Adeno-Associated Virus Vectors in Gene Therapy Market Size 2034 | Clinical Trials, Latest FDA, EMA, PDMA Approvals

Adeno-Associated Virus Vectors in Gene Therapy Market

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DELHI, DELHI, INDIA, June 25, 2024 /EINPresswire.com/ -- DelveInsight's "Adeno-Associated Virus Vector in Gene Therapy Market Insights, Epidemiology, and Market Forecast–2034" report delivers an in-depth understanding of Adeno-Associated Virus Vector in Gene Therapy, historical and forecasted epidemiology as well as Adeno-Associated Virus Vector in Gene Therapy market trends in the United States, EU4 (Germany, France, Italy, and Spain) and the United Kingdom, and Japan.

Explore the intricate details of the Adeno-Associated Virus Vectors in Gene Therapy Market: Uncover drug uptake, treatment dynamics, and epidemiological trends with our comprehensive Adeno-Associated Virus Vectors in Gene Therapy Market Forecast. Click here to stay ahead in healthcare innovation @ Adeno-Associated Virus Vectors in Gene Therapy Market Size

Key Takeaways from the Adeno-Associated Virus Vectors in Gene Therapy Market Report

• June 2024:- Aspa Therapeutics- A Phase 1/2 Open-Label Study of the Safety and Clinical Activity of Gene Therapy for Canavan Disease Through Administration of an Adeno-Associated Virus (AAV) Serotype 9-Based Recombinant Vector Encoding the Human ASPA Gene. The main objective of this trial is to evaluate the safety, tolerability, and pharmacodynamic activity of BBP-812, an investigational AAV9-based gene therapy, in pediatric participants with Canavan disease.

• June 2024:- Grace Science LLC- A Phase 1/2/3 Open-label, Single Arm, Dose-finding Study to Investigate Long-term Safety, Tolerability and Efficacy of GS-100, an Adeno-associated Virus Serotype 9 (AAV9) Vector-mediated Gene Transfer of Human NGLY1, in Patients With NGLY1 Deficiency. A non-randomized, open-label, dose escalation study of a single

intracerebroventricular (ICV) administration of a gene replacement therapy in subjects who are 2 to 18 years old with NGLY1 Deficiency.
• Hemophilia A is more common than Hemophilia B, representing approximately 80–85% of the total hemophilia population.
• The leading Adeno-Associated Virus Vectors in Gene Therapy Companies such as Biomarin Pharmaceutical, Sarepta Therapeutics, Roche (Spark Therapeutics), Sangamo, Pfizer, NightstaRx, Freeline Therapeutics, Horama S.A, MeiraGTx, RegenxBio, Asklepios Biopharmaceutical, Audentes Therapeutics, and others.
• Promising Adeno-Associated Virus Vectors in Gene Therapy Therapies such as BMN 307, AAV9 BBP-812, GS-100, RP-A501, AAV5-hFIXco-Padua, AB-1001 Gene Therapy, and others.

Navigate the complexities of the Adeno-Associated Virus Vectors in Gene Therapy Market: Gain insights into drug trends, treatment scenarios, and epidemiological data through our insightful Adeno-Associated Virus Vectors in Gene Therapy Market Forecast. Click here to get more insights @ Adeno-Associated Virus Vectors in Gene Therapy Treatment Market

Adeno-Associated Virus Vectors in Gene Therapy Epidemiology Segmentation in the 7MM
• Total Prevalent Cases of Selected Indications
• Total Diagnosed Prevalent Cases of Selected Indications
• Total Treated Cases of Selected Indications

Delve deep into the Adeno-Associated Virus Vectors in Gene Therapy Market Landscape: Analyze drug adoption, treatment paradigms, and epidemiological shifts in our detailed Adeno-Associated Virus Vectors in Gene Therapy Market Forecast. Click here to shape the future @ Adeno-Associated Virus Vectors in Gene Therapy Prevalence

Adeno Associated Virus Vectors in Gene Therapy Marketed Drugs
• LUXTURNA: Spark Therapeutics
LUXTURNA (voretigene neparvovec-rzyl) is a suspension of an adeno-associated virus vector-based gene therapy for subretinal injection. LUXTURNA is a live, non-replicating adeno-associated virus serotype 2 which has been genetically modified to express the human RPE65 gene. LUXTURNA is derived from naturally occurring adeno-associated virus using recombinant DNA techniques. LUXTURNA, is a one-time gene therapy for the treatment of patients with vision loss due to a genetic mutation in both copies of the RPE65 gene. The FDA approved Spark Therapeutics' LUXTURNA in December 2017.
• ZOLGENSMA: Novartis
ZOLGENSMA is a suspension of an adeno-associated viral vector-based gene therapy for intravenous infusion. It is a recombinant self-complementary AAV9 containing a transgene encoding the human survival motor neuron (SMN) protein, under the control of a cytomegalovirus enhancer/chicken-β-actin hybrid promoter. ZOLGENSMA an AAV-delivered gene therapy used to treat spinal muscular atrophy (SMA), was approved for clinical use in the US by the FDA.

