CORPORATE FACT SHEET
MAY 2018

Dicerna

DELIVERING RNAi-BASED BREAKTHROUGH THERAPIES

To Improve Lives

HEADQUARTERS

87 Cambridgepark Drive
Cambridge, MA 02140
(617) 621-8097

TRADING SYMBOL

DRNA

MANAGEMENT TEAM

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

James B. Weissman
Chief Business Officer

Ralf Rosskamp, M.D.
Chief Medical Officer

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

John B. Green, CPA
Chief Financial Officer

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

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

CONTACT

James B. Weissman
Chief Business Officer
(617) 612-6214
jweissman@dicerna.com

Media:
Alex Van Rees
SmithSolve Communications
(973) 442-1555 x111
alex.vanrees@smithsolve.com

Investor:
Paula Schwartz
Rx Communications Group
(917) 322-2216
pschwartz@rxir.com

Please visit Dicerna.com for more information

OVERVIEW

- Dicerna is committed to discovering and developing innovative RNAi-based therapeutics for the treatment of critical unmet needs in rare diseases, viral infectious diseases, chronic liver diseases, and cardiovascular diseases.
- Dicerna's GalXC™ RNAi technology creates long-acting, subcutaneously administered product candidates that harness the body's natural biological pathways to silence or "turn off" disease-driving genes with a high degree of selectivity and specificity.
- Dicerna's development programs include:
  - DCR-PHXC for primary hyperoxaluria, currently in Phase 1 trials, called PHYOX.
  - DCR-HBVS for hepatitis B virus (we expect to file regulatory clearances to initiate a clinical trial in Q4 2018).
  - An undisclosed program for NASH (nonalcoholic steatohepatitis) partnered with Boehringer Ingelheim.
  - An undisclosed rare disease program, currently in IND-enabling studies.
  - Multiple discovery stage programs in rare diseases, chronic liver diseases, and cardiovascular diseases.
- Dicerna seeks to retain full or substantial ownership stake in our key rare disease programs, while pursuing partnerships for more complex diseases with multiple gene dysfunctions and larger patient populations.

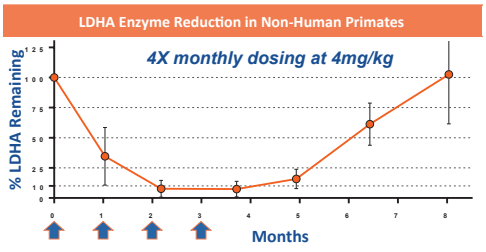
DEVELOPMENT PIPELINE

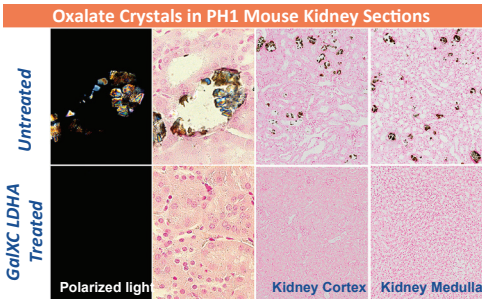
	PRODUCT CANDIDATE	INDICATION	STAGES OF DEVELOPMENT: RESEARCH / PRECLINICAL / CLINICAL POC STUDIES	PARTNERING STATUS
RARE DISEASES	DCR-PHXC	Primary Hyperoxalurias		*Proprietary program*
	DCR-undisclosed	Rare Disease		
	DCR-undisclosed	Rare Disease		
	DCR-undisclosed	Rare Disease		
LARGE POPULATION DISEASE	DCR-HBVS	Hepatitis B Virus		
	DCR-PCSK9	Hypercholesterolemia		
	DCR-LIV1	NASH		*Partnered with Boehringer Ingelheim*
	DCR-undisclosed	Cardiometabolic		
	DCR-undisclosed	Cardiometabolic		
	DCR-undisclosed	Cardiometabolic		
	DCR-undisclosed	Cardiometabolic		

KEY PROGRAMS

Primary Hyperoxaluria

Dicerna is developing DCR-PHXC, a subcutaneously delivered GalXC investigational candidate for the treatment of patients with all forms of primary hyperoxaluria (PH). DCR-PHXC is currently being tested in a Phase 1 trial, called PHYOX, comprised of healthy volunteers and patients with PH type 1 and PH type 2. Proof-of-concept data from this trial are expected in the second half of 2018.





