

CELL GENE

INVESTOR DAY - NEW YORK

March 21, 2019



Welcome Remarks:
Janet Lambert, CEO



Alliance for
Regenerative
Medicine

About ARM

- **International advocacy organization**
 - dedicated to realizing the promise of safe and effective regenerative medicines for patients around the world
- **300 + 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**

ARM Membership

***Members in bold are publicly traded companies*

4D Molecular Tx AABB AATB **Abeona Tx** Accelerated Bio Acera Surgical ACF Bioservices ACRO **Adaptimmune** **Adverum Bio** Aegle Tx **AGTC** Akron Biotech Albumedix Aldevron Alpha-1 Foundation American Gene Technologies **Amicus** Andalusian Initiative for Advanced Therapies ANEMOCYTE Angiocrine Bio ASGCT Aspect Biosystems ASPS Asset Mgmt Company **Astellas** **Asterias** **Atara Bio** **Athersys** **Audentes Tx** AusBiotech **Autolus** Avectas Avery Tx **Avita Medical** AVM Biotech **AVROBIO** **AxoGen** **Axovant** B-MoGen Baylor College of Medicine Be the Match Biotherapies **Becton Dickinson** **Bellicum** **Benitec** BioBridge Global **BioCardia** **BioLife** **BioMarin** **BioStage** Biotech Mountains **BioTime** BCA BlueRock Tx **bluebird bio** **Bone Tx** **BrainStorm Cell Tx** BrainXell Brammer Bio C3i **Caladrius** **Capricor** **Cardinal Health** Caribou Bio Carisma Tx Carpenter Consulting Casebia CASS CCRM CCTA **Celgene / Juno** Celixir Cell Medica Cell Therapies Pty Ltd Cellerant CellGenix Cells for Cells Cellular Technology Ltd Celonic Celsense Cevec CGT Catapult Chemelot CIRM City of Hope Cleveland Clinic Cleveland Cord Blood Center ClinicalMind Cognate BioServices **CombiGene** Cornell University CRC Oncology **CRISPR Tx** **Cryoport Systems** **CSL** CTI CTM CRC **Cynata Tx** Dark Horse Consulting Defined Health DiscGenics Dyno Tx EB Research Partnership **Editas Medicine** **Enochian Biosciences** Enzyvant Tx ERA Consulting Ltd ESGCT EveryLife Foundation Evidera ExCellThera Falcon Tx FARA **Fate Tx** FBRI **Fibrocell** Fight Colorectal Cancer **Flexion Tx** FloDesign Fondazione Telethon Fraunhofer Institute Fred Hutch Frequency Tx Froceth **FUJIFILM Cellular Dynamics** GammaDelta Tx Gates Center for Regenerative Medicine **GE Healthcare** Generation Bio Genprex GENETHON **GenSight Biologics** Gift of Life Marrow Registry **Gilead / Kite** Giner Global Genes GPB Scientific **GSK** **Healios** **K.K.** Histogen **Histogenics** **Hitachi Chemical Adv Tx** Hogan Lovells **Homology Medicines** Huron Consulting Hybrid Concepts International Immusoft INmune Bio InRegen Institut Clayton de la Recherche **Intellia Tx** Invetech Invitria Invitrx IO Biosciences **Iovance**

