Affinia Therapeutics to Present Data on Novel Cardiotropic and BBB-Penetrant AAV Capsids and Preclinical Efficacy and Safety in Genetic Cardiomyopathies and Sporadic ALS at the American Society of Gene & Cell Therapy 2024 Annual Meeting

WALTHAM, Mass. – April 22, 2024 – Affinia Therapeutics ("Affinia"), an innovative gene therapy company with a proprietary platform for rationally designed adeno-associated virus (AAV) vectors and gene therapies for rare and prevalent devastating diseases, today announced that new preclinical data on its novel AAV capsids for genetic cardiomyopathies and diseases of the central nervous system (CNS) such as amyotrophic lateral sclerosis (ALS), as well as the Company's high-yield manufacturing process, will be presented in several oral and poster sessions at the American Society of Gene and Cell Therapy (ASGCT) 2024 Annual Meeting, being held May 7-11, 2024 in Baltimore, MD and virtually.

Affinia has leveraged its proprietary platform to rationally design capsids with increased tropism to cardiac muscle, skeletal muscle, or CNS with more uniform tissue distribution than AAV9. This improved biodistribution is attained while detargeting the liver and dorsal root ganglia (DRG), both potential sites of toxicity. Affinia's novel capsids have favorable manufacturing yields and levels of preexisting population immunity. Preclinical efficacy and safety results with a cardiotropic vector encoding for the BAG3 protein provide proof of concept for the treatment of multiple genetic cardiomyopathies and with a blood-brain barrier (BBB)-penetrant vector encoding for the knockdown of Ataxin-2 (Atxn2), a gene believed to be involved in sporadic ALS, provide proof of concept for the treatment of multiple CNS diseases including ALS, supporting the development of potential medicines across a spectrum of rare and prevalent diseases.

"We are excited to unveil data on our next-generation bespoke capsids that demonstrate robust cardiac transduction while detargeting the liver and DRG. Similarly, our BBB-penetrant capsid detargets the liver and DRG and shows a promising profile for treating multiple CNS diseases," said Rick Modi, Affinia's Chief Executive Officer. "Our initial preclinical results in genetic cardiomyopathies and sporadic ALS are in line with our purpose to bring life-changing gene therapies to people suffering from life-threatening and disabling diseases."

- One oral presentation showcases data from Affinia's novel, BBB-penetrant AAV capsid with a potential therapeutic application to sporadic ALS. The data show that the capsid delivers an Atxn2 microRNA cargo that adequately decreases Atxn2 mRNA in non-human primates. Another oral presentation features Affinia's proprietary manufacturing process in HEK-293 suspension cells, yielding greater than 50% full capsids with greater than 1e15vg/L at harvest, with broad applicability across conventional and novel AAV capsids and with significant potential cost advantages relative to other gene therapies.
- Affinia will also hold two poster presentations. The first poster showcases data that confirm the novel cardiotropic AAV capsid's safety and therapeutic efficacy in an animal model of cardiac dysfunction with a potential therapeutic application in genetic cardiomyopathies. The company will also present a poster on a screening platform for the discovery of novel, next-generation CNS tropic capsids, based on binding to cell receptors that facilitate BBB transcytosis and with potential applicability to non-AAV targeted delivery modalities.

The oral and poster presentation details are provided below. Abstracts can be found at https://annualmeeting.asgct.org/. Data from the oral presentations are embargoed until 6:00 am ET on the presentation day.

Oral presentations

Title: Reduction of Atxn2, a therapeutic target for sporadic ALS, in non-human primates using a novel, intravenously delivered AAV capsid
Presenter: Giridhar Murlidharan, Ph.D., Senior Director, Head of Vector Translational Biology, Affinia
Session: Neurologic Diseases IV
Date/Time: Friday, May 10, 2024, 4:00-4:15 pm ET
Location: Room 307-308
Abstract Number: 304

Title: A proprietary HEK293 AAV production system can achieve greater than 50% full capsids with greater than 1e15vg/L at harvest enabling scalable chromatography-based polishing with high yield and purity
Presenter: Matt Edwards, MBA, Senior Director, Head of Process Science, Affinia
Session: AAV Manufacturing III
Date/Time: Friday, May 10, 2024, 4:00-4:15 pm ET
Location: Ballroom 3
Abstract Number: 290

Poster presentations

Title: Systemic Administration of a Novel Cardiotropic AAV Vector Encoding BAG3 Rescues Left Ventricle Functional Deficits in a Mouse Model of Myocardial Infarction
Presenter: Lisa Stanek, Ph.D., Vice President, Translational Science, Affinia
Date/Time: Wednesday, May 8, 2024, 12:00-7:00 pm ET
Location: Exhibit Hall
Abstract Number: 605

Title: Identification of Novel CNS-Tropic AAVs Using In Vitro Binding to Candidate Receptors Combined With In Vivo Screening, Generative AI, and Structural Modeling
Presenter: Charles Albright, Ph.D., Chief Scientific Officer, Affinia
Date/Time: Thursday, May 9, 2024, 12:00-7:00 pm ET
Location: Exhibit Hall
Abstract Number: 982

In addition, Affinia has been invited to present on the rational design of cardiotropic capsids that detarget the liver and DRG. Sherry Cao, Ph.D., Senior Vice President, Computational Science will deliver the presentation entitled, “Machine-learning guided rational design of cardiotropic capsids that detarget liver and DRG,” on Saturday, May 11, 2024 at 8:00 am ET in Room 339-342.

The Company also announced members from its leadership team have been invited to chair the following sessions:

Scientific Symposium: “Innovations in Cardiac Gene and Cell Therapy”

Chair: Laura Richman, MBA, D.V.M., Ph.D., DACVP, Chief Development Officer
Date/Time: Saturday, May 11, 2024, 8:00-9:45 am ET
Location: Room 339-342

Category of Abstracts: B5 – Neurologic Diseases Section 4
Chair: Lisa Stanek, Ph.D., Vice President, Translational Science
Date/Time: Friday, May 10, 2024, 3:45-5:00 pm ET
Location: Room 307-308

About Affinia Therapeutics
Affinia Therapeutics is pioneering a shift to a new class of rationally designed gene therapies that treat rare and prevalent diseases. Affinia Therapeutics' proprietary Affinia Rationally designed Therapeutics (ART) platform is intended to synergistically improve the efficacy, safety, and manufacturability of adeno-associated virus (AAV)-based gene therapies through the development of next-generation capsids, promoters, and manufacturing approaches. For more information, visit https://www.affiniatx.com.

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