

Advancements in Gene Therapy

Tearing Down Barriers to Discovery in Rare Disease

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Alliance for Regenerative Medicine

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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**
 - 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**

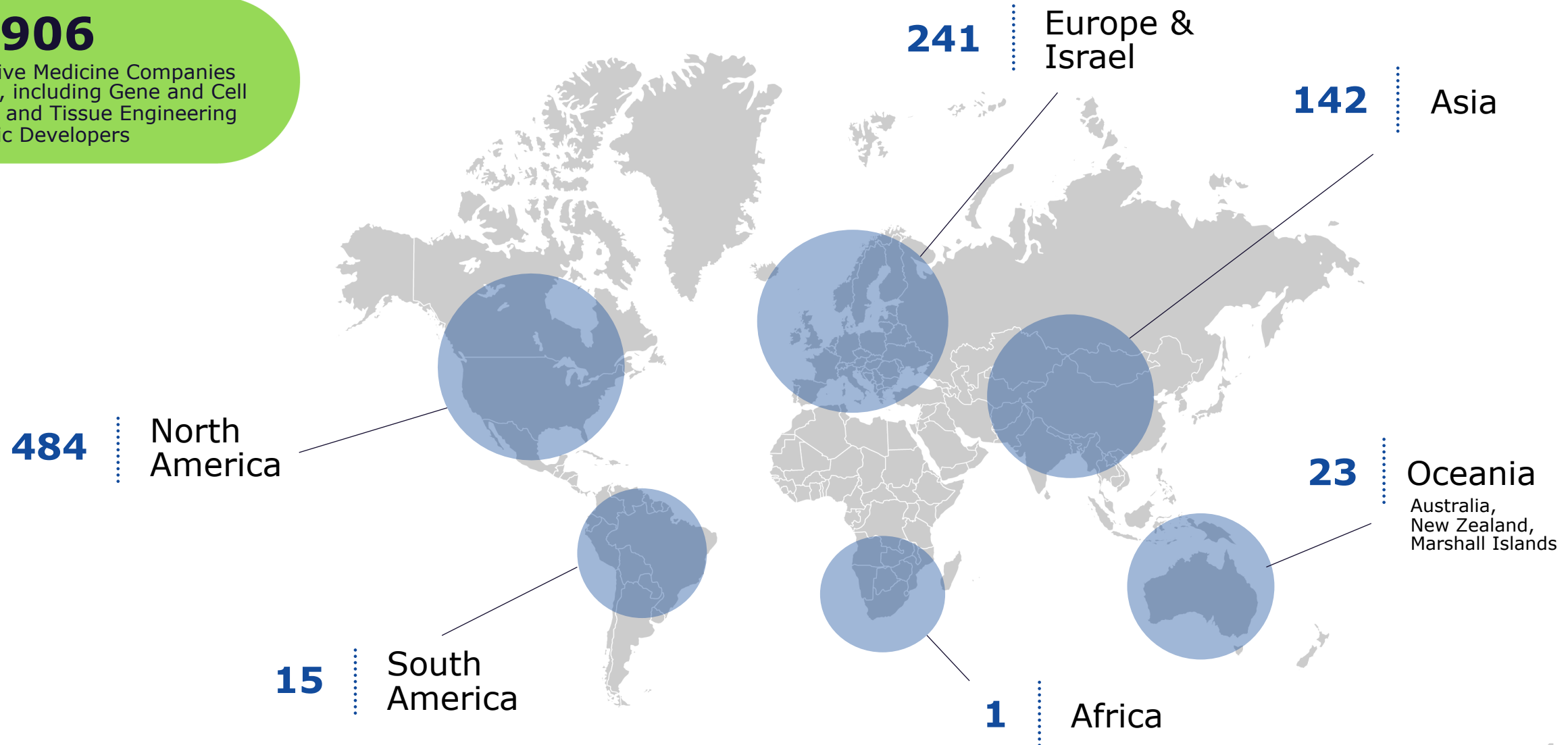
Global Sector Overview



Current Global Sector Landscape: All Regenerative Medicine

906

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



Current Global Sector Landscape: All Gene-Based Medicine

