As the leading global advocate for the regenerative medicine and advanced therapies sector, ARM enables acceleration of research, development, investment and commercialization of transformational treatments and cures for patients worldwide.

- Drives regulatory, scientific and policy advancement
- Maximizes market access and reimbursement
- Enables sustainable access to capital
ARM & Informa Data Partnership

Began in 2014 to provide regularly-updated RM/AT sector trends and metrics
  • ARM quarterly and annual reports
Close collaboration has ensured a continuously refined database
  • Newly-formed companies added quarterly
Continued validation of data with other ARM members
  • Ensures accuracy and reliability
672+
Regenerative Medicine Companies Worldwide, Including Gene and Cell Therapies

185
Europe & Israel

112
Asia

349
North America

10
South America

1
Africa

15
Australia & New Zealand

Data provided by: informa
8:00am – 8:20am | Program Introduction & Industry Update

Presented by: Morrie Ruffin and Edward Lanphier, ARM

8:20am – 9:05am

The 2016 Sector Forecast: Upcoming Clinical Data Events

Featuring: AGTC, Athersys, bluebird bio, GSK, Maxim Group & Mesoblast

9:05am – 9:50am

The Promise of Regen Med & Advanced Therapies in Oncology

Featuring: Argos, Cellectis, Cell Medica, Novartis & Piper Jaffray

Clinical Progress: 2015

Anticipated Clinical Data Events: 2016


Looking Ahead: 2016
Includes both industry & investigator-initiated clinical trials

Clinical Progress: End of 2015

20 Approved RM/AT Products Worldwide

631 Total Clinical Trials

Data provided by: informa
Clinical Trials by Therapeutic Category

- More than 40% of current clinical trials are in oncology
- More than 12% are in cardiovascular
CAR-T & Other Cell-Based Immunotherapies

Major companies and research institutions active in this space:

**CAR-T**
- Bellicum Pharmaceuticals
- bluebird bio / Celgene / Baylor College of Medicine – Center for Cell & Gene Therapy
- Cellectis
- Cellular Biomedicine Group
- Celyad
- Editas Medicine (via Juno collaboration)
- Juno Therapeutics
- Kite Pharma
- MaxCyte
- Memorial Sloan Kettering Cancer Center
- Novartis / UPenn / Oxford BioMedica / GE Life Sciences / Intellia Therapeutics
- Opexa Therapeutics
- Pfizer (via Cellectis collaboration)
- Poseida/Janssen
- University College London
- Unum Therapeutics
- ZIOPHARM/Intrexon / UT Texas MD Anderson Cancer Center

**TCRs**
- Adaptimmune
- Bellicum Pharmaceuticals
- Cellular Biomedicine Group
- Editas (via Juno collaboration)
- Fate Therapeutics
- GSK (via Adaptimmune collaboration)
- Immunocore
- Juno Therapeutics
- Kite Pharma
- Nexcelmed
- Takara Bio
- Unum Therapeutics

**NK cells**
- Celyad
- Fate Therapeutics
- Gamida Cell Ltd.
- Glycostem Therapeutics
- NantKwest
- NK Therapeutics
- Cyto-sen / UT Texas MD Anderson Cancer Center

**TILs**
- Adaptive Biotechnologies
- GE Healthcare
- Lion Biotechnologies
- Tilt Biotherapeutics Ltd.
In separate trials, Juno’s investigational CAR T-Cell product candidates JCAR015 & JCAR014 demonstrate encouraging clinical responses in patients with relapsed or refractory lymphoblastic leukemia – Dec 7, 2015

Kite Pharma receives FDA Breakthrough Therapy designation for KTE-C19 for treatment of refractory, aggressive non-Hodgkin lymphoma – Dec 7, 2015

Novartis releases new CTL019 phase II data demonstrating 93% complete remission in pediatric patients with relapsed/refractory acute lymphoblastic leukemia – Dec 7, 2015

Great Ormond Street Hospital announces world’s first use of gene-edited immune cells to treat ‘incurable’ acute lymphoblastic leukemia in one-year-old child, use of Cellectis’ TALEN gene edited allogeneic UCART19 product candidate – Nov 5, 2015
Gene therapy programs

