

# Business of Regenerative Medicine

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## Sector Overview

**Janet Lambert, CEO**

*July 15, 2019*



# About ARM

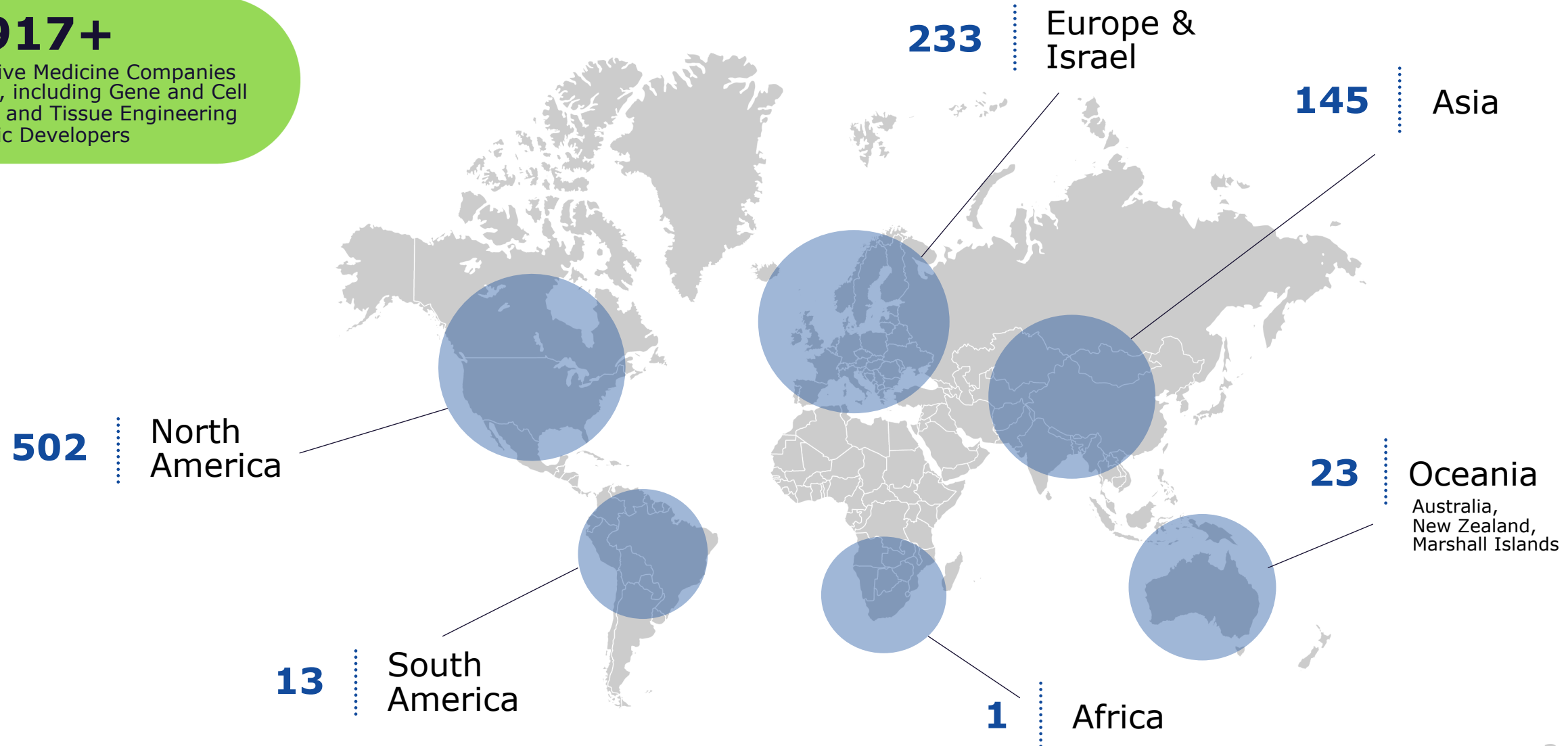
- **International advocacy organization**
  - dedicated to realizing the promise of safe and effective regenerative medicines for patients around the world
- **330+ members**
  - Small and large companies, non-profit research institutions, patient organizations, and other sector stakeholders
- **Priorities:**
  - Clear, predictable, and harmonized **regulatory** pathways
  - Enabling market access and value-based **reimbursement** policies
  - Addressing industrialization and **manufacturing** hurdles
  - Conducting key stakeholder outreach, **communication**, and education
  - Facilitating sustainable access to **capital**



