



Quarterly Data Report

# *Regenerative Medicine & Advanced Therapies*

# ALLIANCE for *Regenerative Medicine*

*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.*

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

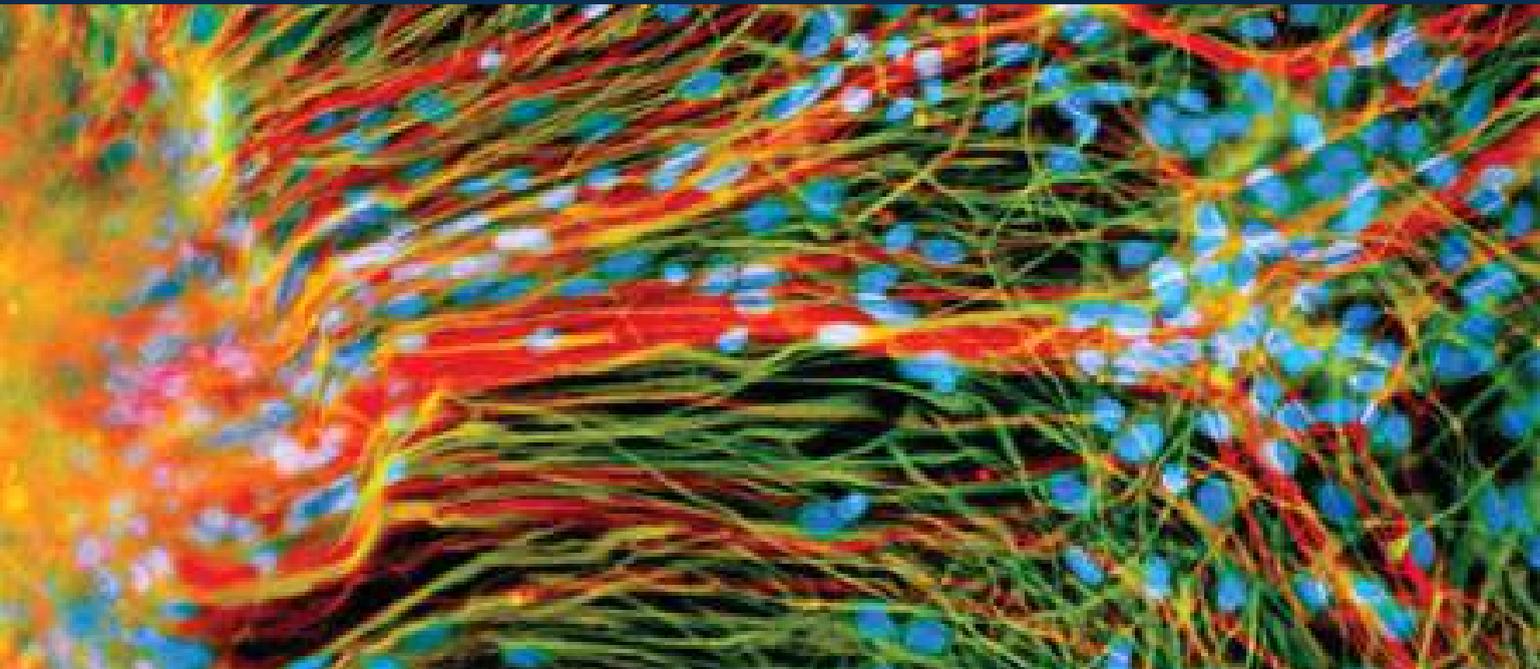
*Special thanks to our data partner:*

**informa**

*Informa is one of the world's leading knowledge providers. We create and deliver highly specialized information through publishing, events, training, market intelligence and expertise, providing valuable knowledge to individuals, businesses and organizations around the world.*

*Informa provides authoritative research and analysis and up-to-the-minute business news, comment and events for all sectors of the healthcare, medical and life sciences communities. Informa Business Information (IBI) is one of the world's leading providers of industry and drug news, analysis and data to the global pharmaceutical industry.*

