Affinia Therapeutics Announces Collaboration with the Institute of Molecular and Clinical Ophthalmology Basel (IOB) To Rationally Design Novel CNS Promoters for Gene Therapy

- *Botond Roska, M.D., Ph.D., world-renowned scientist and Director at IOB and scientific co-founder of Affinia Therapeutics, will oversee collaboration –*

- *Collaboration will expand upon company's leading capsid library approach to broaden the reach of gene therapy beyond rare diseases –*

WALTHAM, Mass. – July 20, 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 a multi-year sponsored research collaboration with the Institute of Molecular and Clinical Ophthalmology Basel (IOB) focused on the central nervous system (CNS). The company will collaborate with IOB to develop novel, next-generation, rationally designed promoters that can enhance gene expression, a key limitation to date in the field. The company has option rights to acquire exclusive licenses to the resulting promoters from the collaboration.

The collaboration with IOB will expand upon Affinia Therapeutics' platform of novel capsids by applying its proprietary capsid discovery approach to promoter design. Key features of that approach include rational in silico design of a barcoded library of synthetic promoters, high throughput screening in non-human primates, and large dataset analytics to determine structure-activity relationships. By applying this unique approach, the collaboration is intended to improve upon promoter technologies that optimize when, where, and how much a gene is expressed.

"The gene therapy field today has very few promoters for CNS applications, and even those promoters are limited in their control of expression levels and cellular specificity. In partnership with IOB, our team will build upon our leading gene therapy platform and develop promoters that significantly improve expression control in the CNS," said Charlie Albright, Ph.D., chief scientific officer of Affinia Therapeutics.

The collaboration with Affinia Therapeutics and IOB will build on IOB's proof of principle research in the retina using pre-clinical models that have successfully translated to humans. In collaboration with Affinia Therapeutics, IOB will initially focus on extending this work to the cortex, with the potential to expand more broadly in the CNS and other tissues over time.

"By focusing on developing rationally designed synthetic promoters, we aim to fill a void in the field that will address the well-known limitations in regulating protein expression in the CNS," said Botond Roska, M.D., Ph.D., Director at IOB and scientific co-founder of Affinia Therapeutics. "I am excited to lead the team and to apply our extensive knowledge of synthetic promoters to the cortex. With this, IOB will also expand its research strategy."

Founded in 2019, Affinia Therapeutics' proprietary capsid discovery platform originates from work done by Luk Vandenberghe, Ph.D., associate professor at Massachusetts Eye and Ear and Harvard Medical School and a co-inventor of AAV9.

About IOB
At the Institute of Molecular and Clinical Ophthalmology Basel (IOB), basic researchers and clinicians work hand in hand to advance the understanding of vision and its diseases, and to develop new therapies for vision loss. IOB started its operations in 2018. The Institute is constituted as a foundation, granting academic freedom to its scientists. Founding partners are the University Hospital Basel, the University of Basel and Novartis. The Canton of Basel-Stadt has granted the institute substantial financial support.

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 capsids and gene therapies with potentially improved tissue tropism, cell specificity, safety, and yields. 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 nervous system and muscle diseases with significant unmet need. For more information, visit https://www.affiniatx.com/.

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