



Preparing for the Gene & Cell Therapy Wave

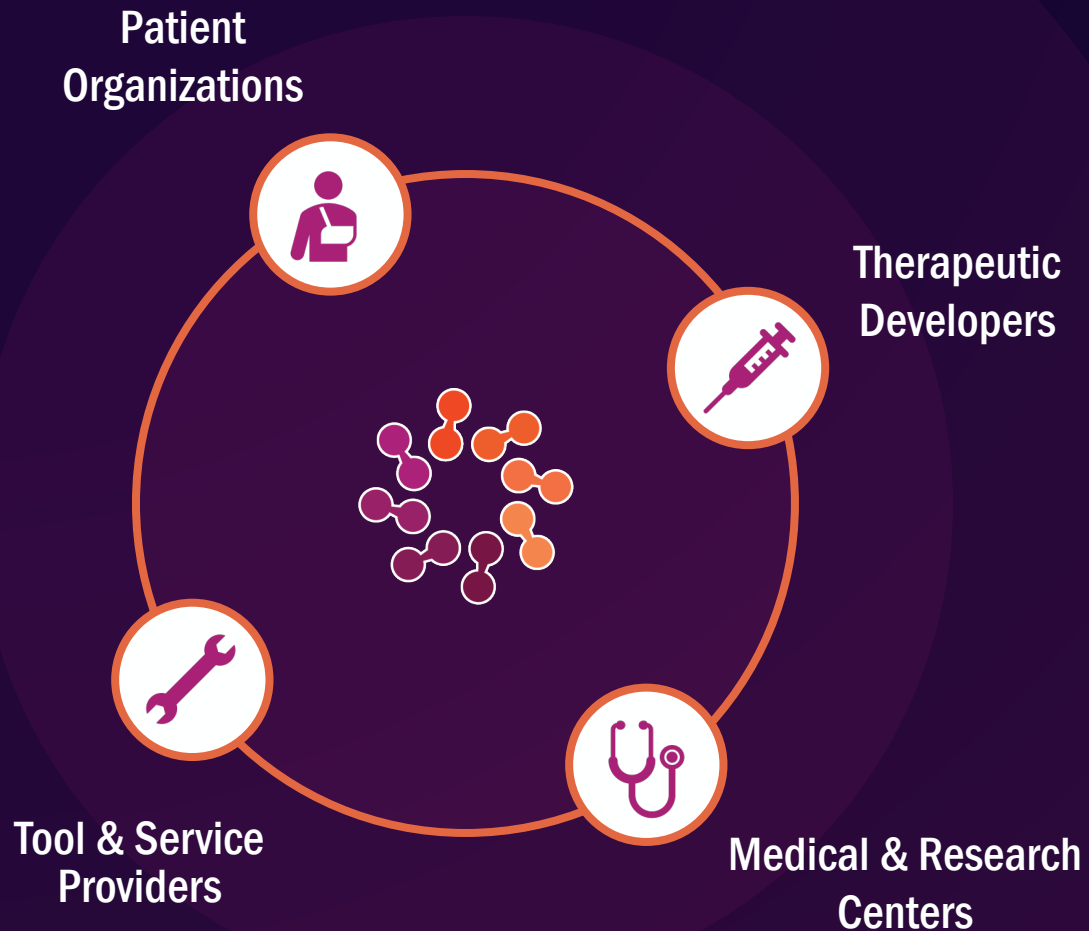
Stephen Majors, Director of Public Affairs

PartnerRe Webinar

March 10, 2021

ARM is the Global Voice of the Sector

Representing 380+ members worldwide



Promote Clear Regulation
Enable Innovative Reimbursement
Address Manufacturing Barriers
Educate Stakeholders



How is Regenerative Medicine Different?

Conventional Chronic Care Therapy

Addresses: Symptoms of diseases

.....

*Dosage: May be administered regularly
over patient's lifetime*

.....

Cost: Throughout a patient's lifetime

.....

*Outcomes: Last for short periods;
repeated dosing needed*

Regenerative Medicine

Addresses: Root cause of diseases

.....

*Dosage: Potentially curative, single or
very limited number of injections*

.....

*Cost : Largely upfront, packed into a
small number of doses or a single dose*

.....

