Sector Overview

Gene Therapy for Rare Disorders

Morrie Ruffin, Co-Founder & Senior Adviser; Alliance for Regenerative Medicine
& Executive Director, Alliance for Regenerative Medicine Foundation

Lyndsey Scull, Vice President, Communications, Alliance for Regenerative Medicine

Alliance *for* Regenerative Medicine

March 28, 2019

About ARM

- **International advocacy organization**
  - Dedicated to realizing the promise of safe and effective regenerative medicines for patients around the world
- **300 + 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**

Alliance for Regenerative Medicine

State of the Industry

- Global Sector Overview
- Clinical Progress
- Anticipated Clinical Events
- Sector Financings
- Policy Environment
- Projects and Initiatives

A Quick Note -

This presentation will be available via:

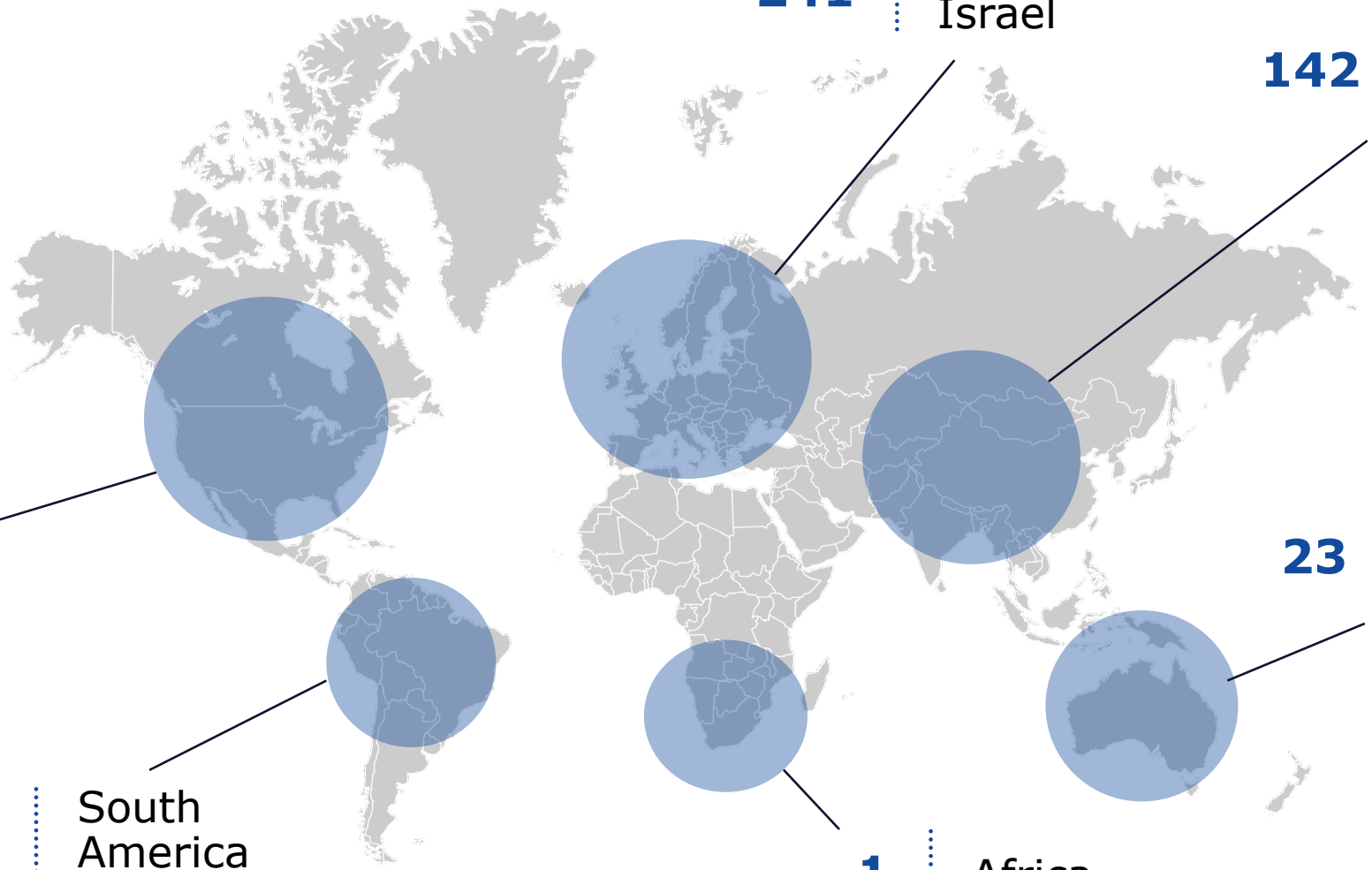
- **ARM's website: www.alliancerm.org**
- **Twitter @alliancerm**

