Affinia Therapeutics Presents New Preclinical Data on Novel AAV Capsids for Skeletal Muscle, Cardiac Muscle, and CNS at the American Society of Gene & Cell Therapy 26TH Annual Meeting

WALTHAM, Mass. – May 18, 2023 – Affinia Therapeutics, an innovative gene therapy company with a proprietary platform for rationally designed adeno-associated virus (AAV) vectors and gene therapies for both rare and prevalent devastating diseases, today announced the presentation of new preclinical data on novel AAV capsids for skeletal muscle, cardiac muscle, and central nervous system (CNS). This research will be presented in an oral session today at 1:30pm PT at the American Society of Gene and Cell Therapy (ASGCT) 26th Annual Meeting, being held May 16-20, 2023 in Los Angeles, CA.

Affinia Therapeutics leveraged its proprietary platform to determine structure-activity relationships (SAR) that guided the design of next-generation AAV capsids with increased tropism to the desired tissues while detargeting the liver and dorsal root ganglia (DRG) which are potential sites of toxicity. These capsids were discovered using rational design techniques based on structural modeling and mechanistic hypotheses to optimize AAV backbones and peptides and have greatly increased mRNA expression in skeletal muscle, cardiac muscle, and CNS, while significantly detargeting the liver and DRG with more uniform tissue expression relative to AAV9. Further, these novel capsids have acceptable manufacturing yields and levels of pre-existing population immunity to support development of potential medicines across a broad spectrum of diseases.

In an oral presentation later today, Affinia Therapeutics will present nonhuman primate data showing that the company's skeletal muscle capsids increased mean mRNA expression by more than 500-fold in myofibrils, and the cardiac muscle capsids increased mean mRNA expression by approximately 75-fold relative to AAV9 with a uniform profile while detargeting the liver. These capsids were administered intravenously (IV). The CNS capsids crossed the blood-brain barrier via IV administration, reaching both cortical and deep brain tissues in nonhuman primates, transducing multiple cell types, including up to 60 percent of neurons. These CNS capsids showed a 100- to 1000-fold mean mRNA expression relative to AAV9.

"We have designed next-generation bespoke capsids with increased skeletal and cardiac muscle tropism while significantly reducing liver tropism," said Charles Albright, Ph.D., Chief Scientific Officer at Affinia Therapeutics. "In addition, potentially developing a blood-brain barrier-penetrant capsid with widespread distribution across the brain and into the deeper brain regions is extremely exciting. We look forward to advancing these capsids in additional preclinical studies to understand their potential to deliver transformative results for patients suffering from a host of life-threatening and disabling diseases."

The oral presentation details are as follows:

Title: Structure-Activity Relationships Guided Engineering of AAV Capsid with Optimized Skeletal Muscle, Cardiac Muscle, and CNS Tropism
Presenter: Charles Albright, Ph.D.
Session: AAV Engineering for CNS Targeting
Date/Time: Thursday, May 18, 2023, 1:30-1:45pm PT

Location: West Hall B
Abstract Number: 102

Affinia Therapeutics also presented additional new preclinical data in the following poster sessions during ASGCT.

Title: Limitations of Marmosets as an Animal Model for AAV Mediated Liver Gene Transfer
Presenter: Bryan Mastis
Session: Wednesday Poster Session
Poster Board Number: 362

Title: Improvement of Yield and Critical Quality Attributes Through Process Development of a Novel Adeno-Associated Viral Vector Capsid (Anc80L65)
Presenter: Thomas M. Edwards
Session: Thursday Poster Session
Poster Board Number: 990

Abstracts can be accessed via the conference website at https://annualmeeting.asgct.org/abstracts.

About Affinia Therapeutics
Affinia Therapeutics is pioneering a shift to a new class of rationally designed gene therapies that treat rare and prevalent devastating diseases. Affinia Therapeutics' proprietary Affinia Rationally designed Therapeutics (ART) platform consists of three pillars 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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Contact Information

Investors:
investors@affiniatx.com

Media:
Kathy Vincent
media@affiniatx.com