*Outcomes: Last for long periods,
possibly for a lifetime*



Examples of Regenerative Medicine



Gene Therapy

Introduces a functioning gene into a patient's cells

One approved durable therapy for 'SMA1', a devastating genetic disorder that kills 90% of children who have it before their second birthday



Gene Editing

Inserts, replaces, removes or modifies DNA

Shows promise in clinical trials to durably treat or cure diseases including sickle-cell, which affects 1 in every 365 Black babies born in the US



Cell Therapy

Transfers cells into a patient to treat disease

4 approved therapies to treat leukemias and lymphomas that are unresponsive to other treatments



Tissue-Based Therapy

Replaces damaged tissue with natural or synthetic tissue

Currently approved products can treat severe burns and wounds; may also be used to repair or replaced damaged organs





\$19.9B raised in 2020

Shattering previous annual financing records

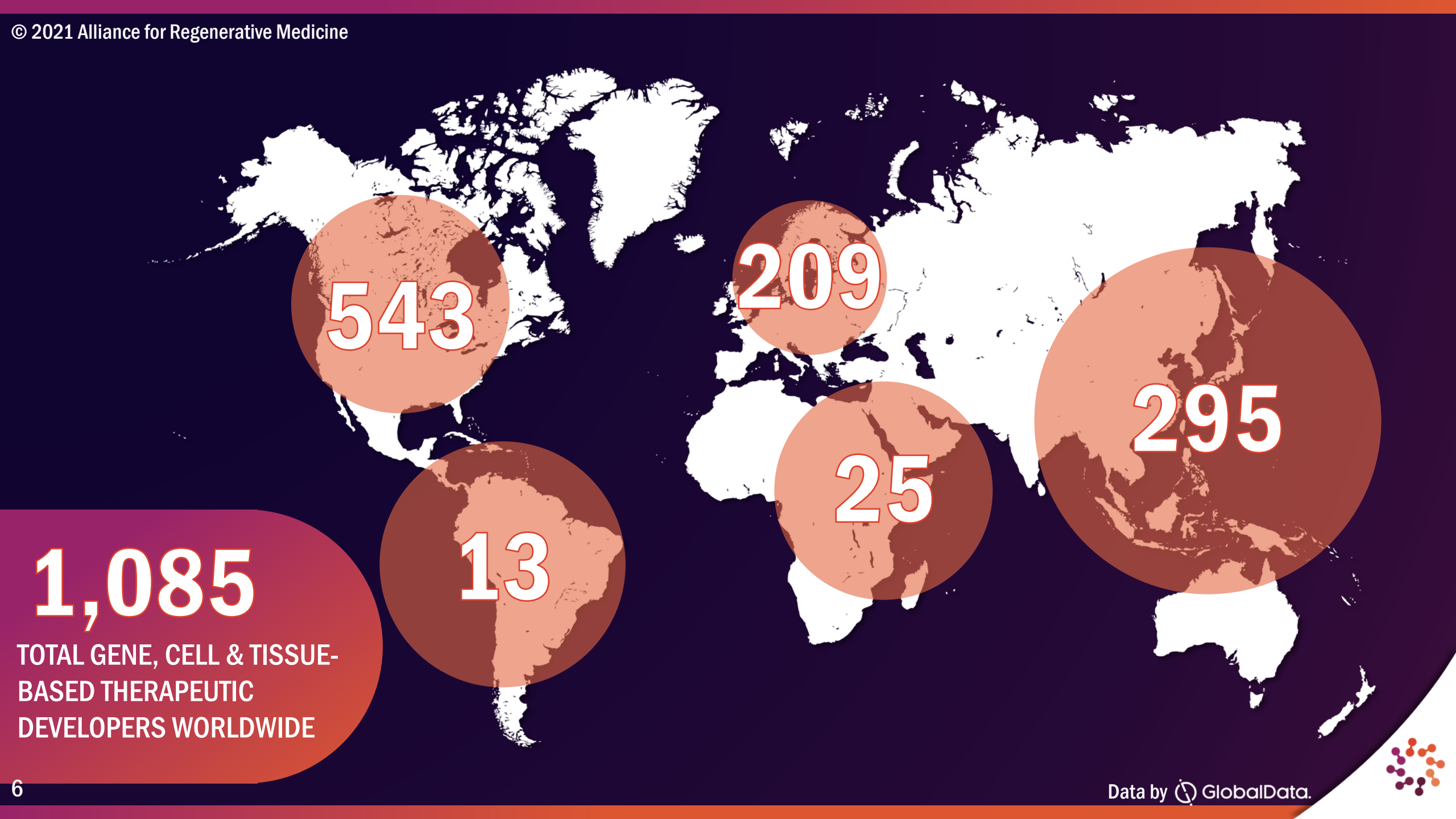
\$9.8B RAISED
IN 2019

\$13.3B RAISED
IN 2018

\$7.5B RAISED
IN 2017

Data by  GlobalData.





