CAMBRIDGE, Mass., Aug 06, 2015 (BUSINESS WIRE) -- Dicerna Pharmaceuticals, Inc. DRNA, -3.64% a leading developer of RNA interference (RNAi) therapeutics, today announced that the European Medicines Agency (EMA) granted Orphan Drug Designation to DCR-PH1, the company's therapeutic candidate for the treatment of primary hyperoxaluria type 1 (PH1). PH1 is a severe, rare, inherited disorder of the liver that often results in kidney failure and for which there are no approved therapies.

Orphan Drug Designation by the EMA provides regulatory and financial incentives under Regulation (EC) No. 141/2000 for companies to develop and market therapies that treat a life-threatening or chronically debilitating condition affecting no more than five in 10,000 persons in the European Union (EU), and for which no satisfactory treatment is available. In addition to a 10-year period of marketing exclusivity in the EU after product approval, Orphan Drug Designation provides incentives for companies seeking protocol assistance from the EMA during the product development phase and direct access to the centralized marketing authorization procedure.

DCR-PH1 is Dicerna's proprietary therapeutic candidate in development for the treatment of PH1. Preclinical experiments indicate that DCR-PH1 knocks down hydroxyacid oxalyase-1 (*HAO1*), the gene transcript that encodes for the enzyme glycolate oxidase (GO), which, in turn, reduces the excretion of oxalate in the urine.

"We are very pleased to have received Orphan Drug Designation in the EU for DCR-PH1. This is an important regulatory milestone for our team working to bring this therapy to patients with PH1," said Ted Ashburn, M.D., Ph.D., senior vice president of product strategy and operations at Dicerna. "We are encouraged by the progress of this program to date and our aim is to rapidly advance the development of DCR-PH1 as a potential new treatment option that will have a meaningful impact on reducing the disease burden for PH1 patients."

DCR-PH1 incorporates a proprietary, lipid nanoparticle (LNP) technology that allows for efficient delivery to the liver after intravenous (IV) administration. Dicerna obtained rights to this delivery technology by way of a licensing agreement with Tekmira Pharmaceuticals Corporation signed in November 2014.

About Primary Hyperoxaluria Type 1 (PH1)

PH1 is a rare, inherited disorder of the liver in which excess oxalate production can result in severe damage to the kidneys and other organs. Patients with this disease often undergo combined liver and kidney transplant, a major surgical procedure, and subsequently must take immunosuppressant drugs for the rest of their lives. Currently there are no approved therapies for the treatment of PH1 in the EU.

PH1 is characterized by a genetic mutations in the *AGXT* gene, which encodes for the liver enzyme alanine:glyoxylate-aminotransferase (AGT). AGT deficiency causes overproduction of oxalate by the liver, which can result in the deposition of calcium oxalate crystals in the kidneys. The deposition of calcium oxalate crystals can lead to both nephrolithiasis (the presence of kidney stones in the kidney) and nephrocalcinosis (calcification in the renal parenchyma, the functional part of the kidney), the latter of which has been linked to more rapid progression to end-stage renal disease (ESRD) and the need for intensive hemo- and/or peritoneal dialysis and a kidney transplant. In addition, PH1 patients in ESRD also need an orthotopic liver transplant (liver removal followed by replacement in the normal position) to correct the overproduction of oxalate. Patients with decreased renal function may also experience oxalosis, which involves a build-up of oxalate in other organs such as the bone, skin, heart and retina, possibly causing other concomitant, debilitating complications. It is estimated that up to three people per one million have PH1.1 The median age at first symptoms is 5.8 years.2 The median age at diagnosis is between 4.2 and 11.5 years depending on whether or not nephrocalcinosis is present.3 Fifty percent of patients with PH1 reach ESRD by their mid-30s.4

About DCR-PH1

Dicerna is developing DCR-PH1, which is in preclinical development, for the treatment of PH1. DCR-PH1 is engineered to address the pathology of PH1 by targeting and destroying the messenger RNA (mRNA) produced by *HAO1*, a gene implicated in the pathogenesis of PH1. *HAO1*
encodes glycolate oxidase (GO), an enzyme involved in producing oxalate. By reducing oxalate production, this approach seeks to prevent the complications of PH1. Preclinical studies indicate that DCR-PH1 induces potent and long-term inhibition of
HAO1
and significantly reduces levels of urinary oxalate, while demonstrating long-term efficacy and tolerability in animal models of PH1.

About Dicerna's Dicer Substrate Technology

Dicerna's proprietary RNAi molecules are known as Dicer substrate short-interfering RNA molecules, or DsiRNAs, so called because they are processed by the Dicer enzyme, which is the initiation point for RNAi in the human cell cytoplasm. Dicerna's discovery approach is believed to maximize RNAi potency because the DsiRNAs are structured to be ideal for processing by Dicer. Dicer processing enables the preferential use of the correct RNA strand of the DsiRNA, which may increase the efficacy of the RNAi mechanism, as well as the potency of the DsiRNA molecules relative to other molecules used to induce RNAi.

About Dicerna

Dicerna Pharmaceuticals, Inc., is a biopharmaceutical company focused on the discovery and development of innovative treatments for rare, inherited diseases involving the liver and for cancers that are genetically defined. The company is using its proprietary RNA interference (RNAi) technology platform to build a broad pipeline in these therapeutic areas. In both rare diseases and oncology, Dicerna is pursuing targets that have been difficult to address using conventional approaches, but where connections between targets and diseases are well understood and documented. The company intends to discover, develop and commercialize novel therapeutics either on its own or in collaboration with pharmaceutical partners.

Cautionary Note on 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. DCR-PH1 is in preclinical development, and the process by which a preclinical therapeutic candidate could potentially lead to an approved drug is long and subject to significant risks and uncertainties. Orphan Drug Designation does not assure a faster or more probable regulatory path. Applicable risks and uncertainties include those relating to our preclinical and clinical research and other risks identified under the heading "Risk Factors" included in our most recent Form 10-K filing 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.

References

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