# Current Global Sector Landscape

**917+**

Regenerative Medicine Companies  
Worldwide, including Gene and Cell  
Therapies, and Tissue Engineering  
Therapeutic Developers



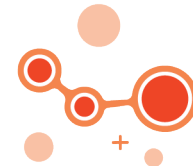
# 59,575

Total Targeted Enrollment of Patients  
in Current Regenerative Medicine  
Clinical Trials Worldwide



## 9,533

Target Enrollment of  
Phase I Clinical Trials



## 29,069

Target Enrollment of  
Phase II Clinical Trials



## 20,973

Target Enrollment of  
Phase III Clinical Trials

# Patient Impact of Recently Approved Products



**40%** of patients with R/R DLBCL treated with Novartis's **Kymriah** experienced a complete response

**60%** of patients with R/R B-Cell ALL treated with Novartis's **Kymriah** experienced a complete response

**58%** of patients with R/R B-Cell NHL treated with Kite / Gilead's **Yescarta** experienced a complete response

**55%** of patients treated with Spark Therapeutics' **Luxturna** showed an improvement of 2+ light levels darker after treatment



**93%** of patients with AveXis / Novartis's SMA Type 1 treated with **Zolgensma** were alive without permanent ventilation at 24 months post-treatment



**75%** of patients with TDT without  $\beta 0/\beta 0$  genotype treated with bluebird bio's **Zynteglo** achieved transfusion independence

# Select Anticipated Near-Term Approvals (Global)



## Gene Therapy

### **Zolgensma** (AveXis / Novartis)

- Spinal muscular atrophy type 1
- Decision expected: mid 2019 (EU & Japan)

### **GT-AADC** (PTC Therapeutics)

- AADC deficiency
- Expects to file: late 2019 (US)

### **Zynteglo** (bluebird bio)

- Beta thalassemia
- Expects to file: 2019 (US)

### **Valoctocogene roxaparvovec**

- (BioMarin)
- Hemophilia A
  - Expects to file: Q4 2019 (US & EU)

### **GS010** (GenSight Biologics)

- Leber hereditary optic neuropathy
- Expects to file: Q4 2019 (EU) / H2 2020 (US)

### **OTL-101** (Orchard Therapeutics)

- ADA-SCID
- Expects to file: 2020 (US)

### **OTL-200** (Orchard Therapeutics)

- Metachromatic leukodystrophy
- Expects to file: 2020 (US & EU)



## Cell-Based IO

### **Rivo-cel** (Bellicum Pharmaceuticals)

- HSCT to treat blood cancers
- Expects to file: EOY 2019 (EU)

### **ATIR101** (Kiadis Pharma)

- HSCT to treat blood cancers
- Decision expected: 1H 2020 (EU)

### **Remestemcel-L** (Mesoblast)

- Acute graft versus host disease
- Decision expected: 2020 (US)

### **P-BCMA-101** (Poseida Therapeutics)

- Multiple myeloma
- Expects to file: 2020 (US)

### **Lifileucel** (Iovance)

- Advanced metastatic melanoma
- Expects to file: 2020 (US)

### **LN-145** (Iovance)

- Advanced metastatic cervical cancer
- Expects to file: 2H 2020 (US)



## Cell Therapy

### **SB623** (SanBio)

- Traumatic brain injury
- Expects to file: January 2020 (Japan)

### **TEMCELL** (Mesoblast / JCR Pharma)

- Epidermolysis bullosa
- Decision expected: 2020 (Japan)