1,085

TOTAL GENE, CELL & TISSUE-BASED THERAPEUTIC DEVELOPERS WORLDWIDE



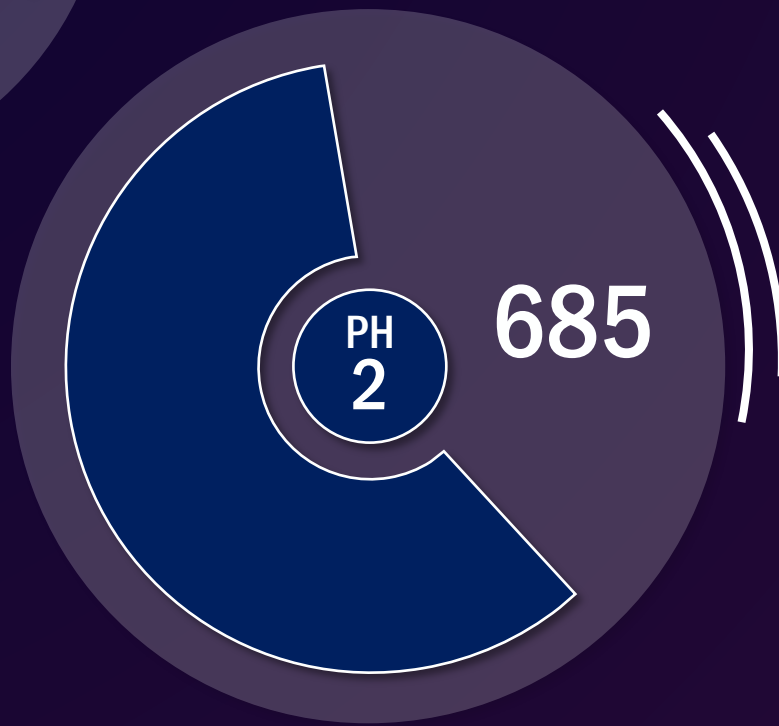
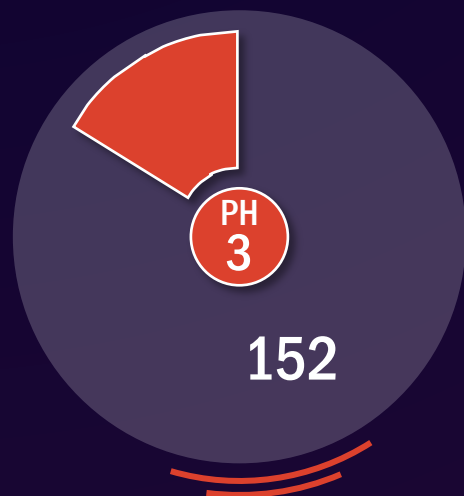
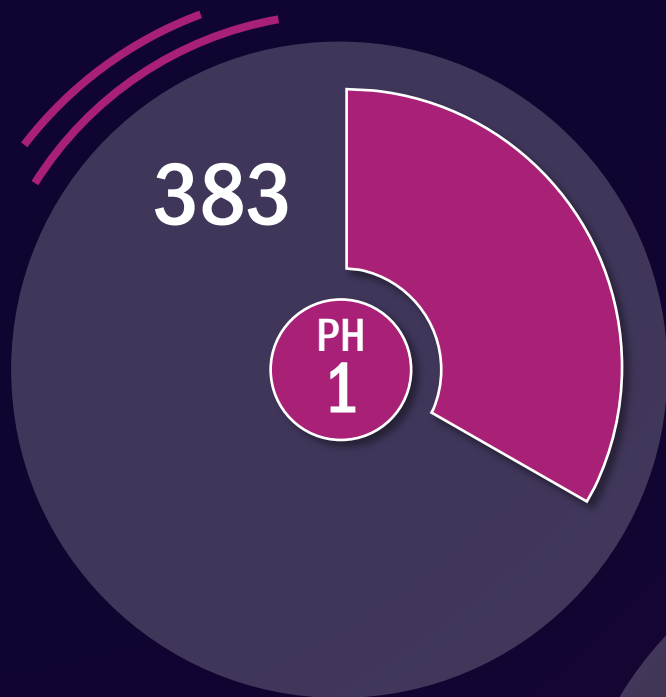


Recent Approvals:

