



Innovative technologies for rare diseases

2 March 2021

International Rare Diseases Conference

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The Bio Revolution has started

Domain and examples

Human health and performance



Health optimization in future generations



Gene drives to reduce vector-borne diseases



Cell-, gene-, and RNA-based approaches to prevent, diagnose, and treat diseases

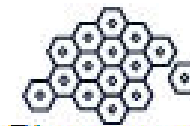


Improvements in drug development and delivery

Arenas of innovation



Biomolecules



Biosystems



Biomachine interfaces

Transformational capabilities

Increased control and precision

Enhanced ability to engineer and reprogram human and non-human organisms

Increased throughput and productivity of R&D

Growing potential for interfaces between biological systems and computers

Source: The Bio Revolution McKinsey Global Institute May 2020



MORE THAN
100
DISEASES BEING EXPLORED FOR
POTENTIAL TREATMENT WITH CELL
AND GENE THERAPIES

362²
CELL AND GENE THERAPIES
IN DEVELOPMENT

132
A THIRD OF CELL AND GENE
THERAPIES ARE IN DEVELOPMENT
FOR RARE DISEASES

MEDICINES IN DEVELOPMENT | 2020 UPDATE

CELL AND GENE THERAPY

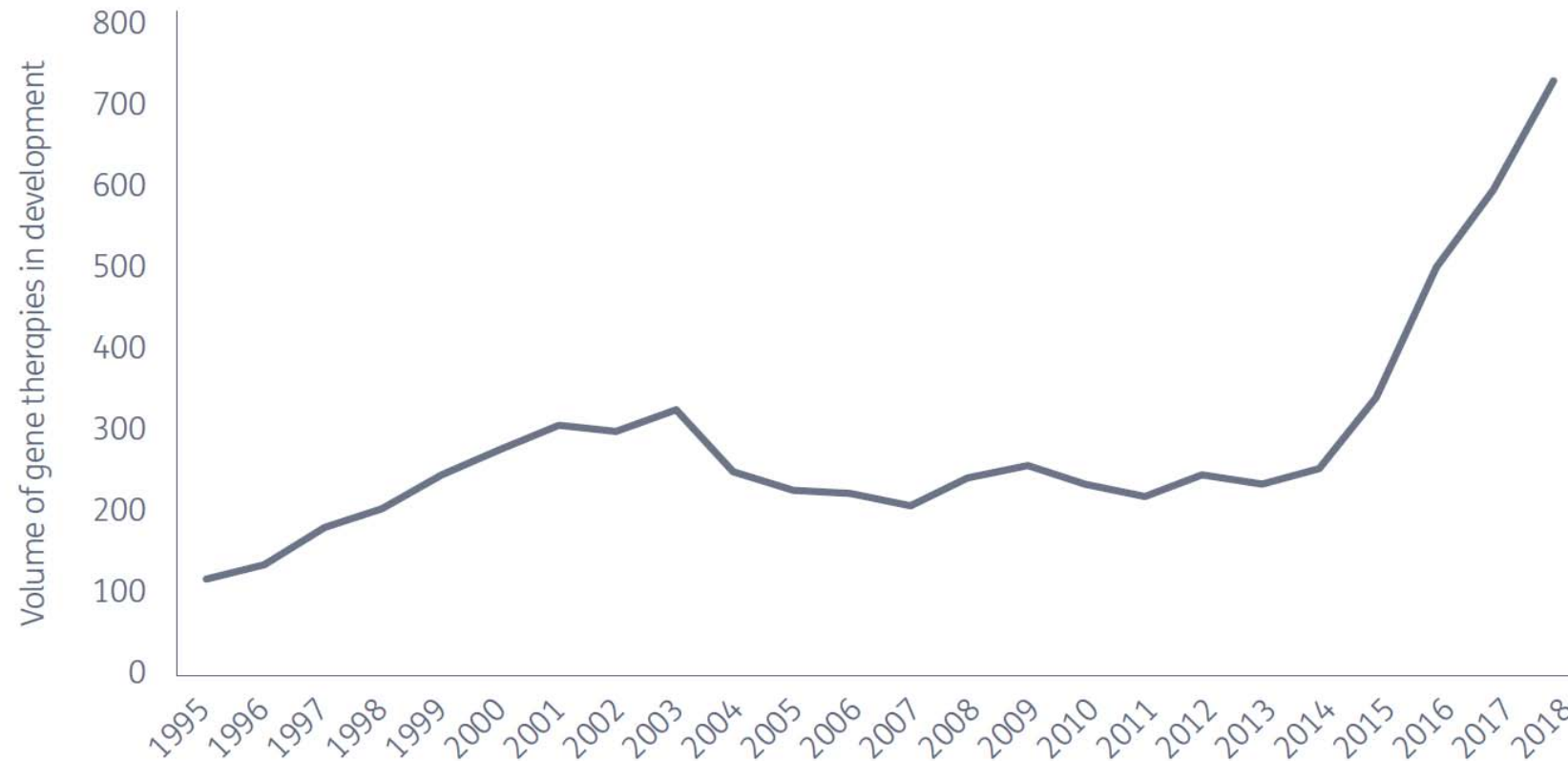
Nearly **400** Cell and Gene
Therapies in Development Target
a Broad Range of Diseases

