*CORPORATE FACT SHEET*
*AUGUST 2017*

Dicerna

## HEADQUARTERS

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## FOUNDED

2007

## LISTED ON NASDAQ

2014

## TRADING SYMBOL

DRNA

## MANAGEMENT TEAM

**Douglas Fambrough, Ph.D.**
President & Chief Executive Officer

**Bob Brown, Ph.D.**
Chief Scientific Officer,
Senior Vice President

**Jack Green, CPA**
Chief Financial Officer

**Jennifer Lockridge, Ph.D.**
Vice President,
Program Development

**David Miller, Ph.D.**
Senior Vice President,
Corporate Operations

**James B. Weissman**
Chief Business Officer

**Ralf Rosskamp, M.D.**
Chief Medical Officer

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**Please visit Dicerna.com for more information**

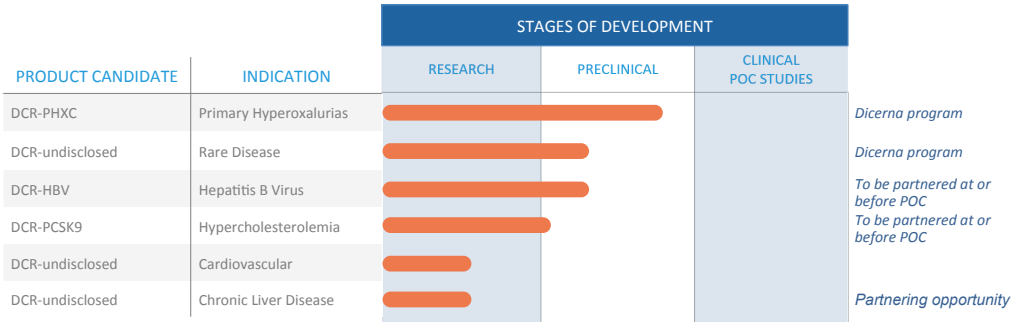
## OVERVIEW

- Dicerna is a biopharmaceutical company focused on the discovery and development of innovative subcutaneously delivered ribonucleic interference RNAi-based therapeutics for the treatment of diseases involving the liver, including rare diseases, chronic liver diseases, cardiovascular diseases, and viral infectious diseases.
- The Company's proprietary GalXC™ RNAi technology uses the body's natural biological pathways to silence or "turn off" disease-driving genes with a high degree of selectivity and specificity.
- The Company has qualified dozens of disease-associated genes in clinical indications where it believes an RNAi-based inhibitor may provide substantial benefit to patients, providing expansive therapeutic opportunities. Dicerna has a growing pipeline of product candidates with four preclinical programs in development, which are outlined below:
  - DCR-PHXC for primary hyperoxaluria (lead candidate, planning to enter clinical development in Q1 2018)
  - DCR-undisclosed for an undisclosed rare disease program (IND or CTA to be filed in Q2 2018)
  - DCR-HBVS for hepatitis B virus (IND or CTA to be filed by end of 2018)
  - DCR-PCSK9 for hypercholesterolemia (advance into formal preclinical development in 2018)
- The Company's strategy is to retain a full or substantial ownership stake and invest internally in disease areas with focused patient populations, such as certain rare diseases, and to pursue partnerships for more complex diseases with multiple gene dysfunctions and larger patient populations.

## ABOUT GalXC™

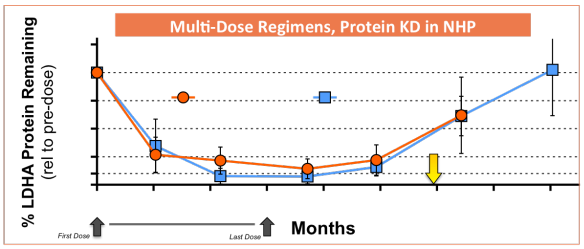
- Fully enabled RNAi drug discovery engine with potentially powerful capabilities:
  - Subcutaneous delivery for liver targets – simple, single shot subcutaneous dosing
  - Long duration of action – expect many programs to be dosed quarterly
  - Well tolerated, high therapeutic index
  - Highly specific binding to gene targets
  - Deep IP and freedom to operate
- GalXC enables rapid discovery and efficient advancement of research activities.
  - Within 30 days of nominating a gene target, we can design, synthesize and validate an *in vivo* GalXC construct
- The Company's goal is to advance five programs into the clinic by the end of 2019.

