CORPORATE FACT SHEET
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Dicerna

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FOUNDED

2007

LISTED ON NASDAQ

2014

TRADING SYMBOL

DRNA

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Senior Vice President

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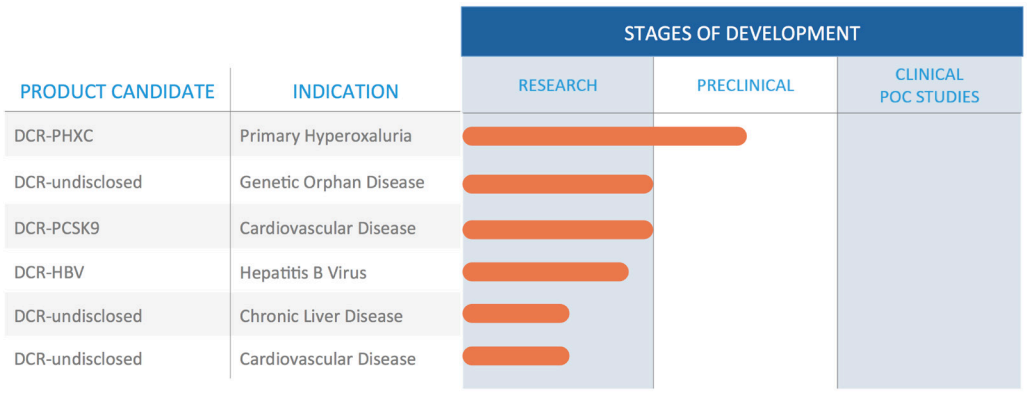
OVERVIEW

- Dicerna is a biopharmaceutical company focused on the discovery and development of innovative subcutaneously delivered RNA 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 developed a growing pipeline of GalXC product candidates and has qualified over 30 disease-associated genes, providing expansive therapeutic opportunities.
- 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

DEVELOPMENT PIPELINE

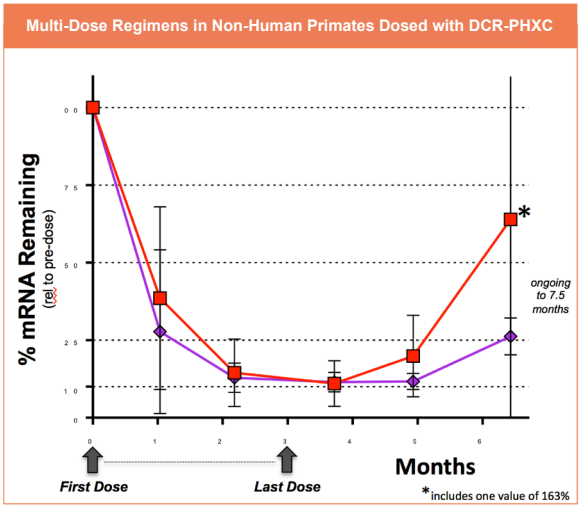


Dicerna has developed an extensive library of GalXC molecules across our Tx areas with high potency in rodents, ready for optimization and full candidate qualification.

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 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 1 in 58,000 individuals worldwide. Type 1 is the most common form, accounting for approximately 80% of cases. Types 2 and 3 each account for about 10% of cases. [1]



Genetic Orphan Disease

Dicerna launched a GalXC research program, DCR-undisclosed, in 2016 in an orphan genetic disease in which there is a high unmet medical need. The compound is currently in preclinical development.

Cardiovascular Disease

Dicerna launched its GalXC research program, DCR-PCSK9, in cardiovascular disease focused on the PCSK9 gene. The PCSK9 gene provides instructions for making a protein that helps regulate the amount of cholesterol in the bloodstream. The PCSK9 protein appears to control the number of low-density lipoprotein receptors, which are proteins on the surface of cells. These receptors play a critical role in regulating blood cholesterol levels. Studies suggest that the PCSK9 protein helps control blood cholesterol levels by breaking down low-density lipoprotein receptors before they reach the cell surface. [2]

Mutations in the PCSK9 gene can cause hypercholesterolemia (high cholesterol). Other mutations in the PCSK9 gene result in reduced blood cholesterol levels (hypocholesterolemia). [3]

More than 34 million American adults have elevated blood cholesterol levels. Inherited forms of hypercholesterolemia, which cause even higher levels of cholesterol, occur less frequently. The most common inherited form of high cholesterol is called familial hypercholesterolemia. Hypercholesterolemia affects about 1 in 500 people in most countries. [4]

Hepatitis B Virus

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) and sustained HBV deoxyribonucleic acid (DNA) suppression. Based on preclinical studies, Dicerna believes that its GalXC RNAi platform can produce an experimental HBV-targeted therapy that eliminates HBsAg expression in HBV patients and that has the potential to be delivered in a commercially attractive subcutaneous dosing paradigm.

Additional Research Programs

Dicerna has the capacity to launch up to three programs every year, with the intent to advance five programs into the clinic by the end of 2019. In 2017, Dicerna expects to launch a chronic liver disease program and another undisclosed program in the area of cardiovascular disease.

References

[1] Genomics Home Reference, https://ghr.nlm.nih.gov/condition/primary-hyperoxaluria#
[2] Genomics Home Reference, https://ghr.nlm.nih.gov/gene/PCSK9#normalfunction
[3] Genomics Home Reference, https://ghr.nlm.nih.gov/condition/familial-hypobetalipoproteinemia
[4] Genomics Home Reference, https://ghr.nlm.nih.gov/condition/hypercholesterolemia#statistics