Adeno-Associated Virus (AAV) Gene Therapy Pipeline | 180+ Companies Advancing Innovative Treatments

Leading pharmaceutical companies are advancing the AAV vector gene therapy pipeline and its future growth potential.

LAS VEGAS , NV, UNITED STATES, February 7, 2025 /EINPresswire.com/ -- DelveInsight's 'Adeno-Associated Virus (AAV) Vectors in Gene Therapy Pipeline Insight 2024' report provides comprehensive global coverage of pipeline Adeno-Associated Virus (AAV) Vectors in Gene Therapy therapies in various stages of clinical development, major pharmaceutical companies are working to advance the pipeline space and future growth potential of the Adeno-Associated Virus (AAV) Vectors in Gene Therapy pipeline domain.

For Adeno-Associated Virus (AAV) Vectors in Gene Therapy emerging drugs, the Adeno-Associated Virus (AAV) Vectors in Gene Therapy pipeline analysis report provides a 360° view of the therapeutics landscape by development point, product type, route of administration, molecule type, and MOA. The pipeline research covers business opportunities, challenges, future partnerships, strong competitors, and growth strategies.

Key Takeaways from the Adeno-Associated Virus (AAV) Vectors in Gene Therapy Pipeline Report
• DelveInsight's Adeno-Associated Virus (AAV) Vectors in Gene Therapy Pipeline analysis depicts a robust space with 180+ active players working to develop 200+ pipeline drugs for Adeno-Associated Virus (AAV) Vectors in Gene Therapy treatment.
• The leading Adeno-Associated Virus (AAV) Vectors in Gene Therapy companies include Belief BioMed, Gensight Biologics, Johnson & Johnson Innovative Medicine/MeiraGTx, Passage Bio, InnoVec Biotherapeutics, Ultragenyx Pharmaceutical, MeiraGTx, Biogen, REGENXBIO, Beacon Therapeutics, Genethon, 4D Molecular Therapeutics, Adverum Biotechnologies, Rocket Pharmaceuticals, Innostellar Biotherapeutics, Aspa Therapeutics, iECURE, AviadoBio, Sarepta Therapeutics, Ray Therapeutics, Genascence Corporation, Exegenesis Bio, DiNAQOR, Tenaya

Therapeutics, InnoVec Biotherapeutics, Jaguar Gene Therapy, LLC, Neurophth, and others are evaluating their lead assets to improve the Adeno-Associated Virus (AAV) Vectors in Gene Therapy treatment landscape.

• Key Adeno-Associated Virus (AAV) Vectors in Gene Therapy pipeline therapies in various stages of development include BBM-H901, GS010, AAV-CNGA3, PBGM01, IVB102, BMN 307, AGTC-402, ADVM-022, AAV - CNGB3, AAV9 BBP-812, SPK-9001, AAV RPE65, VG901, rAAV1-CB-hAAT, AAV2hAQP1, valoctocogene roxaparvovec, GS030-DP, and others.

• In January 2025, MeiraGTx UK II Ltd initiated Phase 1 of a dose-escalation study evaluating the subretinal administration of the AAV5-hRKp.RPGR vector in participants with X-linked retinitis pigmentosa (XLRP) caused by RPGR mutations. Participants were assigned to dose groups based on sequential enrollment.

• In January 2025, ViGeneron GmbH achieved two key milestones for VG901, its gene therapy for CNGA1-related retinitis pigmentosa. The FDA granted it Rare Pediatric Disease Designation, and the Data Safety Monitoring Board approved dose escalation in the ongoing Phase 1b trial.

• In January 2025, Beacon Therapeutics announced that the FDA granted Regenerative Medicine Advanced Therapy (RMAT) designation to laru-zova (laruparetigene zovaparvovec) for treating X-linked retinitis pigmentosa (XLRP), reinforcing its commitment to restoring vision for patients with blinding retinal diseases.

• In January 2025, Janssen Pharmaceuticals Inc. launched a study to assess the safety and tolerability of subretinal delivery of the AAV5-hRKp.RPGR gene therapy in both adult and pediatric patients with XLRP.

• In January 2025, CSL Behring commenced a Phase III, open-label, single-dose, multinational trial investigating the AAV5-hFIXco-Padua (AMT-061) gene therapy. This therapy utilizes a serotype 5 adeno-associated viral vector containing the Padua variant of a codon-optimized human Factor IX gene for adult patients with severe or moderately severe Hemophilia B.

Request a sample and discover the recent breakthroughs happening in the AAV Vectors in Gene Therapy pipeline landscape @ Adeno-Associated Virus (AAV) Vectors in Gene Therapy Pipeline Outlook

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

Adeno-Associated Virus (AAV) vectors are a leading gene therapy delivery system, known for their precision in transferring therapeutic genes with minimal immune response. As non-pathogenic viruses naturally present in humans, AAVs offer a safe and effective approach for in vivo gene delivery. Their ability to integrate into cells ensures long-term gene expression, making them ideal for treating conditions requiring sustained therapeutic effects.

Different AAV serotypes enable targeted treatments, with AAV9 crossing the blood-brain barrier for CNS disorders and AAV2 proving effective in ocular therapies. This adaptability supports the treatment of genetic disorders like hemophilia, SMA, and inherited retinal diseases. Approved AAV-based therapies, including Luxturna and Zolgensma, highlight gene therapy's growing impact. However, AAV vector production remains a major challenge due to high costs and scalability issues. Innovations like baculovirus-insect cell systems aim to enhance manufacturing

efficiency, but further advancements are crucial to improving accessibility.

