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# GENE THERAPIES: OVERCOMING THE BIGGEST HURDLES IN MANUFACTURING

The last decade saw a great rise in gene therapy popularity. Today, gene therapies have reached a momentum with hundreds in clinical development and several therapies approved in recent years. However, the rapid growth of the sector and the complexity of gene therapies has resulted in a number of challenges in process development, scale-up, manufacturing, and regulatory guidelines.

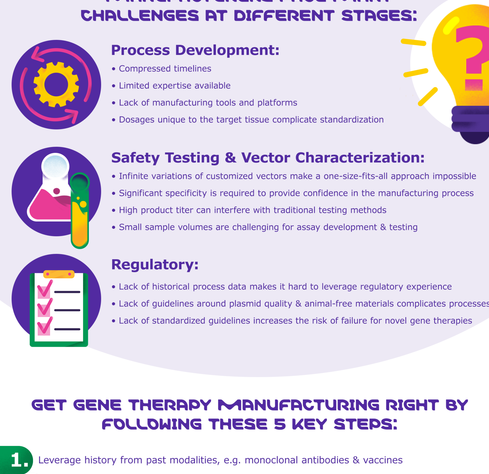
**370+**
Global gene therapy clinical trials in 2019

**~15**
Gene therapies approved globally

**$13B**
Estimated worth of the gene therapy market by 2024

## The gene therapy sector is booming, but manufacturing timelines are greatly compressed!

**Average drug development takes 8 - 10 years, gene therapy drugs take 3 - 5 years!**




## MANUFACTURERS FACE MANY CHALLENGES AT DIFFERENT STAGES:

### Process Development:

- Compressed timelines
- Limited expertise available
- Lack of manufacturing tools and platforms
- Dosages unique to the target tissue complicate standardization

### Safety Testing & Vector Characterization:

- Infinite variations of customized vectors make a one-size-fits-all approach impossible
- Significant specificity is required to provide confidence in the manufacturing process
- High product titer can interfere with traditional testing methods
- Small sample volumes are challenging for assay development & testing

### Regulatory:

- Lack of historical process data makes it hard to leverage regulatory experience
- Lack of guidelines around plasmid quality & animal-free materials complicates processes
- Lack of standardized guidelines increases the risk of failure for novel gene therapies

## GET GENE THERAPY MANUFACTURING RIGHT BY FOLLOWING THESE 5 KEY STEPS:

1. Leverage history from past modalities, e.g. monoclonal antibodies & vaccines
2. Adopt a risk-based approach using a quality by design (QbD) strategy
3. Ensure reproducible product quality, safety, yield, titer & purity
4. Create synergy between development & manufacturing teams
5. Stay up to date with evolving regulatory guidelines

## Draw on MilliporesigMa's experience to help Master gene therapy Manufacturing

MilliporeSigma* focuses its expertise on developing products, testing, and manufacturing to bring gene therapies to life. As the only CDMO inspected by the FDA, EMA, and TGA, MilliporeSigma has products that help biotechs to scale up easily to any batch size. The company's GMP testing services support every stage of a therapy from cell banking and product characterization through lot release testing.

**>10,000**
Cell & gene therapy samples tested in 2019

**3**
Gene therapy products developed through to commercialization

**500+**
Batches of virus production, including Lentivirus, Adenovirus, Adeno Associated Virus, Herpes Simplex Virus, Retrovirus, Dengue

## WHAT TO EXPECT IN THE FUTURE: STEPPING INTO THE NEXT DECADE OF GENE THERAPY MANUFACTURING

**Learn how to tackle the challenges in gene therapy manufacturing and visit MilliposeSigma at emdmillipore.com/genetherapy!**

*The life science business of Merck KGaA, Darmstadt, Germany operates as MilliporeSigma in the U.S. and Canada.

**SOURCES**


- MilliporeSigma
- BusinessWire
- Alliance for Regenerative Medicine
- American Society of Gene & Cell Therapy
- Labiotech.eu
- Nature


Want to work with us?
Reach out to contact@labiotech.eu


Text: Larissa Warneck
Design and images: Elena Resko



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