400

Gene Therapy & Gene-based Medicine
Companies Worldwide

214

North
America

2

South
America

104

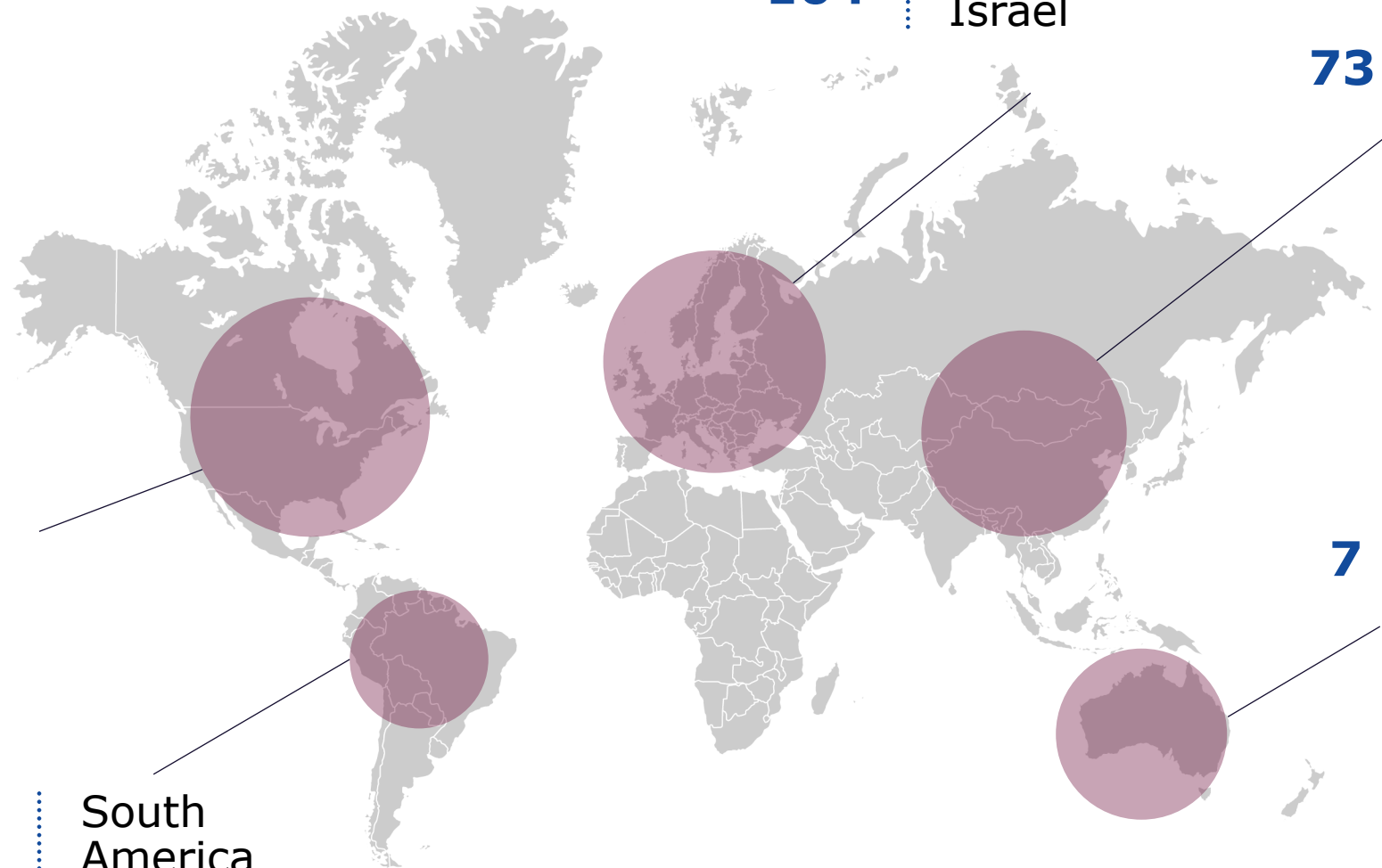
Europe &
Israel

73

Asia

7

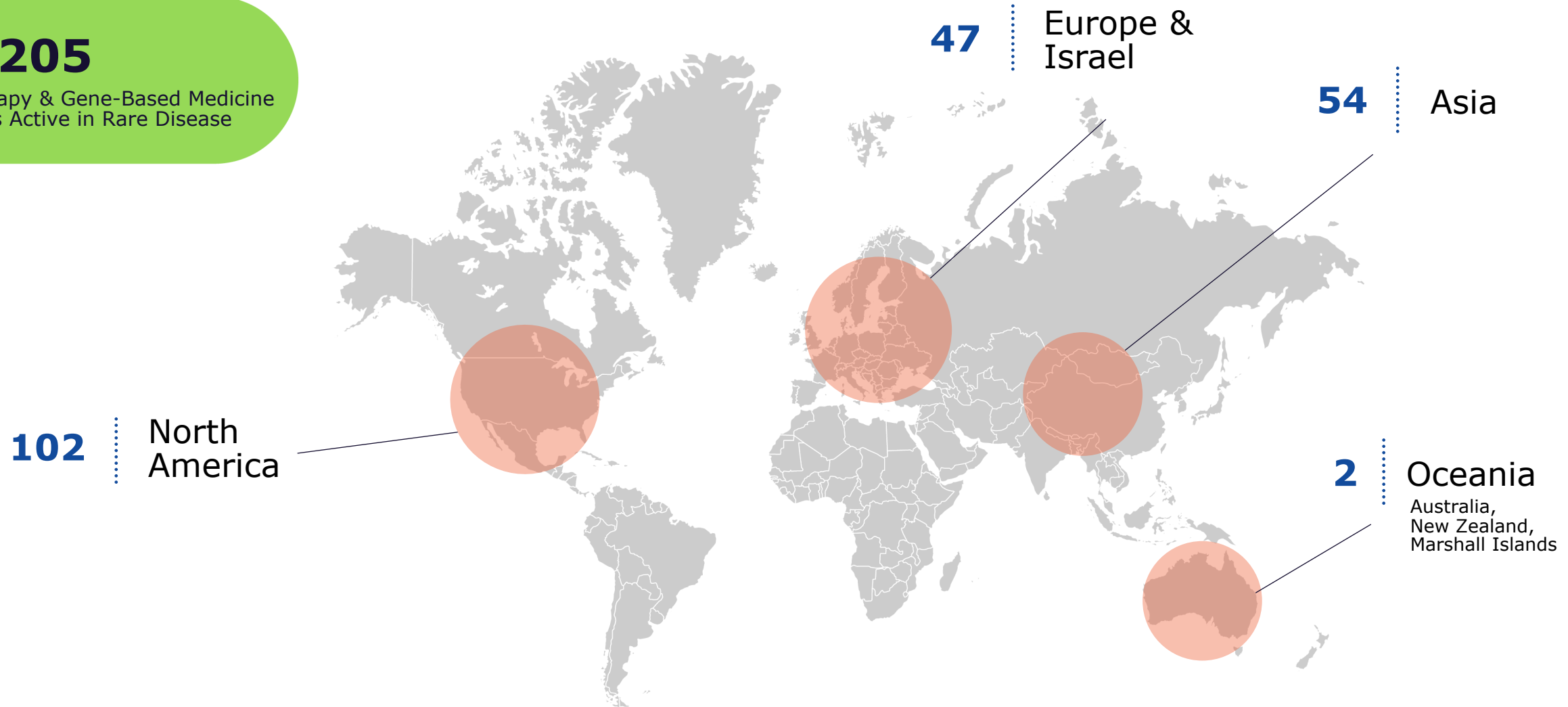
Oceania
Australia,
New Zealand,
Marshall Islands



Current Global Sector Landscape: Gene-Based Medicine for Rare Disease

205

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



Clinical Progress

Select Anticipated Near-Term Approvals: Gene Therapy for Rare Disease



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



