

Cell & Gene Meeting on the Mediterranean

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

Follow Us on Twitter!

@alliancerm

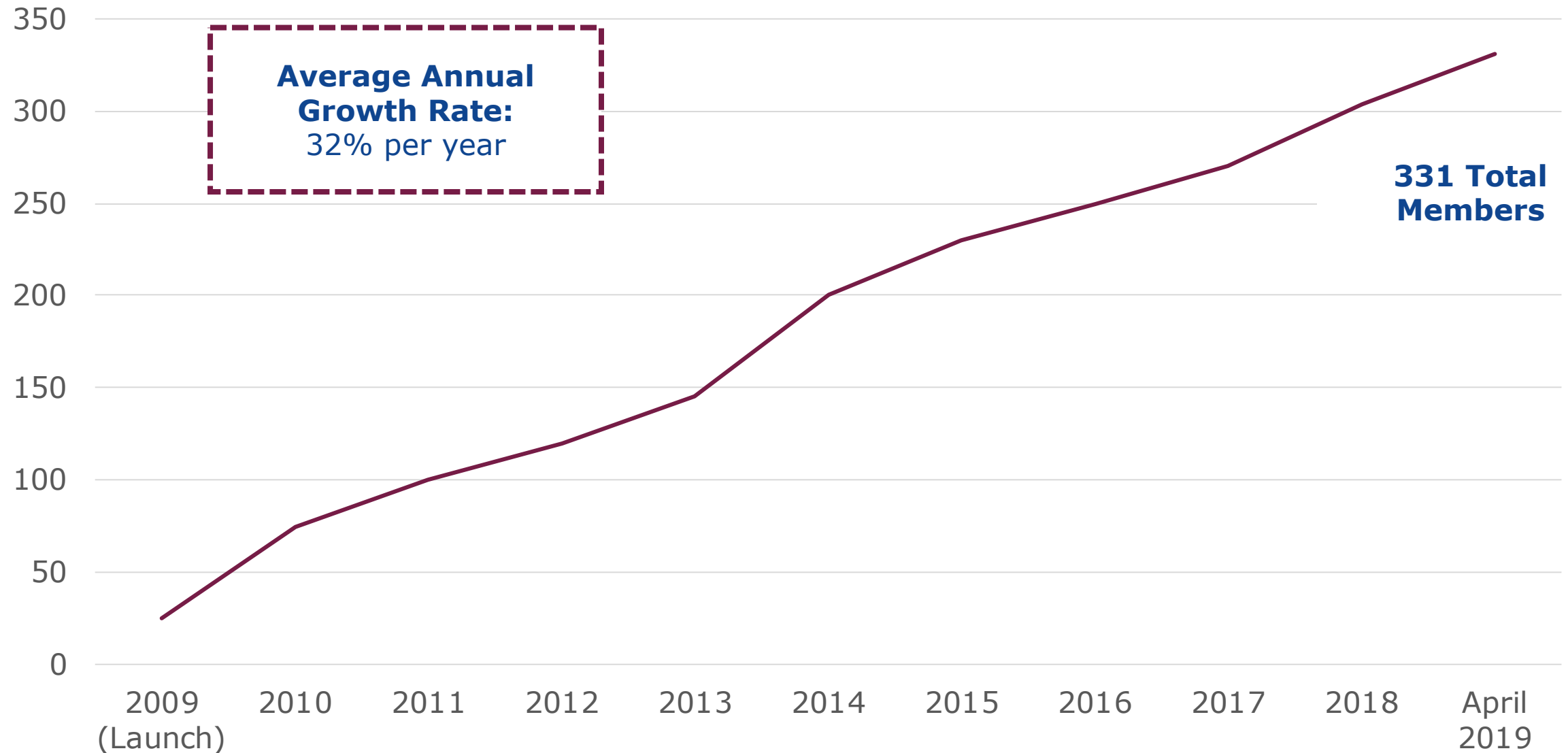
@JLynchLambert

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

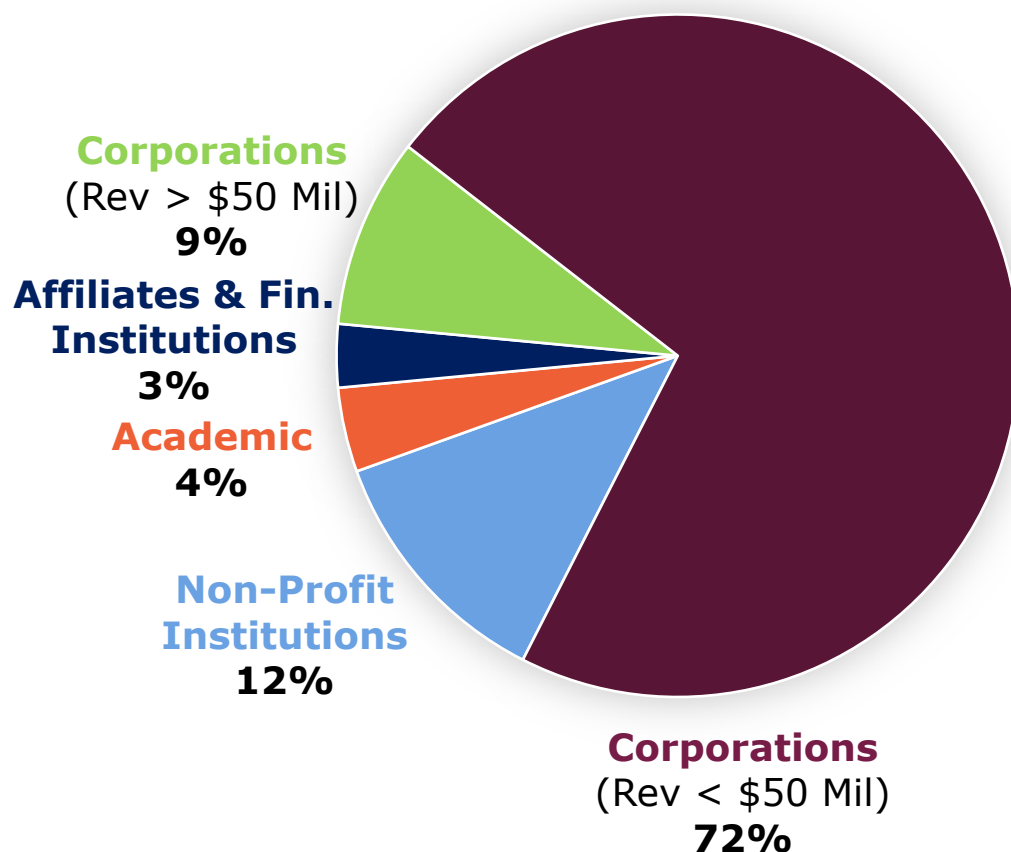
