## Modalis Obtains Access to Foundational CRISPR IP

## Modalis Therapeutics and Editas Medicine conclude license

April 1, 2020

TOKYO —Modalis Therapeutics Corporation (Modalis) today announced that the company has entered into a license agreement with Editas Medicine, Inc., under which Modalis has obtained a license to certain intellectual property that is controlled by Editas Medicine. Modalis is utilizing its proprietary epigenetic gene modulation technology, CRISPR-GNDM (Guide Nucleotide Directed Modulation), to treat patients with serious genetic disorders. Additional details including financial terms of the agreement were not disclosed.

"Our goal is to create CRISPR based gene therapies for genetic disorders, most of which fall into the orphan disease category. There should be no disease that is ignored because of its small patient population, and our mission to develop disease modifying treatments for these diseases reflects our belief that 'Every Life Deserves Attention'. We are proud to be the pioneer in CRISPR based gene modulation therapy," said Haru Morita, Chief Executive Officer of Modalis.

"We are pleased to establish this license agreement with Modalis Therapeutics as their mission is aligned with our mission to make transformative medicines for people living with serious diseases of unmet clinical need. CRISPR technology has many uses and applications, and we are pleased to include Modalis in our expanding portfolio of licensees so the greatest number of patients may benefit in the future from transformative medicines," said Cynthia Collins, president and chief executive officer, Editas Medicine.

## **About Modalis**

Modalis Therapeutics is developing precision genetic medicines through epigenetic gene modulation. Founded by Osamu Nureki and leading scientists in CRISPR gene editing from University of Tokyo, 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, the company is backed by leading Japanese investors including Fast Track Initiative, SBI Investment, UTokyo-IPC, SMBC Venture Capital, and Mizuho Capital. For additional information, visit www.modalistx.com.

## モダリス社、CRISPR 基本特許のライセンスを獲得

モダリスーエディタスでライセンス契約を締結

発表日: 2020 年 4 月 1 日

モダリス株式会社(本社:東京都中央区代表取締役森田晴彦)は本日、エディタス社 (Editas Medicine Inc.米国マサチューセッツ州)との間でライセンス契約を締結したことを発表しました。本契約によってモダリスはエディタスの保有するゲノム編集技術である CRISPR の知財へのアクセスを獲得します。モダリスは独自の CRISPR-GNDM(ガイド核酸誘導型遺伝子制御)技術により、遺伝子を切断することなく、エピジェネティックスを制御する「切らない CRISPR」により深刻な遺伝子疾患に苦しむ患者様のための治療薬開発を目指しています。

モダリス CEO の森田は次のように述べております。「我々が目指すのは CRISPR を用いた遺伝子治療により、そのほとんどが希少疾患に属する遺伝子疾患に対して有効な治療法を作る事です。私たちは社のミッションである"Every Life Deserves Attention(すべての命に光を)"の通り、希少疾患にも光が当たるような治療法開発を目指します。私たちは CRISPR による遺伝子制御技術のパイオニアとして開発を行っていることを誇りに思っております。」

今回の提携についてエディタス CEO であるシンシア・コリンズは次のように述べております。「我々は深刻な病気に苦しむ患者様のアンメット・メディカルニーズ (有効な治療方法がない疾患に対する医療ニーズ)に対して革新的な治療法を開発することをミッションとしており、このミッションに共鳴するモダリスとの提携ができることを歓迎しています。 CRISPR 技術は様々な用途やアプリケーションが存在し、今回の提携を通じて当社の提携ポートフォリオの拡大し、さらに多くの患者様を救う可能性があることを喜んでいます。」