Global Sector Overview

Alliance for Regenerative Medicine

Current Global Sector Landscape

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



241 Europe & Israel

142 Asia

484 North America

23 Oceania
Australia, New Zealand, Marshall Islands

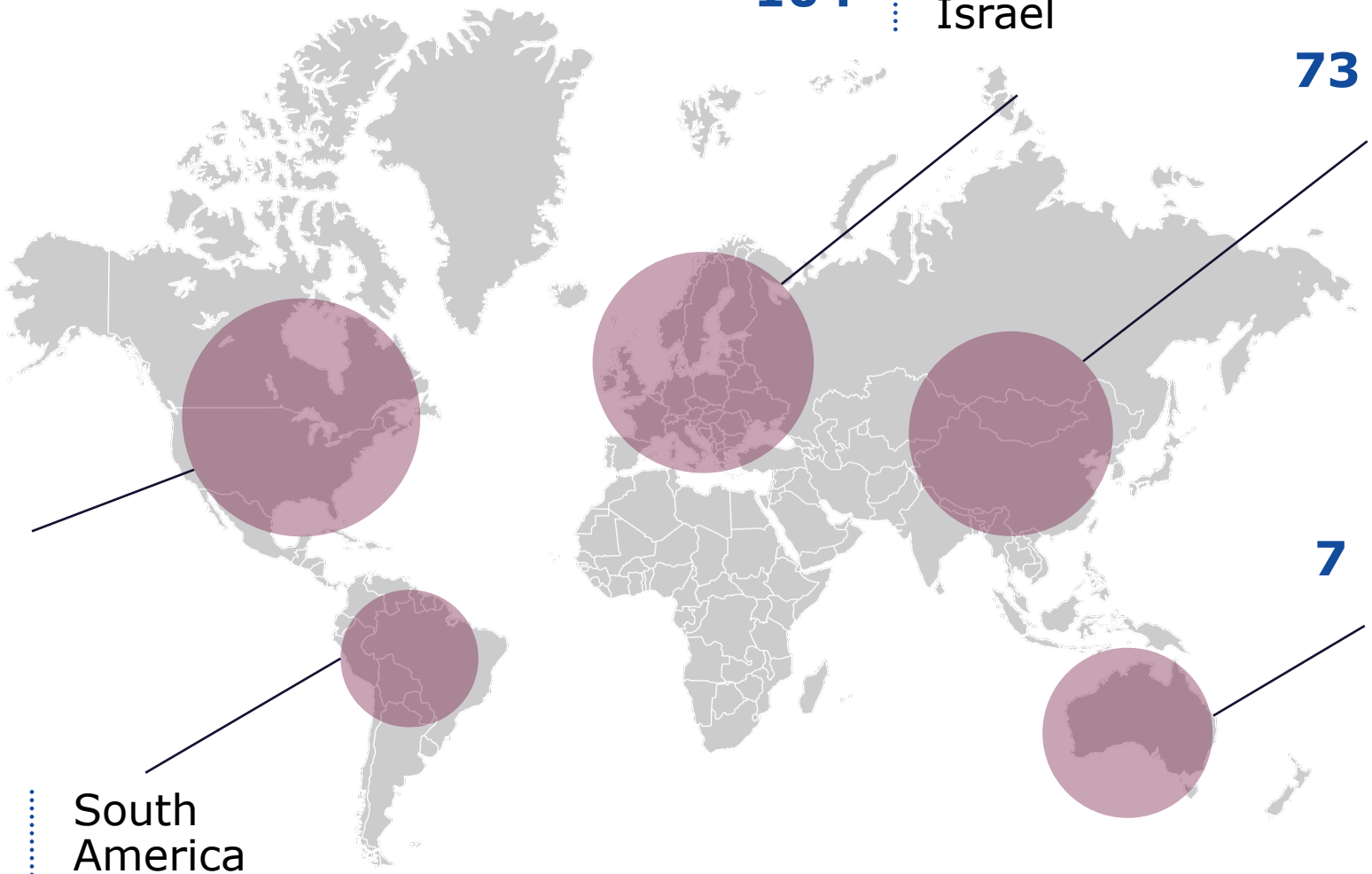
15 South America

1 Africa

Source data provided by: informa

Current Global Sector Landscape

400
Gene Therapy & Gene-based Medicine Companies Worldwide



104 Europe & Israel

73 Asia

214 North America

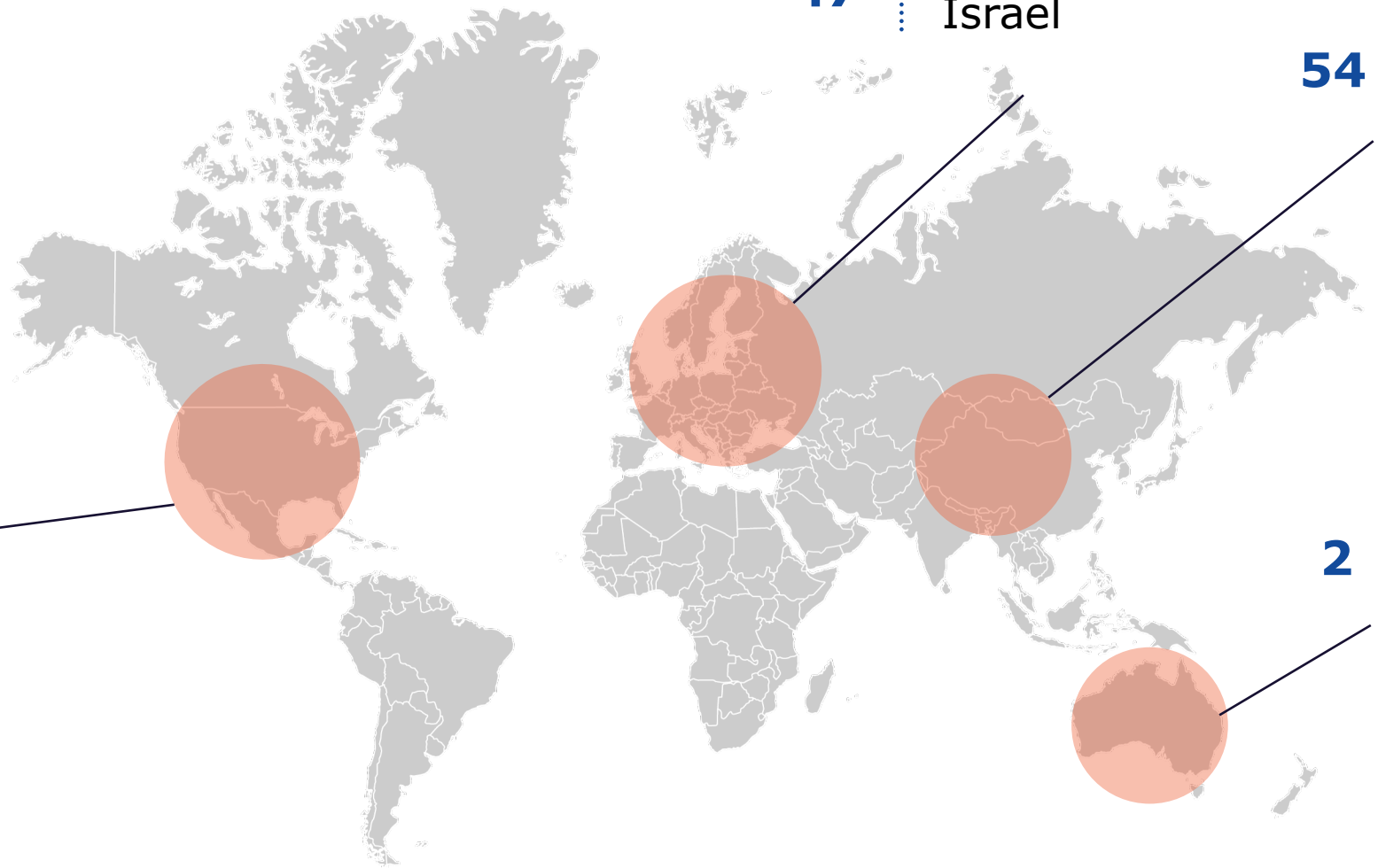
7 Oceania
Australia, New Zealand, Marshall Islands

