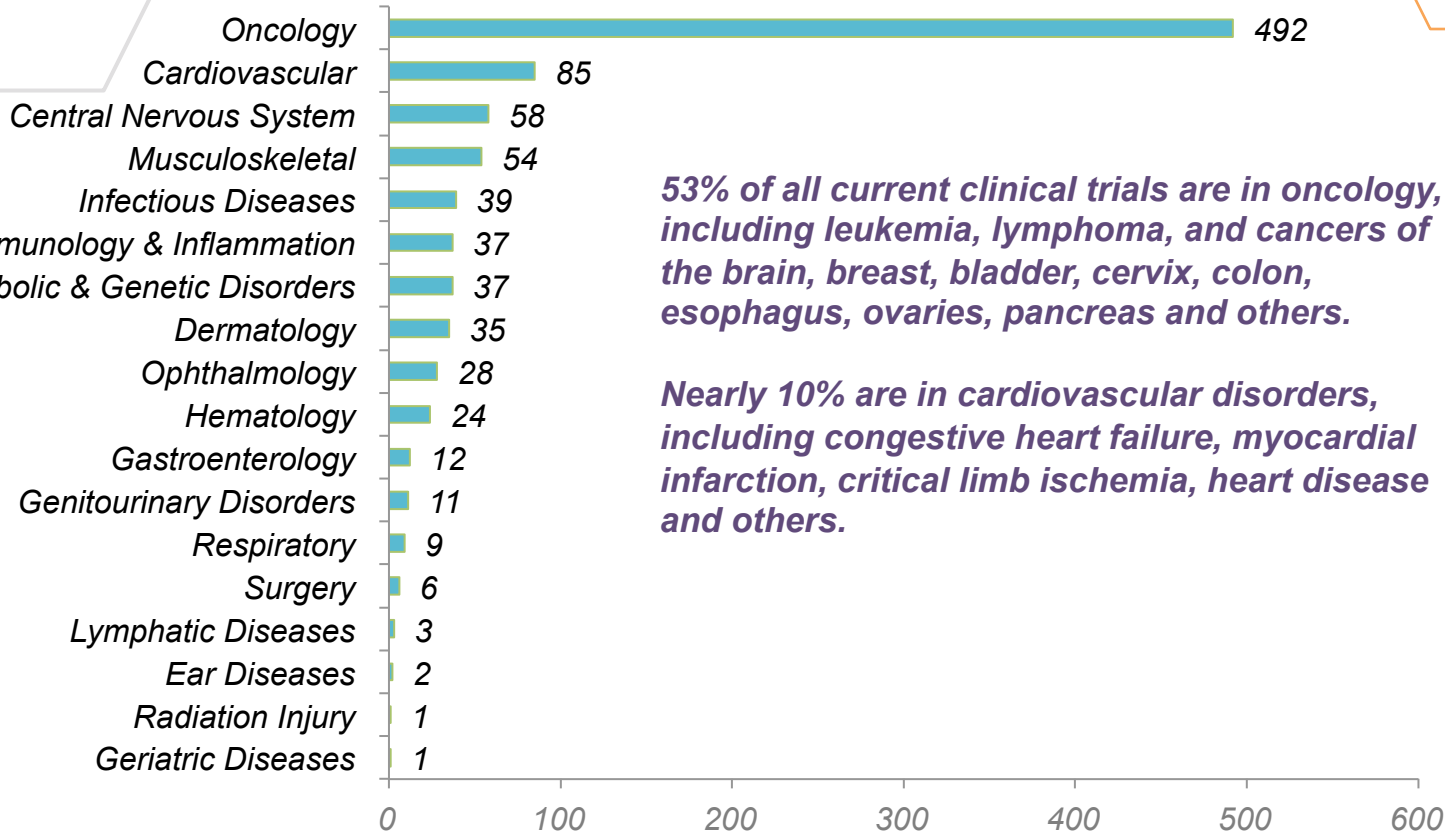


79
Phase III



*As of end Q3 2017
Data provided by: **informa**

Clinical Trials by Therapeutic Category



53% 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 10% are in cardiovascular disorders, including congestive heart failure, myocardial infarction, critical limb ischemia, heart disease and others.

*As of end Q3 2017

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Examples of Key Clinical Milestones & Data Events



Approval Updates:

- **Novartis submits MAA to EMA for Kymriah** for treatment of children and young adults with relapsed or refractory B-cell acute lymphoblastic leukemia and for adults with r/r diffuse large B-cell – Nov 6, 2017
- **Kite's Yescarta CAR T-cell therapy approved by U.S. FDA** for treatment of adult patients with relapsed/refractory large B-cell lymphoma after two or more lines of systemic therapy – Oct 18, 2017
- **U.S. FDA Advisory Committee unanimously recommends approval of Spark Therapeutics' LUXTURNA** for biallelic RPE65-mediated inherited retinal disease – Oct 12, 2017
- **Novartis receives first-ever FDA approval for CAR-T cell therapy, Kymriah**, for children and young adults with refractory/relapsed B-cell acute lymphoblastic leukemia – Aug 30, 2017

U.S. FDA RMAT Designation:

- Thus far, has been awarded to: Humacyte's Humacyl; Enzyvant's RVT-802; jCyte's jCell; Vericel's ixmyelocel; Mallinckrodt's Stratagraft; Kiadis's ATIR101; Asterias's AST-OPC1; Athersys's MultiStem; Cellvation's CEVAL101

Recent European Company Data Events:

- **TiGenix granted Orphan Drug Designation from the U.S. FDA for Cx601** – Oct 23, 2017
- **U.S. FDA grants Pluristem Orphan Drug Designation for PLX-R18 cell therapy for acute radiation syndrome** – Oct 19, 2017
- **uniQure announces U.S. FDA Orphan Drug Designation for AMT-130 in Huntington's Disease** – Oct 6, 2017
- **Orchard Therapeutics announces OTL-101 receives Promising Innovative Medicine designation** – Aug 22, 2017



ALLIANCE for
Regenerative Medicine