



Alexion Exercises Option for Two Additional GalXC™ RNAi Programs for Complement-Mediated Targets Under Collaboration With Dicerna™

December 16, 2019

– Option Exercise Triggers \$20 Million Payment to Dicerna –

BOSTON & LEXINGTON, Mass.--(BUSINESS WIRE)--Dec. 16, 2019-- [Alexion Pharmaceuticals, Inc.](#) (NASDAQ:ALXN) and [Dicerna™ Pharmaceuticals, Inc.](#) (NASDAQ:DRNA) today announced that Alexion has exercised its option for exclusive rights to two additional targets within the complement pathway for the discovery and development of GalXC™ RNAi molecules. This expands the companies' existing research collaboration and license agreement to now encompass four targets within the complement pathway. In connection with the option exercise, Alexion will pay Dicerna a total of \$20 million, or \$10 million in option exercise fees per additional new target.

This press release features multimedia. View the full release here: <https://www.businesswire.com/news/home/20191216005150/en/>

"Our collaboration with Dicerna to discover and develop GalXC RNAi therapies, which have the potential to inhibit uncontrolled activation of the complement system in new ways, represents an exciting opportunity for Alexion to expand its leadership in developing new medicines for complement-mediated diseases," said John Orloff, M.D., Executive Vice President and Head of Research & Development at Alexion. "We are encouraged by the collaboration's progress and the strong relationship our teams have developed over the past year, and we are pleased to expand our efforts to include additional targets in the complement pathway."

"With more than 20 years of experience, Alexion is a clear leader in the field of complement biology, and we are very pleased by our collaboration's success to date in identifying potential new GalXC RNAi-based therapeutic approaches to treating complement-related diseases. Alexion's decision to exercise its option, along with our other new collaborations in different therapeutic areas, underscores the growing recognition of the significant potential for RNAi therapies as a new modality in the treatment of a broad range of diseases," said Douglas M. Fambrough, Ph.D., President and Chief Executive Officer of Dicerna. "We are very proud to be at the forefront of RNAi innovation and look forward to continued advancement of our core development portfolio and progress across our collaborative programs in 2020."

The collaboration between Alexion and Dicerna, which was initiated in October 2018, is focused on the discovery and development of subcutaneously delivered GalXC RNAi molecules directed to a total of four complement pathway targets for the treatment of complement-mediated diseases. Dicerna is leading the joint discovery and research efforts through the preclinical stage, and Alexion will lead development efforts beginning with Phase 1 studies. The agreement provides Alexion with exclusive worldwide licenses and commercial rights to the GalXC RNAi molecules developed in the collaboration in exchange for development- and approval-related milestones, sales milestones and mid-single to low-double-digit royalties on future product sales.

About the Complement System

The complement system is a part of the immune system that enhances, or complements, the ability of antibodies to clear small organisms or damaged cells from an organism, promote inflammation and attack the pathogen's cell membrane. While the complement system is essential to maintaining health, it can also become over-activated, contributing to the development of numerous devastating diseases.

About Dicerna's GalXC™ RNAi Technology Platform

Dicerna's proprietary RNA interference (RNAi) technology platform, called GalXC™, aims to advance the development of next-generation RNAi-based therapies designed to silence disease-driving genes in the liver and other tissues. Liver-targeted GalXC-based compounds enable subcutaneous delivery of RNAi therapies that are designed to bind specifically to receptors on liver cells, leading to internalization and access to the RNAi machinery within the cells. The GalXC approach seeks to optimize the activity of the RNAi pathway so that it operates in the most specific and potent fashion. Compounds produced via GalXC are intended to be broadly applicable across multiple therapeutic areas, including both liver and non-liver indications.

About Alexion

Alexion is a global biopharmaceutical company focused on serving patients and families affected by rare diseases through the discovery, development and commercialization of life-changing therapies. As the global leader in complement biology and inhibition for more than 20 years, Alexion has developed and commercializes two approved complement inhibitors to treat patients with paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS), as well as the first and only approved complement inhibitor to treat anti-acetylcholine receptor (AChR) antibody-positive generalized myasthenia gravis (gMG) and neuromyelitis optica spectrum disorder (NMOSD). Alexion also has two highly innovative enzyme replacement therapies for patients with life-threatening and ultra-rare metabolic disorders, hypophosphatasia (HPP) and lysosomal acid lipase deficiency (LAL-D). In addition, the company is developing several mid-to-late-stage therapies, including a second complement inhibitor, a copper-binding agent for Wilson disease and an anti-neonatal Fc receptor (FcRn) antibody for rare Immunoglobulin G (IgG)-mediated diseases as well as several early-stage therapies, including one for light chain (AL) amyloidosis and a second anti-FcRn therapy. Alexion focuses its research efforts on novel molecules and targets in the complement cascade and its development efforts on the core therapeutic areas of hematology, nephrology, neurology, metabolic disorders and cardiology. Headquartered in Boston, Massachusetts, Alexion has offices around the globe and serves patients in more than 50 countries. This press release and further information about Alexion can be found at: www.alexion.com.

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About Dicerna™ Pharmaceuticals, Inc.

Dicerna™Pharmaceuticals, Inc., is a biopharmaceutical company using ribonucleic acid interference (RNAi) to develop medicines that silence genes that cause disease. The company is applying its proprietary GalXC™ technology to develop potent, selective and safe RNAi therapies for treatment of rare diseases, chronic liver diseases, cardiovascular diseases, neurodegenerative diseases, pain and viral infectious disease. Dicerna aims to treat disease by addressing the underlying causes of illness with capabilities that extend beyond the liver to address a broad range of diseases, focusing on target genes where connections between gene and disease are well understood and documented. Dicerna intends to discover, develop and commercialize novel therapies either on its own or in collaboration with pharmaceutical partners. Dicerna has strategic collaborations with Novo Nordisk A/S*, Roche*, Eli Lilly and Company, Alexion Pharmaceuticals, Inc. and Boehringer Ingelheim International GmbH. For more information, please visit www.dicerna.com.

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Forward-Looking Statements

This press release includes forward-looking statements. Such forward-looking statements are subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statements. Examples of forward-looking statements include, among others, statements we make regarding: (i) the receipt of option exercise fees; (ii) the therapeutic and commercial potential of GalXC™, including its potential to inhibit uncontrolled activation of the complement system in new ways; (iii) research and development plans of Alexion related to GalXC; (iv) the potential of RNAi therapies for the treatment of complement-mediated diseases and a broad range of diseases; & (v) the potential for the collaboration between Alexion and Dicerna and this collaboration representing an exciting opportunity for Alexion to expand its leadership in developing new medicines for complement-mediated diseases. The process by which an early-stage platform such as GalXC could potentially lead to an approved product is long and subject to highly significant risks, particularly with respect to a preclinical research collaboration and the anticipated benefits of the platform may not be realized. In addition, the four targets of the collaboration may not result in any product and this may be after significant investments by the parties to the collaboration. Applicable risks and uncertainties include those relating to preclinical research and other risks identified under the heading "Risk Factors" included in Alexion's and Dicerna's most recent Form 10-Q filings and in other future filings with the SEC. The forward-looking statements contained in this press release reflect Alexion's and Dicerna's current views with respect to future events, and neither Alexion nor Dicerna undertakes and specifically disclaims any obligation to update any forward-looking statements, except as required by law.

* Novo Nordisk A/S and Roche transactions are subject to clearance under the Hart-Scott-Rodino Antitrust Improvements Act of 1976 and other customary closing conditions.

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