2 South America

Source data provided by: informa

Current Global Sector Landscape

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



47 Europe & Israel

54 Asia

102 North America

2 Oceania
Australia, New Zealand, Marshall Islands

Source data provided by: informa

Major Therapeutic Platforms & Enabling Technologies

- **Viral vectors:** retroviruses, adenoviruses, herpes simplex, vaccinia, and adeno-associated virus (AAV)
- **Non-viral vectors:** nanoparticles, nanospheres, transposons, electroporation, and others
- **Genetically modified cell therapies:** chimeric antigen receptors (CAR) T cell therapies, T cell receptor (TCR) therapies, natural killer (NK) cell therapies, tumor infiltrating lymphocytes (TILs), marrow derived lymphocytes (MILs), gammadelta T cells, and dendritic vaccines.
- **Genome editing:** meganucleases, homing endonucleases; zinc finger nucleases (ZFNs); transcription activator-like effector-based nucleases (TALEN); nucleases such as Cas9 and Cas12a that derive from the Clustered Regularly Interspaced Short Palindromic Repeats (CRISPR/Cas); homologous recombination of adeno-associated virus (AAV)-derived sequences.
- **Next-gen expression constructs:** novel capsids; innovative regulatory elements, including synthetic promoters that enable specificity, strength, and improve capacity; inducible elements to regulate gene expression temporally or in response to external stimuli: molecular kill switches to improve safety; etc.

Clinical Progress

Alliance for Regenerative Medicine

Product Approvals in 2018 – Gene Therapies for Rare Diseases

- Spark Therapeutics' **LUXTURNA** gene therapy for biallelic RPE65-mediated inherited retinal disease received EC approval – November 23

- Gilead / Kite Pharma's **Yescarta** cell therapy received approval from the European Commission for the treatment of DLBCL– August 27; approval from the European Commission to treat adult patients with r/r DLBCL and PMBCL – August 27

- Novartis's **Kymriah** cell therapy received FDA approval for a second indication: treatment of adult patients with r/r large B-cell lymphoma – May 1; approval from the European Commission for adult patients with r/r DLBCL and patients under the age of 25 with ALL – August 27; approval from the Australian TGA for adult patients with r/r DLBCL and patients under the age of 25 with ALL – December 18

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
Decisions expected mid-2019

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

bluebird bio's **LentiGlobin**, a gene therapy for the treatment of beta thalassemia
Decision expected: mid-2019 (EU)
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)

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

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

Full list of anticipated near-term clinical milestones & data readouts available online: https://alliancerm.org/anticipated-data-events

Select Anticipated Data Readouts: 2019

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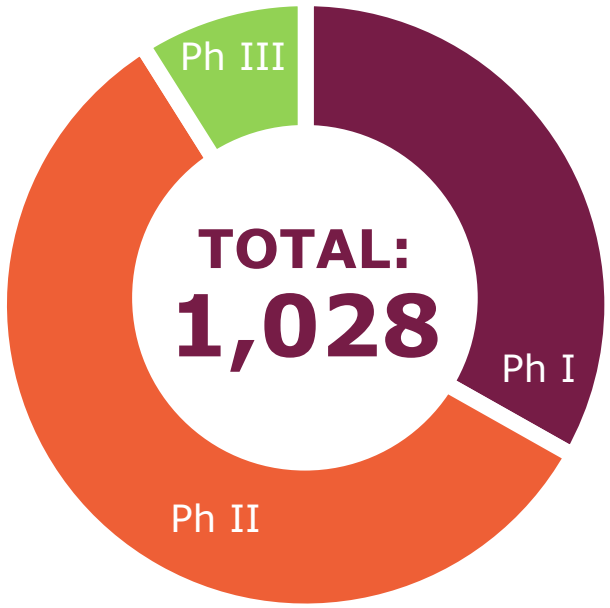
Company	Product	Therapeutic Modality	Indication	Clinical Stage	Expected Reporting Date
GenSight Biologics	GS010	AAV-vector Gene Therapy	Leber Hereditary Optic Neuropathy	Ph III	Topline data Q1 2019
Audentes Tx	AT342	Gene therapy	Crigler-Najjar Syndrome	Ph I/II	Interim data Q1 2019
Axovant	AXO-AAV-GM2	Gene therapy	GM2 gangliosidosis	Ph I	Initial data Q1 2019
Fibrocell	FCX-007	Gene Therapy	Recessive Dystrophic Epidermolysis Bullosa	Ph I/II	Interim data Q1 2019
Pfizer	PF-06939926	Gene therapy	Duchenne Muscular Dystrophy	Ph Ib	Early data 1H 2019
Celyad	CYAD-01	CAR-T therapy	R/r AML/MDS	Ph I	Preliminary data mid-2019
REGENXBIO	RGX-121	Gene therapy	MPS II	Ph I/II	2019
Homology Medicines	HMI-102	Gene therapy	Phenylketonuria	Pre-Ph I	Initial data 2019

Source: Company-provided or publicly-available information

Regenerative Medicine Clinical Trials



Phase I: 341
across all tech types
and indications

Gene Therapy: 120
Gene-Modified Cell Therapy: 158
GT & GMCT Total: 278

Phase II: 595
across all tech types
and indications

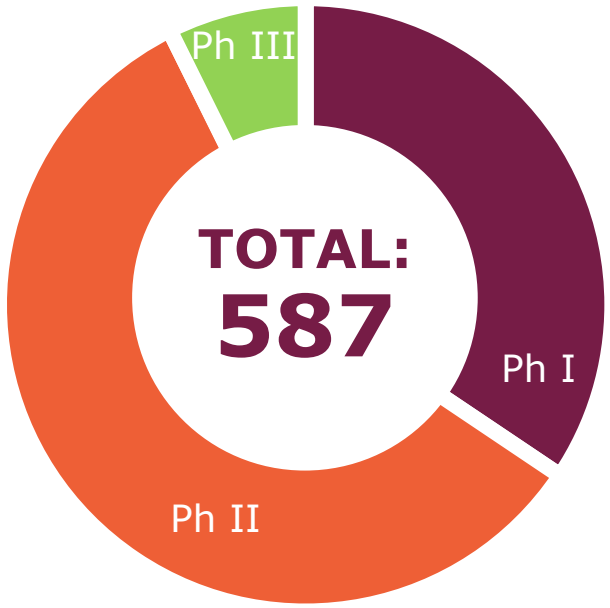
Gene Therapy: 210
Gene-Modified Cell Therapy: 188
GT & GMCT Total: 398