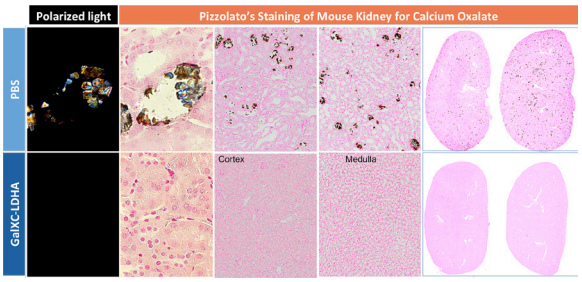
## DEVELOPMENT PIPELINE




*Dicerna has developed an extensive library of GalXC molecules across our Tx areas with high potency in rodents, ready for optimization and full candidate qualification*

## FOCUSED PATIENT POPULATIONS (RARE DISEASES)




### Primary Hyperoxaluria

DCR-PHXC, a subcutaneously delivered GalXC clinical candidate for the treatment of patients with all forms of primary hyperoxaluria (PH), is Dicerna's most advanced GalXC platform. Dicerna is preparing to file a clinical trial application in Europe in the fourth quarter, 2017, and plans to begin Phase 1 clinical trials in early 2018 as the Company pursues its goal of developing new therapies that address the full range of patients with PH.

PH is a family of severe, rare, genetic liver disorders characterized by overproduction of oxalate, a natural chemical in the body that is normally eliminated as waste through the kidneys. In patients with PH, the kidneys are unable to eliminate the large amount of oxalate that is produced, and the accumulation of oxalate can result in severe damage to the kidneys and other organs. Currently, there are no approved therapies for the treatment of PH.

### Genetic Orphan Disease

Dicerna launched a GalXC research program that targets a liver-expressed gene involved in a serious rare disease. The compound is currently in preclinical development.

## COMPLEX DISEASES WITH LARGE PATIENT POPULATIONS

### Hypercholesterolemia (PCSK9-targeted therapy)

We are using our GalXC RNAi platform to develop a therapeutic that targets the PCSK9 gene and will be evaluated for the treatment of statin-refractory patients with hypercholesterolemia. Based on the Company's candidate development work during the fourth quarter of 2016, Dicerna is positioned to advance DCR-PCSK9, which targets the PCSK9 gene and will be evaluated for the treatment of statin-refractory patients with hypercholesterolemia, into formal preclinical development. PCSK9 is a validated target for hypercholesterolemia, and there are FDA-approved therapies targeting PCSK9 that are based on monoclonal antibody technology. Based on preclinical studies, we believe that our GalXC RNAi platform can produce a PCSK9-targeted therapy with attractive commercial properties, such as small subcutaneous injection volumes and less frequent dosing.

### Chronic Hepatitis B Virus Infection

Dicerna continued to progress its DCR-HBVS program, which targets HBV directly, and has initiated formal IND-enabling activities. Current therapies for HBV rarely lead to a long-term immunological cure as measured by the clearance of HBV surface antigen (HBsAg). Based on findings from its preclinical studies, Dicerna is evaluating whether its GalXC RNAi platform can produce an experimental HBV-targeted therapy that significantly reduces HBsAg expression in affected patients and that has the potential to be delivered in a subcutaneous dosing paradigm. The Company expects to file an IND application in the U.S. or CTA in Europe for this program at approximately the end of 2018.

### Chronic Liver Disease

Dicerna is using its GalXC RNAi platform to investigate potential pharmaceutical therapeutic options for the treatment of chronic liver diseases (CLDs) such as nonalcoholic fatty liver disease (NAFLD), nonalcoholic steatohepatitis (NASH), primary sclerosing cholangitis (PSC), primary biliary cirrhosis (PBC), autoimmune hepatitis (AIH), progressive familial intrahepatic cholestasis (PFIC), and other indications. Estimates suggest that more than three million Americans live with some form of chronic liver disease. Based on preclinical studies, Dicerna believes that its GalXC RNAi platform enables exquisite targeting of hepatocytes and the silencing of injury-responsive mRNAs that result in release of profibrotic damage signals offering a novel approach to developing potential therapeutics for the treatment of CLD.

### An Undisclosed Rare Disease Involving the Liver

We are developing a GalXC-based therapeutic, targeting a liver-expressed gene involved in a serious rare disease. For competitive reasons we have not yet publicly disclosed the target gene or disease. We have selected this target gene and disease based on criteria that include having a strong therapeutic hypothesis, a readily-identifiable patient population, the availability of a potentially predictive biomarker, high unmet medical need, favorable competitive positioning, and what we believe is a rapid projected path to approval. Dicerna is on track to file an IND application in the U.S. and/or CTA in Europe for this program in the second quarter of 2018.