Affinia Therapeutics Presents New Data and Updates on AAV Platform at American Society of Gene and Cell Therapy Annual Meeting

Data shows multiple vectors with superiority to AAV9 in CNS, systemic approaches to target muscle while detargeting liver, and improvements in manufacturability

WALTHAM, Mass. – May 13, 2021 – Affinia Therapeutics, an innovative gene therapy company with a proprietary platform for rationally designed adeno-associated virus (AAV) vectors and gene therapies for rare and non-rare diseases, today announced results from preclinical studies presented in poster and oral sessions at the American Society of Gene and Cell Therapy (ASGCT) 24th Annual Meeting.

Challenges in achieving adequate expression in the central nervous system (CNS) with conventional AAV vectors have limited the use of gene therapy to address many devastating rare and non-rare CNS diseases. Affinia Therapeutics presented data in a poster session on its proprietary AAV vector Anc80L65 showing superiority to AAV9 in various non-human primate (NHP) CNS regions after a single intrathecal injection. When delivered via lumbar puncture, Anc80L65 demonstrated up to 32-fold higher RNA expression versus AAV9 delivered via the more invasive intracisternal route of administration. Anc80L65 led to higher expression than AAV9 in both the cortex and deep brain nuclei. The company also presented results in a poster session from a screen for CNS vectors with even greater superiority to AAV9, particularly in AAV delivery to deep CNS regions. Through this screen with an intrathecal route, Affinia Therapeutics has identified novel vectors with vector copy numbers in NHPs up to 256-fold higher than AAV9.

In neuromuscular diseases, the need for systemic administration of high doses of conventional AAV vectors to achieve therapeutic levels of expression may lead to liver toxicity. Data presented in a poster session on Affinia Therapeutics' AAVmod2 demonstrated the application of the company's platform to this problem in a rationally designed manner: the vector contains one modification that detargeted the liver and a second modification that increased muscle expression in mice compared to AAV9.

In an oral presentation later today, Affinia Therapeutics will present data on the use of its platform to characterize AAV binding to the AAVX resin, which is frequently used in vector manufacturing.

"Since the company's inception, our team of gene therapy experts has focused on tackling some of the greatest challenges in the AAV field. We are working to identify differentiated vectors to expand the promise of AAV in areas including CNS and neuromuscular diseases as well as improve AAV manufacturing," said Charlie Albright, chief scientific officer of Affinia Therapeutics. "The first fruit of this work is Anc80L65, which we intend to use in our initial clinical program."

Title: CSF Delivery of Anc80L65 in Nonhuman Primates Results in More Widespread Gene Transfer Throughout the Central Nervous System Compared to AAV9

Presenter: Lisa Stanek, Ph.D.
Abstract Number: 362

Title: Network Analysis of Complex Novel AAV Vector Library Datasets for De-risking Gene Therapy Candidate Selection
Presenter: Deepak Grover, Ph.D.
Abstract Number: 285

Title: AAVmod2, an AAV Capsid Engineered to Independently Detarget the Liver and Enhance Gene Delivery to Skeletal Muscle
Presenter: Kevin Olivieri, Ph.D.
Abstract Number: 286

Title: AAVX Resin Binding Site Identification Via Library Screening Analysis on Novel AAV Vectors
Presenter: Christopher Tipper, Ph.D.
Session: Downstream Process of Vector Manufacturing, Room 9
Time: Thursday May 13, 6:30-6:45 p.m. ET
Abstract Number: 170

About Affinia Therapeutics
Affinia Therapeutics' purpose is to develop gene therapies that can have a transformative impact on people affected by devastating rare and non-rare diseases. Our proprietary platform enables us to methodically engineer novel AAV vectors and gene therapies with potentially improved tissue tropism, cell specificity, immunogenicity, and safety. With our innovative science, we are working to broaden the reach of life-changing gene therapies to meaningful numbers of patients with an initial focus on central nervous system (CNS) and muscle diseases with significant unmet need. www.affiniatx.com.

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