ARM Membership

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IQVIA ISSCR Johns Hopkins **Johnson & Johnson** Juventas Tx **Kawasaki Heavy Industries** Key Biologics **Kiadis Pharma** **Krystal Biotech** Kytopen LabConnect Lake Street Capital Markets LatticePoint Consulting Legend Biotech LogicBio Longeveron **Lonza Biologics** Ludwig Boltzmann Institute Lynch Biologics **Lysogene** **Magenta Tx** MaSTherCell **MaxCyte** Medeor TxBiotech MEDIPOST America **Medpace** MeiraGTx **Mesoblast Ltd** Michael J. Fox Foundation **MilliporeSigma** **MiMedx** Minerva Biotech Miromatrix Missouri Cures **MolMed** Monarch Biosciences MSK Cancer Center MTF **Mustang Bio** Myonex Tx Navan Tech NCLC NDRI **Neuralstem** NexImmune **Nightstar Tx** NJII NMSS Nohla Tx Northwestern University Novadip Biosciences **Novartis / Avexis** **Novellus** Novitas Capital **Novo Nordisk** NSCF NSCI NYSCF Ophthotech Opsis Tx Orbit Biomedical Orbsen Tx **Orchard Tx** Organabio **Organovo** Orig3n **Orthocell** **Oxford BioMedica** Pancella PDC*line Pharma **Pfizer** **Pluristem Tx** **PolarityTE** Poseida Tx Precision Bio Promethera Bio Regenerative Patch Tech Regenerex **ReGenesys** **Regeneus** REGENXBIO REMEDI ReNeuron RepliCel Rescue Hearing Rexgenero **Rocket Pharma** RoosterBio Roslin Cell Therapies RxGen **Saint-Gobain** **SanBio** Sanford Health Sanford Stem Cell Center **Sangamo Tx / TxCell** **Sanofi / Bioverativ** **Sarepta** Sartorius Scinogy SCM LifeScience Semma Tx Senti Biosciences Sentien Biotech Seraxis **Sernova** Sigilon Skingenix Skyland Analytics SNBTS **Solid Bio** **Spark Tx** SSSCR STEL Technologies StemBioSys StemCyte Stop ALD Foundation Synpromics Tenaya TERMIS-Americas Terumo BCT Tessa Tx Texas Heart Institute **Thermo Fisher** Thrive Bioscience **TiGenix** TikoMed Tmunity Tx TrakCel Trizell Tulane University UC Irvine UCSD Stem Cell Program **Ultragenyx** UM Stem Cell Institute UMass Medical School Unicyte **uniQure** Unite 2 Fight Paralysis United Spinal Assn of VA **Universal Cells** Abramson Cancer Center **Vericel** VERIGRAFT ViaCyte VidaCel Videregen VINETI ViveBiotech Vivet Tx Voisin Consulting **Voyager Tx** WindMIL Tx World Courier Wuxi **Xintela** Yposkesi Zelluna