Membership Growth



ARM European Membership Composition

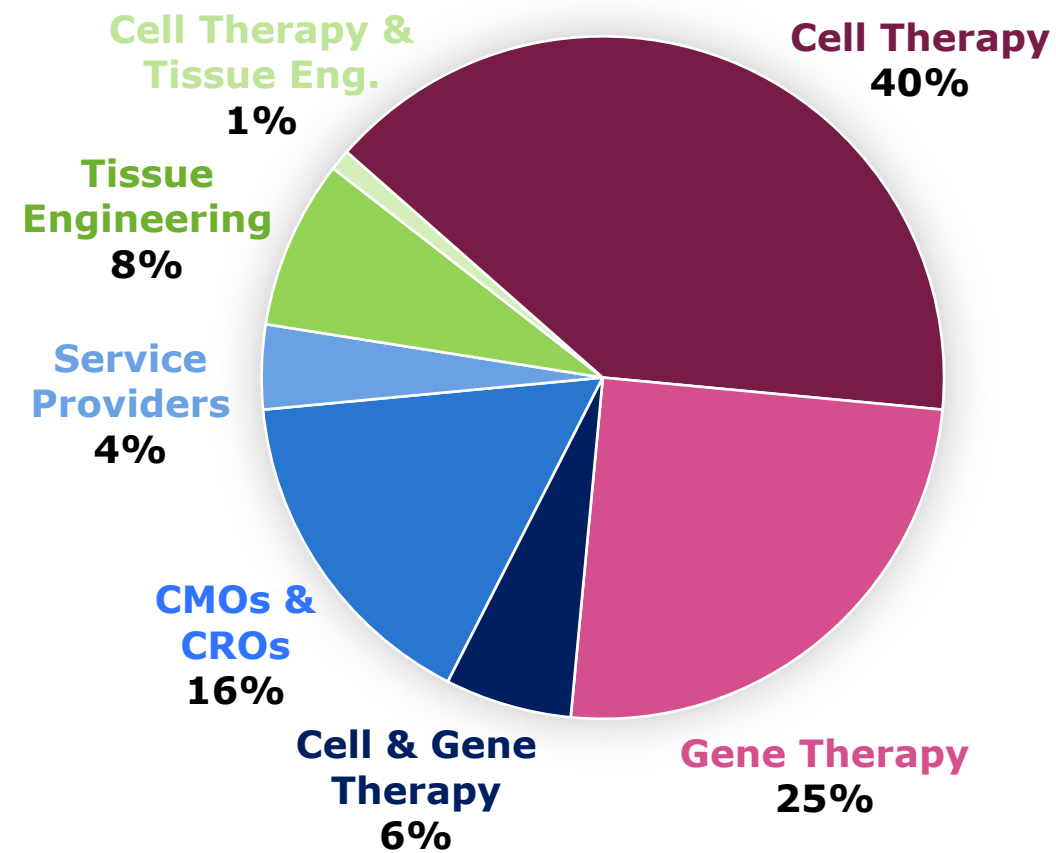
Diversity of Membership

N = 67 Organizations



Corporate Membership

N = 55 Organizations



Inaugural Meeting on the Mediterranean

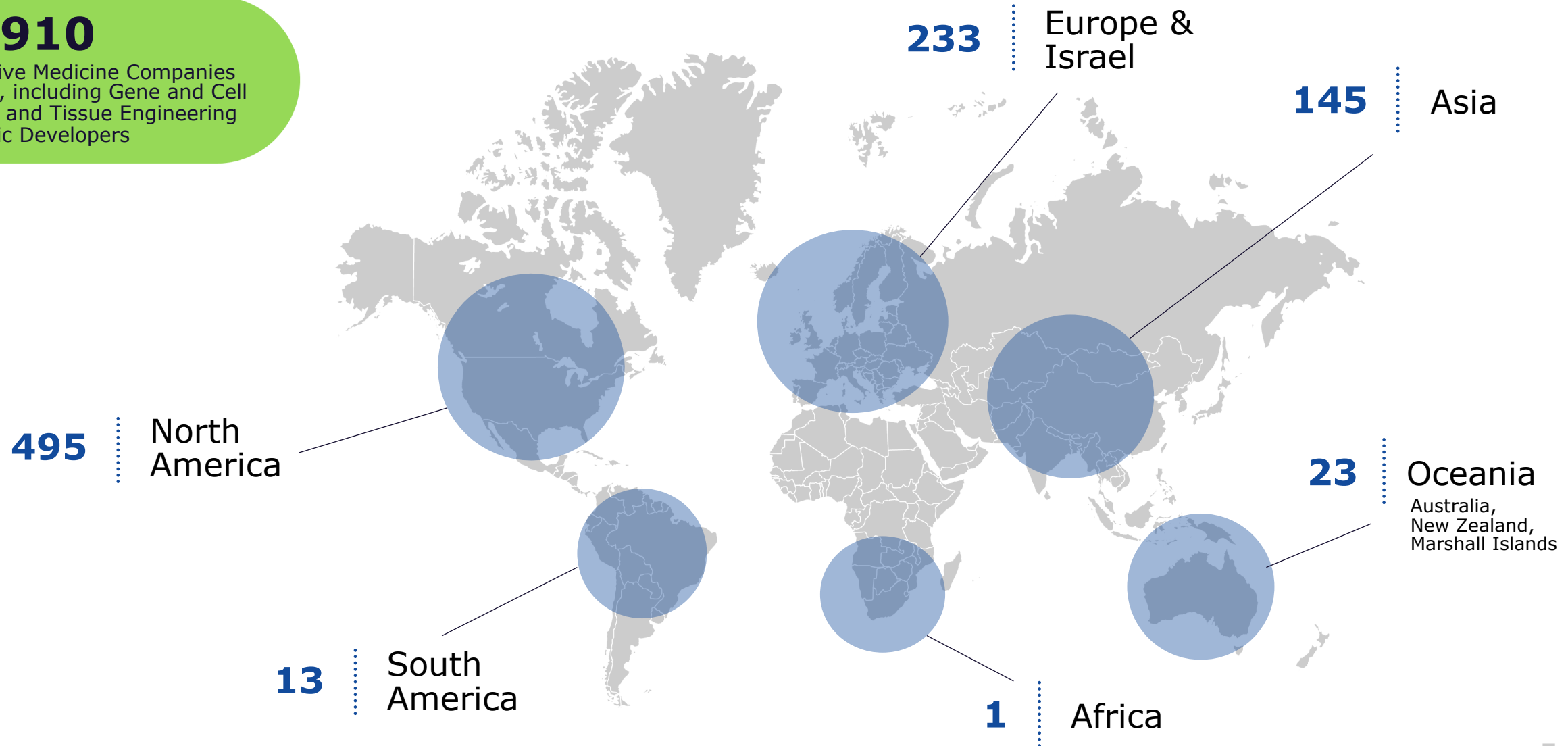


