

\*\*\*\*Published January 2026\*\*\*\*

## Approved gene therapies situation review (CAT: VAMVG001)

<b>Product Name</b>	:	<b>MarketVIEW:</b> Approved gene therapies situation review
<b>Description</b>	:	Global gene therapy approved landscape assessment
<b>Contents</b>	:	Executive presentation (~150 slides .pdf) + workbook(s) (.xls)
<b>Therapeutic Area</b>	:	Gene therapies
<b>Publication date</b>	:	January 2026
<b>Catalogue No</b>	:	VAMVG001

### Background

**Gene therapies** are a type of **Advanced Therapy Medicinal Product (ATMP)** designed to deliver a functional gene or to permanently modify cell progenitors. They utilize vectors such as adeno-associated virus (AAV), lentiviral vectors, or others for directly administered *in vivo* use or *ex vivo* engineered (CD34+) autologous hematopoietic stem cell products. These therapies aim to provide one-time treatments for long-term disease control or cure in monogenic disorders, and, importantly, are in favour with new decision-making appointees at the USA FDA.

On the US market, **15 gene therapies** that meet this classification are FDA-approved, with the first being **Luxturna** (voretigene neparvovec) in 2017/18, marketed by **Spark Therapeutics** for Inherited retinal disease (RPE65 mutation). Other gene therapies approved since this time target a range of indications, including transfusion-dependent beta-thalassemia (**Zenteglo**), sickle cell disease (**Lyfgenia/Casgevy**), and other inherited hematologic or immune disorders. Novartis **Zolgensma/Itivisma** (approved in 2019) for spinal muscular atrophy (SMA) is currently the bestselling gene therapy with ~\$1.2bn in revenues in 2024.

**Market analysts** predict the gene therapy market (currently estimated by VacZine Analytics at **\$2.9bn** in FY2024) will expand greatly by the mid-2030s as indications expand from ultrarare diseases into more prevalent conditions. Newborn screening/diagnosis and technological improvements in next-generation capsid and vector engineering will also drive growth. However, high product pricing coupled with uncertain reimbursement policies still pose issues for the market outlook. Manufacturers are faced with constrained capacity, especially in the autologous space (CD34+). The threat of competition from non-viral delivery systems, e.g., **lipid nanoparticles** and off-the-shelf cell or antibody-based therapies, is likely to moderate uptake and keep access concentrated in specialized centers. Safety concerns that have arisen with certain products **Elevidys/Skysona** cast a shadow over the sector.

This **MarketVIEW** product is a comprehensive Executive Presentation (.pdf) plus data worksheet (.xls), which gives a detailed situation review of the currently FDA-approved gene therapy landscape. The analysis includes a disease

Continued.....

profile for the 11 relevant indications, 15 gene therapy product audits, along with their competitor/commercial performance, and total market assessment. All the latest relevant issues are covered, ranging from adoption case studies/technological advancements. This analysis is the ideal starting point for any client who wishes to familiarize themselves with the **gene therapy space** for opportunity scanning.

## Methodology

**VacZine Analytics** has closely monitored all significant source material pertaining to gene therapies and in each respective market. Source materials used are academic literature articles, government websites, medical bodies and associations, conference proceedings, social media etc. Previously published research by **VacZine Analytics** in the field of genetic therapies has been utilized. Some elements of the research process have utilized enterprise level AI models with human oversight and refinement.

### PRODUCT CONTENTS:

**Published January 2026 (CAT No: VAMVG001)**

\*\*\*\*This product is a [summary presentation \(pdf\)](#), [an MS-workbook \(.xls\)](#)

**Contents – Summary presentation (.pdf)**



Contents

Author's notes

**Executive summary**

**[SECTION 1]** Background to gene therapy

**[SECTION 2]** Gene therapy products (x 15) disease indications/profiles, pricing/reimbursement commercial performance

**[SECTION 3]** Market assessment of approved gene therapy products

**[SECTION 4]** Future drivers/challenges to the gene therapy market

About **VacZine Analytics**

Disclaimer

&

**PAGES: >150 slides fully referenced/sourced. Available in .pdf form**

**Contents – MS-Excel workbook (.xls)**



**SNAPSHOT**

**PRODUCT COST:**

**VacZine Analytics** will grant a [enter region] license to [enter client name], for the price of:

- FULL PRODUCT - USD **\$contact us**/ GBP **£contact us**<sup>#</sup> (Global license only)\*

\*Global = North America, Europe or ROW (non-exclusive, non-transferable license)

For orders in the UK, VAT at 20% will be added to final invoice total

*# - indicative prevailing rate will be applied on date of transaction*

**HOW TO ORDER:**

To order please contact your region account manager or order direct at [orders@vaczine-analytics.com](mailto:orders@vaczine-analytics.com) This report can also be purchased on-line. Please review the **TERMS and CONDITIONS** of purchase.