Current Global Sector Landscape

906

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

484

North
America

15

South
America

241

Europe &
Israel

142

Asia

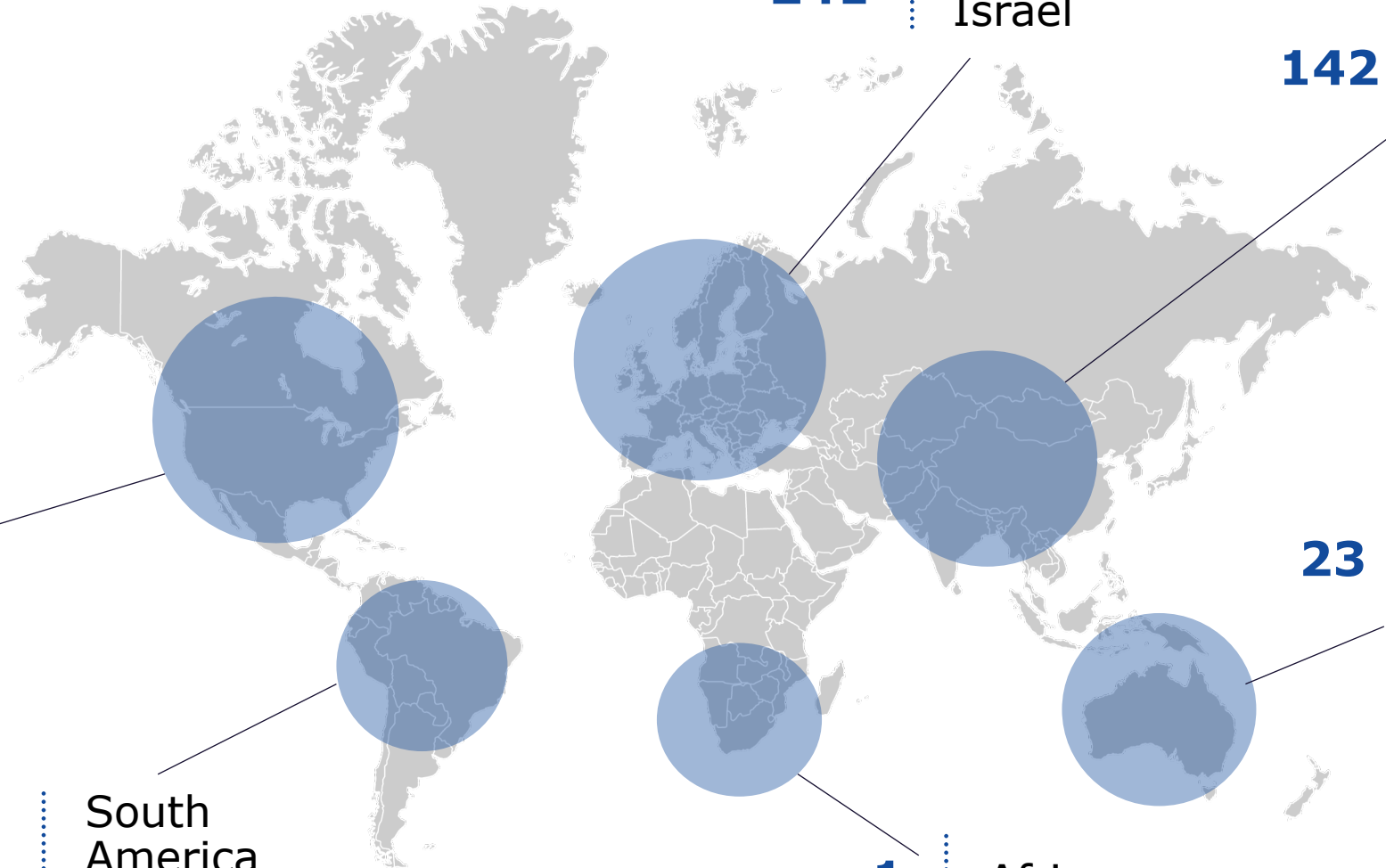
23

Oceania

Australia,
New Zealand,
Marshall Islands

1

Africa



Total Clinical Trials by Phase as of EOY 2018



Phase I
341



Phase II
595



Phase III
92

TOTAL:
1,028

