



Dicerna Sells OXLUMO™ (Iumiasiran) Royalty Interest to Royalty Pharma for Up to \$240 Million

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– Dicerna To Receive \$180 Million Upfront and Up to \$60 Million in Potential Additional Milestone Payments –

LEXINGTON, Mass. & NEW YORK--(BUSINESS WIRE)--Apr. 8, 2021-- [Dicerna Pharmaceuticals, Inc.](#) (Nasdaq: DRNA) ("Dicerna") and [Royalty Pharma plc](#) (Nasdaq: RPRX) ("Royalty Pharma") today announced that Royalty Pharma has acquired Dicerna's royalty interest in OXLUMO™ (Iumiasiran) for an upfront cash payment of \$180 million and up to \$60 million in contingent sales-based milestone payments. OXLUMO, which has been approved by the U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA) for the treatment of primary hyperoxaluria (PH) type 1, is marketed by Alnylam Pharmaceuticals, Inc. ("Alnylam").

"We are very pleased to have entered into this agreement with Royalty Pharma, which is recognized as the leader in royalty acquisitions across the life sciences industry," said Douglas Fambrough, Ph.D., President and Chief Executive Officer of Dicerna. "This agreement with Royalty Pharma reflects our mutually held recognition of the importance of this market and the high unmet need for patients with PH – a population that Dicerna is also seeking to address with our lead product candidate, nedosiran, which we are developing for PH types 1, 2 and 3."

"We are delighted to accomplish this transaction with Dicerna, an emerging leading player in RNAi therapeutics," said Pablo Legorreta, founder and Chief Executive Officer of Royalty Pharma. "RNAi represents a promising new approach to medicine, and OXLUMO is consistent with our focus on highly innovative therapies addressing areas of significant unmet need. We are gratified to have established this initial relationship with Dicerna, which could open the door for future potential collaboration opportunities to bring new RNAi therapies to patients."

Dicerna became entitled to royalties on worldwide net product sales of OXLUMO as part of a 2020 non-exclusive intellectual property cross-license agreement between Dicerna and Alnylam related to the companies' PH programs. Dicerna is entitled to royalties in the mid to high single digits based on OXLUMO global net sales.

This transaction, together with Dicerna's cash, cash equivalents, held-to-maturity investments and anticipated milestone and other payments from existing collaborations, is expected to extend Dicerna's projected cash runway and be sufficient to fund the execution of its current clinical and operating plan into 2024.

J. Wood Capital Advisors acted as Dicerna's sole financial advisor and Latham & Watkins acted as legal advisor to Dicerna on the transaction. Gibson Dunn, Jones Day and Maiwald acted as legal advisors to Royalty Pharma.

About Primary Hyperoxaluria (PH)

Primary hyperoxaluria (PH) is a family of ultra-rare, life-threatening genetic disorders that initially manifest with complications in the kidneys. There are three known types of PH (PH1, PH2 and PH3), each resulting from a mutation in one of three different genes. These genetic mutations cause enzyme deficiencies that result in the overproduction of a substrate called oxalate. Abnormal production and accumulation of oxalate leads to recurrent kidney stones, nephrocalcinosis and chronic kidney disease that may progress to end-stage renal disease requiring intensive dialysis. Compromised renal function eventually results in the accumulation of oxalate in a wide range of organs including the skin, bones, eyes and heart. In the most severe cases, symptoms start in the first year of life. A combined liver-kidney transplant may be undertaken to resolve PH1 or PH2, but it is an invasive solution with limited availability and high morbidity that requires lifelong immune suppression to prevent organ rejection. Genetic studies suggest approximately 8,500 people in the U.S. are affected by PH, and researchers estimate that more than 80% of patients remain undiagnosed.¹ There is currently only one approved therapy available specifically for PH that is for the treatment of patients with PH1.

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 RNAi technologies, GalXC™ and GalXC-Plus™, Dicerna is committed to developing RNAi-based therapies with the potential to treat both rare and more prevalent diseases. By silencing disease-causing genes in the liver, Dicerna's GalXC technology has the potential to address conditions that are difficult to treat with other modalities. Dicerna has continued to innovate and is exploring new applications of its RNAi technology beyond specific hepatocytes, targeting additional tissues and enabling new therapeutic applications with GalXC-Plus. 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 rare, 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, please visit www.dicerna.com.

About Royalty Pharma

Founded in 1996, Royalty Pharma is the largest buyer of biopharmaceutical royalties and a leading funder of innovation across the biopharmaceutical industry, collaborating with innovators from academic institutions, research hospitals and not-for-profits through small and mid-cap biotechnology companies to leading global pharmaceutical companies. Royalty Pharma has assembled a portfolio of royalties which entitles it to payments based directly on the top-line sales of many of the industry's leading therapies. Royalty Pharma funds innovation in the biopharmaceutical industry both directly and indirectly - directly when it partners with companies to co-fund late-stage clinical trials and new product launches in exchange for future royalties, and indirectly when it acquires existing royalties from the original innovators. Royalty Pharma's current portfolio includes royalties on more than 45 commercial products, including AbbVie and J&J's Imbruvica, Astellas and Pfizer's Xtandi, Biogen's Tysabri, Gilead's Trodelvy, Merck's Januvia, Novartis' Promacta, and Vertex's Kalydeco, Symdeko, Orkambi and Trikafta, and five development-stage product candidates. For more

information, visit www.royaltypharma.com.