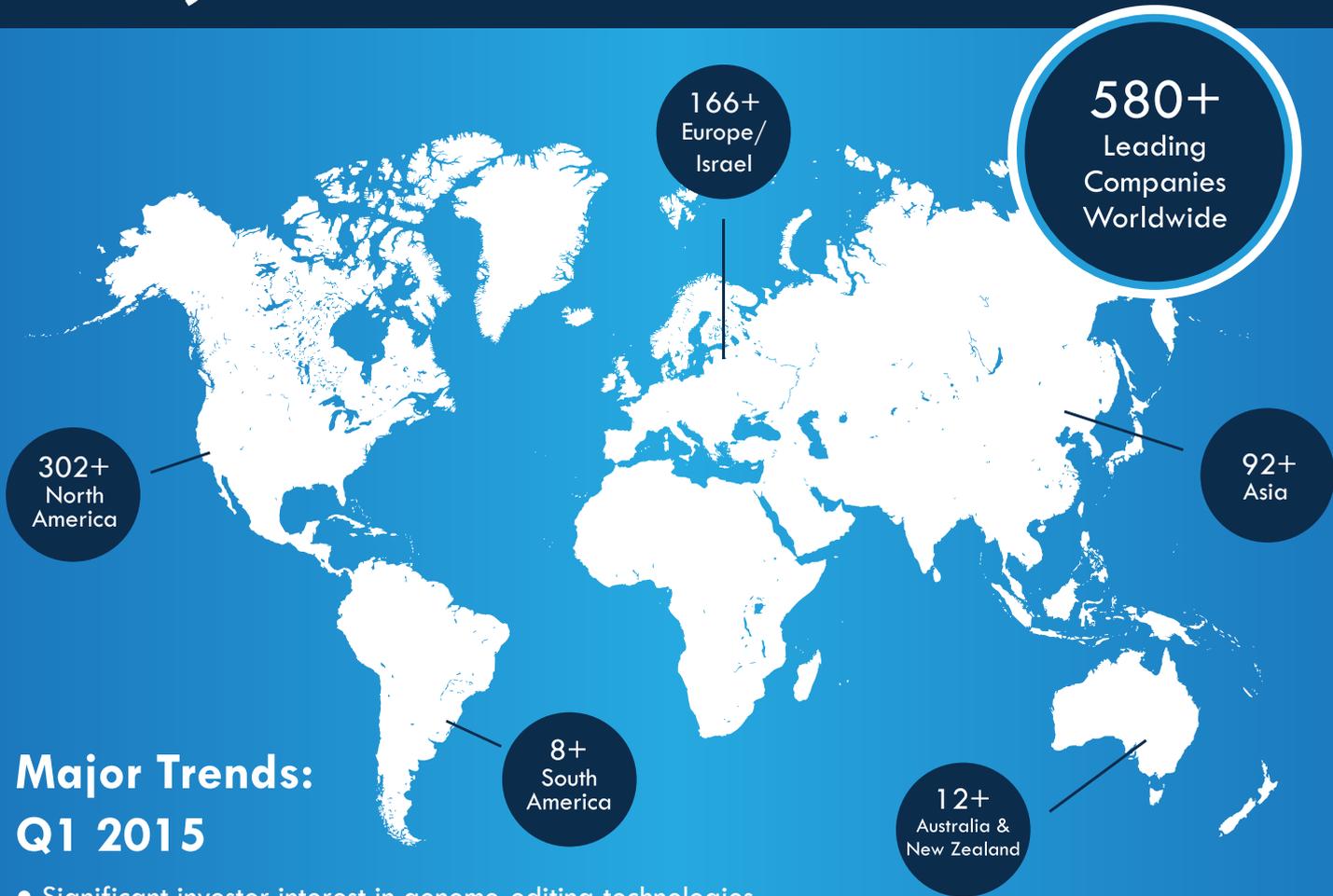
# Table of Contents



<i>Industry Overview.....</i>	<i>2-3</i>
<i>Financings.....</i>	<i>4-5</i>
<i>Corporate Partnerships &amp; Acquisitions.....</i>	<i>6</i>
<i>Clinical Trials &amp; Data Events.....</i>	<i>7-8</i>
<i>Current Regulatory Priorities.....</i>	<i>9</i>