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

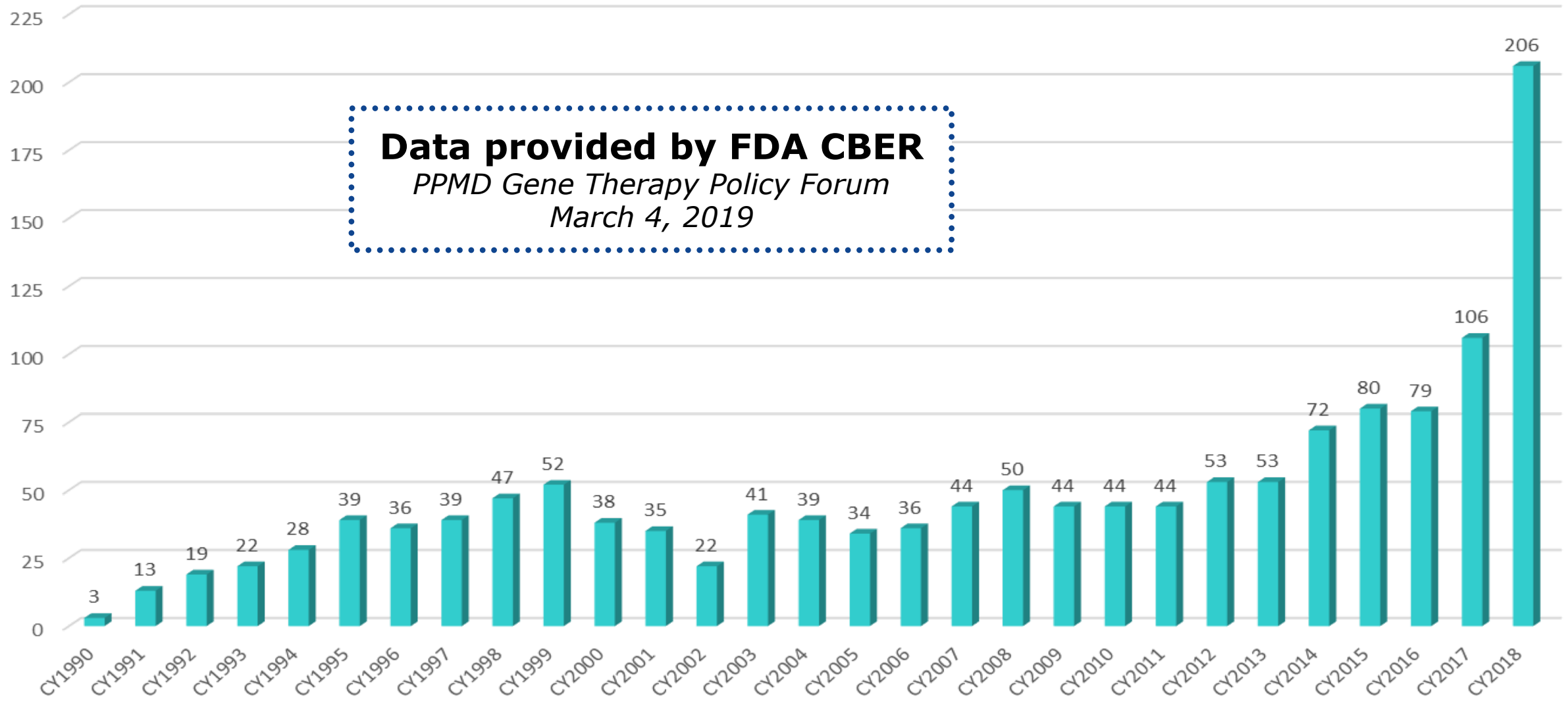


20,973

Target Enrollment of
Phase III Clinical Trials

New Gene Therapy IND Approvals

Data provided by FDA CBER
PPMD Gene Therapy Policy Forum
March 4, 2019



Total Global Financings: 2018



\$13.3B

**Total Global
Financings 2018**

▲ 73%
from 2017



\$9.7B

**Gene-Based Therapies
2018 Financings**

▲ 64%
