MilliporeSigma

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GENE THERAPY

Making rare disease therapies less rare

70% of rare diseases are caused by a single genetic mutation. This makes gene therapy a promising approach to treat rare diseases.

However, a number of challenges remain to be addressed. How can we unlock the **full potential** of gene therapy for patients in need?

RARE DISEASES AT A GLANCE

Rare diseases affect the lives of over **300 M** people worldwide.

7,000 rare diseases have been identified so far

70% Start in early childhood

90% Have no approved therapy

30% Of patients die before the age of five

THE PROMISE OF GENE THERAPY

Gene therapy provides enormous opportunity for patients with rare genetic diseases.

2,400 Gene therapies in the global pipeline

9 Lentiviral and adeno-associated viral vector therapies currently approved

A number of gene therapies for rare diseases have been approved recently:

- Abecma® to treat the rare bone marrow cancer multiple myeloma
- Zolgensma® for the neuromuscular disorder spinal muscular atrophy
- Zynteglo®/LentiGlobin™ for the blood disorder beta thalassemia
- Luxturna® to treat Leber congenital amaurosis

CURRENT CHALLENGES HAMPERING GENE THERAPY MANUFACTURING

A shortage of manufacturing capability

- Lack of personnel with experience in viral vector and gene therapy manufacturing
- Growing imbalance in the demand and availability of viral vector manufacturing capacity
- Urgent need for more manufacturing facilities

A lack of standardized platforms for manufacturing

- Development processes are still being researched
- No industry-standard template exists
- Development protocols and equipment vary between companies
- Dose requirements vary significantly based on the route of administration
- Characterization challenges due to the complex composition of gene therapies

A complex regulatory landscape

- A lack of historical precedent for developers and regulatory authorities
- Uncertain and nascent regulatory approval pathways
- Frequently evolving guidelines

The price tag

- Gene therapy manufacturing is costly
- Reimbursement strategies are new and often complicated
- Gene therapies aim to cure a disease with a single treatment dose

All this results in high one-time costs, leading to difficulties in patient access and affordability.

$0.28 For one tablet of Paracetamol

$124 Average prescription drug price (across 11 countries)

$2.1 M For one dose of Zolgensma®

THE SOLUTION

MAKE GENE THERAPIES MORE COMMONPLACE

At MilliporeSigma, we're giving shape to gene therapy development every day by focusing on advancements of viral vector manufacturing.

Currently, viral vectors are the preferred vehicles of gene delivery due to their high efficiency. In fact, 89% of gene therapy clinical trials are based on viral vectors.

As a viral vector manufacturing pioneer, our products, services, and expertise help to overcome current challenges in gene therapy development.

- **Ratish Krishnan**, Senior Strategy Consultant, MilliporeSigma

HOW MILLIPORESIGMA ADDRESSES THE CHALLENGES IN GENE THERAPY DEVELOPMENT AND MANUFACTURING

Reducing development & manufacturing costs

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

Growing manufacturing capacity

Scaling up BioReliance® Viral and Gene Therapy Manufacturing capacities 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 MilliporeSigma specialists

Biomanufacturing experts like us can accelerate gene therapy development from the clinic to commercialization, resulting in more scalable and affordable breakthrough gene therapies … this will have significant impact on the future of medicine and will bring gene therapy to the millions of patients suffering from rare genetic diseases.

- **David Loong**, Senior Strategy Consultant, MilliporeSigma

To learn how MilliporeSigma is bringing gene therapy to life, click here!

EMDMillipore.com/genetherapy

Want to work with us?
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Sources:
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American Society of Gene & Cell Therapy
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