## Tissue-Based

### **RVT-802** (Enzyvant Therapeutics)

- Complete DiGorge anomaly
- Decision expected: 2019 (US)

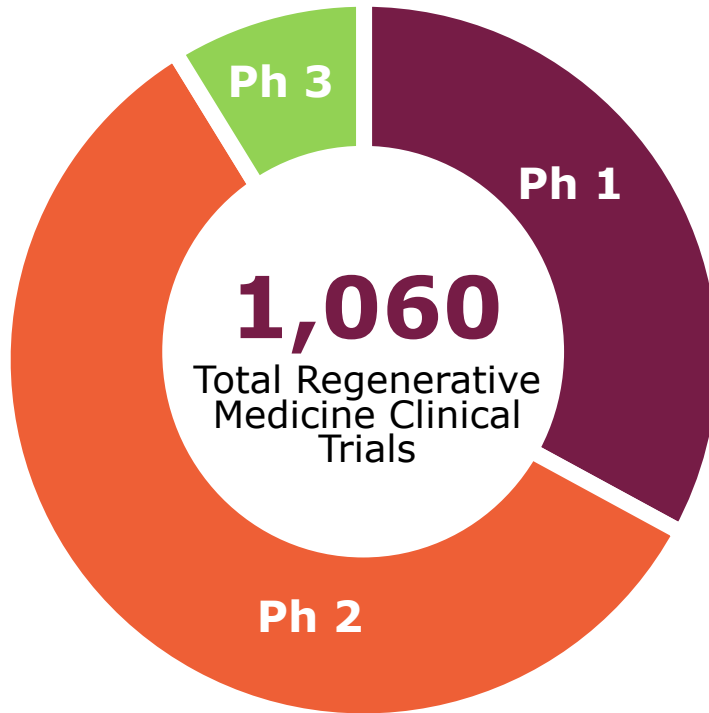
### **Humacyl** (Humacyte)

- End stage renal disease
- Expects to file: 2020 (US)

### **Stratagraft** (Mallinckrodt)

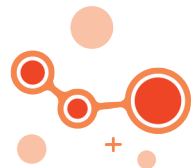
- Deep partial thickness burns
- Expects to file: 2020 (US)