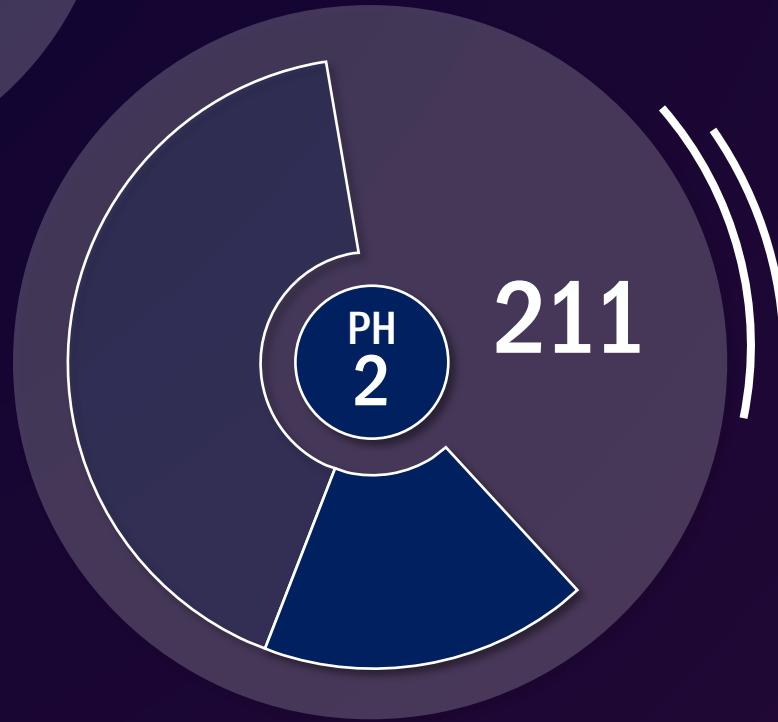
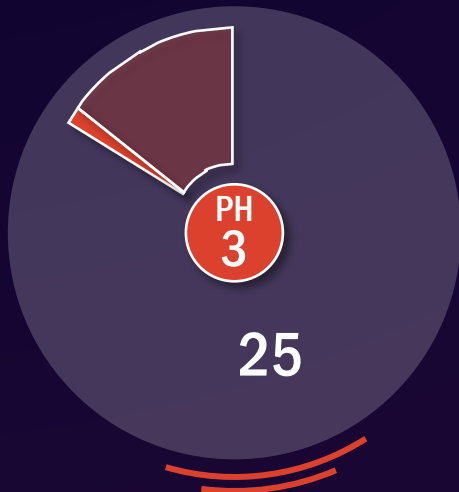
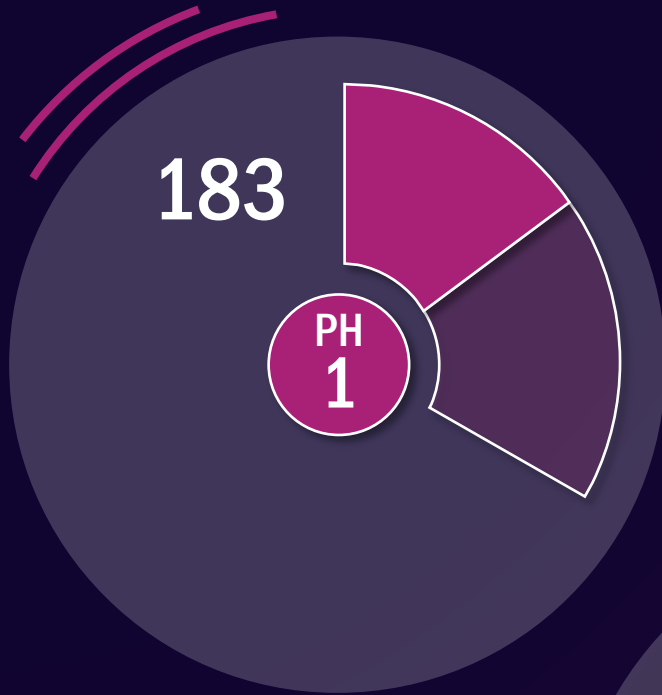
- Breyanzi® (Bristol Myers Squibb) – US
- Tecartus® (Kite, a Gilead company) – US
- Libmeldy® (Orchard Tx) – Europe
- Zolgensma® (Novartis Gene Therapies) – Europe, Japan, Canada
- Luxturna® (Spark / Roche) – Canada