- Spark Therapeutics announces positive top-line results from pivotal phase III trial of SPK-RPE65 for genetic blinding conditions – Oct 5, 2015
- uniQure announces preliminary topline results from low-dose cohort in hemophilia B phase I/II gene therapy clinical trial – Jan 7, 2016
- GSK, Fondazione Telethon and Ospedale San Raffaele submit applications to EMA for gene therapy to treat rare immunodeficiency disease ADA-SCID – May 5, 2015
- Dimension Therapeutics, Inc. announces FDA Fast Track designation for lead candidate DTX101 in patients with hemophilia B – Sept 17, 2015
- Audentes Therapeutics, Inc. receives orphan drug designation from the U.S. & EU’s EMA for AT001 for treatment of x-linked myotubular myopathy – Aug 26, 2015
- Sangamo BioSciences announces FDA clearance of IND application for SB-FIX, the first in vivo protein replacement platform for treatment of hemophilia B – Dec 1, 2015
- Baxalta reports continued progress on Phase I/2 clinical trial of BAX335, investigational gene therapy treatment for hemophilia B – June 24, 2015
TiGenix announces Cx601 meets primary endpoint in pivotal phase III ADMIRE-CD trial in Crohn’s patients with complex perianal fistulas – Aug 23, 2015

Mesoblast Limited licensee JCR Pharmaceuticals Co. Ltd. Receives full product approval in Japan – Sept 17, 2015

The European Commission approves Chiesi’s autologous stem cell product Holoclar for the treatment of patients with severe cornea damage – Feb 20, 2015

U.S. FDA grants Fast Track designation to ReNeuron’s retinitis pigmentosa cell therapy candidate – May 22, 2015

U.S. FDA grants orphan drug designation to Pluristem’s PLX-PAD cells for treatment of severe preeclampsia – Dec 31, 2015
<table>
<thead>
<tr>
<th>Company</th>
<th>Therapy / Product</th>
<th>Therapeutic Modality</th>
<th>Indication</th>
<th>Clinical Stage</th>
<th>Expected Reporting Date</th>
</tr>
</thead>
<tbody>
<tr>
<td>GSK, Fondazione Telethon, Ospedale San Raffaele</td>
<td>GSK2696273</td>
<td>Ex vivo gene therapy therapy</td>
<td>ADA-SCID</td>
<td>EMA Marketing Approval</td>
<td>Q1 2016</td>
</tr>
<tr>
<td>Spark Therapeutics</td>
<td>SPK-RPE65</td>
<td>AAV-mediated gene therapy</td>
<td>RPE65-mediated inherited retinal dystrophies</td>
<td>Final results from Ph III trial</td>
<td>2H 2016; anticipate submitting BLA, 2H 2016</td>
</tr>
<tr>
<td>Celyad</td>
<td>CHART-1</td>
<td>Autologous stem cells</td>
<td>Congestive heart failure</td>
<td>Ph III</td>
<td>Q3 2016</td>
</tr>
<tr>
<td>Mesoblast</td>
<td>MSC-100-IV</td>
<td>MLCs</td>
<td>Steroid-refractory acute GVHD in children</td>
<td>Ph III</td>
<td>Trial completion, top line results Q3 2016</td>
</tr>
<tr>
<td>Mesoblast/Teva Pharmaceutical</td>
<td>MPC-150-IM</td>
<td>MLCs</td>
<td>Class II-III chronic heart failure</td>
<td>Ph III</td>
<td>First interim analysis results Q2 2016</td>
</tr>
<tr>
<td>Mesoblast</td>
<td>MPC-06-ID</td>
<td>MLCs</td>
<td>Chronic low back pain due to degenerative disc disease</td>
<td>Ph III</td>
<td>Enrollment completed Q4 2016 First interim analysis Q4 2016</td>
</tr>
<tr>
<td>bluebird bio</td>
<td>Lenti-D</td>
<td>Ex vivo gene therapy</td>
<td>CCALD</td>
<td>Ph II / III</td>
<td>1H 2016</td>
</tr>
<tr>
<td>Opexa Therapeutics</td>
<td>Tcelna</td>
<td>T-cell immunotherapy</td>
<td>SPMS</td>
<td>Ph IIb</td>
<td>2H 2016</td>
</tr>
<tr>
<td>Brainstorm Therapeutics</td>
<td>NurOwn</td>
<td>Autologous adult stem cell/ MSC</td>
<td>ALS</td>
<td>Ph II</td>
<td>1H 2016</td>
</tr>
<tr>
<td>Kiadis Pharma</td>
<td>ATIR101</td>
<td>Adult stem cell</td>
<td>AML, ALL, MDS</td>
<td>Ph II</td>
<td>Q2 2016</td>
</tr>
<tr>
<td>ReNeuron</td>
<td>CTX</td>
<td>Stem cell</td>
<td>Disability post-stroke</td>
<td>Ph II</td>
<td>Q2 2016</td>
</tr>
<tr>
<td>Mesoblast</td>
<td>MPC-300-IV</td>
<td>MLCs</td>
<td>Rheumatoid arthritis (biologic refractory)</td>
<td>Ph II</td>
<td>Top line first cohort data Q1 2016; full trial results Q3 2016</td>
</tr>
</tbody>
</table>
## Major Anticipated Clinical Data Events: 2016