# Regenerative Medicine Clinical Trials by Phase and Technology Type



**Phase 1: 349**  
across all tech types  
and indications

**Gene Therapy: 123**  
**Gene-Modified Cell Therapy: 160**  
**Cell Therapy: 55**  
**Tissue Engineering: 11**



**Phase 2: 618**  
across all tech types  
and indications

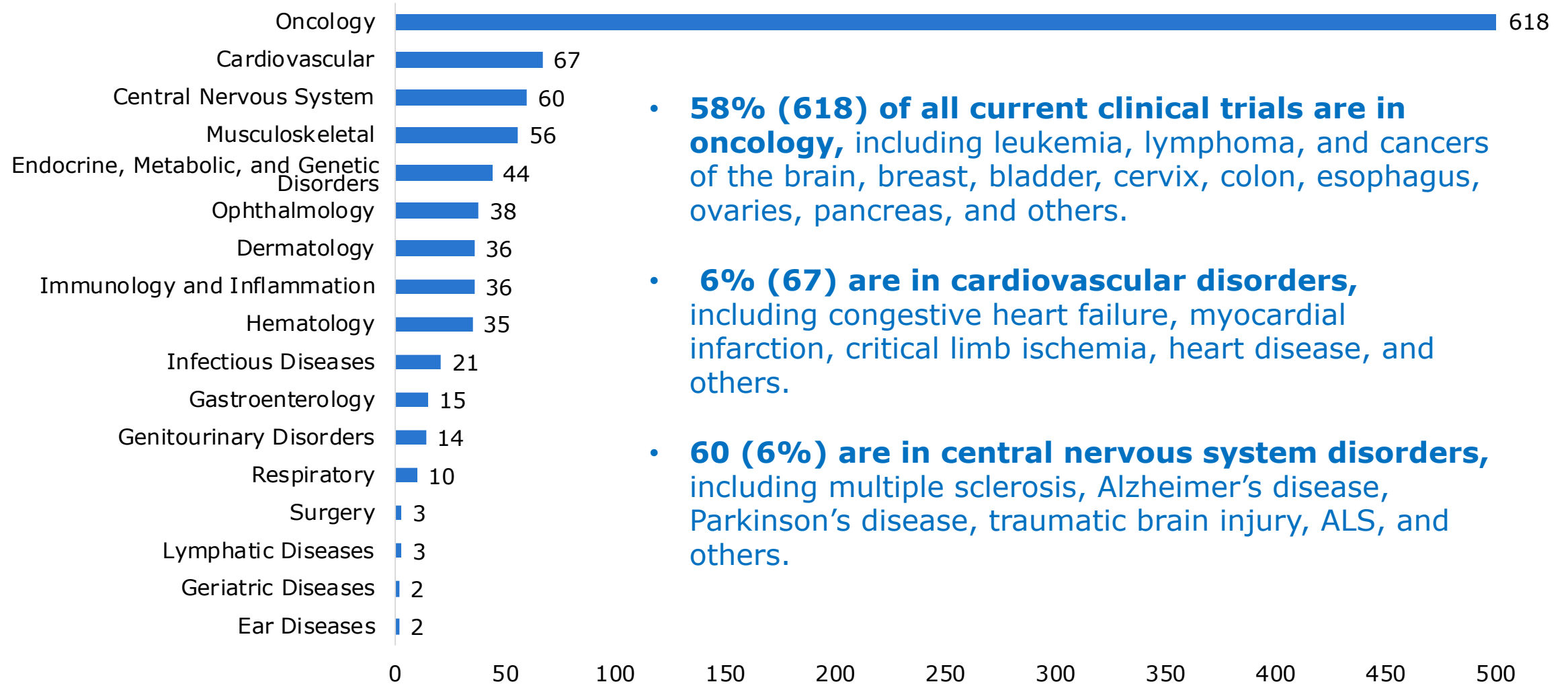
**Gene Therapy: 217**  
**Gene-Modified Cell Therapy: 197**  
**Cell Therapy: 182**  
**Tissue Engineering: 22**