1,220 ONGOING REGENERATIVE MEDICINE &
ADVANCED THERAPY TRIALS WORLDWIDE



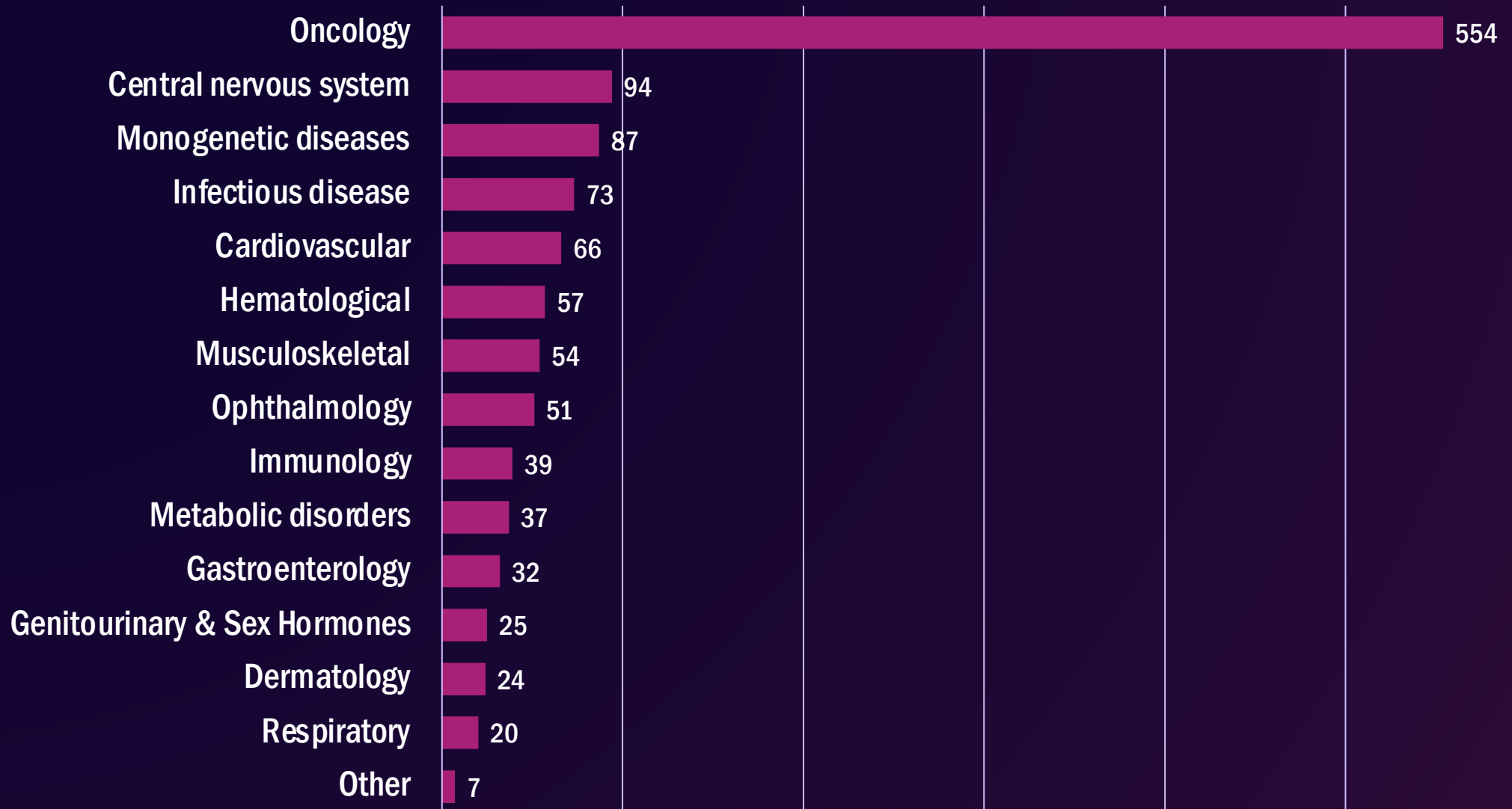
1,220 ONGOING REGENERATIVE MEDICINE &
ADVANCED THERAPY TRIALS WORLDWIDE



419 in cell-based IO



Clinical Trials by Indication





The Scientific and Clinical Landscape

Integrating cutting edge technologies

A broader range of cell types

New milestones for allogeneic therapies

Breaking into the solid tumor space



A Challenging Environment

COVID-driven operational and clinical disruptions