<table>
<thead>
<tr>
<th>Company</th>
<th>Therapy / Product Name</th>
<th>Therapeutic Modality</th>
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<th>Clinical Stage</th>
<th>Expected Reporting Date</th>
</tr>
</thead>
<tbody>
<tr>
<td>Mesoblast licensee, JCR Pharmaceuticals</td>
<td>TEMCELL HS Inj.</td>
<td>MLCs</td>
<td>GVHD in children &amp; adults</td>
<td>Approved</td>
<td>To be launched in Japan Q1 2016</td>
</tr>
<tr>
<td>Sutter Neurosciences Institute (sponsored by Cord Blood Registry)</td>
<td>Autologous cord blood infusion</td>
<td>Adult stem cell</td>
<td>Autism</td>
<td>Ph II</td>
<td>Q1 2016</td>
</tr>
<tr>
<td>Histogenics</td>
<td>NeoCart</td>
<td>Tissue-engineering product</td>
<td>Cartilage</td>
<td>Ph II 5-year follow up</td>
<td>2016</td>
</tr>
<tr>
<td>Cells for Cells</td>
<td>UC-MSCs / Cellistem</td>
<td>Systemic IV administration</td>
<td>SLE</td>
<td>Ph IIa / IIb RCT; partial results</td>
<td>Q2 2016</td>
</tr>
<tr>
<td>MEDIPOST Co., Ltd.</td>
<td>PNEUMOSTEM</td>
<td>Adult stem cell</td>
<td>Prevention of Bronchopulmonary Dysplasia</td>
<td>Ph II</td>
<td>Q2 2016</td>
</tr>
<tr>
<td>GenSight Biologics</td>
<td>GS010</td>
<td>In vivo gene therapy;</td>
<td>Leber Hereditary Optic Neuropathy</td>
<td>Ph I / Ila - Safety and tolerability study with 48-week follow up</td>
<td>Q3 2016</td>
</tr>
<tr>
<td>uniQure</td>
<td>AAv5/FIX</td>
<td>In vivo gene therapy</td>
<td>Hemophilia B</td>
<td>Ph I / II</td>
<td>Q1 2016; further analysis Q2 2016</td>
</tr>
<tr>
<td>AGTC</td>
<td>XLRS</td>
<td>In vivo gene therapy</td>
<td>X-linked Retinoschisis</td>
<td>Ph I / II</td>
<td>2H 2016</td>
</tr>
<tr>
<td>Celyad</td>
<td>NKG2D</td>
<td>CAR T-cell therapy</td>
<td>Acute myeloid leukemia or multiple myeloma</td>
<td>Ph I</td>
<td>2H 2016</td>
</tr>
<tr>
<td>Athersys</td>
<td>MultiStem</td>
<td>Human stem cell</td>
<td>ARDS &amp; AMI</td>
<td>Ph II (AMI), Ph I (ARDS) – initial data</td>
<td>2016</td>
</tr>
</tbody>
</table>
Examples of Key Corporate Partnerships: 2015