**Phase 3: 93**  
across all tech types  
and indications

**Gene Therapy: 32**  
**Gene-Modified Cell Therapy: 17**  
**Cell Therapy: 31**  
**Tissue Engineering: 13**

# Regenerative Medicine Clinical Trials by Therapeutic Area





# Total Global Financings: Q2 2019



## Total Global Financings

**\$2.5B**  
Q2 2019

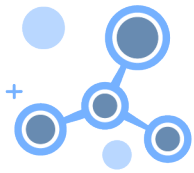
**\$4.7B**  
YTD 2019



## Gene-Based Therapies

**\$2.2B**  
Q2 2019

**\$4.3B**  
YTD 2019



## Cell Therapy

**\$691M**  
Q2 2019

**\$1.5B**  
YTD 2019



## Tissue Engineering

**\$53M**  
Q2 2019

**\$67M**  
YTD 2019

*\*YTD financings calculated as of the end of Q2 2019*

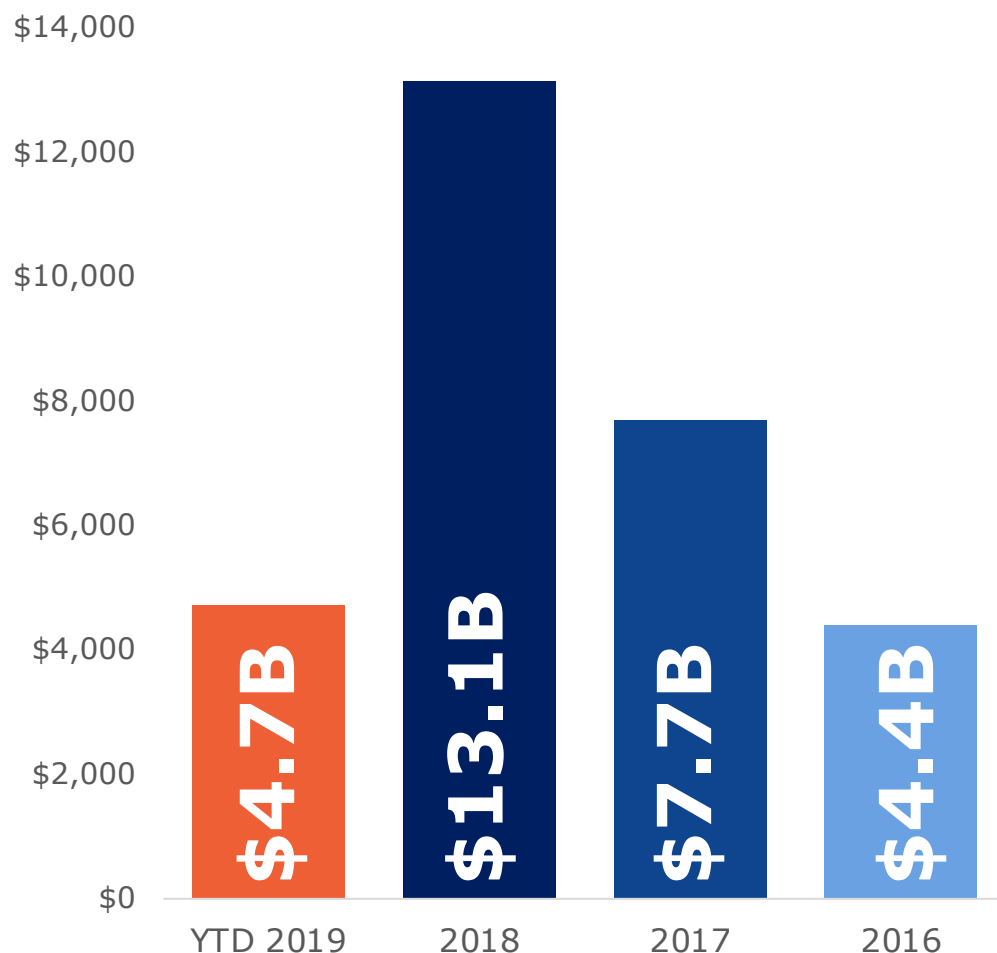
*\*both Gene-Based Therapies & Cell Therapy categories include financings from companies active in developing gene-modified cell therapies*

# Total Global Financings: YTD 2019

## 2018 was a watershed year for regenerative medicine financings.

- Highest total financings raised in recent years
- In 2018, there were **eight IPOs** for regenerative medicine companies that raised **\$100M+**
- The public performance averages for all RM/AT companies, cell-based IO companies, and gene therapy companies were higher than the Nasdaq Index and Nasdaq Biotech Index for the majority of the year.

