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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

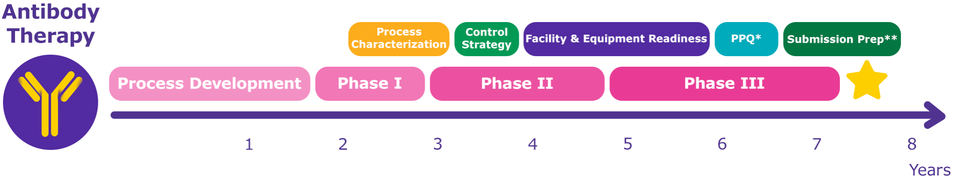
$13B

Estimated worth of the gene therapy market by 2024

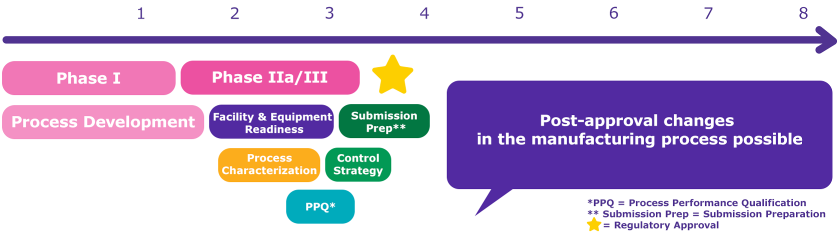
The gene therapy sector is booming,

but manufacturing timelines are greatly compressed!

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



Gene Therapy



*PPQ = Process Performance Qualification
** Submission Prep = Submission Preparation
★ = Regulatory Approval

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 Merck's experience to help master gene therapy manufacturing

Merck* 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, Merck 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

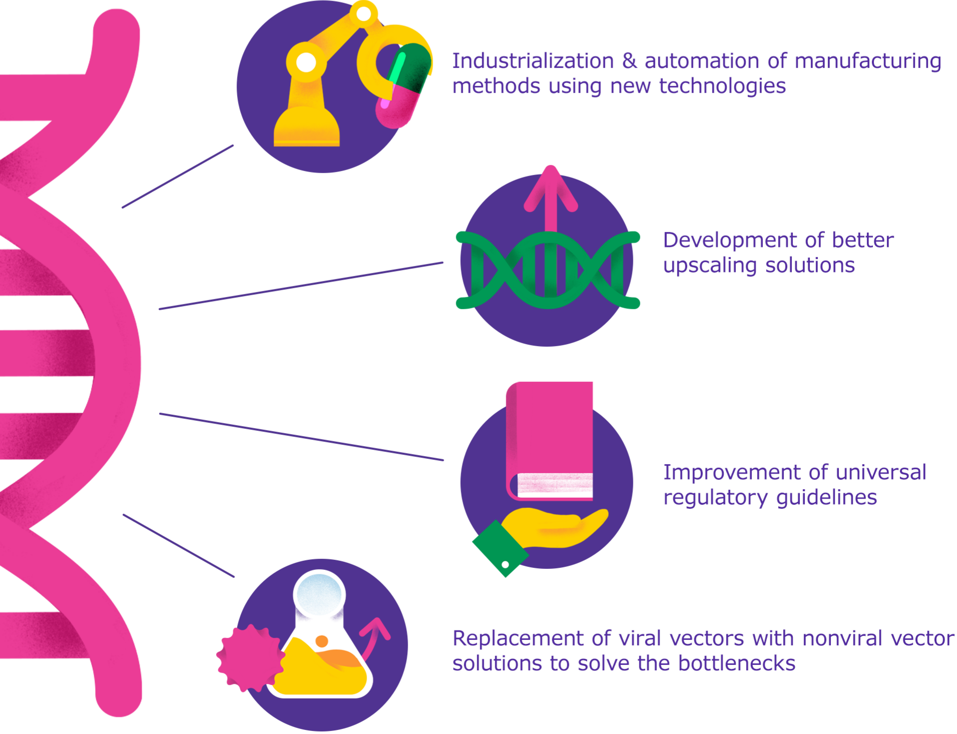
Gene therapy products developed through to commercialization

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



Industrialization & automation of manufacturing methods using new technologies

Development of better upscaling solutions

Improvement of universal regulatory guidelines

Replacement of viral vectors with nonviral vector solutions to solve the bottlenecks

Learn how to tackle the challenges in gene therapy manufacturing and visit Merck at merckmillipore.com/genetherapy!

*The life science business of Merck 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?
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