Find out more about Adeno-Associated Virus (AAV) Vectors in Gene Therapy medication @ https://www.delveinsight.com/report-store/adeno-associated-virus-aav-vectors-in-gene-therapy-pipeline-insight?utm_source=einpresswire&utm_medium=pressrelease&utm_campaign=jpr

Adeno-Associated Virus (AAV) Vectors in Gene Therapy Treatment Analysis: Drug Profile

BBM-H901: Belief BioMed
BBM-H901 is indicated for prophylactic treatment of bleeding in adults with hemophilia B. BBM-H901 is designed to restore the production of factor IX (FIX) the blood-clotting protein that is faulty or missing in people with hemophilia B by delivering a functional copy of the F9 gene to liver cells, which are the main producers of clotting factors in the body. Gene therapy components are delivered to liver cells by a modified, harmless adeno-associated virus (AAV) that works as a vehicle. The drug is currently being registered for the treatment of hemophilia B.

GS010: Gensight Biologics
LUMEVOQ (GS010; lenadogene nolparvovec) targets Leber Hereditary Optic Neuropathy (LHON) by leveraging a mitochondrial targeting sequence (MTS) proprietary technology platform, arising from research conducted at the Institut de la Vision in Paris, which, when associated with the gene of interest, allows the platform to specifically address defects inside the mitochondria using an AAV vector (Adeno-Associated Virus). The gene of interest is transferred into the cell to be expressed and produces the functional protein, which will then be shuttled to the mitochondria through specific nucleotidic sequences in order to restore the missing or deficient mitochondrial function. “LUMEVOQ” was accepted as the invented name for GS010 (lenadogene nolparvovec) by the European Medicines Agency (EMA) in October 2018. Currently, the drug is in the Phase III stage of its development for the treatment of Leber Hereditary Optic Neuropathy.

Key Adeno-Associated Virus (AAV) Vectors in Gene Therapy Therapies and Companies
• BBM-H901: Belief BioMed
• GS010: Gensight Biologics
• AAV-CNGA3: Johnson & Johnson Innovative Medicine/ MeiraGTx
• PBGM01: Passage Bio
• IVB102: InnoVec Biotherapeutics
• BMN 307: BioMarin Pharmaceutical
• AGTC-402: Beacon Therapeutics
• ADVM-022: Adverum Biotechnologies
• AAV - CNGB3: MeiraGTx
• SPK-9001: Pfizer/Spark Therapeutics
• AAV RPE65: MeiraGTx
• VG901: ViGeneron
• rAAV1-CB-hAAT: Beacon Therapeutics

• AAV2hAQP1: MeiraGTx
• valoctocogene roxaparvovec: BioMarin Pharmaceutical
• GS030-DP: GenSight Biologics

Learn more about the novel and emerging Adeno-Associated Virus (AAV) Vectors in Gene Therapy pipeline therapies @ https://www.delveinsight.com/report-store/adeno-associated-virus-aav-vectors-in-gene-therapy-pipeline-insight?utm_source=einpresswire&utm_medium=pressrelease&utm_campaign=jpr

Adeno-Associated Virus (AAV) Vectors in Gene Therapy Therapeutics Assessment

By Product Type
• Mono
• Combination
• Mono/Combination.

By Stage
• Late-stage products (Phase III)
• Mid-stage products (Phase II)
• Early-stage product (Phase I) along with the details of
• Pre-clinical and Discovery stage candidates
• Discontinued & Inactive candidates

By Route of Administration
• Intravenous
• Subcutaneous
• Oral
• Intramuscular

By Molecule Type
• Monoclonal antibody
• Small molecule
• Peptide

Scope of the Adeno-Associated Virus (AAV) Vectors in Gene Therapy Pipeline Report
• Coverage: Global
• Key Adeno-Associated Virus (AAV) Vectors in Gene Therapy Companies: Belief BioMed, Gensight Biologics, Johnson & Johnson Innovative Medicine/MeiraGTx, Passage Bio, InnoVec Biotherapeutics, Ultragenyx Pharmaceutical, MeiraGTx, Biogen, REGENXBIO, Beacon Therapeutics, Genethon, 4D Molecular Therapeutics, Adverum Biotechnologies, Rocket Pharmaceuticals, Innostellar Biotherapeutics, Aspa Therapeutics, iECURE, AviadoBio, Sarepta Therapeutics, Ray Therapeutics, Genascence Corporation, Exegenesis Bio, DiNAQOR, Tenaya Therapeutics, InnoVec Biotherapeutics, Jaguar Gene Therapy, LLC, Neurophth, and others

• Key Adeno-Associated Virus (AAV) Vectors in Gene Therapy Pipeline Therapies: BBM-H901, GS010, AAV-CNGA3, PBGM01, IVB102, BMN 307, AGTC-402, ADVM-022, AAV - CNGB3, AAV9 BBP-812, SPK-9001, AAV RPE65, VG901, rAAV1-CB-hAAT, AAV2hAQP1, valoctocogene roxaparvovec, GS030-DP, and others.

Dive deep into rich insights for drugs used for Adeno-Associated Virus (AAV) Vectors in Gene Therapy treatment; visit @ Adeno-Associated Virus (AAV) Vectors in Gene Therapy Drugs

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Jatin Vimal
DelveInsight Business Research LLP
+1 469-945-7679
info@delveinsight.com
