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Gene therapies R&D targets review (CAT: VAMVG002)

Product Name	:	MarketVIEW: Gene therapies R&D targets review
Description	:	Global gene therapy R&D pipeline assessment
Contents	:	Executive presentation (~150 slides .pdf) + workbook(s) (.xls)
Therapeutic Area	:	Gene therapies
Publication date	:	January 2026
Catalogue No	:	VAMVG002

Background

Research and Development in new **gene therapies** (including genetically modified cell therapies e.g. CAR-T) is reportedly at its highest level to date. A recent 2025 analysis (ASCGT/Citeline) indicated ~2,200 gene therapy programs are active globally (ranging from preclinical through pre-registration) with ~800 being either in vivo or ex vivo clinical assets (mostly in Phase 1 and Phase 2). Rare diseases and anticancer are key areas in the gene therapy pipeline with rare diseases itself having oncology and non-oncology subcategories. The most common non oncology rare diseases include Beta thalassemia | Duchenne Muscular Dystrophy | Retinitis Pigmentosa | Amyotrophic lateral sclerosis | Scleroderma | Lupus | Myasthenia Gravis | Hemophilia A. Some of these indications already have FDA-approved gene therapy products e.g. **Elevidys** (*delandistrogene moxeparvovec.rokl*) marketed by **Sarepta Therapeutics**. There are important issues with first generation gene therapies with regarding to safety and efficacy which necessitates further research and development. Importantly, regulatory momentum has accelerated significantly with the **US FDA** committing to approving **10-20** cell and gene therapies annually.

VacZine Analytics has conducted a separate new review of the gene therapy pipeline for the aforementioned 8 non-oncology rare disease indications. Our analysis finds that 436 trials are registered globally with around 50% currently active/ongoing. 50% of the trials are operated by the industrial sector. 25 countries and 245 organisations are represented in our analysis and overall, USA-based companies such as **Pfizer**, **Spark Therapeutics Inc**, **PTC Therapeutics Inc**, BioMarin and others still dominate the field. Gene therapy modalities varied by disease indications with adenovirus-associated (AAV) vectors, autologous approaches, and stem cell therapies predominating. Competitive and platform intensity varies widely across indications.

This **MarketVIEW** product is a comprehensive Executive Presentation (.pdf) plus data worksheet (.xls) outlining an up-to-date review of the gene therapy R&D sector. 8 rare **disease indications** are profiled along with key gene late-stage therapy programs and their mechanism of action (MOA)/technology. This review digs deeper and investigates current drivers/potential whitespace areas for any client wishing an opportunity assessment of this exciting future area of transformative medicine.

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:

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

Contents – Summary presentation (.pdf)



Contents

Author's notes

Executive summary

[SECTION 1] Analysis of the gene therapy landscape for rare diseases

[SECTION 2] Company involvement/investment in the gene therapy landscape for rare diseases

[SECTION 3] Disease and R&D landscape profiles: per 8 key indications

Beta thalassemia | Duchenne Muscular Dystrophy | Retinitis Pigmentosa | Amyotrophic lateral sclerosis | Scleroderma | Lupus | Myasthenia Gravis | Hemophilia A

[SECTION 4] Future technological drivers, new generation platforms for rare-disease gene therapies

References/bibliography

About **VacZine Analytics**

Disclaimer

SNAPSHOT

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

Contents – MS-Excel workbook (.xls)



Pipeline and company analysis

Beta thalassemia

Duchenne Muscular Dystrophy

Retinitis Pigmentosa

Amyotrophic lateral sclerosis

Scleroderma

Lupus

Myasthenia Gravis

Hemophilia A

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Table 1: companies covered in this report