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



bluebird bio's **Zynteglo**,
a gene therapy for the
treatment of beta thalassemia
Expects to file (US) in 2019



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



GenSight's **GS010**, a gene therapy
for the treatment of Leber hereditary
optic neuropathy
Expects to file: 2019 (EU), 2020 (US)



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



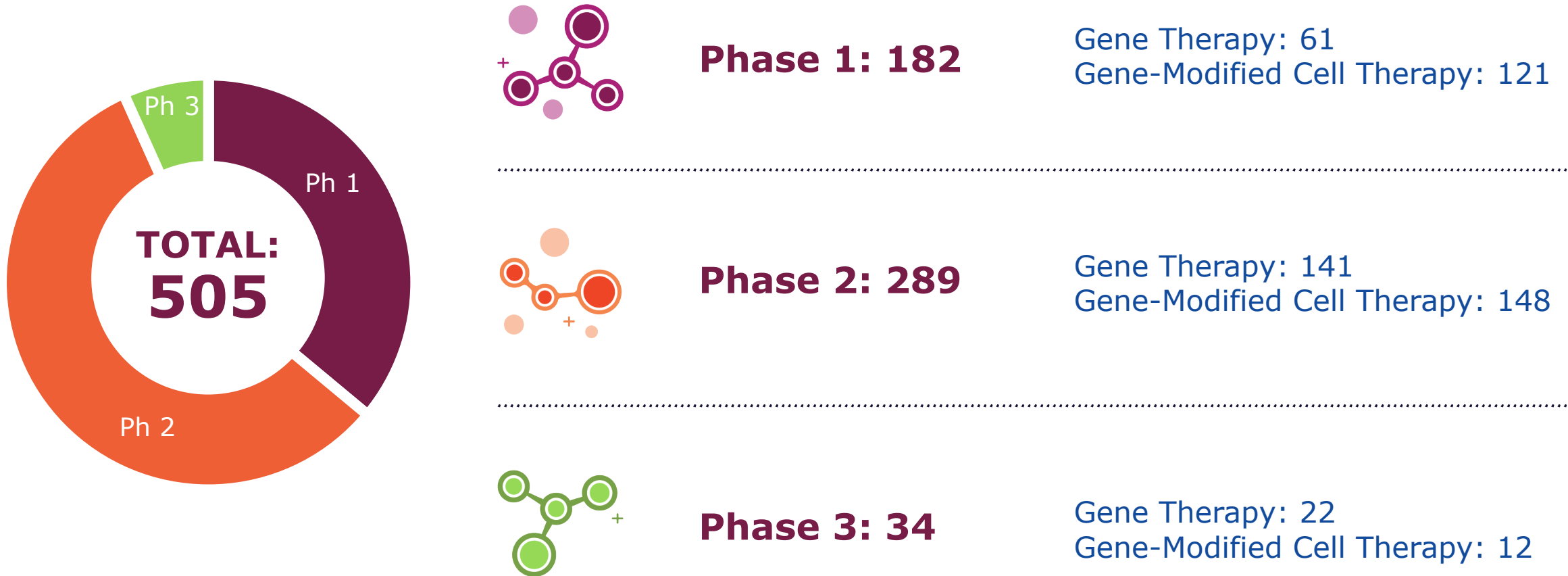
Orchard Therapeutics' **OTL-101**, a
gene therapy for the treatment of
ADA Deficiency / ADA-SCID
Expects to file: 2020 (US)



