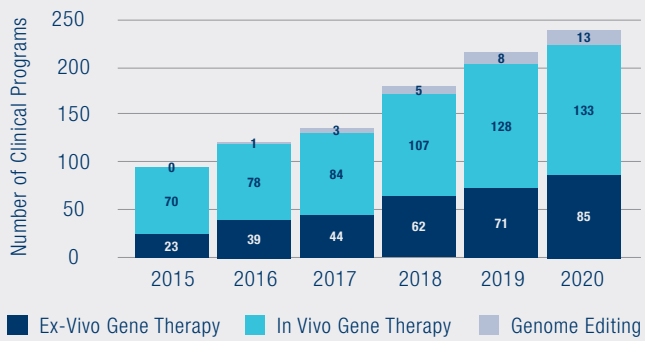
Biotechnology Innovation Organization

THE NEXT WAVE OF TRANSFORMATIVE THERAPIES

Over the last five years, there has been a **dramatic increase** in the global development of transformative gene therapies.* Given this increase and the global development, **there is a clear need for harmonization across global regulatory authorities to ensure that these therapies can be delivered to the patients that need them as quickly as possible.** Such harmonization efforts may include regulatory terminology and definitions, and nonclinical, quality, and clinical considerations.

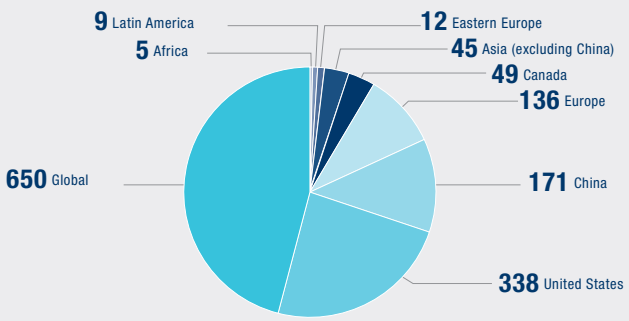
While there are a small number of gene therapies already approved, **there are more than 100 currently under development:**

CLINICAL PIPELINE FOR GENE THERAPIES 2015 – MARCH 2020



Additionally, **clinical trials for gene therapies are occurring across the globe:**

CURRENT ACTIVE CLINICAL TRIALS FOR GENE THERAPIES BY COUNTRY



*Data current as of March 2020

POTENTIAL AREAS FOR HARMONIZATION

Regulatory Terminology and Definitions: Opportunities for harmonizing terminology related to regulatory submission. For example, definition of viral vector, definition of genetic modification, and definition of sameness.

Nonclinical Considerations: Opportunities for harmonizing nonclinical aspects of development. For example, species selection, duration and redosing, study endpoints, and juvenile toxicity studies.

Quality Considerations: Opportunities for harmonizing quality aspects of development. For example, raw and starting material considerations, scale-up and scale-out challenges for gene therapy products; identification and management of critical quality attributes; and commercial life-cycle management.

Clinical Considerations: Opportunities for harmonizing clinical aspects of development. For example, long-term follow-up (LTFU) observation requirements, registry sampling/testing.

*For the purposes of this graphic the term gene therapy includes in vivo gene therapies, ex-vivo gene therapies (i.e., CAR-T) and gene editing therapies.

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METHODS

Clinical Program Pipeline Data

BIO used data from the Informa Biomedtracker database and annotated each clinical program as either gene editing, in-vivo gene therapy, or ex-vivo gene therapy. Gene editing therapies are therapies delivering a transgene that is incorporated into the genome of living humans. In-vivo gene therapies are therapies aimed at delivering a transgene to living somatic cells, and ex-vivo gene therapies are those that are categorized as therapies aimed at delivery of a transgene to isolated human cells (typically blood cells), that are later delivered back to the patient. Please note that this data does not include preclinical programs.

Trial Data

Data from the Informa Citeline database includes active clinical trials for gene therapy products. This category for gene therapy includes both in-vivo and ex-vivo gene therapies.