Dicerna Announces Poster Presentations at American Association for the Study of Liver Diseases (AASLD) The Liver Meeting® 2021 in November

October 14, 2021

LEXINGTON, Mass.--(BUSINESS WIRE)--Oct. 14, 2021-- Dicerna Pharmaceuticals, Inc. (Nasdaq: DRNA), a leading developer of investigational ribonucleic acid interference (RNAi) therapeutics, today announced that two abstracts related to the Company's clinical development programs have been accepted for poster presentations at the American Association for the Study of Liver Diseases (AASLD) The Liver Meeting® taking place Nov. 12-15, 2021.

The first abstract provides clinical data from the Company's Phase 1 trial of belcesiran, an investigational GalXC™ RNAi therapeutic in development for the treatment of alpha-1 antitrypsin (AAT) deficiency-associated liver disease (AATLD).

Session: Metabolic and Genetic Disease: Hemochromatosis, Wilson Disease, α-1 Antitrypsin Deficiency
Poster Title: *Belcesiran Was Well-Tolerated and Reduced Serum AAT Levels in Healthy Volunteers (Phase 1 Interim Results)*
Poster #: 27962
Abstract #: 1549

An additional abstract provides results of a subpopulation pharmacokinetic and safety analysis from the Phase 1 trial of RG6346 (RO7445482), an investigational GalXC RNAi therapeutic that Dicerna is developing in collaboration with Roche for the treatment of chronic hepatitis B virus (HBV) infection.

Session: Hepatitis B: Therapeutics: New Agents
Poster Title: *The Pharmacokinetic and Safety Profiles of RO7445482 siRNA Are Similar Between Asian and Non-Asian Healthy Volunteers and Chronic Hepatitis B Patients in a Phase 1 Study*
Poster #: 28577
Abstract #: 850

Both posters will be available for the duration of the meeting beginning on Friday, Nov. 12.

About Alpha-1 Antitrypsin (AAT) Deficiency and Alpha-1 Antitrypsin Deficiency-Associated Liver Disease (AATLD)

Alpha-1 antitrypsin (AAT) deficiency is a rare genetic condition caused by mutations in the *SERPINA1* gene that results in disease of the liver and lungs. AAT protein is produced in hepatocytes and circulates in the bloodstream; AAT protects the lungs and other parts of the body by neutralizing neutrophil elastase, an enzyme that fights infection but can also damage healthy tissues if not adequately regulated by AAT. The majority of people with severe AAT deficiency are homozygous for the Z allele (PiZZ genotype).[1] In the liver, misfolding of the mutant Z-AAT protein causes the protein to aggregate in liver cells, leading to liver injury, including fibrosis, cirrhosis and hepatocellular carcinoma. An estimated 10% or more of adults with AAT deficiency develop clinically meaningful liver disease.[2,3] People with AAT deficiency may also develop lung disease, including emphysema.

About Belcesiran

Belcesiran is a clinical-stage, subcutaneously administered, investigational GalXC™ RNAi therapy targeting alpha-1 antitrypsin (AAT) that is in development for the treatment of AAT deficiency-associated liver disease (AATLD). Belcesiran is designed to target the gene responsible for production of the abnormal AAT protein in order to reduce AAT production in the liver. Dicerna is currently investigating the use of belcesiran for the treatment of AATLD in the SHINE clinical development program.

About Chronic Hepatitis B Virus (HBV) Infection

Hepatitis B virus (HBV) is the world's most common serious liver infection, with nearly 300 million people living with chronic HBV. [4] According to the Hepatitis B Foundation, 1.5 million people become newly infected with HBV each year, and it is estimated that 820,000 people die annually from hepatitis B and related complications such as liver cancer.[5]