Phase III: 92
across all tech types
and indications

Gene Therapy: 32
Gene-Modified Cell Therapy: 16
GT & GMCT Total: 48

Source data provided by: informa

Regenerative Medicine Clinical Trials in Rare Disease



Phase I: 202
across all tech types
in rare disease

Gene Therapy: 61
Gene-Modified Cell Therapy: 121
GT & GMCT Total: 182

Phase II: 342
across all tech types
in rare disease

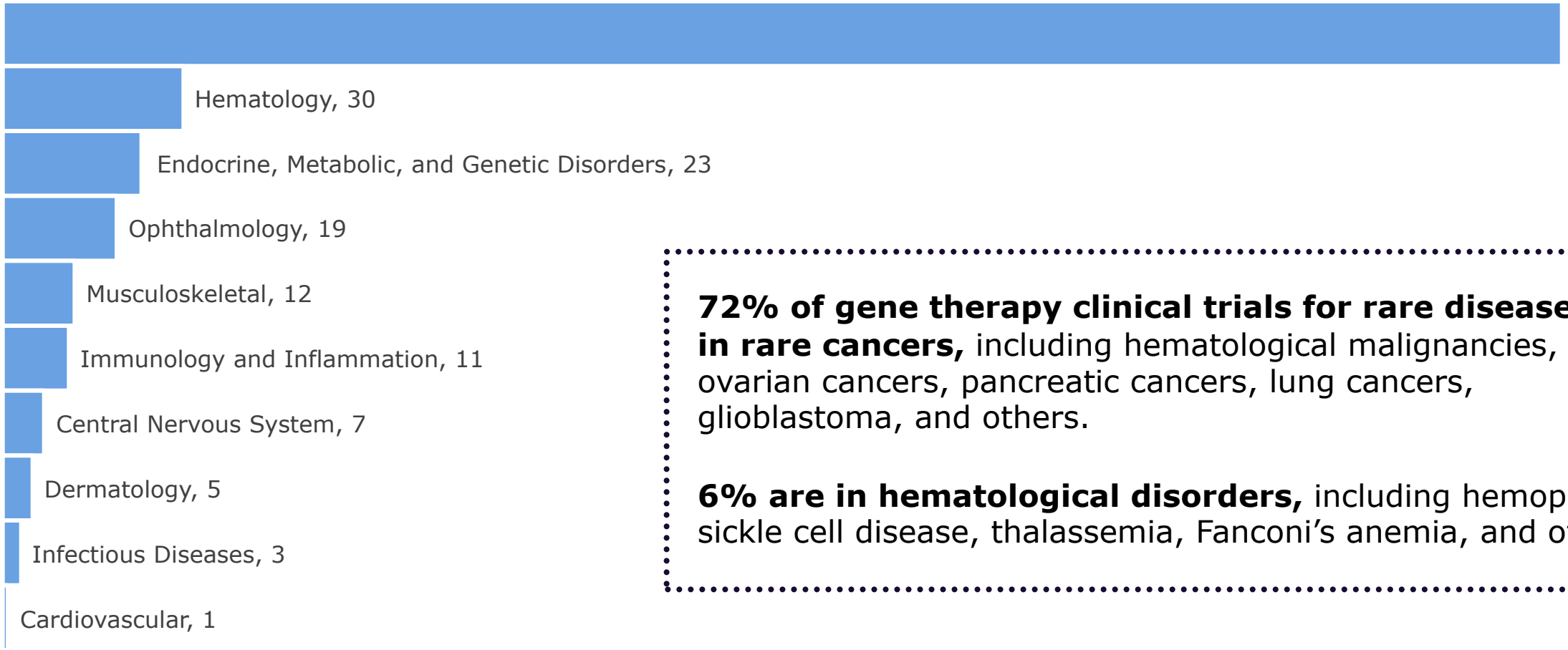
Gene Therapy: 141
Gene-Modified Cell Therapy: 148
GT & GMCT Total: 289

Phase III: 43
across all tech types
in rare disease

Gene Therapy: 22
Gene-Modified Cell Therapy: 12
GT & GMCT Total: 26

Source data provided by: informa

Gene Therapy Clinical Trials for Rare Disease



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.

Sector Financings

Alliance for Regenerative Medicine

Global Financings

$13.3B
2018

Total Global Financings
All Technologies & Indications

+73%
increase from 2017

$9.7B
2018

Total Global Financings
All Technologies, Companies Active in Rare Disease

+48%
increase from 2017

$9.7B
2018

Total Global Gene-Based Therapies Financings
All Indications

+64%
increase from 2017

$8.2B
2018

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

+39%
increase from 2017

Policy Environment

Alliance for Regenerative Medicine

Supportive Regulatory Environment

*"We anticipate that by 2020 we will be receiving **more than 200 INDs per year**, building upon our total of more than 800 active cell-based or directly administered gene therapy INDs currently on file with the FDA. And by 2025, we predict that the FDA will be approving **10 to 20 cell and gene therapy products a year** based on an assessment of the current pipeline and the clinical success rates of these products."*

- FDA Commissioner Scott Gottlieb and CBER Director Peter Marks, January 2019

The FDA is actively involved in creating a positive regulatory environment for regenerative medicines and advanced therapies:

- Plans to hire additional reviewers, leverage expedited pathways, and issue new guidances for different areas of product development of cell and gene therapies
- Two CMC specific draft guidances for cell and gene therapies released July 2018
- Disease-specific draft guidances on hemophilia, rare diseases, retinal disorders
- 31 products have received RMAT designation to date

Expeditated Pathways for Therapies for Orphan Indications

336

regenerative medicines have received Orphan Designation in the U.S.

58%

of publicly announced RMAT designation recipients are for rare disorders

29

regenerative medicines have received Orphan Designation in the E.U.

