Joint Statement on Modalis Therapeutics' MDL-101 Program for LAMA2-Related Dystrophy

Cure CMD, Voor Sara, Modalis Therapeutics

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In light of ongoing press regarding development of the MDL-101 gene therapy treatment for those living with LAMA2-Related Dystrophy (LAMA2-RD), and keen interest from the affected community, we'd like to share the details that are currently available.

While Modalis Therapeutics is working toward initiation of a Phase 1/2 study, no enrollment timeline has been finalized. Several development and regulatory milestones remain before the study can open for enrollment. So, for now, there is no specific date that can be provided.

Protocol and eligibility criteria for the initial trial has not been finalized, the company is still preparing to submit an application to the U.S. Food and Drug Administration (FDA) regarding the planned clinical trial, but has not yet submitted the application and has not received clearance for the study. Early plans for an initial Phase 1/2 trial include:

- Identification of a study site in the United States capable of enrolling patients from outside the United States.
- Recruitment of 10 or fewer young individuals with a confirmed diagnosis of LAMA2-RD who test seronegative for AAV9 antibodies*****.

*** Please note** there is nothing that can be done to influence seronegativity; it is expected that approximately 20% of the population naturally have antibodies that preclude successful administration of AAV9-related therapies.

All other eligibility requirements, such as age, pulmonary function, mutations/mutation types, and phenotype severity are under active consideration. Outcome measures are also still under consideration.

Depending on early progress, study sites may expand beyond the United States to include a larger recruitment target.

While we know the information we can currently provide will not satisfy all of your questions, please be assured that we will share more details as they become available. In the meantime, ensuring you are fully registered (completed consent, profile, and submission of genetic report) in the **Congenital Muscle Disease International Registry** (**cmdir.org**) means that you will be directly notified about any updates on the MDL-101 Program, as well as opportunities to participate in other studies or trials for which you or your child may be eligible.

About MDL-101
MDL-101 is an investigational epigenetic gene-activation therapy being developed for the treatment of LAMA2-Related Dystrophy (LAMA2-RD).

The therapy utilizes:

- a guide RNA targeting LAMA1, a highly homologous compensatory gene to LAMA2
- a nuclease-null Cas9 (dCas9) fused to a transcriptional activator
- a muscle-specific promoter packaged within a muscle-tropic AAV vector

MDL-101 is designed to upregulate endogenous LAMA1 expression to compensate for loss-of-function caused by LAMA2 mutations. The program has the potential to be a one-time, durable treatment for individuals living with LAMA2-RD.

About Modalis Therapeutics
Modalis Therapeutics develops precision genetic medicines using

epigenome editing technology. Modalis is pursuing therapies for orphan genetic diseases using its proprietary CRISPR-GNDM® technology which enables the gene/locus-specific modulation of gene expression or epigenetic editing without the need for DNA cleavage or altering DNA sequence. Headquartered in Tokyo with laboratories and facilities in Waltham Massachusetts, the company is listed on Tokyo Stock Exchange's Growth market. For additional information, visit **modalistx.com/en**.

About Voor Sara

Stichting Voor Sara is a Dutch charity founded to fund and accelerate research into the cure and treatment of the rare, incurable muscle disease MDC1A (LAMA2-RD). Learn more at **voorsara.nl**.

About Cure CMD

Cure CMD is a U.S. registered, global nonprofit organization with a mission to advance research toward treatments for the congenital muscular dystrophies and empower those living with CMD. Learn more at **curecmd.org**.