About RG6346

RG6346 is an investigational GalXC™ RNAi therapeutic candidate in development in collaboration with Roche for the treatment of chronic hepatitis B virus (HBV) infection. Current therapies for HBV, such as nucleos(t)ide analogs, can provide long-term viral suppression if taken continuously, but they rarely lead to long-term functional cures, as measured by the clearance of HBV surface antigen (HBsAg) and sustained HBV deoxyribonucleic acid (DNA) suppression in patient plasma or blood. By contrast, RG6346 is designed to employ RNAi to knock down selectively HBsAg messenger RNA (mRNA) and protein expression in hepatocytes, which is required for the HBV virus lifecycle. Preclinical data have demonstrated greater than 99.9% reduction in circulating HBsAg, as observed in mouse models of HBV infection. Results from a Phase 1 trial of RG6346 demonstrated that four monthly doses of RG6346 treatment resulted in substantial and durable reductions in HBsAg levels lasting up to one year following the last dose. Dicerna believes RG6346 has the potential to deliver a functional cure as part of a combination regimen for patients living with chronic HBV.

About Dicerna Pharmaceuticals, Inc.

Dicerna Pharmaceuticals, Inc. (Nasdaq: DRNA) is a biopharmaceutical company focused on discovering, developing and commercializing medicines that are designed to leverage ribonucleic acid interference (RNAi) to silence selectively genes that cause or contribute to disease. Using our proprietary GalXC™ and GalXC-Plus™ RNAi technologies, Dicerna is committed to developing RNAi-based therapies with the potential to treat both rare and more prevalent diseases. By silencing disease-causing genes, Dicerna's GalXC platform has the potential to address conditions that are

difficult to treat with other modalities. Initially focused on disease-causing genes in the liver, Dicerna has continued to innovate and is exploring new applications of its RNAi technology with GalXC-Plus, which expands the functionality and application of our flagship liver-targeted GalXC technology to tissues and cell types outside the liver, and has the potential to treat diseases across multiple therapeutic areas. In addition to our own pipeline of core discovery and clinical candidates, Dicerna has established collaborative relationships with some of the world's leading pharmaceutical companies, including Novo Nordisk A/S, Roche, Eli Lilly and Company, Alexion Pharmaceuticals, Inc., Boehringer Ingelheim International GmbH and Alnylam Pharmaceuticals, Inc. Between Dicerna and our collaborative partners, we currently have more than 20 active discovery, preclinical or clinical programs focused on cardiometabolic, viral, chronic liver and complement-mediated diseases, as well as neurodegenerative diseases and pain. At Dicerna, our mission is to interfere – to silence genes, to fight disease, to restore health. For more information, visit www.dicerna.com.

Cautionary Note on Forward-Looking Statements

This press release includes forward-looking statements pertaining to the Company's planned participation at a scientific conference, which may include discussion of the Company's business and operations, including the discovery, development and commercialization of our product candidates and technologies, and the therapeutic potential thereof, the success of our collaborations with partners and any potential future collaborations. 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. Applicable risks and uncertainties include those relating to our preclinical research and clinical programs and other risks identified under the heading "Risk Factors" included in our most recent Form 10-Q and Form 10-K filings and in other future filings with the SEC. The forward-looking statements contained in this press release reflect Dicerna's current views with respect to future events, and Dicerna does not undertake and specifically disclaims any obligation to update any forward-looking statements.

1. Stoller JK, Hupertz V, Aboussouan LS. Alpha-1 Antitrypsin Deficiency. 2006 Oct 27 [updated 2020 May 21]. In: Adam MP, Ardinger HH, Pagon RA, Wallace SE, Bean LJH, Mirzaa G, Amemiya A, editors. GeneReviews [Internet]. Seattle (WA): University of Washington, Seattle; 1993–2021. PMID: 20301692.

2. Tanash & Piitulainen. J Gastroenterol. 2019 Jun;54(6):541-548. doi: 10.1007/s00535-019-01548-y. Epub 2019 Jan 24.

3. Clark et al. J Hepatol. 2018 Dec;69(6):1357-1364. doi: 10.1016/j.jhep.2018.08.005. Epub 2018 Aug 21.

4. Polaris Observatory Collaborators. Global prevalence, treatment, and prevention of hepatitis B virus infection in 2016: a modelling study. *The Lancet Gastroenterology and Hepatology*. 2018;3(6):383-403.

5. Hepatitis B Foundation. Facts and Figures. Available at: http://www.hepb.org/what-is-hepatitis-b/what-is-hepb/facts-and-figures/. Accessed on Oct. 14, 2021.

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