# Industry Overview



## Major Trends: Q1 2015

- Significant investor interest in genome-editing technologies
- Ongoing major investment in CAR-T and other adoptive T cell therapies to treat cancer
- Interest from large pharma expanding and accelerating
- Major disease focus areas include: hematological diseases; ophthalmology – dry AMD; cardiovascular disease; and stroke
- Further discussion regarding new pricing models and market access for curative therapies
- Several data events from later-stage cell therapy clinical trials expected throughout 2015

"Strategic partnerships with leading biopharmas are central to building a sustainable industry in regenerative medicine."



Silviu Itescu, MBBS, FRACP  
CEO & Managing Director  
Mesoblast Limited

# Industry Overview

The big stories in the first quarter of 2015 were investing and dealmaking, up significantly compared with the first quarter of 2014. While dealmaking was spread across the board, two sectors stood out. Gene editing technologies were of particular interest to investors, with strong interest in the CAR-T (chimeric antigen receptor T cells) space as a rapidly progressing approach to therapeutics. Partnerships have remained steady and included one standout in the first quarter. Genzyme's option agreement with Voyager Therapeutics to license several AAV products for CNS indications with \$100 million upfront and \$745 million in potential development and sales milestone payments is a major strategic move for Genzyme, a company that has been working in gene therapy since 1991.

This sector has come off a strong year in 2014 and 2015 is off to an even stronger start. As 2015 continues to unfold, we anticipate more high-value deals, and increased investor and public interest in what advanced therapies can offer.

-Patricia Reilly  
Executive Director, Medtrack

-Nancy Dvorin  
Managing Editor – IN VIVO, Start-Up  
and Medtech Insight

Informa Business Intelligence,  
Pharma and Healthcare

60+  
Number  
of Approved  
and/or  
Marketed  
Products

486  
Total Number of  
Clinical Trials  
(Currently in  
Phase I, II or III)



Phil Vanek, Ph.D.  
General Manager, Cell Therapy Technologies  
GE Healthcare

“Confidence in and throughout the sector has really grown – there’s increasing sophistication in supply chain and production plans, shaping the infrastructure needed to support advanced therapy products.

We expect to see significant movement in the next few years regarding the physical integration of manufacturing workflow unit operations, along with increased digital integration. This digitization will enable huge amounts of data to be mined, which will further inform everything from process optimization to patient stratification, improving systems and outcomes.”

# Financings



**TOTAL RAISED  
IN Q1 2015:**  
\$2.7 Billion  
(up 135% compared  
to Q1 2014)



**GENE &  
GENE-MODIFIED  
CELL THERAPY:**  
\$1.6 Billion raised  
(up 339% compared  
to Q1 2014)



**TISSUE  
ENGINEERING:**  
\$162 Million raised  
(down 50% compared  
to Q1 2014)



**CELL THERAPY:**  
\$926 Million raised  
(up 101% compared  
to Q1 2014)

## Examples of key financings: Q1 2015

### IPO:

- Collectis, Inc. raises \$228M - March 25, 2015
- Spark Therapeutics, Inc. raises \$185.2M - February 4, 2015
- Bone Therapeutics raises \$36.6M - February 4, 2015

### FOLLOW-ONS:

- Lion Biotechnologies raises \$73.6M in follow-on financing - March 3, 2015
- Intrexon Corporation raises \$116.4M in follow-on financing - January 27, 2015
- Avalanche Biotechnologies, Inc. raises \$162.8M in follow-on financing - January 13, 2015

### VENTURE FINANCING:

- Semma Therapeutics closes \$44M in funding - March 18, 2015
- REGENXBIO, Inc. closes \$30M in Series C financing - January 21, 2015