Adeno Associated Virus Vectors in Gene Therapy Emerging Drugs

- LUMEVOQ: 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. According to Phase III results all treated participants showed sustained improvement over 4 years, but that patients treated with a bilateral injection of the gene therapy continued to have a better visual acuity than the patients treated with a unilateral injection, a disparity that had been seen in REFLECT since 1.5 years posttreatment. The company is planning to submit to MHRA in the second half of 2024 and aiming to receive a decision from MHRA on LUMEVOQ in the second half of 2025, in the hopes of commercializing the product in the UK that same year.

- Giroctocogene fitelparvovec: Sangamo and Pfizer

Giroctocogene fitelparvovec comprises of a recombinant AAV6 encoding the complementary deoxyribonucleic acid for B-domain deleted human FVIII. It is being developed as part of a collaboration agreement for the global development and commercialization of gene therapies for hemophilia A between Sangamo and Pfizer. Currently, the company is conducting Phase III trials to attain better and clear results about the efficacy of SB-525. A Phase III clinical trial (NCT03587116) evaluates the effectiveness and safety of preventive replacement therapy in the usual care setting in hemophilia A patients. A pivotal readout is expected in mid-2024, with Pfizer anticipating BLA and MAA submissions in the second half of 2024 if the pivotal readout is supportive.

Unlock insights into the Adeno-Associated Virus Vectors in Gene Therapy Market: discover drug uptake patterns, treatment landscapes, and epidemiological insights with our exclusive Adeno-Associated Virus Vectors in Gene Therapy Market Forecast. Click here @ Adeno-Associated Virus Vectors in Gene Therapy Market Drivers and Barriers- https://www.delveinsight.com/sample-request/adeno-associated-virus-vectors-in-gene-therapy-market?utm_source=einpresswire&utm_medium=pressrelease&utm_campaign=ypr

Adeno-Associated Virus Vector in Gene Therapy Market Outlook

Adeno Associated Virus Vectors in Gene Therapy has provided a unique opportunity to treat and even cure degenerative diseases, offering hope to the millions of people either affected by inherited disorders or carrying disease-causing mutations. Addressing optimal intervention timing, standardized outcome assessments, inflammation mitigation, awareness enhancement, and equitable access are key to advancing inherited retinal disease treatments and reshaping the landscape of visual impairment. Gene therapies have brought about a change in the treatment paradigm for genetic diseases by providing lasting therapeutic effects with a single intervention. Gene therapy holds significant potential for addressing various eye diseases. However, individuals considering this treatment should be mindful of certain challenges and crucial factors. Although the recent successes in rare disease therapy approvals have provided

momentum for AAV therapy research and funding, several limitations make this a difficult, capital-intensive platform to develop.

Adeno-Associated Virus Vectors in Gene Therapy Drug Market
The report provides insights into therapeutic candidates in Phase III, Phase II/III, and Phase II. It also analyzes key players involved in developing targeted therapeutics. Companies like Sangamo and Pfizer, MEIRAGTx/J&J, Johnson & Johnson, and others actively engage in late and mid-stage research and development efforts for Adeno-Associated Virus Vector in Gene Therapy pipeline possesses potential drugs. However, there is a positive outlook for the therapeutics market, with expectations of growth during the forecast period (2024–2034).

Gain a strategic edge in the Adeno-Associated Virus Vectors in Gene Therapy Market: explore comprehensive drug insights, treatment updates, and epidemiological forecasts in our in-depth Adeno-Associated Virus Vectors in Gene Therapy Market Forecast. Click here to lead in advancements @ Adeno-Associated Virus Vectors in Gene Therapy Clinical Trials Assessment- https://www.delveinsight.com/sample-request/adeno-associated-virus-vectors-in-gene-therapy-market?utm_source=einpresswire&utm_medium=pressrelease&utm_campaign=ypr

Scope of the Adeno-Associated Virus Vectors in Gene Therapy Market Report
- Coverage- 7MM
- Adeno-Associated Virus Vectors in Gene Therapy Companies- Biomarin Pharmaceutical, Sarepta Therapeutics, Roche (Spark Therapeutics), Sangamo, Pfizer, NightstaRx, Freeline Therapeutics, Horama S.A, MeiraGTx, RegenxBio, Asklepios Biopharmaceutical, Audentes Therapeutics, and others.
- Adeno-Associated Virus Vectors in Gene Therapy Therapies- BMN 307, AAV9 BBP-812, GS-100, RP-A501, AAV5-hFIXco-Padua, AB-1001 Gene Therapy, and others.
- Adeno-Associated Virus Vectors in Gene Therapy Market Dynamics: Adeno-Associated Virus Vectors in Gene Therapy Market Drivers and Barriers
- Adeno-Associated Virus Vectors in Gene Therapy Market Access and Reimbursement, Unmet Needs and Future Perspectives

Dive deeper into the comprehensive insights and projections for the Adeno-Associated Virus Vectors in Gene Therapy market by accessing the full Adeno-Associated Virus Vectors in Gene Therapy drug market report @ https://www.delveinsight.com/sample-request/adeno-associated-virus-vectors-in-gene-therapy-market?utm_source=einpresswire&utm_medium=pressrelease&utm_campaign=ypr

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DelveInsight is a leading Healthcare Business Consultant, and Market Research firm focused exclusively on life sciences. It supports Pharma companies by providing comprehensive end-to-end solutions to improve their performance. It also offers Healthcare Consulting Services, which benefits in market analysis to accelerate the business growth and overcome challenges with a practical approach.

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