YOUR GENE THERAPY MANUFACTURING SUCCESS RECIPE

GENE THERAPIES: A BOOMING AND DYNAMIC MARKET LANDSCAPE

Since its beginnings in the early 2000s, gene therapy has been regarded as the cutting edge of medicine. With vast therapeutic potential, cell and gene therapies are anticipated to overtake conventional drug therapies in the not-so-distant future, with sales projected to significantly increase by 2026.

An increasing number of therapies in the global pipeline as well as advances in gene therapy manufacturing reflect the growing demand:

20 gene therapies approved globally[1]

Estimated combined revenues projected from the cell[2] and gene[1] therapy market by 2028 exceed **€51B**

600+ active global gene therapy clinical trials reported in 2021 of which a majority - **309** - are in phase 1[1]

300 biotech and pharma companies in Europe, over **600** in North America working in the gene therapy field[1]

KICKSTARTING YOUR UPSTREAM PRODUCTION PROCESS CORRECTLY

Gene therapies in the market today use viral vectors to introduce the modified genetic material into target cells. **This means that gene therapy manufacturing starts with the upstream process of vector and cell line development, and cell culturing which culminates in harvesting of the virus produced.[3]**

This feeds into downstream purification and concentration to ensure the product has high efficacy and safety to pass regulatory

Therefore making the right upstream process decisions not only impacts immediate upstream outputs, but also downstream processes, timelines and regulatory acceptance.

THE FOUR MAJOR CHALLENGES THAT FACE UPSTREAM MANUFACTURING TODAY INCLUDE:

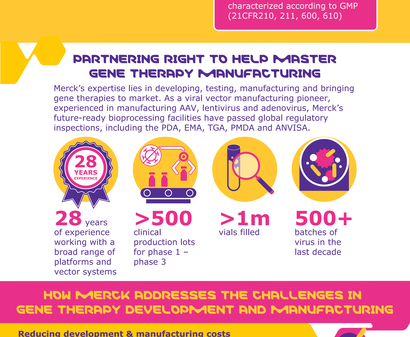
Delivering on speed-to-market: Gene therapies have highly compressed development timelines of 3–5 years that need to be met vs 8–10 years for traditional biologic modalities.[4]

Product safety: Developing a multi-faceted contamination risk mitigation strategy is critical to the development of your AAV process.

Increasing productivity: The ability to deliver high quality virus is paramount to the success of your therapy. Templated solutions can help maintain product efficiency as well as ensure quality.

Ensuring scalability: The rapid growth in gene therapy's demand can only be met by starting out with sufficient upstream outputs. Currently the ability to scale adherent cell culture to efficient volumes is limited.

THE MAIN CONSIDERATIONS THAT BIOTECHS NEED TO FOCUS ON WHEN LOOKING AT UPSTREAM PROCESSES INCLUDE:



CHOOSING YOUR RECIPE FOR SUCCESS: USING PROVEN UPSTREAM PLATFORMS

Using optimized, time-effective upstream production templates can help address key upstream hurdles. Companies like Merck, provide multiple scalable upstream solutions for your gene therapy manufacturing process:

SF-RVN® Platform leverages an insect cell line cultured with an optimized medium to produce AAVs.

Benefits:

- Sf9 rhabdovirus-negative cell line: enhanced risk mitigation and biosafety profile
- High performance: optimized to get low doubling time, high cell viability and high AAV titers
- Cell bank manufactured according to GMP (21CFR210, 211, 600, 610) and fully characterized
- Full traceability and documentation for regulatory filings
- Technical user guide with detailed protocols for optimal performances

VirusExpress® platforms leverages a transfection-based solution using suspension-adapted HEK cell lines to produce AAVs.

Benefits:

- Reduced time in process development and scale-up by approximately 40%
- A suspension adapted cell line of 293 AAV Production Cells optimized for production of AAV vectors for gene therapy applications
- Proven process performance at 3 L scale with genome titer exceeding 2.4 X 10^{15} gc/mL for AAV2 production
- Comprehensive user protocols to guide from seed train through to at-scale transfection and virus production, allowing for seamless scale-up
- Cell banks manufactured and characterized according to GMP (21CFR210, 211, 600, 610)

PARTNERING RIGHT TO HELP MASTER GENE THERAPY MANUFACTURING

Merck's expertise lies in developing, testing, manufacturing and bringing gene therapies to market. As a viral vector manufacturing pioneer, experienced in manufacturing AAV, lentivirus and adenovirus, Merck's future-ready bioprocessing facilities have passed global regulatory inspections, including the FDA, EMA, TGA, PMDA and ANVISA.

28 years of experience working with a broad range of platforms and vector systems

>500 clinical production lots for phase 1 – phase 3

>1m vials filled

500+ batches of virus in the last decade

HOW MERCK ADDRESSES THE CHALLENGES IN GENE THERAPY DEVELOPMENT AND MANUFACTURING

Reducing development & manufacturing costs

More efficient processes enabled by expertise in both products and services may result in lower costs and shorter timelines

Growing manufacturing capacity

Continuing investments in Millipore® CTDMO Services to meet the growing demands of gene therapy

Standardizing platforms for manufacturing

- Providing standardized upstream platform solutions such as the scalable and high-yield VirusExpress® viral vector production platforms
- Data-driven downstream solutions for connected unit operations

Unique solutions within process development

- Equipment and consumables designed specifically for viral vector manufacturing
- On-site technical process development support
- BioReliance® biosafety and characterization services

Providing expert regulatory support

Comprehensive knowledge of regulatory guidelines and expectations provided by Merck specialists

Are you interested in learning how Merck can help you develop your life-saving therapy? Contact the experts at Merck or check out the website for more information!

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Sources:

1. Gene Therapy Industry Report 2021
2. Cell Therapy Industry Report 2022
3. Merck
4. Labiotech

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