



Corporate Overview

March 2020

Forward-looking Statements

This presentation has been prepared by Dicerna Pharmaceuticals, Inc. (“we,” “us,” “our,” “Dicerna,” or the “Company”) and includes forward-looking statements. Forward-looking statements are subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statements. Examples of forward-looking statements include, among others, statements we make regarding: (i) the therapeutic and commercial potential of nedosiran (DCR-PHXC), RG6346 (DCR-HBVS), DCR-A1AT and the GalXC™ platform; (ii) expectations that our current cash and future collaborative revenue will fund operations into 2023; (iii) research and development plans and timelines, as well as regulatory pathways and plans, related to nedosiran, RG6346, DCR-A1AT and GalXC; (iv) the potential of Dicerna’s technology and drug candidates in the Company’s research and development pipeline; (v) the Company’s collaborations with Novo Nordisk A/S; Roche; Eli Lilly and Company; Alexion Pharmaceuticals, Inc.; Boehringer Ingelheim International GmbH; and (vi) the Company’s strategy, business plans and focus. The process by which an early-stage investigational therapy such as nedosiran and an early-stage platform such as GalXC could potentially lead to an approved product is long and subject to significant risks. Applicable risks and uncertainties include those relating to Dicerna’s clinical research and other risks identified under the heading “Risk Factors” included in the Company’s most recent Form 10-K filing and in other subsequent filings with the Securities and Exchange Commission. These risks and uncertainties include, among others, the cost, timing and results of preclinical studies and clinical trials and other development activities; the likelihood of Dicerna’s clinical programs being executed within timelines provided and reliance on the Company’s contract research organizations and predictability of timely enrollment of subjects and patients to advance Dicerna’s clinical trials; the potential for future data to alter initial and preliminary results of early-stage clinical trials; the unpredictability of the duration and results of the regulatory review of Investigational New Drug (IND) applications and Clinical Trial Applications that are necessary to continue to advance and progress the Company’s clinical programs and the regulatory review of submissions relevant to regulatory agencies for marketing approvals, including New Drug Applications (NDAs); market acceptance for approved products and innovative therapeutic treatments; competition; the possible impairment of, inability to obtain and costs of obtaining needed intellectual property rights; possible safety or efficacy concerns that could emerge as new data are generated in R&D; that the Company may not realize the intended benefits of its collaborations; general business, financial and accounting risks; and the risks and potential outcomes from litigation.

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.

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VISION


Maximize the impact
of RNAi on medicine

STRATEGY

Develop and commercialize our core
high-probability-of-success programs
either alone or in collaboration
with partners

Broadly enable the use of our GalXC™
technology by collaborating with
therapeutic area leaders on
non-core opportunities

Dicerna Pipeline of Core and Collaborative Programs

	CANDIDATE	TARGET INDICATION	DISCOVERY/RESEARCH	PRECLINICAL	CLINICAL PROOF-OF-CONCEPT TRIALS	REGISTRATION TRIALS	DICERNA'S PRODUCT RIGHTS
Dicerna™ Core Programs	Nedosiran (DCR-PHXC)	Primary Hyperoxaluria					100%
	RG6346* (DCR-HBVS) 	Hepatitis B Virus					U.S. opt-in
	DCR-A1AT	A1AT Liver Disease					100%
	DCR-undisclosed	Undisclosed					100%

*Under its agreement with Roche, Dicerna has the option to co-fund pivotal development for heightened royalties and co-promotion rights in U.S.

● ORPHAN ● PREVALENT



<p>● NASH</p> <p>1 target</p> <p>Discovery/Research</p>	<p>● Complement-mediated</p> <p>● 4 targets</p> <p>● Initially two targets; ALXN exercised option for two more targets in Dec. 2019</p>	<p>● Cardiometabolic (CM; liver and non-liver), neurodegeneration and pain</p> <p>● Up to 8 CM targets</p> <p>● DRNA retains rights to certain neuro orphan indications</p> <p>● First IND/CTA expected late 2020 (LY3561774)</p>	<p>● HBV infection</p> <p>● RG6346 and multiple potential targets</p> <p>● DRNA option to co-fund dev. and co-promote in U.S.</p> <p>Discovery/Research anticipated 2020 start</p>	<p>● Liver-related cardiometabolic diseases</p> <p>● 30+ potential targets</p> <p>● 2 Novo programs: DRNA opt-in</p> <p>● DRNA retains rights to 2 new orphan programs (Novo retains opt-in rights)</p> <p>Initial targets selected</p>
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● Currently active discovery or preclinical-stage liver program