**VacZine Analytics** © is a trading division of Assay Advantage Ltd, UK Company Number: 5807728

**VacZine Analytics** © and the “**spiral logo**” are UK Registered Trademarks, 2009

**Table 1: Products and indications covered in this report**

Product	Company	Indication
<b>Casgevy</b> <sup>™</sup>	Vertex (CRISPR)	Sickle cell disease (SCD), beta-thalassemia
<b>Elevidys</b> <sup>®</sup>	Sarepta Therapeutics (Roche)	Duchenne muscular dystrophy (DMD)
<b>Hemgenix</b> <sup>®</sup>	CSL Behring (UniQure)	Hemophilia B (congenital Factor IX deficiency)
<b>Lenmeldy</b>	Orchard Therapeutics	Children with early onset metachromatic leukodystrophy (MLD)
<b>Luxturna</b> <sup>™</sup>	Spark Therapeutics	Inherited retinal disease (RPE65 mutation)
<b>Lyfgenia</b> <sup>™</sup>	Bluebird	Sickle cell disease (SCD)
<b>Roctavian</b> <sup>™</sup>	BioMarin	Hemophilia A
<b>Skysona</b> <sup>®</sup>	Bluebird Bio	Cerebral adrenoleukodystrophy (CALD)
<b>Zolgensma</b> <sup>®</sup>	Novartis	Spinal muscular atrophy (SMA)
<b>Zynteglo</b> <sup>™</sup>	Bluebird Bio	Beta-thalassemia
<b>Adstiladrin</b>	Ferring Pharmaceuticals	High risk non-muscle invasive bladder cancer (NMIBC)
<b>Beqvez</b>	Pfizer	Hemophilia B
<b>Vyuvek</b>	Krystal Biotech	Dystrophic epidermolysis bullosa (DEB) a rare genetic skin disorder
<b>Itvisma</b>	Novartis	Spinal muscular atrophy (SMA)
<b>Kebilidi</b>	PTC therapeutics	Aromatic L amino acid decarboxylase (AADC) deficiency.
<b>Waskyra</b>	Fondazione Telethon	Wiskott–Aldrich syndrome

**TERMS and CONDITIONS:**

VacZine Analytics – a trading division of Assay Advantage Ltd UK Company Number: 5807728 (Herein referred to as “The Company”). (Herein [enter client name] to as “The Client”).

1. All Rights Reserved. This finished research product is a licensed product. It may not be reproduced, stored in a retrieval system or transmitted in any form without the written permission of the Company **VacZine Analytics** (of division of Assay Advantage Ltd).
2. The license granted by the Company to the Client will be non-exclusive, non-transferable and should only be used for the Client business purposes at the agreed Client sites/location in accordance with this agreement. The Client will have no ownership rights over the research product.
3. Invoicing will **100%** after submission of the deliverables (.pdf) and (.xls) to the Client.
4. If not purchased online invoices are payable within **thirty days** of the invoice date.
5. All proposals are quoted in **\$USD dollars or £GBP or €euro** and invoices are to be settled in the same currency.
6. The Company agrees not to disclose to any third-party confidential information acquired while providing the research product listed without the prior written consent of the Client. Exception occurs when the information is already in the public domain or when disclosure is necessary to help the Company’s employees and agents with the performance of the Company’s obligations to achieve satisfactory completion of the project and approved in writing by the Client.
7. Force Majeure: The Company will not be liable for any delay or failure to perform any obligation under this Agreement insofar as the performance of such obligation is prevented by an event beyond our reasonable control, included by not limited to, earthquake, fire, flood or any other natural disaster including pandemic, labour dispute, riot, revolution, terrorism, acts of restraint of government or regulatory authorities, failure of computer equipment and failure or delay of sources from which data is obtained.
8. Please also refer to Master **TERMS and CONDITIONS** available upon request.

**VacZine Analytics**

A division of Assay Advantage Ltd  
Warren (Carlton) House  
Bells Hill  
Bishops Stortford  
Herts  
CM23 2NN  
United Kingdom  
Tel: +44 (0) 1279 927049  
E-mail: [info@vaczine-analytics.com](mailto:info@vaczine-analytics.com)

## About VacZine Analytics:

**VacZine Analytics** is an established strategic research agency based in the United Kingdom. Its aim is to provide disease and commercial analysis for the vaccine industry and help build the case for developing new vaccines and biologics.

For more information, please visit our website [www.vacZine-analytics.com](http://www.vacZine-analytics.com)

**VacZine Analytics** © is a trading division of Assay Advantage Ltd, UK Company Number: 5807728

**VacZine Analytics** © and "the spiral logo" are UK Registered Trademarks, 2009



*Since 2007*

