Welcome Remarks 2019

Janet Lynch Lambert, CEO
April 23, 2019
Resources

All presentations, talks, & panels available through our livestream:  
www.meetingonthemed.com/webcast

Recordings will be accessible shortly at  
www.meetingonthemed.com

ARM presentations, publications, webinars, and other materials:  www.alliancerm.org

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About ARM

• **International advocacy organization**
  • Dedicated to realizing the promise of safe and effective regenerative medicines for patients around the world

• **330+ members across 25 countries**
  • 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

Average Annual Growth Rate: 32% per year

331 Total Members

Membership Growth

ARM European Membership Composition

Diversity of Membership
N = 67 Organizations

- Corporations (Rev > $50 Mil) 9%
- Affiliates & Fin. Institutions 3%
- Academic 4%
- Non-Profit Institutions 12%
- Corporations (Rev < $50 Mil) 72%

Corporate Membership
N = 55 Organizations

- Cell Therapy 40%
- Gene Therapy 25%
- Cell & Gene Therapy 6%
- Tissue Engineering 8%
- Service Providers 4%
- CMOs & CROs 16%
- Cell Therapy & Tissue Eng. 1%
Inaugural Meeting on the Mediterranean

- Modeled after ARM’s **highly successful** Meeting on the Mesa event each October.
- Recognizes the **global impact** of Europe’s rapidly growing ATMP sector and provides an **opportunity for stakeholders to convene** around key issues such as clinical development, manufacturing, commercialization, patient access, and more.
- **450+ attendees**, including company executives, investors, patient advocates, and large pharma representatives.
- **500+ meetings** scheduled through the partnering system.
- **85+ industry experts** discussing topics ranging from go-to-market strategies to the latest discoveries in T-cell therapies.
- **50+ presentations** by the field’s most prominent publicly traded and emerging private companies.
Regenerative Medicine Companies Worldwide, including Gene and Cell Therapies, and Tissue Engineering Therapeutic Developers

- **910** Regenerative Medicine Companies
- **495** North America
- **233** Europe & Israel
- **13** South America
- **145** Asia
- **23** Oceania: Australia, New Zealand, Marshall Islands
- **2** Africa

Source data provided by: informa
ATMP Therapeutic Developers

233 Regenerative Medicine Companies in Europe and Israel, of 904 Companies Worldwide

Source data provided by: informa
Select Anticipated Near-Term EMA ATMP Approvals

bluebird bio’s **ZYNTEGLO**
gene therapy for the treatment for transfusion-dependent beta thalassemia
Positive CHMP Opinion received March 2019; EC decision expected end Q2

Kiadis Pharma’s **ATIR101**
gene-modified cell therapy for the treatment of leukemia
Decision expected: 1H 2019

AveXis / Novartis’s **Zolgensma**
gene therapy for the treatment of spinal muscular atrophy type 1
Decision expected: late 2019

Orchard’s **OTL-200**
gene therapy for the treatment of meta-chromatic leukodystrophy
Anticipated filing MAA in 2020
ATMP Clinical Trials
Sponsored by European and Israeli Developers

324
Active clinical trials sponsored by European and Israeli developers

Phase I: 78 across all tech types and indications

Phase II: 196 across all tech types and indications

Phase III: 50 across all tech types and indications

230 are industry-related
94 are from academic/other organizations
<table>
<thead>
<tr>
<th>Category</th>
<th>Q1 2019 Financings</th>
<th>Change from Q1 2018</th>
</tr>
</thead>
<tbody>
<tr>
<td>Total</td>
<td>€488.3M</td>
<td>▲ 143%</td>
</tr>
<tr>
<td>Gene-Based Therapies</td>
<td>€451.4M</td>
<td>▲ 198%</td>
</tr>
<tr>
<td>Cell Therapy</td>
<td>€291.1M</td>
<td>▲ 1020%</td>
</tr>
<tr>
<td>Tissue Engineering</td>
<td>€12.8M</td>
<td>▼ 46%</td>
</tr>
</tbody>
</table>

*Source data provided by: informa

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

- Improve market access to promote patient access to ATMPs
- Drive robust and effective real-world data infrastructure
- Promote policy changes to improve the clinical trial process for ATMPs
- Promote regulatory convergence of requirements for the development and commercialization of ATMPs
Thank You!
Dr. Guido Rasi
Director General
European Medicines Agency