Company	Company	Company
4D Molecular Therapeutics	A.O.U. Città della Salute e della Scienza	AAVantgarde Bio Srl
ALS Therapy Development Institute	ASC Therapeutics	AbbVie
Abdullah Kutlar	Accelaron Pharma, Inc., a wholly-owned subsidiary of Merck & Co., Inc., Rahway, NJ USA	Acibadem University
Ambulanzpartner Soziotechnologie APST GmbH	American Thrombosis and Hemostasis Network	Amgen
Anhui Provincial Hospital	Arrowhead Pharmaceuticals	Artgen Biotech
Assistance Publique - Hôpitaux de Paris	Assistance Publique Hopitaux De Marseille	Assiut University
Astellas Pharma Inc	AstraZeneca	Avigen
Barrow Neurological Institute	Baxalta now part of Shire	Bayer
Baylor College of Medicine	Be Biopharma	Beacon Therapeutics
Beijing Tiantan Hospital	Belief BioMed (Beijing) Co., Ltd	BioMarin Pharmaceutical
Biocad	Biogen	Biomedical Research Foundation, Academy of Athens
Bioray Laboratories	Blood Care Clinic	Boston Children's Hospital
Boston Pharmaceuticals	Boston University	Bristol-Myers Squibb
CRISPR Therapeutics	CSL Behring	CSPC ZhongQi Pharmaceutical Technology Co., Ltd.
Cairo University	Centre Hospitalier National d'Ophtalmologie des Quinze-Vingts	Centre Hospitalier Régional d'Orléans
Centre Hospitalier Universitaire de Liege	Centre Hospitalier Universitaire de Nîmes	Chigenovo Co., Ltd
Children's Hospital of Philadelphia	Chongbo Zhao	Christian Medical College, Vellore, India
Columbia University	Craig McDonald, MD	Cure Rare Disease, Inc
CytoCares Inc	Da, Yuwei, M.D.	Daishi Tian
Daniel Bauer	David Williams	DxTerty Diagnostics
Edgewise Therapeutics, Inc.	Editas Medicine, Inc.	Edward Kasaraskis
Eli Lilly and Company	Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD)	Expression Therapeutics, LLC
First Affiliated Hospital of Guangxi Medical University	Fondazione Angelo Bianchi Bonomi	Fondazione Don Carlo Gnocchi Onlus
Fondazione I.R.C.C.S. Istituto Neurologico Carlo Besta	Fondazione Policlinico Universitario Agostino Gemelli IRCCS	Foundation Fighting Blindness

Frontera Therapeutics	GenSight Biologics	Genentech, Inc.
Genervon Biopharmaceuticals, LLC	Genetix Biotherapeutics Inc.	Georgetown University
GlaxoSmithKline	Great Ormond Street Hospital for Children NHS Foundation Trust	Gritgen Therapeutics Co., Ltd.
Groupe Maladies hémorragiques de Bretagne	Haemnet	Hoffmann-La Roche
Hong Chen	Hospices Civils de Lyon	Hu Peng
HuidaGene Therapeutics Co., Ltd.	IRCCS Eugenio Medea	IRCCS San Raffaele
ITF Therapeutics LLC	Imperial College London	InCor Heart Institute
Insmad Gene Therapy LLC	Institut National de la Santé Et de la Recherche Médicale, France	Institute of Hematology & Blood Diseases Hospital, China
Iron Horse Diagnostics, Inc.	Istituti Clinici Scientifici Maugeri SpA	Istituto Auxologico Italiano
Istituto Superiore di Sanità	Jaeb Center for Health Research	Janssen Research & Development, LLC
Jerry R. Mendell	Jinnah Burn and Reconstructive Surgery Centre, Lahore	Johns Hopkins University
Juventas Cell Therapy Ltd.	Kanglin Biotechnology (Hangzhou) Co., Ltd.	Kevin Flanigan
King Khaled Eye Specialist Hospital	Kobe University	Laboratoires Thea
Lantu Biopharma	Masonic Cancer Center, University of Minnesota	Massachusetts General Hospital
MedImmune LLC	Mediar Therapeutics	Medical College of Wisconsin
Megan Waldrop	MeiraGTx UK II Ltd	Memorial Sloan Kettering Cancer Center
Meyer Children's Hospital IRCCS	NHS Greater Glasgow and Clyde	NS Pharma, Inc.
NYU Langone Health	Nanfang Hospital, Southern Medical University	Nanoscope Therapeutics Inc.
National Cancer Institute (NCI)	National Center for Research Resources (NCRR)	National Eye Institute (NEI)
National Heart, Lung, and Blood Institute (NHLBI)	National Human Genome Research Institute (NHGRI)	National Institute of Allergy and Infectious Diseases (NIAID)