- Celgene and Juno announce 10-year collaboration (with initial payment of ~$1B) to advance immunotherapies for cancer and autoimmune diseases – June 29, 2015
- Vertex Pharmaceuticals and CRISPR Therapeutics establish 4-year collaboration (valued at $2.6B, $105M upfront) to discover and develop new treatments for genetic disease – Oct 26, 2015
- uniQure N.V. and Bristol-Myers Squibb enter into exclusive strategic collaboration (valued at $2.3B, $100M upfront) to develop gene therapies for cardiovascular disease – May 26, 2015
- Voyager Therapeutics signs agreement (valued at $845M, $100M upfront) with Genzyme for CNS disorder gene therapies – February 11, 2015
- Amgen and Kite Pharma enter strategic cancer immunotherapy collaboration (valued at $1.1B, $60M upfront) to advance CAR-T cell therapies – January 5, 2015
Examples of Key IPOs: 2015

- NantKwest, Inc. closes $238.3M IPO – July 31, 2015
- Cellectis IPO raises $228M to advance its CAR-T pipeline – March 25, 2015
- Adaptimmune closes $191.3M IPO – May 11, 2015
- Spark Therapeutics IPO raises $185.2M in IPO – February 4, 2015
- REGENXBIO closes $159.4M IPO – September 22, 2015
- Aduro Biotech closes $136.9M IPO – April 20, 2015
- Celyad SA raises $100.1M with NASDAQ IPO – June 19, 2015
- Voyager Therapeutics closes $80.5M IPO – October 12, 2015

Data provided by: informa
$10.8 Billion
Total Amount Raised in 2015
$5.2 Billion raised in 2014
YoY growth of 106%

$806.8 Million
Tissue Engineering
$293.9 Million raised in 2014
YoY growth of 175%

$6.8 Billion
Gene & Gene-Modified Cell Therapy
$3.6 Billion raised in 2014
YoY growth of 84%

$7.0 Billion
Cell Therapy
$3.4 Billion raised in 2014
YoY growth of 104%

*in USD
Data provided by: informa

**Please note: total amount raised represents sector-wide figures; some companies are active in more than one technology group.
Total Financings by Type: 2015

- M&A: $2,386
- Private Equity: $131
- PIPES: $1,065
- VC Financing: $1,580
- Corporate Partnerships Upfront Pmts: $2,432
- Follow-Ons: $2,229
- IPO: $1,672

*in millions USD

Data provided by: informa
Total Financings by Type: By Year

M&A
- 2013: $570
- 2014: $2,386
- 2015: $2,689

Private Equity
- 2013: $213
- 2014: $177
- 2015: $131

PIPES
- 2013: $881
- 2014: $746
- 2015: $1,065

VC Financing
- 2013: $273
- 2014: $968
- 2015: $1,580

Corporate Partnerships Upfront Pmts
- 2013: $59
- 2014: $314
- 2015: $2,432

Follow-Ons
- 2013: $463
- 2014: $1,225
- 2015: $2,229

IPO
- 2013: $506
- 2014: $1,276
- 2015: $1,672

*in millions USD
Data provided by: informa

Up 675% YoY
Up 82% YoY
Up 63% YoY
Up 31% YoY

Data provided by: informa
Looking Ahead – ARM Focus Areas: 2016

- Global reimbursement issues – payment, coverage & & coding policies for RM/AT products
- 21st Century Cures Act / Medical Innovation Act – Senate version
  - Standards Coordinating Body
  - NIH-RAC
  - Combination products
  - Potential new pathway to market
- Gene editing & related bioethics issues
- PDUFA reauthorization recommendations
- International regulatory convergence
- Capital formation initiatives
- Patient Advocacy Roundtable series
- Increasing education and outreach to key stakeholders worldwide:
  - Legislators, policymakers & regulators
  - Patients and patient advocacy groups
  - Investor community
  - Media, and more
Special thanks to our data partner: informa

Additional thanks to ARM member: Invetech
The 2016 Sector Forecast: Upcoming Clinical Data Events

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