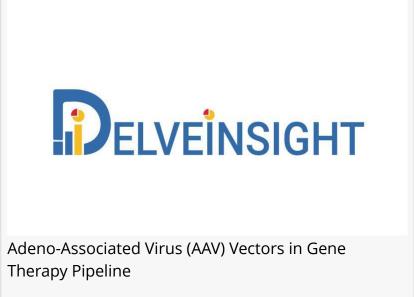


## Adeno-Associated Virus (AAV) Vectors in Gene Therapy Pipeline 2024: Clinical Trials, FDA, EMA, and PMDA Approvals

Adeno-Associated Virus (AAV) Vectors in Gene Therapy Pipeline

DELHI, DELHI, INDIA, July 1, 2024 /EINPresswire.com/ -- Delvelnsight's, "AAV Vectors in Gene Therapy Pipeline Insight 2024" report provides comprehensive insights about 70+ companies and 235+ pipeline drugs in AAV vectors in gene therapy pipeline landscape. It covers the pipeline drug profiles, including clinical and nonclinical stage products. It also covers the therapeutics assessment by product type, stage, route of



vers the therapeutics assessment by

administration, and molecule type. It further highlights the inactive pipeline products in this space.

Request a sample and discover the recent advances in AAV vectors in gene therapy @ <u>AAV Vectors in Gene Therapy Pipeline Outlook</u>

Key Takeaways from the AAV Vectors in Gene Therapy Pipeline Report

- DelveInsight's Adeno-Associated Virus Vectors in Gene Therapy pipeline report depicts a robust space with 70+ active players working to develop 235+ pipeline therapies for Adeno-Associated Virus Vectors in Gene Therapy treatment.
- The leading companies working in the Adeno-Associated Virus Vectors in Gene Therapy Market include BioMarin Pharmaceutical, Gensight Biologics, PTC therapeutics, Ultragenyx Pharmaceutical, MeiraGTx, Pfizer, Biogen, uniQure, Ultragenyx Pharmaceutical, REGENXBIO, Biogen, Spark therapeutics (Roche), Sarepta therapeutics, Neurophth Therapeutics, LYSOGENE, Gyroscope Therapeutics, Nanoscope Therapeutics, Homology medicines, Ultragenyx Pharmaceutical, Passage Bio, Freeline therapeutics, Astellas Pharma, Aspa therapeutics, Adrenas Therapeutics, ESTEVE, Sio Gene Therapies, Amicus therapeutics, 4D Molecular therapeutics, Taysha Gene Therapies, Atsena Therapeutics, BioMarin Pharmaceutical, Abeona Therapeutics,

REGENXBIO, uniQure, Taysha Gene Therapies, Asklepios BioPharmaceutical, Sarepta Therapeutics, Abeona Therapeutics, Forge Biologics, Sangamo therapeutics, and others.

- Promising Adeno-Associated Virus Vectors in Gene Therapy Pipeline Therapies in the various stages of development include Valoctocogene Roxaparvovec, AAV CNGB3, SB-525 (PF-07055480), AAV2/5-RPGR, BMN 307, GC301, rAAV-Olig001-ASPA, and others.
- May 2024:- UniQure Biopharma B.V.- A Phase Ib/II Randomized, Double-Blind Study to Explore Safety, Tolerability, and Efficacy Signals of Multiple Doses of Striatally-Administered rAAV5-miHTT Total Huntingtin Gene (HTT) Lowering Therapy (AMT 130) in Early Manifest Huntington's Disease. This is the second study of AMT-130 in patients with early manifest HD and is designed as part of an integrated two-study phase I/II program under a single data safety monitoring board (DSMB) with staggered enrollment based upon continued demonstration of safety of AMT-130 administration.
- May 2024:- Tenaya Therapeutics- Seroprevalence Study of Pre-existing Antibodies Against Adenovirus-associated Virus Vector (AAV9) in Patients With Plakophilin 2 (PKP2)-Associated Arrhythmogenic Right Ventricular Cardiomyopathy (ARVC). This is a multicenter, non-interventional study to observe the natural progression of the disease and to study the prevalence of pre-existing antibodies to AAV9 used for gene therapy in a population of patients with PKP2 gene-associated ARVC. Participation from all patients is encouraged regardless of interest in or eligibility for gene therapy.

Learn more about the AAV vectors for gene therapy in clinical trials @ <u>New AAV Vectors for Gene Therapy Drugs</u>

AAV vectors in Gene Therapy Emerging Drugs

- GS010: Gensight Biologics
- Valoctocogene roxaparvovec: BioMarin Pharmaceutical
- DTX401: Ultragenyx Pharmaceutical
- AAV5-RPGR: MeiraGTx
- Timrepigene emparvovec: Biogen
- · AMT 061: UniQure
- RGX-314: REGENXBIO
- SPK-8011: Spark Therapeutics
- NFS-01: Neurophth
- GT 005: Gyroscope Therapeutics

Discover more about AAV vectors in gene therapy in development @ <u>AAV Vectors in Gene Therapy Clinical Trials</u>

AAV Vectors in Gene Therapy: Therapeutic Assessment

There are approx. 70+ key Adeno-Associated Virus (AAV) Vectors in Gene Therapy companies developing Adeno-Associated Virus (AAV) Vectors in Gene Therapy therapies. The Adeno-Associated Virus (AAV) Vectors in Gene Therapy companies that have their AAV vectors in gene therapy drug candidates in the most advanced stage, i.e. Preregistration include BioMarin

Pharmaceutical.

