Advancements in Gene Therapy
Tearing Down Barriers to Discovery in Rare Disease

Lyndsey Scull, Vice President, Communications
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

• **330+ members**
  • Small and large companies, non-profit research institutions, patient organizations, and other sector stakeholders
  • More than one-third of therapeutic developers who are members of ARM are active in rare disease

• **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*
Global Sector Overview
Current Global Sector Landscape: All Regenerative Medicine

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

484 North America

241 Europe & Israel

142 Asia

23 Oceania: Australia, New Zealand, Marshall Islands

1 Africa

Source data provided by: informa
Current Global Sector Landscape: All Gene-Based Medicine

Gene Therapy & Gene-based Medicine Companies Worldwide

- **North America**: 214 companies
- **South America**: 2 companies
- **Europe & Israel**: 104 companies
- **Asia**: 73 companies
- **Oceania**: 7 companies (Australia, New Zealand, Marshall Islands)

Source data provided by: informa
Current Global Sector Landscape: Gene-Based Medicine for Rare Disease

205
Gene Therapy & Gene-Based Medicine Companies Active in Rare Disease

North America
102

Europe & Israel
47

Asia
54

Oceania
2
Australia, New Zealand, Marshall Islands

Source data provided by: informa
Clinical Progress
<table>
<thead>
<tr>
<th>Select Anticipated Near-Term Approvals: Gene Therapy for Rare Disease</th>
</tr>
</thead>
<tbody>
<tr>
<td>AveXis / Novartis’s <strong>Zolgensma</strong>, a gene therapy for the treatment of spinal muscular atrophy type 1</td>
</tr>
<tr>
<td>Decision expected: 2019 (Japan/EU)</td>
</tr>
<tr>
<td>bluebird bio’s <strong>Zynteglo</strong>, a gene therapy for the treatment of beta thalassemia</td>
</tr>
<tr>
<td>Expects to file (US) in 2019</td>
</tr>
<tr>
<td>GenSight’s <strong>GS010</strong>, a gene therapy for the treatment of Leber hereditary optic neuropathy</td>
</tr>
<tr>
<td>Expects to file: 2019 (EU), 2020 (US)</td>
</tr>
<tr>
<td>Orchard Therapeutics’ <strong>OTL-101</strong>, a gene therapy for the treatment of ADA Deficiency / ADA-SCID</td>
</tr>
<tr>
<td>Expects to file: 2020 (US)</td>
</tr>
<tr>
<td>Kiadis Pharma’s <strong>ATIR101</strong>, a gene modified cell therapy for the treatment of leukemia</td>
</tr>
<tr>
<td>Decision expected: 1H 2019 (EU)</td>
</tr>
<tr>
<td>PTC Therapeutics’ <strong>GT-AADC</strong>, a gene therapy for the treatment of aromatic L-amino acid decarboxylase (AADC) deficiency</td>
</tr>
<tr>
<td>Expects to file: late 2019 (US)</td>
</tr>
<tr>
<td>Orchard Therapeutics’ <strong>OTL-200</strong>, a gene therapy for the treatment of meta-chromatic leukodystrophy</td>
</tr>
<tr>
<td>Expects to file: 2020 (US &amp; EU)</td>
</tr>
<tr>
<td>Poseida’s <strong>P-BMCA-101</strong>, a CAR-T therapy targeting r/r multiple myeloma</td>
</tr>
<tr>
<td>Expects to file: EOY 2020 (US)</td>
</tr>
</tbody>
</table>

Full list of anticipated near-term clinical milestones & data readouts available online: https://alliancerm.org/anticipated-data-events
Gene Therapy Clinical Trials in Rare Disease
As of End 2018

TOTAL: 505

Phase 1: 182
Gene Therapy: 61
Gene-Modified Cell Therapy: 121

Phase 2: 289
Gene Therapy: 141
Gene-Modified Cell Therapy: 148

Phase 3: 34
Gene Therapy: 22
Gene-Modified Cell Therapy: 12

Source data provided by: informa
Gene Therapy Clinical Trials for Rare Disease
As of End 2018

72% of gene therapy clinical trials for rare disease are in rare cancers, including hematological malignancies, ovarian cancers, pancreatic cancers, lung cancers, glioblastoma, and others.

6% are in hematological disorders, including hemophilia, sickle cell disease, thalassemia, Fanconi’s anemia, and others.

Source data provided by: informa
Sector Financings
Global Financings

Total Global Financings
All Technologies, Companies Active in Rare Disease

$9.7B in 2018

+48% increase YoY from 2017

Total Global Gene-Based Therapies Financings
Companies Active in Rare Disease

$8.2B in 2018

+39% increase YoY from 2017

Source data provided by: informa
Thank you!

To access these slides, as well as other ARM presentations, publications, and sector information, please visit: www.alliancerm.org