- Modeled after ARM's **highly successful** Meeting on the Mesa event each October.
- Recognizes the **global impact** of Europe's rapidly growing ATMP sector and provides an **opportunity for stakeholders to convene** around key issues such as clinical development, manufacturing, commercialization, 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.

Current Global Sector Landscape

910

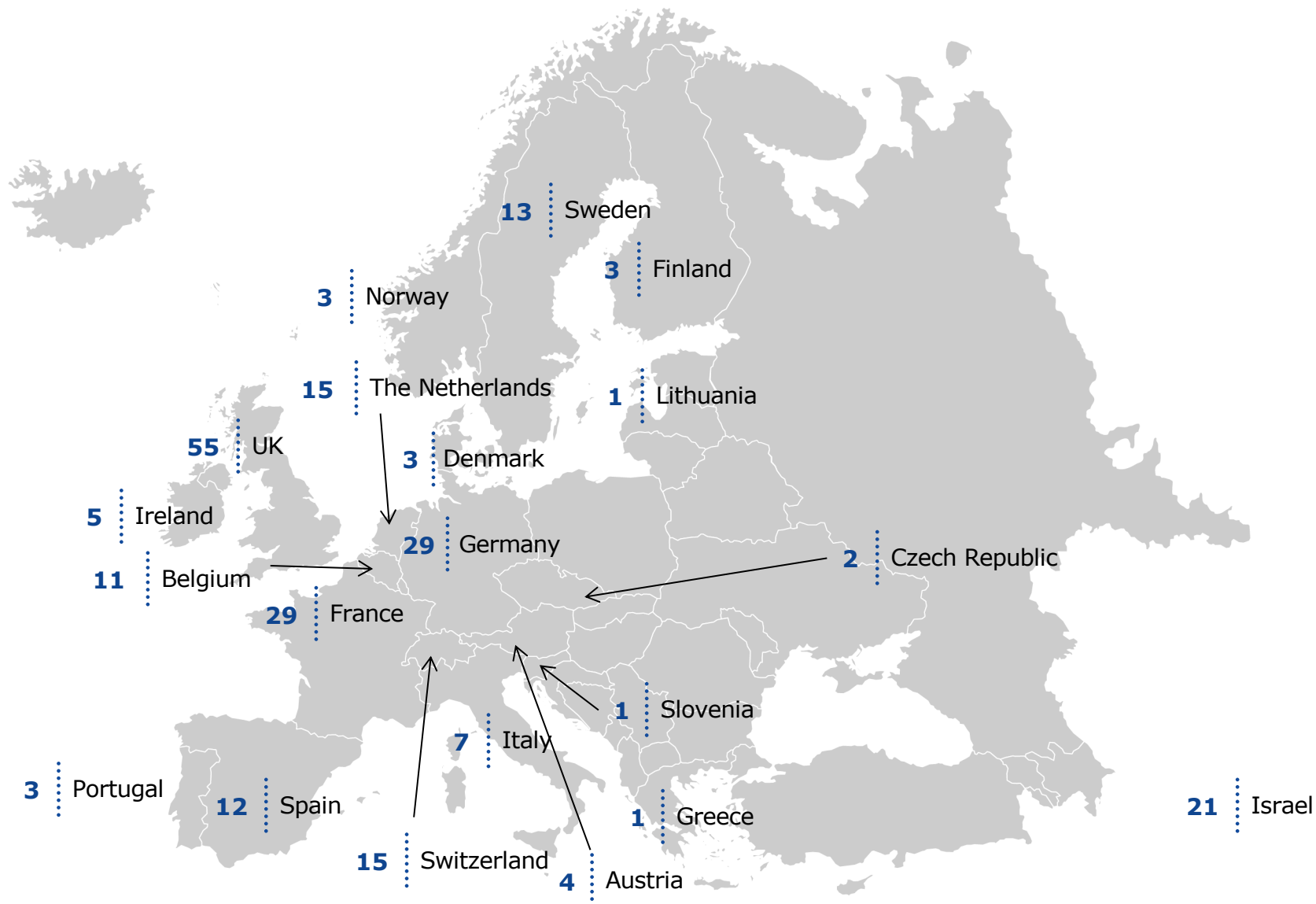
Regenerative Medicine Companies
Worldwide, including Gene and Cell
Therapies, and Tissue Engineering
Therapeutic Developers