Source: American BioPharmaceutical Medicines in Development report

In Gene therapies alone



Figure 1. Gene therapy pipeline volume, preclinical through pre-registration phase, 1995–2018

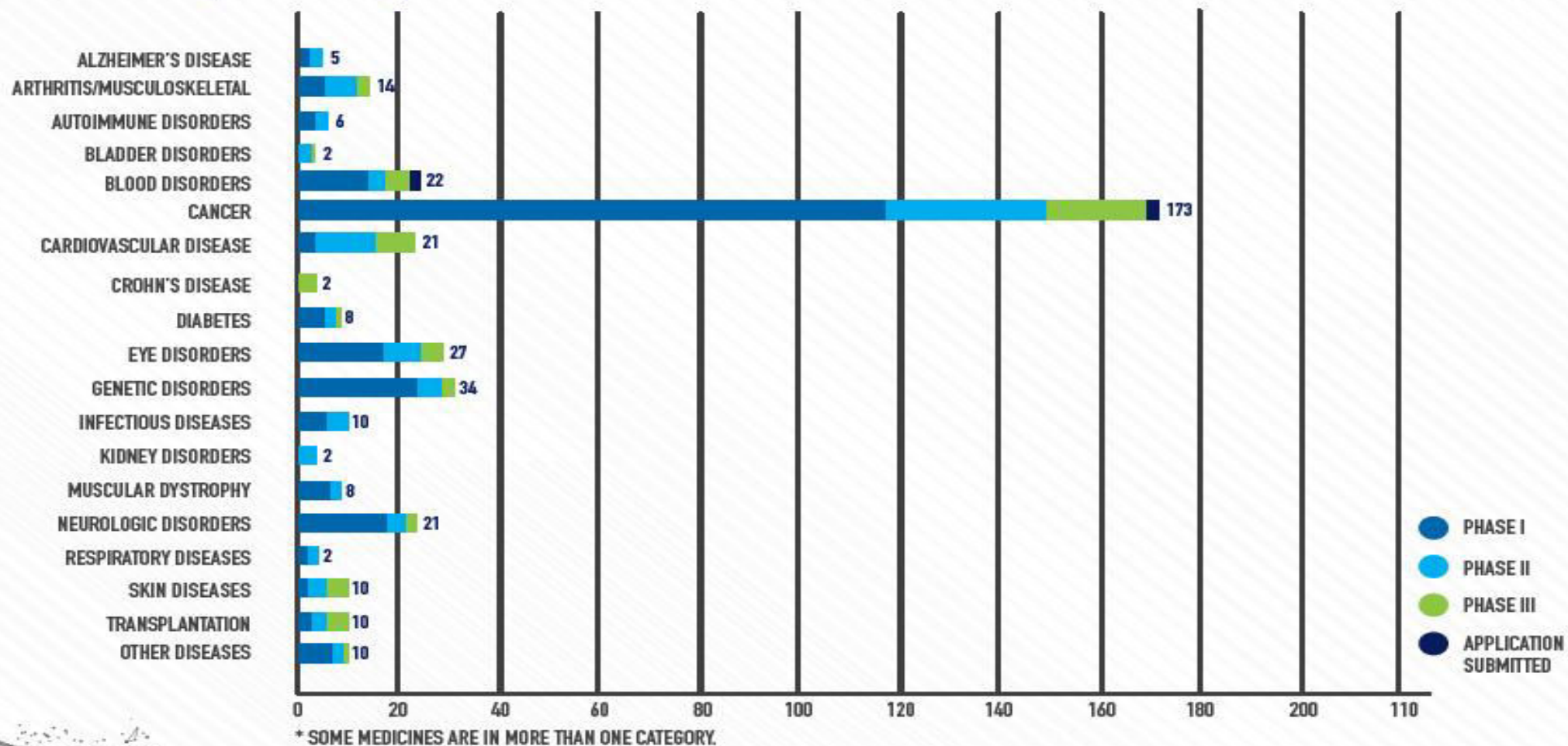


Note: Annual volume snapshots are captured in May of each year.

Source: Pharmaprojects®, August 2018

Pipeline by disease and phase

Medicines in Development by Disease and Phase



- **Apostolia Poimenidou**, Sr Medical Advisor Rare Diseases
Greece-Cyprus-Malta Pfizer

Gene therapy in Rare diseases: opportunities and challenges

- **Thomas Bols**, Head of Government Affairs and Public Policy EMEA and APAC, PTC Therapeutics

The challenging path to bring a gene therapy to patients with an ultra-rare disease

- **Christos Dakas**, Executive Director, Country Manager Greece, Cyprus and Malta, NOVARTIS Gene Therapies

Navigating the Access Pathway with a one-time Gene Therapy



Thank you

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