Market Access Landscape in 8 Countries

As of Q1 2019

Alliance for Regenerative Medicine

Kymriah ★

Yescarta ★

Kymriah ✓

Luxturna ✓

Strimvelis ✓

Yescarta ✓

Zalmoxis ★ ✓

Kymriah ★ ✓

Strimvelis ★ ✓

Yescarta ★ ✓

Strimvelis ★ ✓

Zalmoxis ★ ✓

Kymriah ★ ✓

Luxturna ★ ✓

Yescarta ★ ✓

★ Kymriah

CADTH Assessment: Would be cost effective if price lowered

Yescarta

Undergoing CADTH assessment

Kymriah

Undergoing MSAC assessment

Kymriah

Expected to be covered nationally

★ *Positive HTA Opinion* ✓ *Reimbursed*

As a Reminder…

This presentation will be available via:

- **ARM's website:** www.alliancerm.org
- **Twitter** @alliancerm

Visit **www.alliancerm.org** to access additional resources, including:

- Quarterly sector data reports
- Upcoming near-term clinical trial milestones & data readouts
- Access to slides, graphics, and figures from ARM presentations
- Our weekly sector newsletter, a robust round-up of business, clinical, scientific, and policy news in the sector
- Commentary from experts in the field

In Summary

- 2018 was a year of significant growth in the regenerative medicine sector overall, and in rare disease
- A rich and diverse pipeline is producing positive data
- The impact of early products for patients and families is dramatic
- 2018 saw pronounced and sustained investor interest in the sector
- The policy environment gene therapies for orphan indications and for more common diseases is extremely positive
- 2019 will see the sector address commercialization challenges, particularly focused on new financing models & CMC-related issues

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PROJECT A-GENE

Alliance for Regenerative Medicine

Project A-Gene Overview

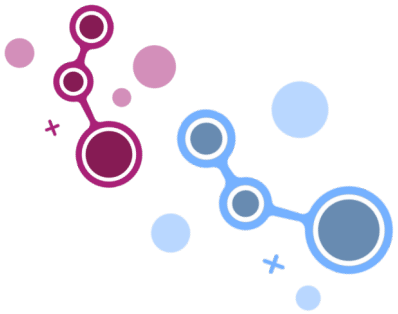
Historical Context

- During the rise of the monoclonal antibody industry, challenges caused by heterogeneity began to inhibit continued growth
- In 2008, a group of leaders in the sector joined to create a case-study based guide to the development and manufacture of monoclonal antibodies, called A-Mab
- Published in 2009, the document advanced the adoption of quality by design best practices including a focus on:
    - Systematic evaluation and iterative process development
    - Development and application of a comprehensive and forward looking control strategy
- Public access to A-Mab helped to improve industry best practices, and continues to play an educational role in the monoclonal antibody industry

Project A-Gene Overview

Alliance for Regenerative Medicine

What is the Current Need?



- Methodologies for the development and manufacture of gene therapies are heterogeneous leading to…
    - Discord in expectations and assays for analytics and characterization
    - Diminished potential for economies of scale in gene therapy costs of good due to prominence of boutique solutions
    - A lack of harmonization of best practices, increasing obstacles to tech transfer and complication GMP workforce training
- Overcoming current challenges requires industry consensus, and application of ‘quality by design’ practices where possible
- A similar effort to A-Mab for gene therapies would provide similar benefits

Project A-Gene Overview

Overview of Current Effort

- ARM launched an effort in May 2018 using the A-Mab product as a model
- Covers the "A to Z" on Gene Therapies, from designing a Target Product Profile, to Process Control, and through Regulatory Considerations for IND filing
- Follows a hypothetical rAAV product, used to highlight key concepts to consider during the course of Gene Therapy product and process development
- To be published for industry use and reference to assist in standardization and harmonization within the industry as regards CMC
- Participation from multiple technology platform developers, including: large biopharma, growth phase therapeutic developers, and other industry stakeholders
- Chapters are drafted in collaboration across technology developers

Project A-Gene Overview

Coordination with Regulatory Authorities

- FDA is considering developing guidance on AAV manufacturing considerations in 2020
- FDA expressed strong interest in “A-Gene” project and ARM has requested through Peter Marks that they appoint a liaison to the project
- ARM is planning a May workshop on comparability where we will use an AAV case study, among others, to frame the conversation

ARM Foundation
for Cell & Gene Medicine

ARM Foundation:
Dedicated to Education

ARM Foundation
for Cell & Gene Medicine

MISSION STATEMENT

The **ARM Foundation** serves as the **educational and information catalyst** on issues fundamental to making gene and cell therapies, tissue-engineered products and other regenerative medicine treatments available to patients.

By examining, quantifying, clarifying and informing stakeholders of the clinical and societal benefits of these therapies, as well as convening discussions to raise awareness about the sector's progress, challenges and results, the Foundation **accelerates patient access** to safe, efficacious and potentially curative therapies.

ARM Foundation
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PROJECT WORK STREAMS:

HEALING GENES
TRANSFORMING MEDICINE, CHANGING LIVES

- Project #1: **Gene Medicine Education Program**
- Project #2: **RM/AT Economic Impact**
- Project #3: **Industrialization of Gene & Cell Therapies**

ARM Foundation
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WHY NOW? THE URGENCY

1 Gene Medicine is Misunderstood

- Complex topic, misunderstood by the public, patients and caregivers.
- Faith-based communities are especially sensitive to issues around responsible use of gene medicine
- However, people who are very knowledgeable about gene medicine are much more in favor of it.
  - ❖ Yet, recent study shows that 90% of Americans have heard "nothing at all" or "little" about gene editing
  - ❖ Stem-cell type backlash by misinformed public possible

2 Press Coverage Has Been Inflammatory

- Negative and inflammatory information in the environment
- Focus of mainstream press often not on transformative, therapeutic use
  - ❖ Rogue scientists, pricing debates and designer babies make headlines

3 Pricing Dominates the Dialogue

- Discussion about how gene medicines will be financed is taking center stage over the value of gene medicine.

4 Industrial Infrastructure is Lacking

- Experts express valid concerns about meeting the manufacturing needs to supply clinical trials and the future demand for these therapies
- Investment is needed in pharmacy, quality control, storage, transportation, etc to optimize the impact these therapies will have on healthcare delivery.