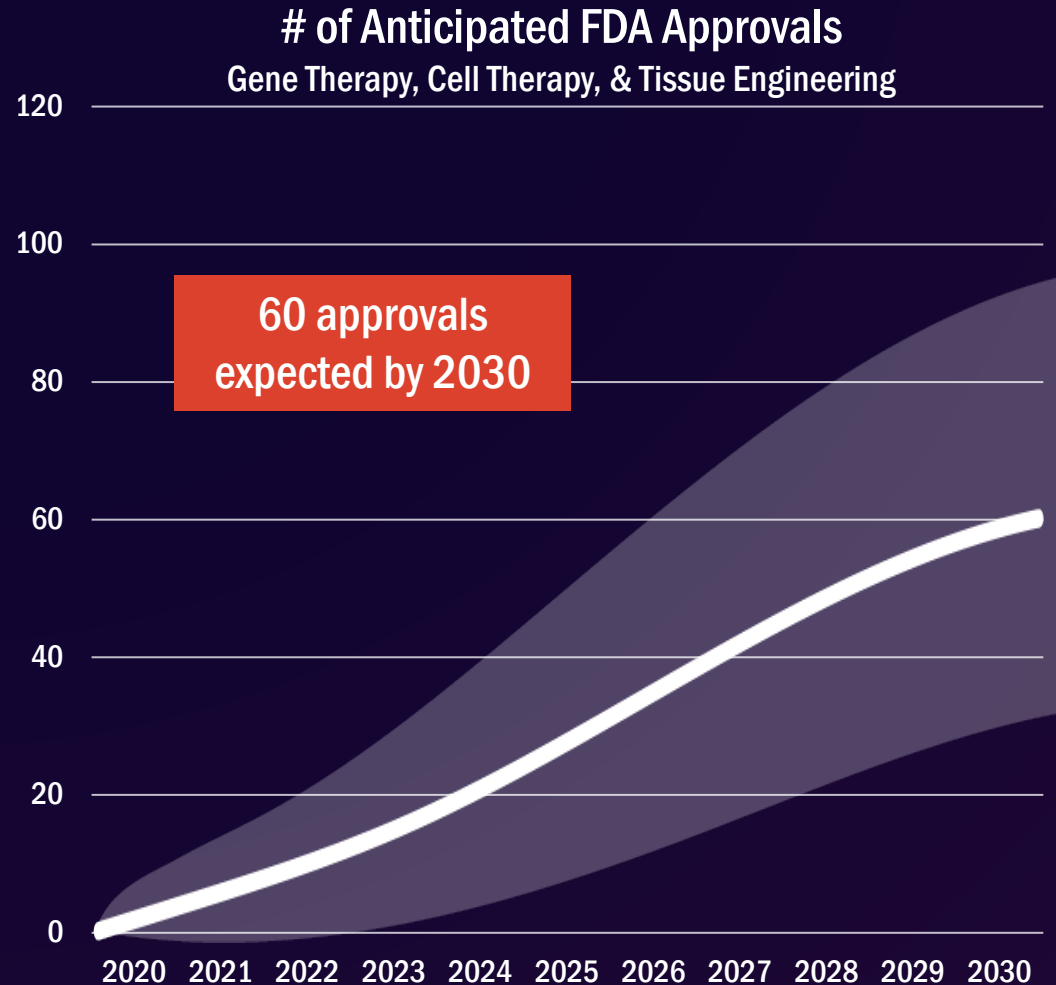
Pandemic limited regulators' CGT bandwidth and travel

The politicization of COVID vaccines

Growing industry demands and fast-moving science



The Coming Wave of Cell & Gene Therapies: Patient Impact



By 2030, more than 500,000 Americans could receive a regenerative medicine product

The FDA is expected to decide on:

- Stratagraft®, a tissue engineered product to treat severe burns (April)
- Ide-cel® (March) & cilta-cel® (late 2021 / early 2022) , CAR-T therapies for multiple myeloma





