



# Dicerna

## Corporate Overview

**Douglas Fambrough, President and CEO**  
**8<sup>th</sup> Annual SVB Leerink Healthcare Conference**  
**March 1, 2019**

# Forward-looking Statements

---

This information may contain projections and other forward-looking statements regarding future events, including statements regarding Dicerna's technology platform, product candidates, preclinical and clinical pipeline and milestones, regulatory objectives, market opportunities, and intellectual property. Such statements are predictions only and are subject to risks and uncertainties that could cause actual events or results to differ materially. These risks and uncertainties include, among others, the cost, timing and results of preclinical studies and clinical trials and other development activities; the unpredictability of the duration and results of regulatory review of New Drug Applications and Investigational NDAs; market acceptance for approved products and innovative therapeutic treatments; competition; the possible impairment of, inability to obtain and costs of obtaining intellectual property rights; and possible safety or efficacy concerns, general business, financial and accounting risks and litigation. More information concerning Dicerna and such risks and uncertainties is available on its website and in its press releases, and in its public filings with the U.S. Securities and Exchange Commission.

Dicerna is providing this information as of this date and does not undertake any obligation to update or revise it, whether as a result of new information, future events or circumstances or otherwise. Additional information concerning Dicerna and its business may be available in press releases or other public announcements and public filings made after the date of this information.

# Dicerna Vision and Strategy: Driving RNAi Therapies Forward for Patients

---

## Vision

Build a fully integrated company by developing innovative RNAi-based therapies for high unmet medical needs and broadly capture the value of our GalXC™ platform

## Strategy

- **Build Value** in rare diseases by independently developing and commercializing programs with high value and high probability of success
- **Capture Value** in common diseases by developing high probability of success programs through clinical proof of concept before seeking development and commercialization partners
- **Enhance Value** by partnering with therapeutic field leaders on discovery stage programs

# Dicerna Development Pipeline



CANDIDATE	INDICATION	RESEARCH	PRECLINICAL	CLINICAL POC TRIALS	REGISTRATION TRIALS	PARTNER
DCR-PHXC	Primary Hyperoxalurias	ORPHAN				—
DCR-HBVS	Hepatitis B Virus	COMMON				—
DCR-undisclosed	Rare Disease	ORPHAN				—
DCR-undisclosed	Undisclosed	COMMON				—
DCR-LIV1	NASH	COMMON				Boehringer Ingelheim
DCR-LIV2	NASH	COMMON				Boehringer Ingelheim
DCR-CM1	Cardiometabolic	COMMON				Lilly
DCR-CM2	Cardiometabolic	COMMON				Lilly
DCR-CM3	Cardiometabolic	COMMON				Lilly
DCR-NEURO1	Neurodegeneration	COMMON				Lilly
DCR-NEURO2	Neurodegeneration	COMMON				Lilly
DCR-COMP1	Complement-mediated	ORPHAN				Alexion
DCR-COMP2	Complement-mediated	ORPHAN				Alexion

● ORPHAN ● COMMON

# Lead Programs Focused on Major Unmet Medical Needs

## Primary Hyperoxalurias (PH)

- PH is family of serious, rare inborn errors of metabolism where excess oxalate production by the liver causes serious damage to the kidneys. Genome sequencing projects predict up to as many as ~16K PH patients in the U.S. and EU who might benefit from DCR-PHXC
- Dicerna's DCR-PHXC is the only RNAi candidate in development for all PH types with convenient, monthly fixed-dose regimen, designed for pre-filled syringes or autoinjectors
- Double-blind, randomized, placebo-controlled trial (2:1 randomization) in approx. 36 patients with PH1 and PH2 (Q1 2019 initiation)
- Phase 1 open label extension study: Patients from PHYOX may be re-enrolled into a long-term, multi-dose open label extension trial, allowing continuous readout of multi-dose data (Q2 2019 initiation)

## Hepatitis B Virus (HBV) Infection

- Massive worldwide economic burden: ~257 million chronically infected and >10th leading cause of death worldwide: 887,000 in 2015 (WHO); Responsible for 80% of primary liver cancers
- Functional cure of chronic HBV is the best treatment outcome currently, but is seldom achieved
- RNAi can simultaneously inhibit multiple viral activities due to overlapping transcripts, and RNAi therapy may be an important component of an effective functional cure for chronic HBV
- Three part Phase 1 proof-of-concept study in healthy volunteers and chronic HBV patients
  - Group A: Single dose dose-ascending study, placebo controlled, in 30 normal healthy volunteers: First volunteer dosed in Q1 2019
  - Group B: Single-dose study, placebo controlled, in 8 NUC-naïve chronic hepatitis B patients
  - Group C: Multiple dose-ascending dose study, placebo-controlled, in 18 NUC-experienced chronic hepatitis B patients: First patient to be dosed in Q2 2019

# Realizing Value Through Strategic Collaborations

---

## Developing GalXC™ candidates with established pharmaceutical partners

- Lilly: cardiometabolic, neurodegenerative and pain targets
- Alexion: complement pathway targets
- Boehringer Ingelheim: NASH targets

## Expanding the range of the GalXC technology platform to go beyond liver tissue

- Neural tissues: exclusive collaboration with Lilly to develop RNAi neural delivery technology; Dicerna retains right to select rare diseases for Dicerna development
- Cardiometabolic tissue: non-exclusive collaboration with Lilly

## Providing resources to drive our strategic programs

- \$252 million in upfront payments and equity at a premium among the three collaborations
- >\$200 million is allocated to our proprietary programs

The Lilly logo is written in a red, cursive script font.The Alexion logo features the word "ALEXION" in a bold, blue, sans-serif font, with a red and blue arc above the letters "X" and "I".The logo for Boehringer Ingelheim consists of a circular icon with vertical bars of varying heights, followed by the text "Boehringer Ingelheim" in a blue, sans-serif font.

# 2019 Milestones

*Building Value, Capturing Value, Enhancing Value*

---

## **Advance DCR-PHXC for the treatment of primary hyperoxaluria (PH)**

- Initiate PHYOX2, a FDA/EMEA registration trial in patients with PH1 and PH2
- Report longer-term clinical data from PHYOX3, an open-label extension of PHYOX1
- Initiate PHYOX4, a Phase 2 open-label trial in patients with PH3

## **Advance DCR-HBVS for the treatment of patients with chronic hepatitis B virus (HBV)**

- Dose first healthy volunteer in Phase 1 trial
- Dose first patient with non-cirrhotic chronic HBV in Phase 1 trial
- Report proof-of concept data for DCR-HBVS

## **Advance investigational GalXC™ therapy for the treatment of an undisclosed rare disease**

- File Clinical Trial Application

## **Achieve anticipated corporate milestones**

- Evolve Board composition and expand leadership team to support continued growth

# Dicerna