5 Critical Challenges Ahead

If we don't fill the education gap, condition the market and build the infrastructure, we may face:

- An environment ranging from an apathetic or unsupportive patient population to public backlash
- Low clinical trial enrollment
- Prohibitive regulatory environment
- Insufficient reimbursement models upon regulatory approval
- Slow uptake of technology upon availability
- Deficient infrastructure to support the industrialization of gene and cell therapies.
- **Patients still waiting for a cure.**

SOURCES:

PEW RESEARCH CENTER, JULY, 2016, "U.S. PUBLIC WAY OF BIOMEDICAL TECHNOLOGIES TO 'ENHANCE' HUMAN ABILITIES"
U.S. ATTITUDES ON HUMAN GENOME EDITING BY DIETRAM A. SCHEUFELE, MICHAEL A. XENOS, EMILY L. HOWELL, KATHLEEN M. ROSE, DOMINIQUE BROSSARD, BRUCE W. HARDY *SCIENCE* 11 AUG 2017 : 553-554

ARM Foundation
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HEALING GENES
TRANSFORMING MEDICINE, CHANGING LIVES

What is it?

An international educational program designed to generate understanding, awareness and appreciation of gene and cell therapy as *transformative medicine* – an important *therapeutic* option for treating chronic and acute disease and addressing major global health challenges.

Who are our target audiences*?

- Primary: Patients/Caregivers, Policymakers, Key NGOs, Public
- Secondary: Providers, Payers

**Primary and secondary reflects timing of reaching audiences, not priority; secondary audiences will still be reached by channels targeting the public, including media.*

ARM Foundation *for* Cell & Gene Medicine

HEALING GENES
TRANSFORMING MEDICINE, CHANGING LIVES

OBJECTIVES & STRATEGIES

- **Increase awareness about the science and benefits**, and also the limitations and risks of therapeutic gene and cell therapy. Why is it exciting and transformative?
  - **Educate** in advance and alongside negative incident(s) that could threaten the development progress of this potentially life-saving technology.
  - **Equip** and encourage news and social media influencers to cover gene medicine in more balanced, less sensational/provocative way.
- **Help audiences understand the differences** between gene editing and gene therapy, and between therapeutic (somatic) gene editing and germline gene editing.
  - **Provide information** in a way that is simple, non threatening, consumer-friendly, engaging and sensitive.
- **Partner with advocacy groups and credible spokespersons** to drive an education program funded co-operatively by gene medicine-related organizations and possibly other sources.
  - **Equip** advocates to educate others; grow the gene medicine advocacy community.
  - **Engage** stakeholders in productive dialogue about sensitive issues.
  - **Refer** audiences to other sources when appropriate.

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DESIRED PERCEPTION SHIFT

SHIFT PUBLIC PERCEPTION FROM UNAWARE AND DOUBTFUL …

CONFUSED:
"I don't know what gene medicine is or who it's for."

UNCERTAIN:
"How do I know this is safe?"

DISTRUSTFUL:
"I have concerns about this new technology."

APATHETIC:
"It sounds complicated, I'd rather wait and see."

FEARFUL:
"I think gene medicine goes against my religious beliefs."

OPPOSED:
"This research should not continue."

GENE MEDICINE EDUCATION PROGRAM

…TO EXCITED, INTERESTED IN LEARNING MORE AND EAGER TO TAKE ACTION

EXCITED:
"Therapeutic use of gene medicine is truly transformative."

CONFIDENT:
"Like pharmaceuticals, trial monitoring will be rigorous & science-based, as will be the review and approval process."

CURIOUS & OPTIMISTIC:
"I'd like to find out how gene medicine could help me/my family."

INTERESTED & ACTION-ORIENTED:
"I want to learn more."
"I want to participate in a clinical trial."
"I will ask my doctor about this."
"I will help educate others."

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HEALING GENES
TRANSFORMING MEDICINE, CHANGING LIVES

2019 PROJECT UPDATE

- Education portal – Spring launch
- Patient Group Research, Workshop and Toolkit – Spring/Summer
- “Speaking of Genes” Nomenclature Consensus Summit and Guidance Publication – Spring Focus Groups, Fall Conference
- Clinical Research Education Project (with nonprofit partner CISCRP) – June launch
- Policymaker Fact Sheet Series (beginning with Sickle Cell Anemia) – Spring
- Science Briefing Series for Journalists (and Science Writers’ Guide) – under development
- “I AM WHY” value project – under development

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HEALING GENES
TRANSFORMING MEDICINE, CHANGING LIVES

EDUCATION PORTAL HIGHLIGHTS

- Genetic Alliance disease info search platform
- "Splash pages" of an initial 100 diseases/conditions within the context of cell and gene medicine
- Three initial videos: Gene Medicine 101, What's Next? and a visual FAQ
- Infographic on differences in cell and gene medicine approaches

- Cell and Gene Medicine 101
- Search
  - By Disease (links to Genetic Alliance)
  - By Injury | Condition
- Resource Library
  - Advocacy
  - Approved Therapies
  - Clinical Trials
  - Insurance Assistance Programs
  - Patient Assistance
  - Research
- News & Updates
- What Happens Next
- Contact Us

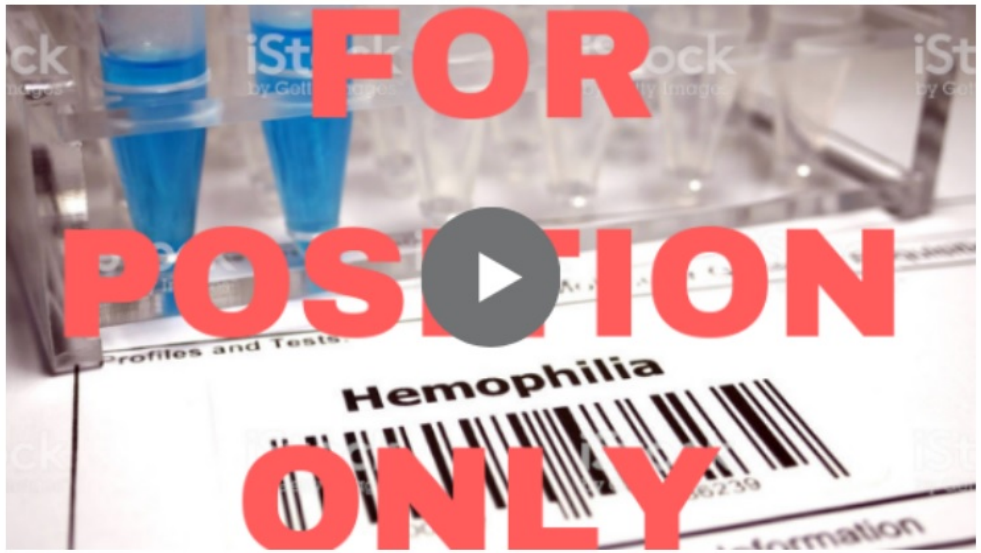
ARM Foundation *for* Cell & Gene Medicine

HEALING GENES PORTAL PREVIEW

HEALING GENES
TRANSFORMING MEDICINE, CHANGING LIVES

Cell and Gene Medicine 101 | Search | Resource Library | News & Updates | What Happens Next | Contact Us

Hemophilia



Hemophilia is an X-chromosome-linked bleeding disorder that affects about 400,000 patients worldwide. Hemophilia A occurs in approximately 1 of every 5,000 male babies worldwide. Hemophilia B occurs in approximately 1 of every 30,000 male babies. Female babies rarely have hemophilia.

