

## MEDIA UPDATE

# Novartis announces collaboration with Alnylam to explore targeted therapy to restore liver function

- *Agreement will bring Alnylam's proprietary siRNA technology to bear on liver target identified by Novartis researchers*
- *Novartis and Alnylam have agreed to collaborate on discovery and development of siRNA-based targeted therapy to restore functional liver cells in patients with end-stage liver disease*
- *Collaboration aims to develop liver-targeted therapy as a potential alternative to transplantation for patients experiencing liver failure*

**Basel, January 6, 2021** — Novartis today announced a collaboration with Alnylam to leverage Alnylam's proven, proprietary siRNA technology to inhibit a target discovered at the Novartis Institutes for BioMedical Research, potentially leading to development of a treatment designed to promote the regrowth of functional liver cells and to provide an alternative to transplantation for patients with liver failure.

"There remains an enormous unmet need for new types of medicines to address end-stage liver disease," said Jay Bradner, President of the Novartis Institutes for BioMedical Research. "Building on a legacy of leadership in regenerative medicine, we have devised a restorative strategy that could potentially deliver a transformative benefit to patients with liver failure. We're delighted now to work alongside Alnylam in this new collaboration, as the Alnylam siRNA platform is optimally suited to translate this concept to clinical investigation."

End-stage liver disease (ESLD) is a progressive illness, most often resulting from cirrhosis, that is characterized by the destruction of healthy liver tissue and the loss of critical liver function.<sup>1</sup> The disease has a profound impact on patients' quality of life, and accounts for over one million deaths globally each year. Currently, liver transplantation is the only treatment for ESLD, but transplants are invasive procedures and there is a limited supply of organs available for patients in need. A significant need exists for medicinal alternatives to transplantation that regenerate liver tissue and restore the essential metabolic and synthetic processes that are managed by the liver.

"We are so pleased to collaborate with Novartis," said Kevin Fitzgerald, Ph.D., Chief Scientific Officer at Alnylam. "We believe collaborations like this serve as an excellent example of how Alnylam's leadership in RNAi can fuel new frontiers of medicine with highly innovative targets coming from some of the most admired pharmaceutical companies."

During the exclusive three-year research collaboration, Alnylam will develop and test potential siRNAs using target-specific assays developed by Novartis. Once a lead candidate is identified, further development and clinical research will be conducted by Novartis.

### **Disclaimer**

This press release contains forward-looking statements within the meaning of the United States Private Securities Litigation Reform Act of 1995. Forward-looking statements can generally be identified by words such as “potential,” “can,” “will,” “plan,” “may,” “could,” “would,” “expect,” “anticipate,” “seek,” “look forward,” “believe,” “committed,” “investigational,” “pipeline,” “launch,” “aims,” “to collaborate,” or similar terms, or by express or implied discussions regarding the collaboration with Alnylam to explore siRNA-based targeted therapy to restore liver in patients with end-stage liver disease. You should not place undue reliance on these statements. Such forward-looking statements are based on our current beliefs and expectations regarding future events, and are subject to significant known and unknown risks and uncertainties. Should one or more of these risks or uncertainties materialize, or should underlying assumptions prove incorrect, actual results may vary materially from those set forth in the forward-looking statements. There can be no guarantee that the expected benefits and synergies from the collaboration described in this media update will be achieved in the expected time frame, or at all. In particular, our expectations regarding the collaboration could be affected by, among other things, the uncertainties inherent in research and development, including clinical trial results and additional analysis of existing clinical data; regulatory actions or delays or government regulation generally; global trends toward health care cost containment, including government, payor and general public pricing and reimbursement pressures and requirements for increased pricing transparency; our ability to obtain or maintain proprietary intellectual property protection; the particular prescribing preferences of physicians and patients; general political, economic and business conditions, including the effects of and efforts to mitigate pandemic diseases such as COVID-19; safety, quality, data integrity or manufacturing issues; potential or actual data security and data privacy breaches, or disruptions of our information technology systems, and other risks and factors referred to in Novartis AG’s current Form 20-F on file with the US Securities and Exchange Commission. Novartis is providing the information in this press release as of this date and does not undertake any obligation to update any forward-looking statements contained in this press release as a result of new information, future events or otherwise.

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### **References**

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2. Asrani SK, Devarbhavi H, Eaton J, Kamath PS. Burden of liver diseases in the world. *J Hepatol*. 2019 Jan;70(1):151-171. doi: 10.1016/j.jhep.2018.09.014. Epub 2018 Sep 26. PMID: 30266282.

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