Alexion and Dicerna Announce Collaboration to Discover and Develop RNAi Therapies for Complement-Mediated Diseases

October 24, 2018

- Collaboration provides opportunity to build on Alexion's more than two decades of complement leadership with expansion into RNAi-based therapies using Dicerna's GalXC™ technology -

- Agreement provides Alexion with exclusive worldwide licenses as well as development and commercial rights for two preclinical GalXC RNAi molecules, with option for two additional candidates -

- Dicerna to receive upfront payment of $22 million and equity investment of $15 million, with potential for additional milestone-dependent and royalty payments -

- Collaboration further strengthens Dicerna's position as a leading developer of RNAi-based therapeutics -

BOSTON & CAMBRIDGE, Mass.--(BUSINESS WIRE)--Oct. 24, 2018-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) and Dicerna Pharmaceuticals, Inc. (NASDAQ:DRNA) today announced a collaboration to discover and develop RNA interference (RNAi) therapies for complement-mediated diseases. An RNAi-based approach to blocking the production of complement pathway factors offers the potential to inhibit the uncontrolled complement activation that leads to many diseases. The agreement provides Alexion with exclusive worldwide licenses as well as development and commercial rights for two of Dicerna's preclinical, subcutaneously delivered GalXC™ RNAi molecules and an exclusive option for other preclinical GalXC RNAi molecules for two additional targets within the complement pathway.

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"With Soliris, Alexion has demonstrated the transformative impact of complement inhibition on multiple serious and debilitating diseases," said John Orloff, M.D., Executive Vice President and Head of Research & Development at Alexion. "This collaboration provides the opportunity to continue building on our more than two decades of complement expertise using Dicerna's proprietary GalXC RNAi technology platform, which provides a potentially promising new way of inhibiting the uncontrolled complement activation that we know plays a significant role in many devastating diseases."

"Dicerna's proprietary GalXC technology is designed to silence the expression of disease-driving genes in a way that is highly specific, generally well tolerated, and allows for convenient, infrequent subcutaneous administration. Having recently demonstrated clinical proof-of-concept for DCR-PHXC, our lead program for the treatment of primary hyperoxaluria, we are eager to expand and advance our pipeline of innovative GalXC therapies, including both proprietary and partnered programs," said Douglas M. Fambrough, Ph.D., President and Chief Executive Officer of Dicerna. "Our collaboration with Alexion provides access to the deep expertise and resources of an established leader in complement-mediated diseases. We look forward to working with Alexion to discover and develop promising new RNAi therapies."

Under the terms of the agreement, Alexion and Dicerna will collaborate on the discovery and development of subcutaneously delivered GalXC RNAi molecules directed to two complement pathway targets for the treatment of complement-mediated diseases. In addition, Alexion will have the right to exercise options, for additional payment, for two additional GalXC RNAi molecules directed to complement pathway targets. Dicerna will lead 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. Dicerna will receive an immediate upfront payment of $22 million, with Alexion making a concurrent $15 million equity investment in Dicerna at a premium to market as of the collaboration effective date. The collaboration also provides for potential additional development and approval-related milestone payments of up to $105 million per target, plus sales milestones and mid-single to low-double digit royalties on future product sales.

About RNAi

RNA interference (RNAi) is a biologic process in which certain double-stranded RNA molecules inhibit the expression of disease-causing genes by destroying the messenger RNAs (mRNAs) of those genes. It reflects a new approach in the development of specific and powerful therapies. Rather than targeting and binding to proteins to inhibit their activity, RNAi exerts its effects one step earlier in the gene silencing process by targeting the mRNA, the instruction set that directs the building of the protein. By attaching to this instruction set, RNAi is believed to have the ability to attack any target, including disease-causing genes that are beyond the reach of conventional antibody and small-molecule modalities. Additionally, RNAi-based therapeutic approaches hold the potential to offer more convenience for patients via infrequent subcutaneous dosing and a long duration of effect.

About Dicerna's GalXC™ RNAi Technology Platform

The proprietary RNAi technology platform called GalXC™, invented by Dicerna, aims to advance the development of next-generation RNAi-based therapies designed to silence disease-driving genes in the liver. GalXC-based therapies are processed by the Dicer enzyme, which is the natural initiation point for RNAi within the human cell. Using GalXC, Dicerna scientists attach N-acetylgalactosamine sugars directly to the extended region of the proprietary Dicer substrate short-interfering RNA (DsiRNA) molecules, yielding multiple conjugate delivery configurations that allow flexible and efficient conjugation to the targeting ligands while stabilizing the RNAi duplex. Dicerna believes this stabilization will enable subcutaneous delivery of RNAi therapies to hepatocytes in the liver, where they are designed to specifically bind to receptors on target cells, potentially leading to internalization and access to the RNAi machinery within the cells. By using the Dicer enzyme as the entry point into RNAi, 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 rare diseases, viral infectious diseases, chronic liver diseases and cardiovascular diseases.

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 the first and only approved complement inhibitor to treat patients with paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome (aHUS), and anti-acetylcholine receptor (AchR) antibody-positive generalized myasthenia gravis (gMG). 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 two late-stage therapies, including a second complement inhibitor and a copper-binding agent for Wilson disease. 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, and metabolic disorders. Alexion has been named to the *Forbes* list of the World's Most Innovative Companies seven years in a row and is headquartered in Boston, Massachusetts' Innovation District. The company also 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

Dicerna is a biopharmaceutical company focused on the discovery and development of innovative, subcutaneously delivered RNAi-based therapeutics for the treatment of diseases involving the liver, including rare diseases, viral infectious diseases, chronic liver diseases, and cardiovascular diseases. Dicerna is leveraging its proprietary GalXC™ RNAi technology platform to build a broad pipeline in these core therapeutic areas, focusing on target genes where connections between target gene and diseases are well understood and documented. Dicerna intends to discover, develop and commercialize novel therapeutics either on its own or in collaboration with pharmaceutical partners. For more information, please visit www.dicerna.com.

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Forward-Looking Statement

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 therapeutic and commercial potential of GalXC™; (ii) research and development plans related to GalXC; (iii) the potential of RNAi therapies for the treatment of complement-mediated diseases; and (iv) the potential for the collaboration between Alexion and Dicerna. 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. 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 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.

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Source: Alexion Pharmaceuticals, Inc. and Dicerna Pharmaceuticals, Inc.

Alexion
Media
Megan Goulart, 857-338-8634
Senior Director, Corporate Communications
or
Investors
Susan Altschuller, Ph.D., 857-338-8788
Vice President, Investor Relations
or
Dicerna Pharmaceuticals
Investors
Rx Communications Group
Paula Schwartz, 917-322-2216
pschwartz@rxir.com
or
Media
SmithSolve
Alex Van Rees, 973-442-1555 ext. 111
alex.vanrees@smithsolve.com