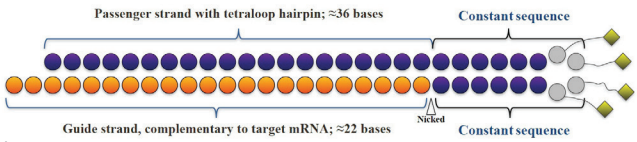
PH is a family of severe, rare, genetic liver disorders characterized by overproduction of oxalate, a natural chemical in the body that is normally eliminated as waste through the kidneys. In patients with PH, the kidneys are unable to eliminate the large amount of oxalate that is produced, and the accumulation of oxalate can result in severe damage to the kidneys (nephrocalcinosis) and other organs (systemic oxalosis). In patients with PH type 1, the median age of kidney failure is in the early 20s. Currently, there are no approved therapies for the treatment of PH. In preclinical studies, DCR-PHXC mediates near complete silencing (>90%) of the oxalate-generating enzyme LDHA when dosed monthly in non-human primates at 4 mg/kg, an easily tolerated dose. Comparable dosing in the mouse genetic model of PH type 1 blocks the production of excess oxalate and prevents the induced nephrocalcinosis symptoms.

Chronic Hepatitis B Virus Infection

We have declared a GalXC RNAi platform-based product candidate for the treatment of HBV, DCR-HBVS, and are conducting formal non-clinical development studies. Current therapies for HBV rarely lead to a long-term immunological cure as measured by the clearance of HBV surface antigen (HBsAg) and sustained HBV deoxyribonucleic acid (DNA) suppression in patient plasma or blood. We expect to file regulatory clearances to initiate a clinical trial during the fourth quarter of 2018. DCR-HBVS targets HBV messenger RNA and leads to greater than 99% reduction in circulating HBsAg in mouse models of HBV infection. Based on preclinical studies, and only if we receive appropriate regulatory approval to begin human clinical trials, we hope to determine the potential of DCR-HBVS to reduce HBsAg and HBV DNA levels in the blood of HBV patients in a commercially attractive subcutaneous dosing paradigm.

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's GalXC RNAi Therapeutic Platform

GalXC-BASED PARTNERING OPPORTUNITIES

Cardiometabolic and Chronic Liver Diseases

Dicerna is investigating potential pharmaceutical therapeutic options with the GalXC RNAi platform for the treatment of cardiometabolic and chronic liver diseases such as hypocholesteremia, hypertriglyceridemia, other forms of dyslipidemias, nonalcoholic fatty liver disease (NAFLD), nonalcoholic steatohepatitis (NASH), and multiple additional liver diseases less frequently diagnosed. Based on preclinical studies, Dicerna believes that its GalXC RNAi platform enables exquisite targeting of hepatocytes and the silencing of injury-responsive mRNAs for the potential treatment of cardiometabolic and chronic liver diseases.

In October 2017, Dicerna entered into collaboration with Boehringer Ingelheim to generate a potential therapeutic for NASH using the GalXC RNAi platform.

An Undisclosed Rare Disease Involving the Liver

Dicerna is developing a GalXC-based product candidate for the treatment of a serious rare liver 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 seeking a risk-sharing collaborator for this program before we file regulatory clearances to initiate a clinical trial, which we expect to be prepared to file in the second half of 2018.

Hypercholesterolemia (PCSK9-targeted therapy)

Dicerna is using the GalXC RNAi platform to develop a therapeutic that targets the PCSK9 gene for the possible treatment of hypercholesterolemia. The Company has selected a provisional clinical candidate. 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 has the potential to produce a PCSK9-targeted therapy with attractive commercial properties, such as small subcutaneous injection volumes and less frequent dosing.