● Currently active discovery or preclinical-stage non-liver program

GalXC™ The Foundation of Our Value



- Proprietary, patented RNA interference (RNAi) technology with potential to extend to diverse tissues beyond the liver
- Clinically compelling pharmaceutical properties

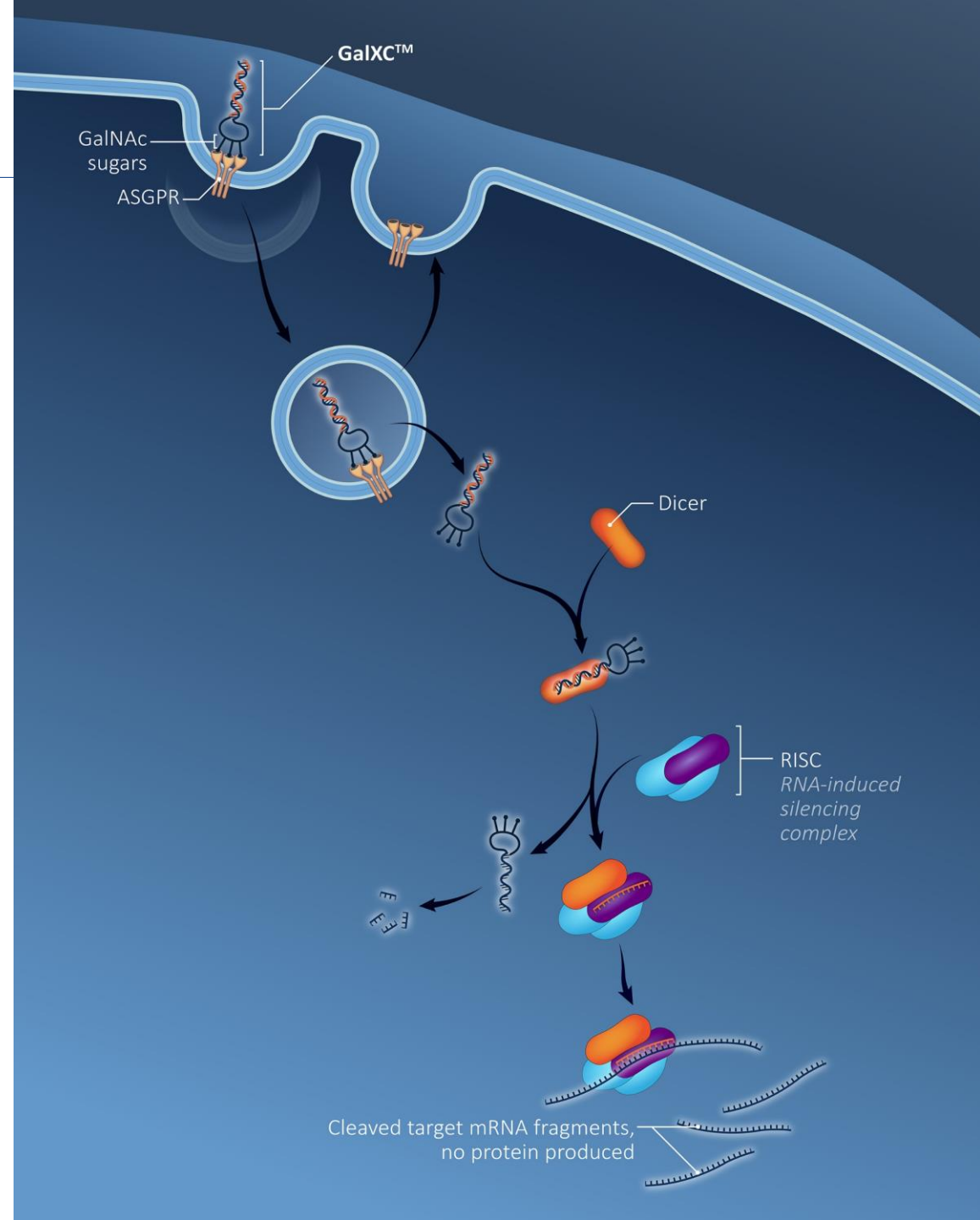
Subcutaneously delivered	→	convenient administration
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Long duration of action	→	infrequent dosing
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High target specificity	→	predictable activity
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High therapeutic index	→	broad applicability
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Established manufacturing	→	scalable
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Targeting Multiple Milestones Throughout 2020