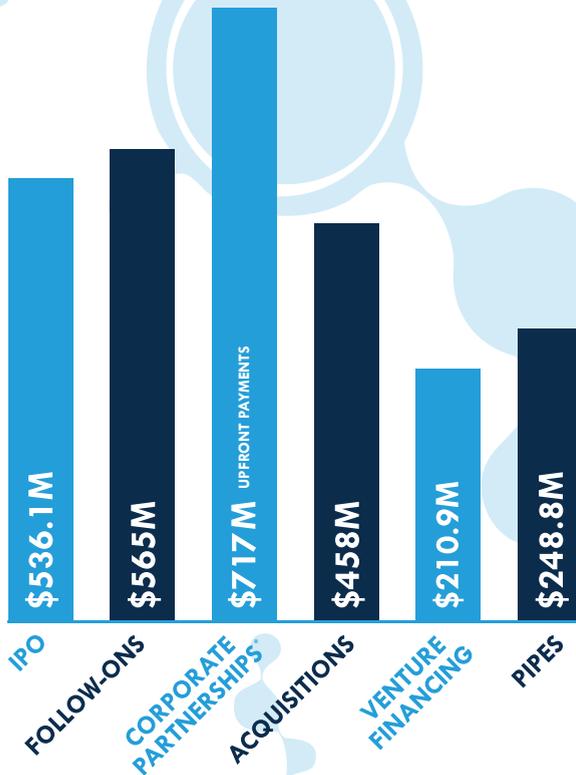
### MAJOR DEVELOPMENT PARTNERSHIPS:

- Aduro Biotech signs \$750M agreement with Novartis, includes \$225M upfront - March 30, 2015
- Intrexon Corporation sign \$941M agreement with Merck Serono, includes \$115M upfront - March 30, 2015
- Voyager Therapeutics signs \$845M agreement with Genzyme Corporation, a Sanofi company, includes \$100M upfront - February 11, 2015

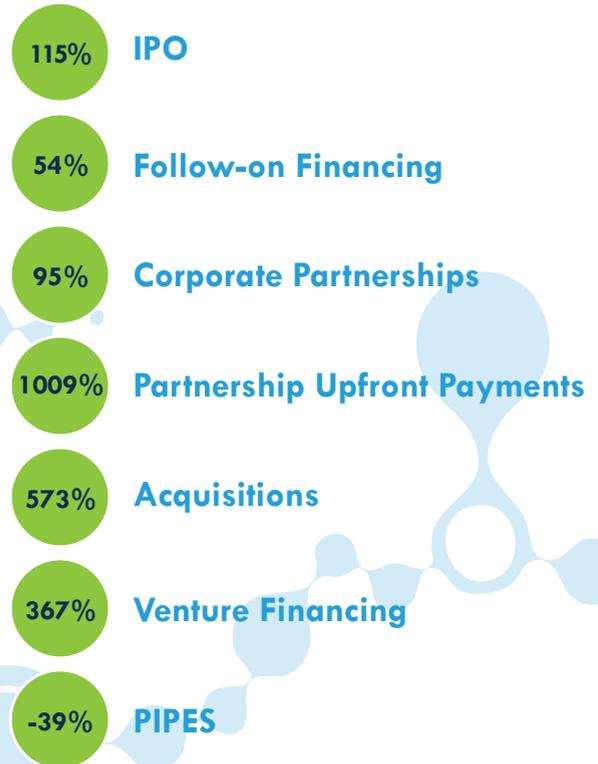
# Financings

## Total financings by type: Q1 2015

Amounts shown in USD



## Financing Comparisons Q1 2014 to Q1 2015



- Q1 2015 total corporate partnerships deal value is \$4.5 billion, compared to Q1 2014 \$2.3 billion total deal value

“Industry and investor interest in gene therapy has experienced a renaissance over the past few years, driven in large part by pioneering clinical work completed by leading academic researchers. Universally, people recognize the transformative potential gene therapy offers to patients suffering from severe disorders, including debilitating CNS diseases. In just the past two to three years, significant capital has been invested in companies like Voyager, focused on the development of novel gene therapies and it’s an exciting time as these groundbreaking therapies advance in development.”



