English translation for reference purposes only

Q&A regarding Notice of Modalis Announces Termination of a Research Collaboration on MDL-204



In case of any discrepancy,
the Japanese version shall prevail

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Modalis Therapeutics Corporation
(TSE：4883)

January 7, 2022

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What is the future of your relationship with Astellas?

A) To date, we have conducted a total of five joint research projects with Astellas, two of which have resulted in licensing agreements. With the conclusion of the MDL-204 collaboration, there are no ongoing research collaborations with Astellas. However, we continue to maintain a good relationship through collaboration on two licensed programs, MDL-201 and MDL-202, as well as exploring other collaboration opportunities.
We've confirmed that Astellas continues to explore the existing programs licensed to it, MDL-201 and MDL-202, currently in the pre-clinical stage.

＊ Please note that this information is subject to change without notice. Please understand.

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What is the difference between MDL-206, which Modalis regained rights and put into internal program in August 2021, and the current MDL-204?

A) While both pipelines use the same CRISPR-GNDM® technology approach to create gene therapies, each program has its own technical challenges specific to the target gene, patient population size, and competition from other modalities.
In the case of MDL-206, the decision to continue the program as our own was based on reasons such as the target Angelman Syndrome's affinity to the technology and the difficulty of approaching it with other modalities, as well as the assumed efficacy based on existing data. However, the decision to discontinue MDL-204 was based on a comprehensive judgment that it was not sufficiently superior or reasonable.

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Do you foresee any increase or decrease in the pipelines in the future?

A) We have several new candidates, including those for which we are currently exploring the possibility of collaboration and those that are being incubated in-house. We plan to promote these to the pipeline at an appropriate stage.
Among existing pipelines, we will promptly discontinue those for which we believe there is no longer a possibility to continue research and development, such as MDL-204.
We believe that it is reasonable to optimize the size and quality of our portfolio by conducting an appropriate metabolism.
We also believe that making decisions at an early stage, especially at the research stage, is effective in increasing the chances of success in the costly development stage.

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