Poseida's **P-BMCA-101**, a CAR-T
therapy targeting r/r multiple
myeloma
Expects to file: EOY 2020 (US)

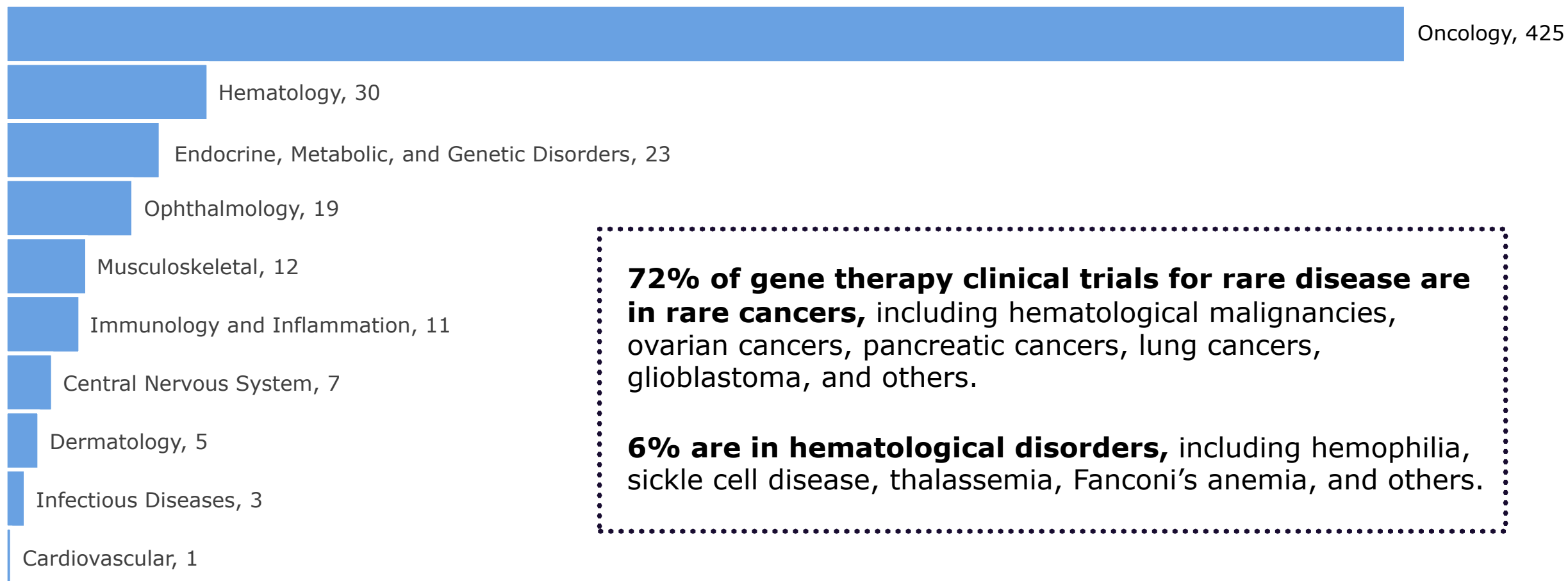
Gene Therapy Clinical Trials in Rare Disease

As of End 2018

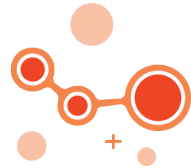


Gene Therapy Clinical Trials for Rare Disease

As of End 2018



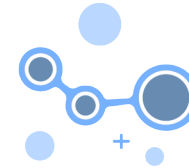
Sector Financings



\$9.7B
in 2018

Total Global Financings
All Technologies, Companies
Active in Rare Disease

+48%
increase YoY from 2017



\$8.2B
in 2018

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

+39%
increase YoY from 2017

Thank you!

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