ATMP Therapeutic Developers

233

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



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



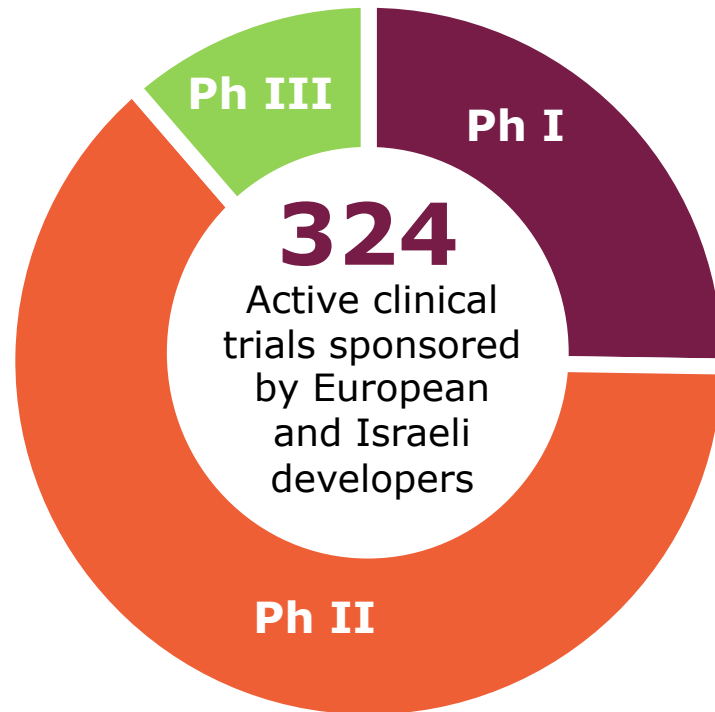
AveXis / Novartis's
Zolgensma
gene therapy for the treatment of
spinal muscular atrophy type 1
Decision expected:
late 2019



Orchard's
OTL-200
gene therapy for the treatment of
meta-chromatic leukodystrophy
Anticipated filing MAA in
2020

ATMP Clinical Trials

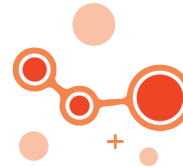
Sponsored by European and Israeli Developers



230 are industry-related
94 are from academic/other organizations



Phase I: 78
across all tech types
and indications

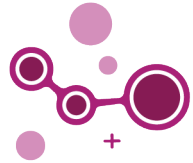


Phase II: 196
across all tech types
and indications



Phase III: 50
across all tech types
and indications

Total Financings: Europe & Israel Q1 2019



€488.3M

**Total Q1 2019
Financings**

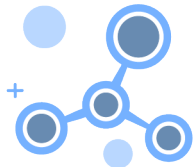
▲ 143%
from Q1 2018



€451.4M

**Gene-Based Therapies
Q1 2019 Financings**

▲ 198%
from Q1 2018



€291.1M

**Cell Therapy
Q1 2019 Financings**

▲ 1020%
from Q1 2018



€12.8M

**Tissue Engineering
Q1 2019 Financings**

▼ 46%
from Q1 2018

 **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