Dicerna Pharmaceuticals, Inc.'s Cautionary Note on Forward-Looking Statements

This press release includes forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this press release that do not relate to matters of historical fact should be considered 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 made regarding Dicerna's product candidates and the development thereof; the potential for additional, escalating payments to Dicerna based on Royalty Pharma's royalty receipts on net sales of OXLUMO; the progress of Dicerna's clinical trials for nedosiran and its potential to address PH1, PH2 and PH3; the sufficiency of Dicerna's cash, cash equivalents, held-to-maturity investments and anticipated milestone and other payments from existing collaborations; the potential for future collaboration opportunities between Dicerna and Royalty Pharma; as well as Dicerna's business and operations, including the discovery, development and commercialization of product candidates and technologies, and the therapeutic potential thereof; Dicerna's collaborations with partners, including the pace and progress of development by collaboration partners; the receipt of anticipated milestone payments therefrom; the estimated number of patients affected by PH; and the ability of the GalXC platform to address difficult-to-treat conditions. Applicable risks and uncertainties include those relating to Dicerna's clinical research and other risks identified under the heading "Risk Factors" included in the Company's most recent filings on Forms 10-K and 10-Q, as may be updated in future filings with the Securities and Exchange Commission. These risks and uncertainties include, among others, the cost, timing and results of preclinical studies and clinical trials and other development activities by Dicerna and its collaborative partners; the likelihood of Dicerna's clinical programs being executed on timelines provided and reliance on the Company's contract research organizations and predictability of timely enrollment of subjects and patients to advance Dicerna's clinical trials; the reliance of Dicerna on contract manufacturers to supply its products for research, development and commercialization and the risk of supply interruption from a contract manufacturer; the potential for future data to alter initial and preliminary results of early-stage clinical trials; the impact of the ongoing COVID-19 pandemic on business operations, including the conduct of research and development activities; the regulatory review and unpredictability of the duration and results of the regulatory review of Investigational New Drug applications (INDs) and Clinical Trial Applications (CTAs) that are necessary to continue to advance and progress Dicerna's clinical programs; the timing, plans and reviews by regulatory authorities of marketing applications such as New Drug Applications (NDAs) and comparable foreign applications for one or more of Dicerna's product candidates; continued alignment with the FDA on the regulatory pathway to approval for nedosiran; the ability to secure, maintain and realize the intended benefits of collaborations with partners; market acceptance for approved products and innovative therapeutic treatments; competition; the possible impairment of, inability to obtain, and costs to obtain intellectual property rights; possible safety or efficacy concerns that could emerge as new data are generated in R&D and following commercialization; and general business, financial, and accounting risks and litigation. 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, except as may be required by law.

Royalty Pharma plc's Forward-Looking Statements

The information set forth herein does not purport to be complete or to contain all of the information you may desire. Statements contained herein are made as of the date of this document unless stated otherwise, and neither the delivery of this document at any time, nor any sale of securities, shall under any circumstances create an implication that the information contained herein is correct as of any time after such date or that information will be updated or revised to reflect information that subsequently becomes available or changes occurring after the date hereof. This document contains statements that constitute "forward-looking statements" as that term is defined in the United States Private Securities Litigation Reform Act of 1995, including statements that express the company's opinions, expectations, beliefs, plans, objectives, assumptions or projections regarding future events or future results, in contrast with statements that reflect historical facts. Examples include discussion of Royalty Pharma's strategies, financing plans, growth opportunities and market growth. In some cases, you can identify such forward-looking statements by terminology such as "anticipate," "intend," "believe," "estimate," "plan," "seek," "project," "expect," "may," "will," "would," "could" or "should," the negative of these terms or similar expressions. Forward-looking statements are based on management's current beliefs and assumptions and on information currently available to the company. However, these forward-looking statements are not a guarantee of Royalty Pharma's performance, and you should not place undue reliance on such statements. Forward-looking statements are subject to many risks, uncertainties and other variable circumstances, and other factors. Such risks and uncertainties may cause the statements to be inaccurate and readers are cautioned not to place undue reliance on such statements. Many of these risks are outside of Royalty Pharma's control and could cause its actual results to differ materially from those it thought would occur. The forward-looking statements included in this document are made only as of the date hereof. Royalty Pharma does not undertake, and specifically declines, any obligation to update any such statements or to publicly announce the results of any revisions to any such statements to reflect future events or developments, except as required by law. Certain information contained in this document relates to or is based on studies, publications, surveys and other data obtained from third-party sources and Royalty Pharma's own internal estimates and research. While Royalty Pharma believes these third-party sources to be reliable as of the date of this document, it has not independently verified, and makes no representation as to the adequacy, fairness, accuracy or completeness of, any information obtained from third-party sources. In addition, all of the market data included in this document involves a number of assumptions and limitations, and there can be no guarantee as to the accuracy or reliability of such assumptions. Finally, while the company believes its own internal research is reliable, such research has not been verified by any independent source. For further information, please reference Royalty Pharma's reports and documents filed with the U.S. Securities and Exchange Commission ("SEC") by visiting EDGAR on the SEC's website at www.sec.gov.

¹ Hopp K, et al. J Am Soc Nephrol. 2015;26(10):2559-2570 and U.S. Census Bureau population on a date: February 20, 2020. United States Census Bureau website, 2020.

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