Regenerative Medicine & Multiple Myeloma



Standard of Care

35k patients diagnosed with multiple myeloma in the U.S. annually

5-year mean survival from diagnosis

Annual healthcare costs: \$280,000 per patient



The Multiple Myeloma Pipeline

117 Developers Active in
Multiple Myeloma

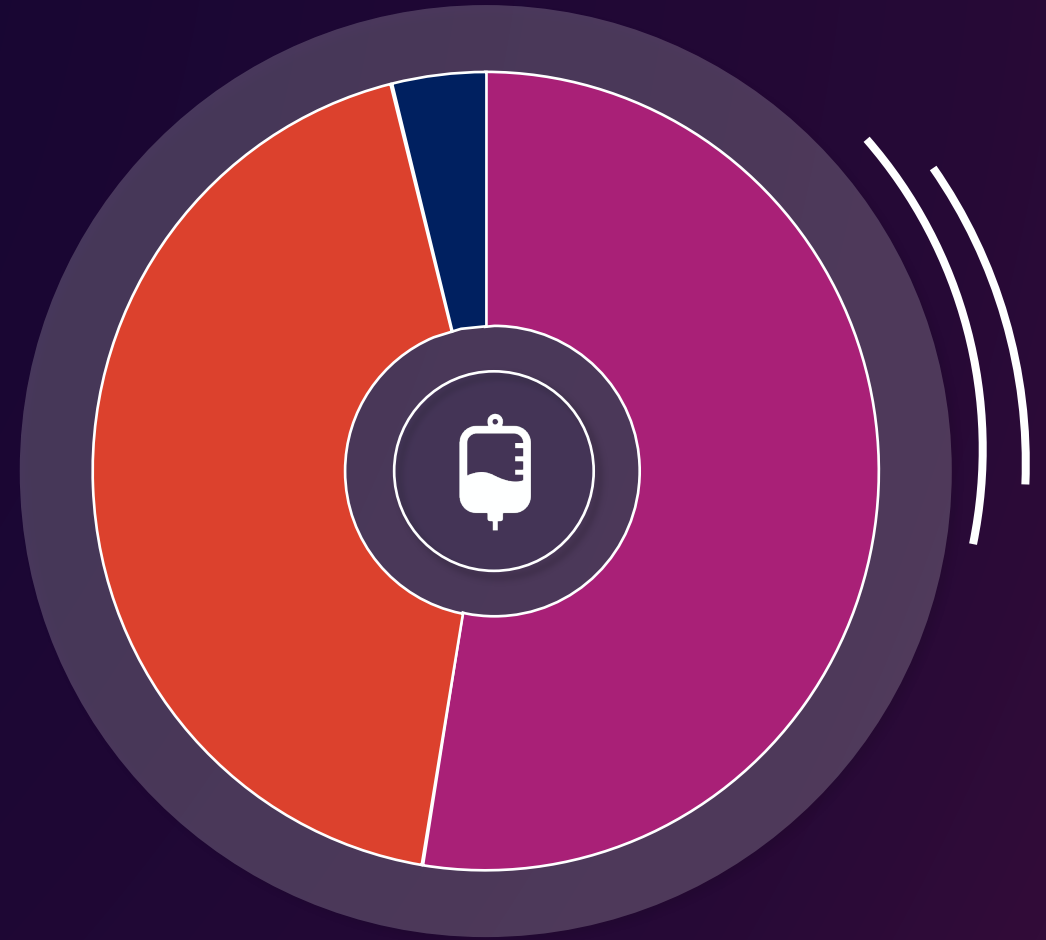
186 Regenerative Medicine
Therapies in Development

