

HEADQUARTERS

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FOUNDED

2007

LISTED ON NASDAQ

2014

TRADING SYMBOL

DRNA

MANAGEMENT TEAM

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President & Chief Executive Officer

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

Jack Green, CPA
Chief Financial Officer

Jennifer Lockridge, M.D.
Vice President,
Program Development

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

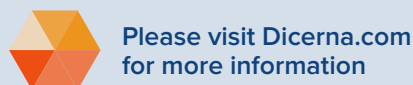
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OVERVIEW

- 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.
- Dicerna 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. In addition, the Company has developed hits and/or optimized GalXC conjugate inhibitors against almost 40 of these qualified targets.
- 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
- Dicerna has the capacity to launch up to three programs annually, with the intent to advance five programs into the clinic by the end of 2019.



DEVELOPMENT PIPELINE

PRODUCT CANDIDATE	INDICATION	STAGES OF DEVELOPMENT		
		RESEARCH	PRECLINICAL	CLINICAL POC STUDIES
DCR-PHXC	Primary Hyperoxaluria	[Progress bar spanning Research and Preclinical stages]		
Undisclosed	Rare Disease	[Progress bar spanning Research and Preclinical stages]		
DCR-PCSK9	Cardiovascular Disease	[Progress bar spanning Research and Preclinical stages]		
DCR-HBV	Hepatitis B Virus	[Progress bar spanning Research and Preclinical stages]		
Undisclosed	Cardiovascular Disease	[Progress bar in Research stage]		
Undisclosed	Chronic Liver Disease	[Progress bar in Research stage]		

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)

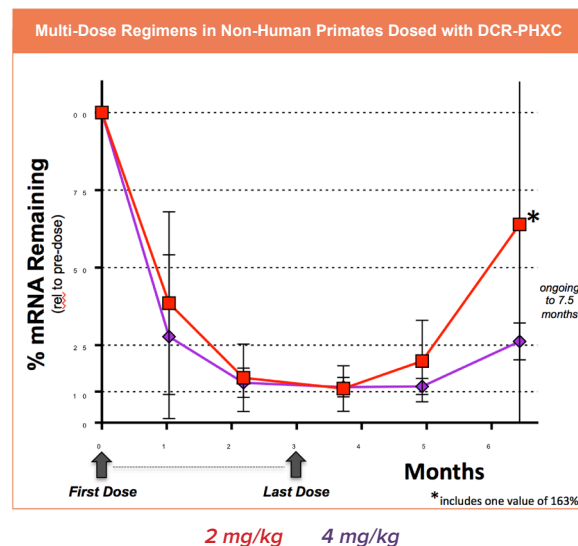
Primary Hyperoxaluria Type 1

DCR-PHXC, a subcutaneously delivered GalXC clinical candidate for the treatment of patients with primary hyperoxaluria type 1 (PH1), is Dicerna's most advanced GalXC program. Dicerna expects to file an Investigational New Drug application and/or a Clinical Trial Application for DCR-PHXC in late 2017 and commence human clinical trials shortly thereafter.

Primary hyperoxaluria (PH) is a severe, rare, inherited genetic disorder of the liver that results in irreparable damage to the kidneys. The disease is often fatal in the absence of a combined liver-kidney transplant. PH is estimated to affect one in 58,000 individuals worldwide. PH1 is the most common form, accounting for approximately 80% of cases. PH2 and PH3 each account for about 10% of cases.

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)

Dicerna is leveraging its GalXC RNAi platform to develop a therapeutic that targets the PCSK9 gene for the treatment of hypercholesterolemia. PCSK9 is a validated target for hypercholesterolemia, and there are United States (U.S.) Food and Drug Administration (FDA)-approved therapies targeting PCSK9 that are based on monoclonal antibody (MAb) technology. Based on preclinical studies, we believe that our GalXC RNAi platform can produce a PCSK9-targeted therapy with more attractive commercial properties than existing MAb therapies, based on comparatively smaller subcutaneous injection volumes and less frequent dosing, while providing equal or superior control of serum cholesterol.

Hepatitis B Virus Infection

Dicerna is using its GalXC RNAi platform to investigate potential pharmaceutical treatments for chronic Hepatitis B Virus (HBV). More than 350 million people are infected with HBV worldwide. Current therapies rarely lead to a long-term immunological cure as measured by the clearance of HBV surface antigen (HBsAg). Based on preclinical studies, Dicerna believes that its GalXC RNAi platform has the potential to produce an experimental HBV-targeted therapy that eliminates HBsAg expression in HBV patients and can be delivered in a commercially attractive subcutaneous dosing paradigm.

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.