To know more about AAV vectors in gene therapy, visit @ AAV Vector Technology Segmentation

## Adeno-Associated Vector Companies

BioMarin Pharmaceutical, Gensight Biologics, PTC therapeutics, Ultragenyx Pharmaceutical, MeiraGTx, Pfizer, Biogen, uniQure, Ultragenyx Pharmaceutical, REGENXBIO, Biogen, Spark therapeutics (Roche), Sarepta therapeutics, Neurophth Therapeutics, LYSOGENE, Gyroscope Therapeutics, Nanoscope Therapeutics, Homology medicines, Ultragenyx Pharmaceutical, Passage Bio, Freeline therapeutics, Astellas Pharma, Aspa therapeutics, Adrenas Therapeutics, ESTEVE, Sio Gene Therapies, Amicus therapeutics, 4D Molecular therapeutics, Taysha Gene Therapies, Atsena Therapeutics, BioMarin Pharmaceutical, Abeona Therapeutics, REGENXBIO, uniQure, Taysha Gene Therapies, Asklepios BioPharmaceutical, Sarepta Therapeutics, Abeona Therapeutics, Forge Biologics, Sangamo therapeutics, and others

Adeno-Associated Vector pipeline report provides the therapeutic assessment of the pipeline drugs by the Route of Administration. Products have been categorized under various ROAs such as

- Intracerebral
- Intracerebroventricular
- Intracisternal
- Intramuscular
- Intraocular
- Intrapancreatic
- Intrastriatal
- Intrathecal
- Intravenous
- Intravitreous
- Introvitinreous
- Oral
- Parenteral
- Retinal
- Intra-arterial
- Molecule Type

Adeno-Associated Vector Products have been categorized under various Molecule types such as

- Monoclonal Antibody
- Peptides
- Polymer
- Small molecule
- Gene therapy
- Product Type

For further information on the AAV vectors in gene therapy pipeline therapeutics, reach out @ AAV Vector Gene Therapy Products Development- <a href="https://www.delveinsight.com/sample-request/adeno-associated-virus-aav-vectors-in-gene-therapy-pipeline-insight?utm\_source=einpresswire&utm\_medium=promotion&utm\_campaign=ypr\_negetime.">https://www.delveinsight.com/sample-request/adeno-associated-virus-aav-vectors-in-gene-therapy-pipeline-insight?utm\_source=einpresswire&utm\_medium=promotion&utm\_campaign=ypr\_negetime.

Scope of the Adeno-Associated Virus (AAV) Vectors in Gene Therapy Pipeline Report

- Coverage- Global
- Adeno-Associated Virus Vectors in Gene Therapy Therapeutic Assessment by Product Type: Mono, Combination, Mono/Combination
- Adeno-Associated Virus Vectors in Gene Therapy Therapeutic Assessment by Clinical Stages: Discovery, Pre-clinical, Phase I, Phase II
- Adeno-Associated Virus Vectors in Gene Therapy Companies- BioMarin Pharmaceutical, Gensight Biologics, PTC therapeutics, Ultragenyx Pharmaceutical, MeiraGTx, Pfizer, Biogen, uniQure, Pfizer, Ultragenyx Pharmaceutical, REGENXBIO, Biogen, Spark therapeuics (Roche), Sarepta therapeutics, Neurophth Therapeutics, LYSOGENE, Gyroscope Therapeutics, Nanoscope Therapeutics, Homology medicines, Ultragenyx Pharmaceutical, Passage Bio, Freeline therapeutics, Astellas Pharma, Aspa therapeutics, Adrenas Therapeutics, ESTEVE, Sio Gene Therapies, Amicus therapeutics, 4D Molecular therapeutics, Taysha Gene Therapies, Atsena Therapeutics, BioMarin Pharmaceutical, Abeona Therapeutics, and others.
- Adeno-Associated Virus Vectors in Gene Therapy Pipeline Therapies- Valoctocogene Roxaparvovec, AAV CNGB3, SB-525 (PF-07055480), AAV2/5-RPGR, BMN 307, GC301, rAAV-Olig001-ASPA, and others.

Discover more about Adeno-Associated Virus (AAV) Vectors in Gene Therapy Drugs in development @ Adeno-Associated Virus (AAV) Vectors in Gene Therapy Unmet Needs-https://www.delveinsight.com/sample-request/adeno-associated-virus-aav-vectors-in-gene-therapy-pipeline-

insight?utm\_source=einpresswire&utm\_medium=promotion&utm\_campaign=ypr

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