National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS)	National Institute of Blood and Marrow Transplant (NIBMT), Pakistan	National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK)
National Institute of Neurological Disorders and Stroke (NINDS)	Nationwide Children's Hospital	Neovacs
Neuromed IRCCS	NightstaRx Ltd, a Biogen Company	Northwell Health
Northwestern University	Novartis Pharmaceuticals	Novo Nordisk A/S
OMNI Medical Services, LLC	Ocugen	Oliver Blanchard

Ontario Neurodegeneration Disease Research Initiative	Orthogonal Neuroscience Inc.	Ospedale Policlinico San Martino
PTC Therapeutics	PYC Therapeutics	Peking Union Medical College Hospital
Peking University Third Hospital	Pfizer	Phoenicia BioScience
Prism Pharma Co., Ltd.	ProQR Therapeutics	QLT Inc.
Qiong Fu	REGENXBIO Inc.	Ractigen Therapeutics.
Rare Partners srl Impresa Sociale	Ray Therapeutics, Inc.	Red de Terapia Celular
Regeneron Pharmaceuticals	RenJi Hospital	Rhythm Pharmaceuticals, Inc.
Richard Bedlack, M.D., Ph.D.	Rigshospitalet, Denmark	STZ eyetrial
Saiful Anwar Hospital	Samus Therapeutics, Inc.	San Rocco Therapeutics
Sanford Health	Sangamo Therapeutics	Sardocor Corp.
Sarepta Therapeutics, Inc.	Sensor Technology for Deafblind	Shanghai BDgene Co., Ltd.
Shanghai Jiao Tong University School of Medicine	Shanghai Siponuoyin Biotechnology Co Ltd	Shanghai Vitalgen BioPharma Co., Ltd.
Shanghai Xinzhi BioMed Co., Ltd.	Shenzhen Geno-Immune Medical Institute	Shenzhen Hemogen
SineuGene Therapeutics Co., Ltd.	Singapore National Eye Centre	Sohag University
Solid Biosciences Inc.	SparingVision	Spark Therapeutics, Inc.
Spur Therapeutics	St. Jude Children's Research Hospital	Stanford University

Sun Yat-sen University	The Affiliated Nanjing Drum Tower Hospital of Nanjing University Medical School	The Children's Hospital of Zhejiang University School of Medicine
The First Affiliated Hospital, Guangzhou University of Traditional Chinese Medicine	The Rogosin Institute	Tianjin Medical University General Hospital
Tongji Hospital	Transposon Therapeutics, Inc.	Truway Health, Inc.
UMC Utrecht	Ultragenyx Pharmaceutical Inc	Ulysses Neuroscience LTD
UniQure Biopharma B.V.	Union Hospital, Tongji Medical College, Huazhong University of Science and Technology	University College, London
University Hospital Tuebingen	University Hospital, Grenoble	University Hospital, Montpellier
University Hospital, Strasbourg, France	University Hospital, Tours	University Medical Center Groningen
University of Aarhus	University of British Columbia	University of Cagliari
University of California, San Francisco	University of Campania Luigi Vanvitelli	University of Cologne

University of Colorado, Denver	University of Göttingen	University of Illinois at Chicago
University of Massachusetts, Worcester	University of North Carolina, Chapel Hill	University of Oxford
University of Texas Southwestern Medical Center	University of Washington	Università degli Studi di Ferrara
Vanderbilt University Medical Center	Vector Y Therapeutics	VeonGen Therapeutics GmbH
Vertex Pharmaceuticals Incorporated	Wake Forest University Health Sciences	Washington University School of Medicine
West China Hospital	World Federation of Hemophilia	XDx
Zhejiang University	Zhongmou Therapeutics	eyeDNA Therapeutics
iCell Gene Therapeutics	yilong Wang	



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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.

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