from 2017



\$7.6B

**Cell Therapy
2018 Financings**

▲ 64%
from 2017

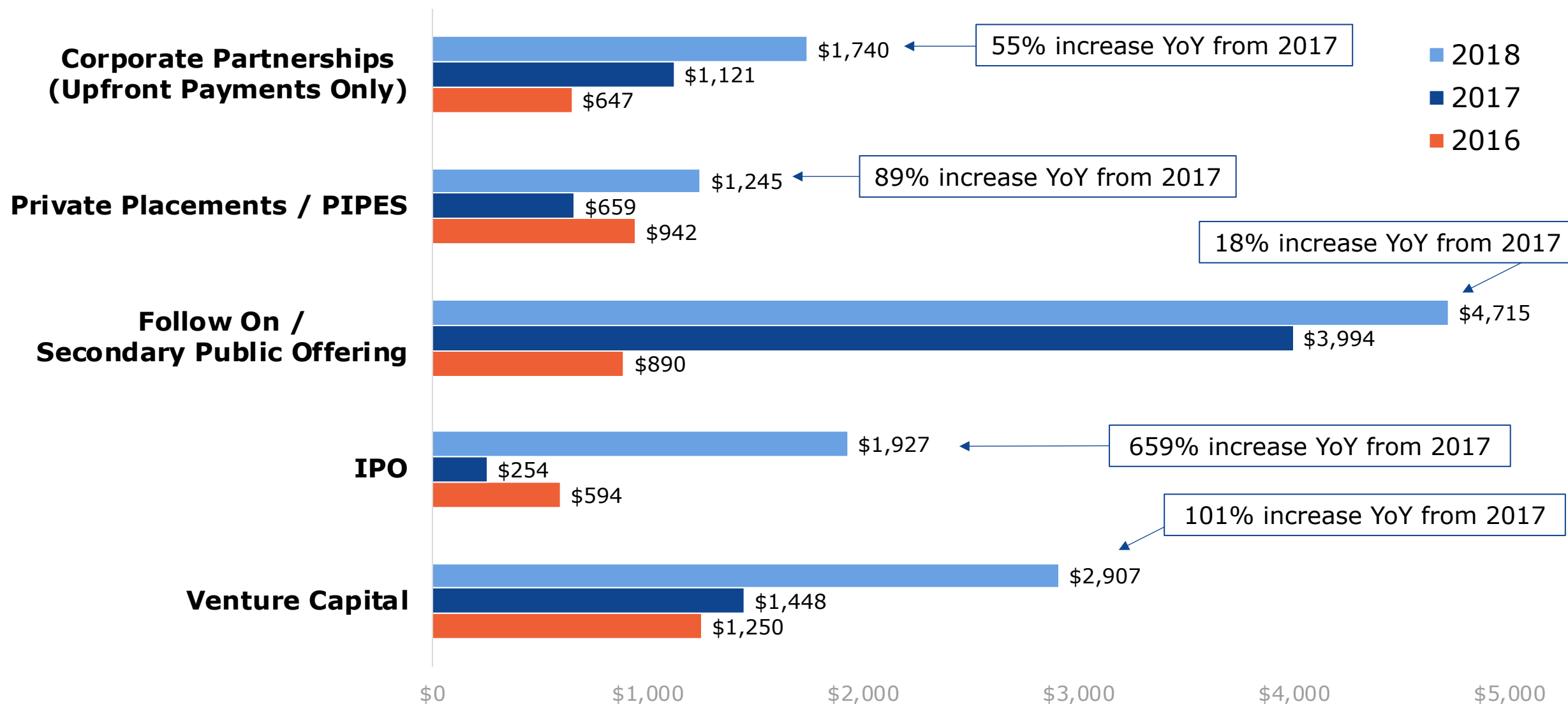


\$936.9M

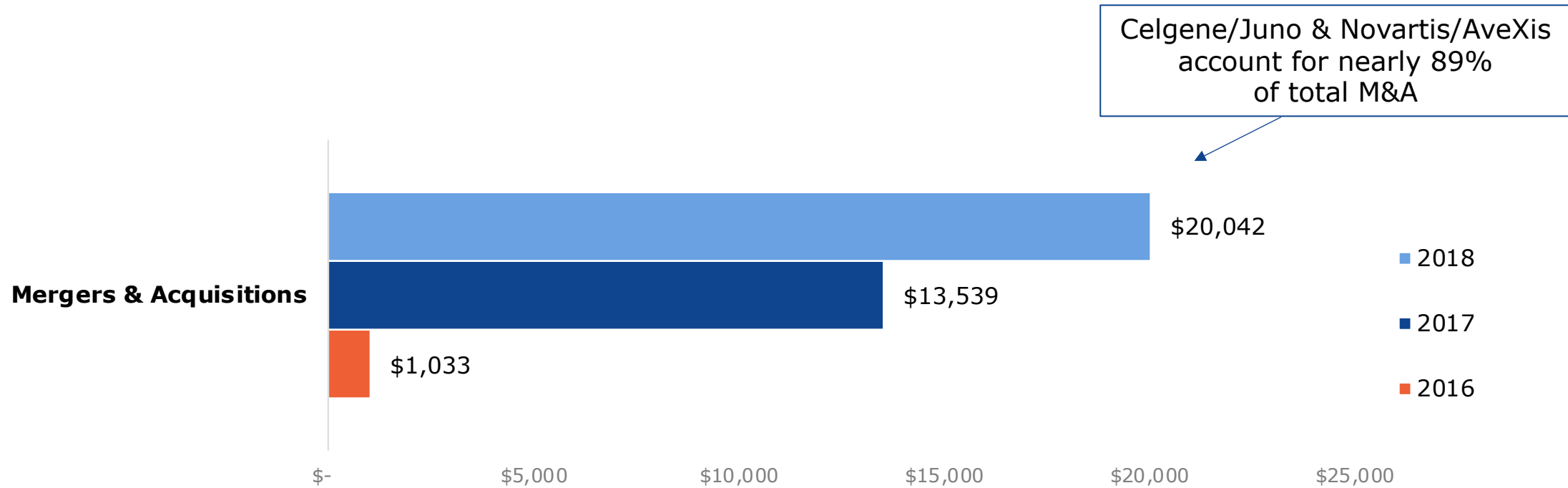
**Tissue Engineering
2018 Financings**

▲ 258%
from 2017

Total Financings by Type, by Year



Total M&A Transactions Values, By Year



Select Corporate Partnerships & Public Financings in 2018

Corporate Partnerships: (Upfront Payments)