74 Clinical Stage Therapies

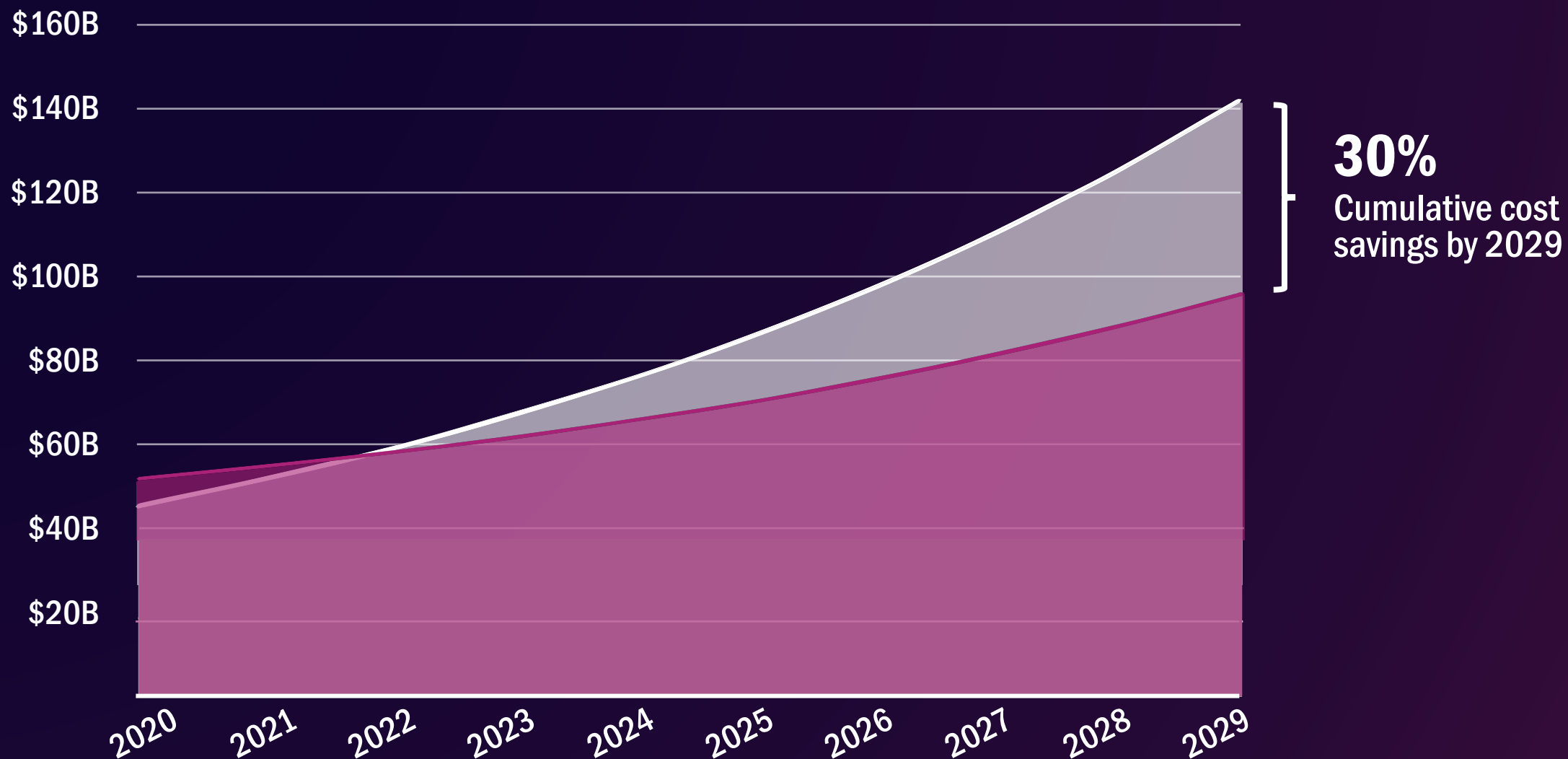
Ph 1

Ph 2

Ph 3

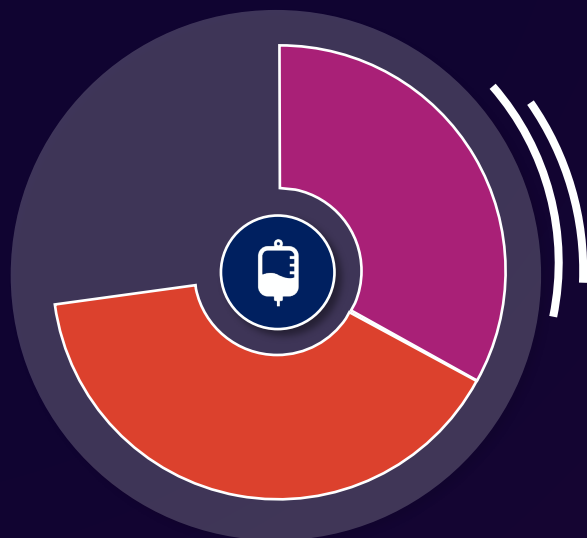


Cost Savings from a Durable Multiple Myeloma Treatment



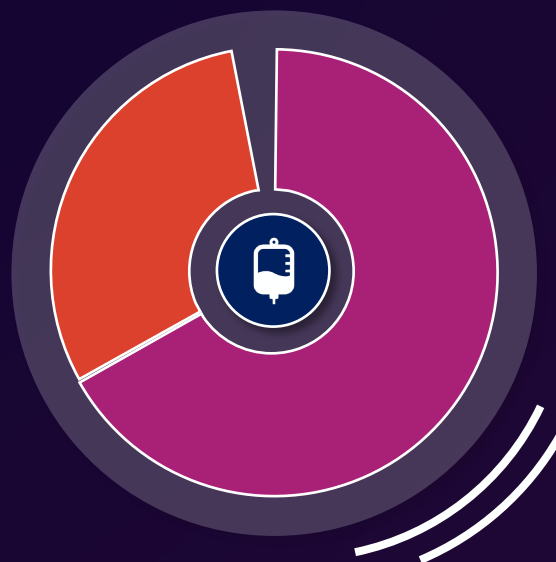
Patient Outcomes

Clinical trials demonstrate deep & durable responses with 12+ months of follow-up



Ide-cel®
bluebird bio, BMS

Under standard of care, most patients with relapsed / refractory multiple myeloma die within 6 months



Cilta-cel®
Janssen, Legend



***Multiple myeloma survivor grateful
CAR T cell therapy clinical trial gives
her another option***



Thank you!

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