



GENE THERRY

Making Rare bisease therapies Less Rare

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

unlock the **full potential** of gene therapy for patients in need?

However, a number of challenges remain to be addressed. How can we

Rare diseases affect the lives of over

RARE DISEASES AT A GLANCE

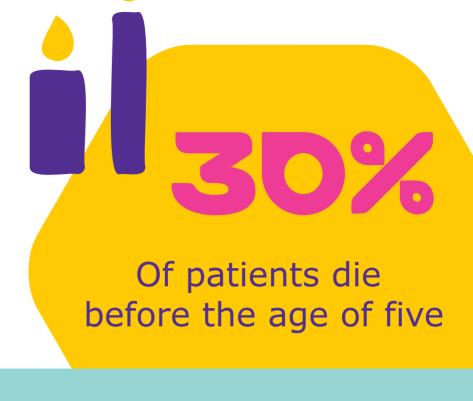


people worldwide.

DDD rare diseases have been identified so far







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







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

Zynteglo®/LentiGlobin™ for the blood disorder beta thalassemia

Luxturna® to treat Leber congenital amaurosis

Abecma® to treat the rare bone marrow cancer multiple myeloma

Zolgensma® for the neuromuscular disorder spinal muscular atrophy

GENE THERAPY MANUFACTURING A shortage of manufacturing capability

Lack of personnel with experience in viral vector and gene therapy manufacturing

CURRENT CHALLENGES HAMPERING

Urgent need for more manufacturing facilities

A lack of standardized platforms for manufacturing

Development protocols and equipment vary between companies

Dose requirements vary significantly based on the route of administration

Growing imbalance in the demand and availability of viral vector manufacturing capacity

Characterization challenges due to the complex composition of gene therapies

A lack of historical precedent for developers and regulatory authorities

Development processes are still being researched

No industry-standard template exists

- Uncertain and nascent regulatory approval pathways Frequently evolving guidelines

The price tag Gene therapy manufacturing is costly Reimbursement strategies are new and often complicated

A complex regulatory landscape

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.



help to overcome current challenges in gene therapy development. - Ratish Krishnan, Senior Strategy Consultant, MilliporeSigma

More efficient processes enabled by expertise in both products and

HOW MILLIPORESIGMA ADDRESSES THE CHALLENGES IN

trials are based on viral vectors.

GENE THERAPY DEVELOPMENT AND MANUFACTURING

At MilliporeSigma, we're giving shape to gene therapy development

Currently, viral vectors are the preferred vehicles of gene delivery

due to their high efficiency. In fact, 89% of gene therapy clinical

As a viral vector manufacturing pioneer, our products, services, and expertise

every day by focusing on advancements of viral vector 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

Providing expert regulatory support

Reducing development & manufacturing costs

services result in lower costs and shorter timelines

Growing manufacturing capacity

manufacturing

- On-site technical process development support BioReliance® biosafety and characterization services
- Comprehensive knowledge of regulatory guidelines and expectations provided by MilliporeSigma specialists
- 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

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