- Kite signs \$150M upfront agreement with Sangamo – February 20
- Intrexon signs \$150M upfront agreement with Merck – December 20
- SQZ signs \$125M upfront agreement with Roche – October 15
- Spark Therapeutics signs \$110M upfront agreement with Jazz Pharmaceuticals – April 30
- Spark Therapeutics signs \$105M upfront agreement Novartis – January 24

Private Placements & Venture Financings:

- Allogene secures \$300M Series A – April 3
- Celularity secures \$250M in venture financing – February 15
- Humacyte raises \$150M in private placement – June 11
- Orchard Tx raises \$150M in Series C – August 13
- TCR Therapeutics raises \$125M in Series B – March 21
- Allogene raises \$120M in venture financing – September 6
- Freeline Tx raises \$116.6M in Series B – June 19
- Adaptimmune Tx raises \$100M in private placement – Sept 7
- bluebird bio raises \$100M in private placement – August 6
- Rubius raises \$100M in private placement – March 1
- Generation Bio raises \$100M in Series B – February 27
- Tmunity Tx raises \$100M in Series A – January 23
- ViaCyte raises \$80M in Series D – November 29

Public Offerings: (IPOs & Follow-Ons)

- bluebird bio raises \$651.3M in follow-on financing – January 8
- bluebird bio raises \$632.5M in follow-on financing – July 7
- AveXis raises \$431.9M in follow-on financing – January 22
- Allogene raises \$372.6M in IPO – October 15
- Rubius Tx raises \$277.3M in IPO – July 23
- Iovance raises \$252.2M in follow-on financing – October 17
- Audentes raises \$231.4M in follow-on financing – January 29
- Sangamo raises \$230M in follow-on financing – April 30
- Orchard Tx raises \$225.5M in IPO – November 5
- REGENXBIO raises \$201.8M in follow-on financing – August 14
- CRISPR Tx raises \$200M in follow-on financing – September 19

Select Anticipated Near-Term Approvals



Enzyvant Therapeutics' **RVT-802**, a tissue-based product for the treatment of complete DiGeorge Anomaly
Decision expected: 2019 (US)



AveXis / Novartis's **Zolgensma**, a gene therapy for the treatment of spinal muscular atrophy type 1
Decision expected: May 2019 (US), mid-2019 (EU), mid-2019 (Japan)



Kiadis Pharma's **ATIR101**, a gene modified cell therapy for the treatments of leukemia
Decision expected: 1H 2019 (EU)



bluebird bio's **LentiGlobin**, a gene therapy for the treatment of beta thalassemia
Decision expected: mid-2019 (EU)
Expects to file (US) in 2019



Mesoblast's **Remestemcel-L**, a stem cell therapy for the treatment of acute graft versus host disease
Expects to file (US) in 2019



Atara's **tab-cel**, a cell therapy for the treatment of EBV+ PTLD
Expects to file: 2H 2019 (EU)



PTC Therapeutics' **GT-AADC**, a gene therapy for the treatment of aromatic L-amino acid decarboxylase (AADC) deficiency
Expects to file: late 2019 (US)



Orchard's **OTL-101**, a gene therapy for the treatment of ADA Deficiency / ADA-SCID
Expects to file: 2020 (US & EU)



Orchard's **OTL-200**, a gene therapy for the treatment of meta-chromatic leukodystrophy
Expects to file: 2020 (US & EU)

This presentation will be available on our website and shared via Twitter at @alliancerm

Visit [**www.alliancerm.org**](http://www.alliancerm.org) to access additional resources, including:

- ✿ Quarterly sector data reports
- ✿ Upcoming near-term clinical trial milestones & data readouts
- ✿ Access to slides, graphics, and figures from ARM presentations
- ✿ Our weekly sector newsletter, a robust round-up of business, clinical, scientific, and policy news in the sector
- ✿ Commentary from experts in the field

Thank You!

FEATURED FIRESIDE CHAT

Gbola Amusa

Partner, Director of Research and
Head of Healthcare Research
Chardan (*Moderator*)

Mark Kay

Dennis Farrey Family Professor, Departments of Pediatrics
& Genetics; Associate Chair for Basic Research - Pediatrics
Stanford University