The disease is caused by missing blood clotting factors:

- Factor VIII (Hemophilia A), or
- Factor IX (Hemophilia B).

Patients may need medical treatment two to three times a week in order to maintain the clotting factor proteins necessary to form protective blood clots after fairly minor injuries, such as capillary breaks. A majority of patients throughout the world lack access to optimal treatment.

Several manufacturers, including BioMarin Pharmaceuticals, Spark Therapeutics, Pfizer, and UniQure, have gene therapy products in Phase III clinical trials. Products being evaluated have restored patients' anticoagulant factor activity levels to normal or near-normal levels and reduced patients' bleeding rates by 90 percent or more. Research will continue to evaluate long-term safety and effectiveness over time (durability). Cost also will be a factor in evaluating and ensuring the gene therapy products are available to those who need them.

Learn More

Current Therapies | Clinical Trials | Organizations | Research Studies

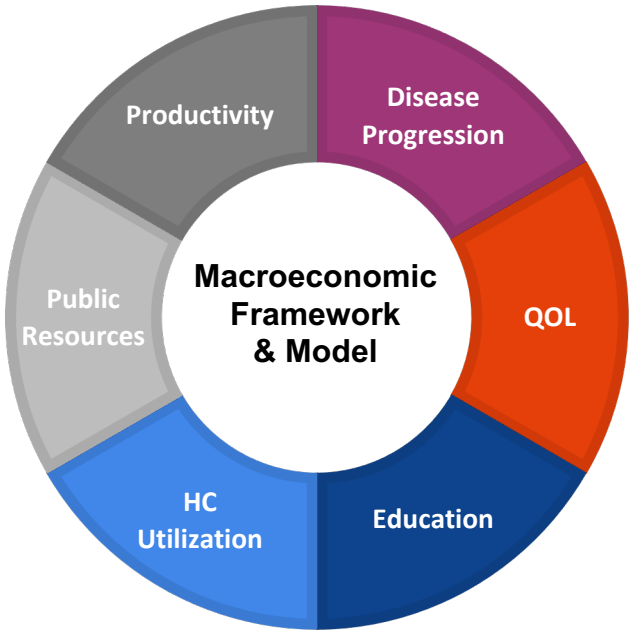
Economic Impact Project

Economic Model Development & Validation

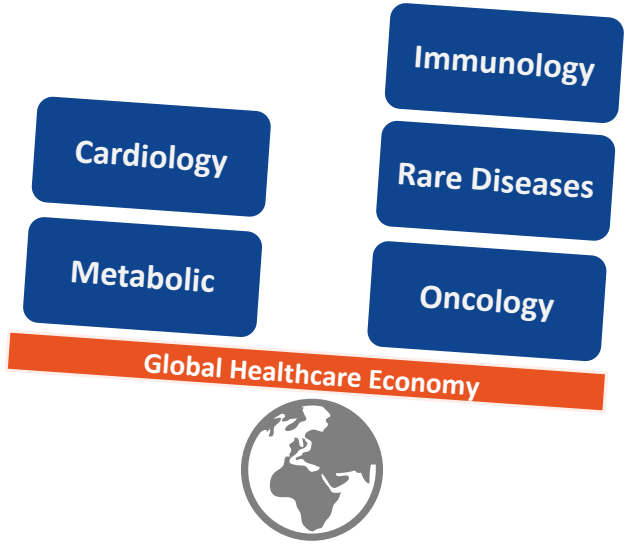
Economic Models & Case Studies

Case Studies & RW Application of Models

Develop framework of burden & treatment value for integration of potentially curative gene and cell therapies across healthcare system, evaluating the interplay between clinical and social factors



Apply framework to specific case studies per disease area to demonstrate the impact of advanced therapies at the macro and microeconomic level on the global healthcare economy



Customizable frameworks and models to present to KOLs and therapeutic developers to help assess the value of individual novel therapies

Comprehensive models to help therapeutic developers, policy makers, medical providers and patients understand the value of RM/ATs

Economic Impact Project

OBJECTIVE -- Develop an in-depth macro-economic impact analysis of cell and gene therapy and other regenerative medicines on the national and international healthcare economies to inform market access initiatives and public awareness.

Key Deliverables:

1 Health Economic Outcomes Landscape

Survey landscape to identify clinical and lifestyle indicators, data sources, and research methodologies across disease areas and healthcare systems relevant to RM

✓ ***Completed***

2 Pharmaco-Economic Analysis and Framework

Employing advanced economic simulation models of RM in several major disease areas and compare to standard of care to quantify the impact and value of curative therapies

3 Stakeholder Awareness Education

Communicating the societal and economic value of RM therapies through data reporting and strategic engagement with payers, legislators, and the public

ARM Foundation for Cell & Gene Medicine

Evidera | PPD IQVIA

Disease Areas and Method for Selecting

Disease areas/diseases must be picked in a manner that is objective/defensible

Criteria

- Disease is chronic or acute mortal or high morbidity; high unmet need/patient burden
- Disease involves high lifetime cost above X threshold
- Disease is a significant health priority or protected class (US)
- Disease is amenable to cell, gene or gene-modified cell treatment

- **Oncology** – top 10 cancers - CDC
- **Rare Diseases** – More than 7,000 diseases
  - Pick a top 10-20 where cell & gene therapies are active (i.e., hemophilia & sickle cell)
- **Neurology** – top neurodegenerative diseases (many are targets of advanced therapy)
  - Alzheimer's disease
  - Amyotrophic lateral sclerosis
  - Friedreich's ataxia
  - Huntington's disease
  - Lewy body disease
  - Parkinson's disease
  - Spinal muscular atrophy
- **Musculoskeletal**
- **Cardiovascular and related:**
  - Chronic angina
  - Myocardial infarction
  - Stroke
  - Critical limb ischemia

Evidera | PPD IQVIA

Why Do We Need A Rare Disease Moonshot?

