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Modalis Therapeutics Corporation
Stock exchange listing: Tokyo Stock Exchange (4883)
Representative: Haruhiko Morita

Modalis Announces Completion of a Research Collaboration on Angelman Syndrome and Regaining Worldwide Rights

05-August-2021 TOKYO & CAMBRIDGE, Mass.- Modalis Therapeutics Corporation (Tokyo Stock Exchange T.4883), a leading company developing innovative products for the treatment of rare genetic diseases utilizing its proprietary CRISPR-GNDM epigenetic gene modulation technology, today announced the expiration of its research collaboration with Astellas, a global pharmaceutical company, for the development of Angelman Syndrome.

Modalis and Astellas Pharma Inc. (hereinafter “Astellas”) have been pursuing their Angelman Syndrome program through discovery activities with Modalis’ proprietary technology, CRISPR-GNDM. Astellas has notified us today of its decision not to extend the joint research program for MDL-206, which has been underway since 2020. All rights to MDL-206 will be returned to Modalis, and the Company will continue to advance the program internally. Additional research programs with Astellas will continue as currently planned.

Upon the separation, Modalis regains all rights to develop gene therapies targeting Angelman Syndrome with CRISPR-GNDM® technology. This allows Modalis to fully leverage its expertise in engineering, biology, regulatory, and manufacturing of gene therapies to expand its wholly owned CRISPR-based portfolio to include Angelman Syndrome. Modalis further regains the right to partner its Angelman Syndrome programs in the future.

"Our collaboration with Astellas has allowed us to advance our understanding of the CRISPR-GNDM® platform for the treatment of Angelman Syndrome and to establish animal Proof-of Concept," said Haru Morita, president and CEO of Modalis. "Regaining worldwide rights to develop this highly differentiated treatment for Angelman syndrome, which is a serious disorder and constitutes an area of high unmet medical need, allows us to expand our efforts with freedom to explore new areas of biology and potential partnerships. We remain committed to bringing novel gene therapies to patients suffering from this disease."

About Modalis:

Modalis Therapeutics is developing precision genetic medicines through epigenetic gene modulation. Modalis is pursuing therapies for orphan genetic diseases using its proprietary CRISPR-GNDM technology which enables the locus specific modulation of gene expression or histone modification without the need for double-stranded DNA cleavage, gene editing or base editing. Modalis is focusing initially on genetic disorders caused by loss of gene regulation – resulting in excess or insufficient protein production – which includes more than 660 genes that are currently estimated to cause human disease due to haploinsufficiency. Headquartered in Tokyo with laboratories and facilities in Cambridge, Massachusetts. For additional information, visit www.modalistx.com.

Forward-Looking Statements:

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