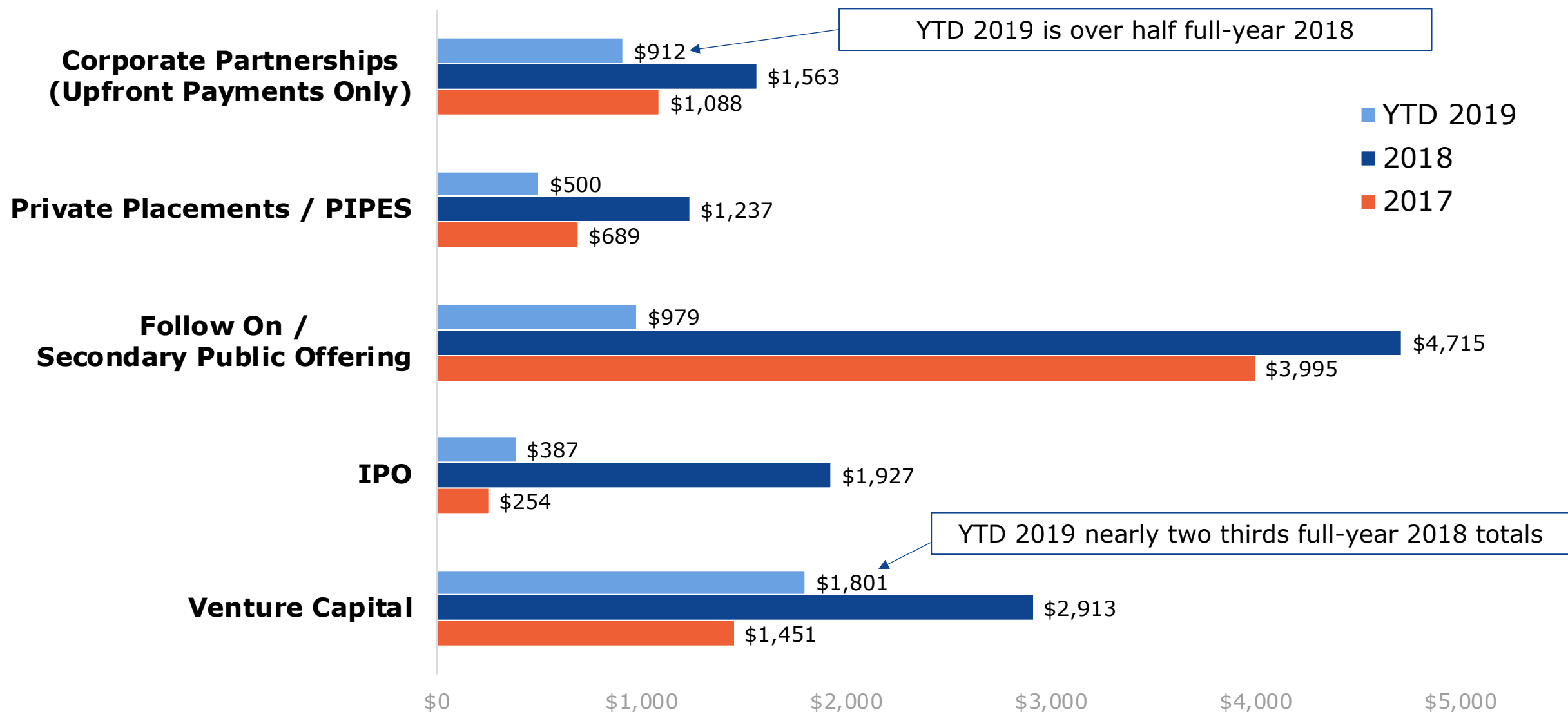
*2019 is on track to reach or exceed 2018 venture capital financings and upfront payments from corporate partnerships.*



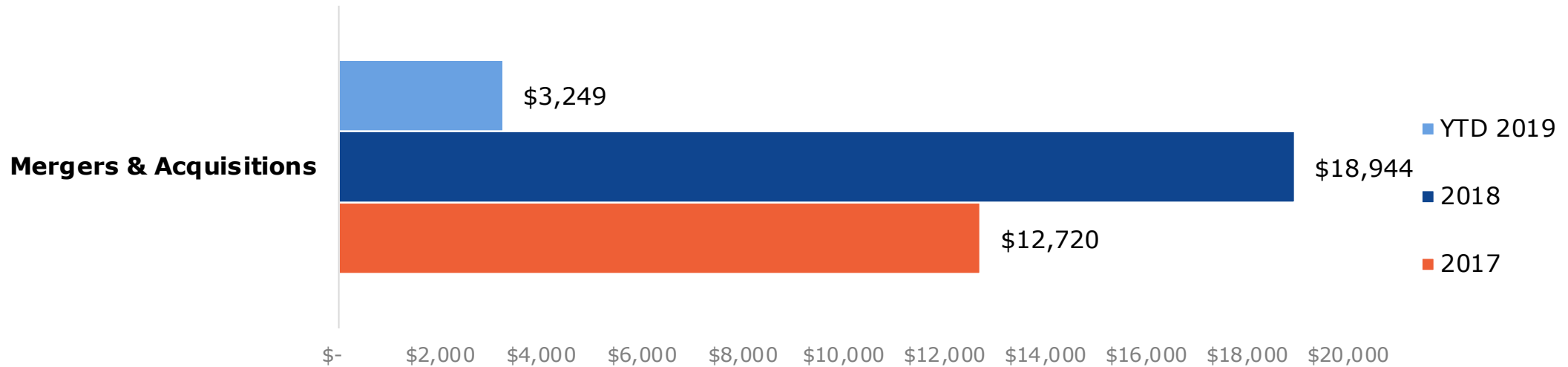
\*YTD financings calculated as of the end of Q2 2019