RARE DISEASE TYPES

7,000+ Identified rare diseases, with more being discovered every day.

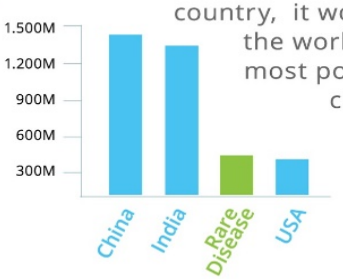
THE CAUSE

80% of rare diseases are caused by faulty genes.

RARE DISEASE EFFECT

30 MILLION AMERICANS

350 MILLION WORLDWIDE



If all of the people with rare diseases lived in one country, it would be the world's 3rd most populous country.

THE IMPACT

Rare diseases impact more people than **CANCER** & **AIDS** combined.

FEW FDA APPROVED CURES

ONLY **5%** of rare diseases have an FDA approved drug treatment.

WHAT IS CONSIDERED "RARE"?

In the United States, a condition is considered "rare" if it affects fewer than **200,000** persons combined in a particular rare disease group.

International definitions on rare diseases vary. For example in the UK, a disease is considered rare if it affects fewer than **50,000** citizens per disease.

RARE DISEASE AFFECTS CHILDREN

Approximately **50%** of the people affected by rare diseases are children.

30% of children with rare disease will not live to see their 5th birthday.

Rare diseases are responsible for **35%** of deaths in the first year of life.

THE DIAGNOSIS

8

On average, it takes most rare disease patients **8 years** to receive an accurate diagnosis—within this time period, they have seen over **10 specialists** and have been misdiagnosed 3 times.

THE SUPPORT

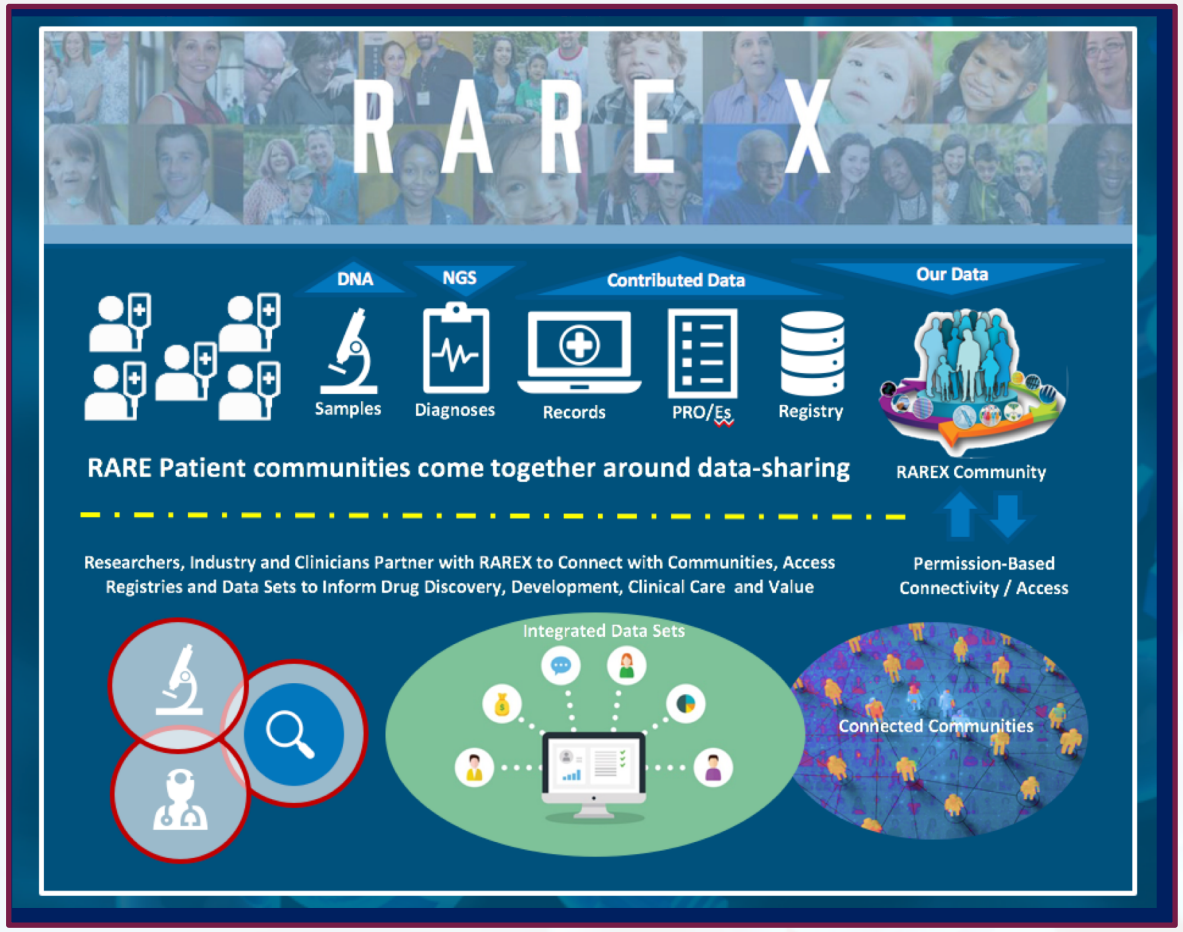
50%

Approximately **50%** of rare diseases do not have a disease specific foundation supporting or researching their rare disease.

How RAREX Would Work

DATA AND ENGAGEMENT PLATFORM: RAREX would be centered around a technology platform, developed by the Broad Institute and tested and used already in support of NIH's All of Us initiative, able to scale and accommodate massive quantities of genomic and other patient data, a best-in-class registry development tool and services, and secure interfaces designed to accommodate queries and deliver analytics across a wide variety of use cases.

SUPPORTING INFRASTRUCTURE AND SERVICES: RAREX would also provide educational, governance, community development and management, data standards, e-consent and other services, templates and tools to help patient communities aggregate, access, assess and share data via the platform, with proper safeguards and permissions, with researchers or others based on their preferences and permission.



Thank You!

Alliance for Regenerative Medicine