Jeff Goater  
SVP, Finance & Business Development  
Voyager Therapeutics, Inc.

# Corporate Partnerships & Acquisitions

“The strategic collaboration between Amgen and Kite unites Amgen’s immuno-oncology experience and array of cancer targets with Kite’s CAR platform and capabilities in T cell manufacturing. We are witnessing a new era in medicine in which the power of the immune system can be harnessed and enhanced by engineering with CARs or TCRs, together with other therapeutic genes, potentially transforming the treatment of cancer.”



Margo Roberts, Ph.D.  
Chief Scientific Officer  
Kite Pharma

## Examples of key corporate partnerships & acquisitions: Q1 2015

Fujifilm Corporation announces agreement to acquire Cellular Dynamics International

**March 30, 2015**

Athersys, Inc. and Chugai Pharmaceutical Co., Ltd. collaborate on cell therapy for ischemic stroke

**March 2, 2015**

Voyager Therapeutics and Genzyme Corporation, a Sanofi company, announce development and commercialization collaboration for novel AAV gene therapies

**February 11, 2015**

Intrexon Corporation partners with ZIOPHARM Oncology, Inc. and The University of Texas MD Anderson Cancer Center in CAR-T pact

**January 13, 2015**

Cardio3 BioSciences acquires Oncyte, LLC CAR-T portfolio

**January 6, 2015**

Kite Pharma and Amgen Inc. announce strategic cancer immunotherapy collaboration

**January 5, 2015**

# Clinical Trials & Data Events

## Examples of major milestones and key data events: Q1 2015

- Editas Medicine reports new data demonstrating potential of CRISPR/Cas9 construct as novel gene repair mechanism - March 2, 2015
- Sangamo BioSciences, Inc. announces new clinical data from trial of ZFP therapeutic designed to provide functional control of HIV, also announces FDA acceptance of IND application for new HIV/AIDS clinical trial using ZFN-modification of hematopoietic stem cells - February 26, 2015
- bluebird bio receives Breakthrough Therapy designation for LentiGlobin for treatment of beta-thalassemia major- February 2, 2015
- Pluristem Therapeutics Inc. announces significant new finding from its Phase I/II muscle injury trial and excellent safety profile for PLX-PAD cells at twelve months - February 2, 2015
- Mesoblast Ltd. reports positive 24-month results in Phase II trial for chronic lower back pain - January 15, 2015

**Ph. I: 150**

**Ph. II: 288**

**Ph. III: 48**

**486**  
Clinical  
trials  
underway



"At Pluristem, we are seeing a significant uptick in interest across the board in cell therapy products. We are actively meeting with other companies throughout Asia, building upon our current partnership with Korea's CHA Biotech and our development strategy in Japan. Pluristem's manufacturing capabilities and our later-stage assets make us an attractive partner in these large, high-potential markets and we expect to make further progress in 2015 towards bringing these products to patients."