\*both Gene-Based Therapies & Cell Therapy categories include financings from companies active in developing gene-modified cell therapies

# Total Financings by Type, by Year



# Total M&A Transactions Values, By Year



*\*Does not include Roche's planned \$4.3B acquisition of Spark Therapeutics (expected to close by EOY 2019).*

# Select Corporate Partnerships & Public Financings in 1H 2019

## Corporate Partnerships: (Upfront Payments)

- Adaptive Biotechnologies signs \$300M upfront agreement with Genentech/Roche – January 4
- Vertex signs \$175M upfront agreement with CRISPR Tx – June 6
- Neurocrine Biosciences completes \$115M upfront agreement with Voyager Tx – January 29
- MeiraGTx signs \$100M upfront agreement with Janssen – January 31
- Voyager Tx \$65M upfront agreement with AbbVie – February 22

## Private Placements & Venture Financings:

- Maze Tx raises \$191M in venture funding – February 28
- Poseida raises \$142M in Series C – April 22
- Beam Tx secures \$135M in Series – March 6
- AlloVir raises \$120M in Series B – May 22
- Passage Bio raises \$115.5 Million in Series A – February 15
- Talaris Tx raises \$100M in Series A – April 18
- Gracell raises \$85M in Series B – February 25
- MeiraGTx raises \$80M in private placement – February 27
- Inovio raises \$70M in private offering – February 19
- Oxford Biomedica raises \$68M in private placement – May 28

## Public Offerings: (IPOs & Follow-Ons)

- Sangamo raises \$145M in follow-on offering – April 8
- Precision Bio raises \$145M in IPO – April 1
- Homology raises \$144M in follow-on offering – April 12
- Orchard Tx raises \$128M in follow-on offering – June 3
- Prevail Tx raises \$125M in IPO – June 24
- Autolus raises \$115.9M in follow-on offering – April 15
- Krystal Bio raises \$115M in follow-on offering – June 24
- Rocket Pharma raises \$91M in follow-on offering – April 19
- TCR2 Tx raises \$86.25M in IPO – February 19

# Market Access Landscape in 8 Countries

As of July 2019

France (TC/CEESP)	Germany (IQWiG/ G-BA)	UK (NICE/SMC)	Italy (AIFA/ regional)	Netherlands (ZIN/CVZ)	U.S. (CMS)	Canada (CADTH)	Australia (MSAC)
Kymriah ★	Alofisel ★ ✓	Holoclار ★	Holoclار ★	Imlygic ★ ✓	Imlygic ★ ✓	Kymriah <i>CADTH Assessment: Would be cost effective if price lowered</i>	Kymriah ✓
Yescarta ★	Imlygic ✓	Imlygic ★ ✓	Imlygic <i>Authorized, hospital only</i>		Kymriah ★ ✓		
	Kymriah ✓	Kymriah ★ ✓	Strimvelis ★ ✓		Luxturna ★ ✓		
	Luxturna ✓	Strimvelis ★ ✓	Zalmoxis ★ ✓		Yescarta ★ ✓		
	Strimvelis ✓	Yescarta ★ ✓			Zolgensma ★ ✓		
	Yescarta ✓						
	Zalmoxis ★ ✓						

Zynteglo, which was approved in Europe in June 2019, is currently working with payers in UK, Italy, Germany, France for its initial commercial rollout in 2020.

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# Thank You!