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](image)

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

![CURRENT ACTIVE CLINICAL TRIALS FOR GENE THERAPIES BY COUNTRY](image)

*Data current as of March 2020

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**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.

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*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.*
## 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.