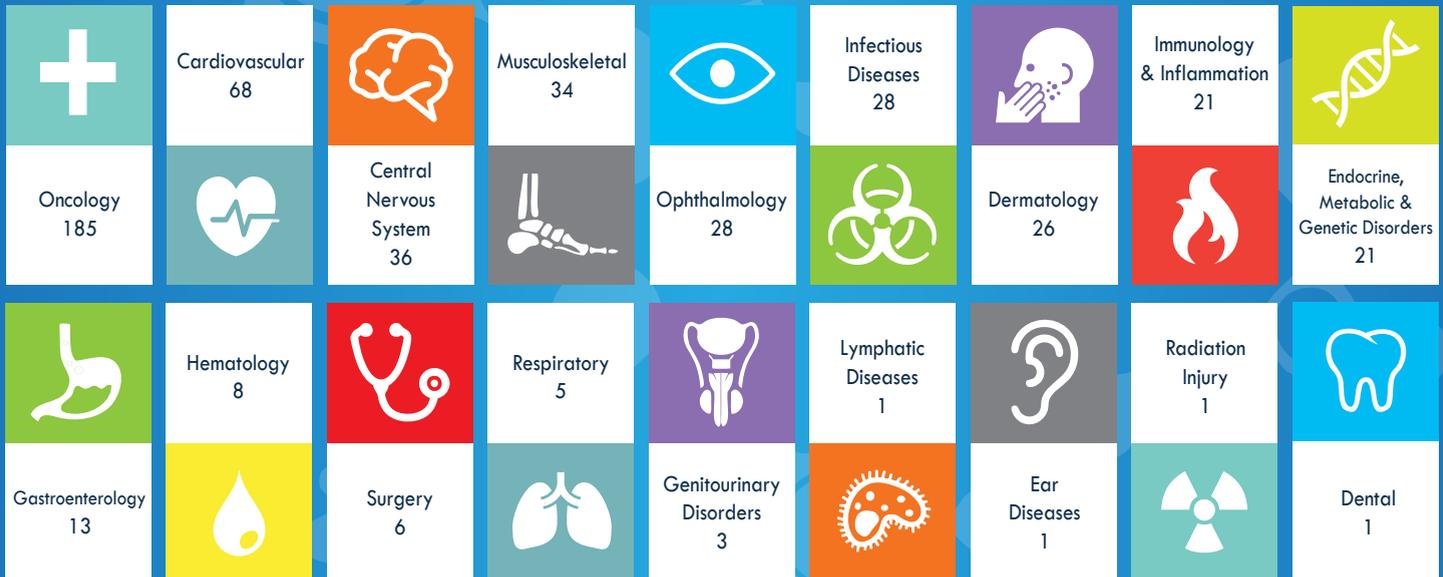


Hillit Mannor Shachar, M.D.  
Vice President Business Development  
Pluristem Therapeutics Inc.

# Clinical Trials & Data Events

## Current Clinical Trials by Therapeutic Category: Q1 2015

- More than  $\frac{1}{3}$  of current clinical trials are in oncology.
- More than  $\frac{1}{10}$  are in cardiovascular.



Thomas Chalberg, Ph.D.  
Founder & CEO  
Avalanche Biotechnologies, Inc.

"Investors are eager to learn about the use of AAV vectors for ophthalmologic applications, including AVA-101 for wet age-related macular degeneration and our recently-announced candidates for the treatment of color vision deficiency. At Avalanche Biotechnologies, we're looking forward to continuing to develop the use of our gene therapy and Directed Evolution platforms, to preserve patients' sight, and implementing our scalable manufacturing capabilities to bring these products to market and transform lives."

# Current Regulatory Priorities



Matthew Patterson  
President and CEO  
Audentes Therapeutics, Inc.

"An important goal for us at Audentes is to understand the current state of awareness regarding advanced therapy products and to collaborate with a range of stakeholders to ensure we establish a reimbursement environment that accommodates unique pricing models for these incredibly innovative products."



## ARM Regulatory Focus Areas: Q1 2015

- Continuing to push our proposals to be included in the U.S. House of Representatives' 21st Century Cures initiative and the U.S. Senate's Medical Innovation initiative, such as:
  - Facilitating a clear path to market, including expedited product review
  - U.S. Regenerative Medicine Promotion Act and provisions regarding coordinated federal policymaking and its impact on the regenerative medicine and advanced therapies sector
- Reimbursement models and supportive payment structure
- We are also engaged in EU issues, such as the anticipated EU Committee for Advanced Therapies' guidelines on:
  - Advanced therapies medicinal products (ATMPs) classification procedure
  - Requirements for conducting clinical trials with ATMPs
  - Investigations with cell-based medicinal products
- Building on relationship with Japan's FIRM to better understand early market access opportunities for member companies



ALLIANCE<sup>for</sup>  
*Regenerative Medicine*

Alliance for Regenerative Medicine

Contact: Lyndsey Scull | Director, Communications | [lscull@alliancerm.org](mailto:lscull@alliancerm.org)

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