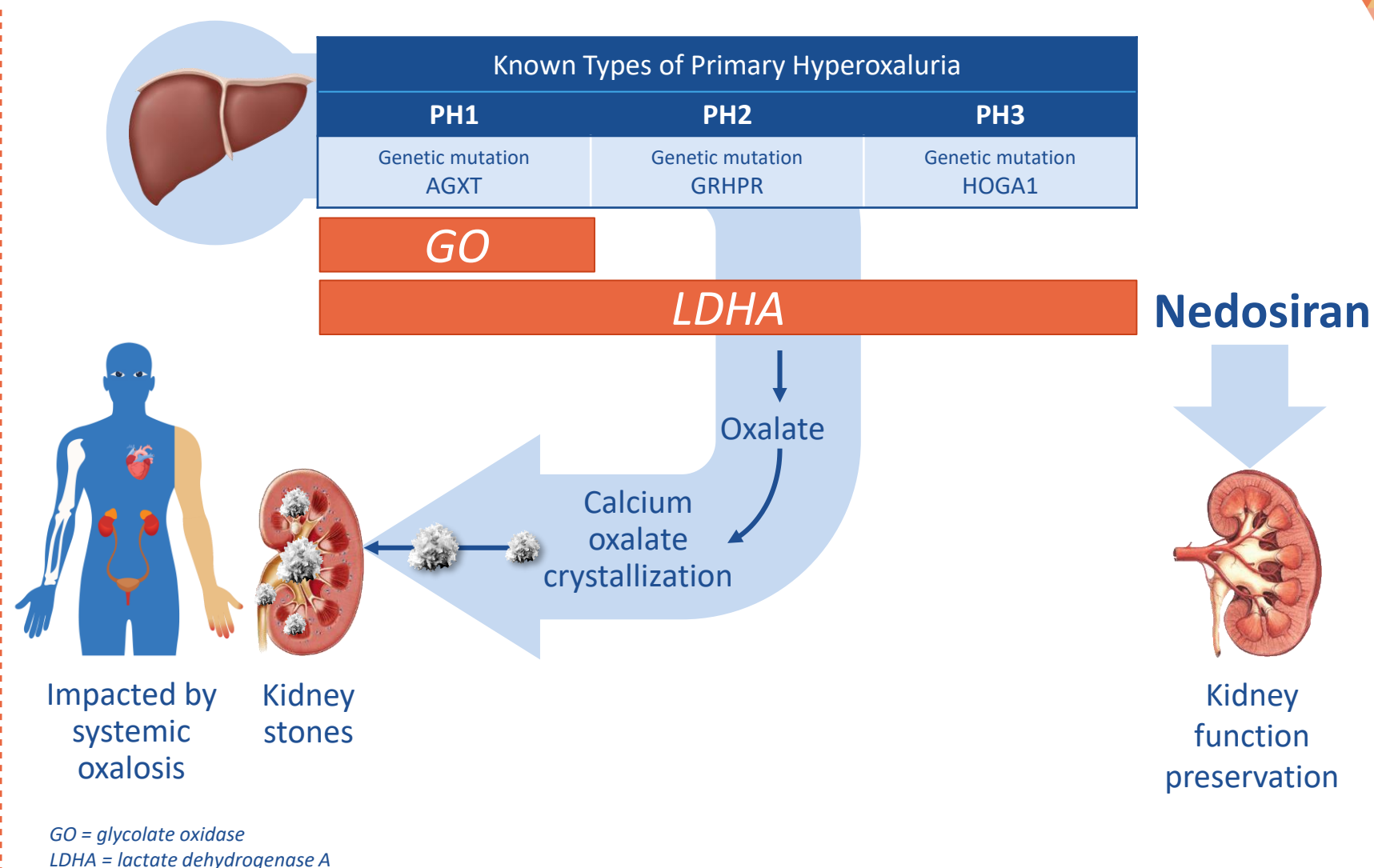
- ❑ **Nedosiran:** First multi-dose data from PHYOX™3 open-label clinical trial – OxalEurope International Congress, March 31, 2020
- ❑ **Nedosiran:** PHYOX2 pivotal clinical trial enrollment completion – Q2 2020
- ❑ **RG6346:** Phase 1 proof-of-concept data from all existing cohorts – Q3 2020 R&D Day
- ❑ **GalXC:** Present data for extending GalXC technology to additional tissues – Q3 2020 R&D Day
- ❑ **DCR-A1AT:** First patient dosing in Phase 1/2 trial – 2H 2020
- ❑ **Collaborative Program:** IND or CTA filing for LY3561774 – late 2020
- ❑ **Nedosiran:** PHYOX2 last patient out – by YE 2020

Primary Hyperoxaluria

Primary Hyperoxaluria (PH)

A family of ultra-rare, life-threatening genetic disorders resulting in renal complications

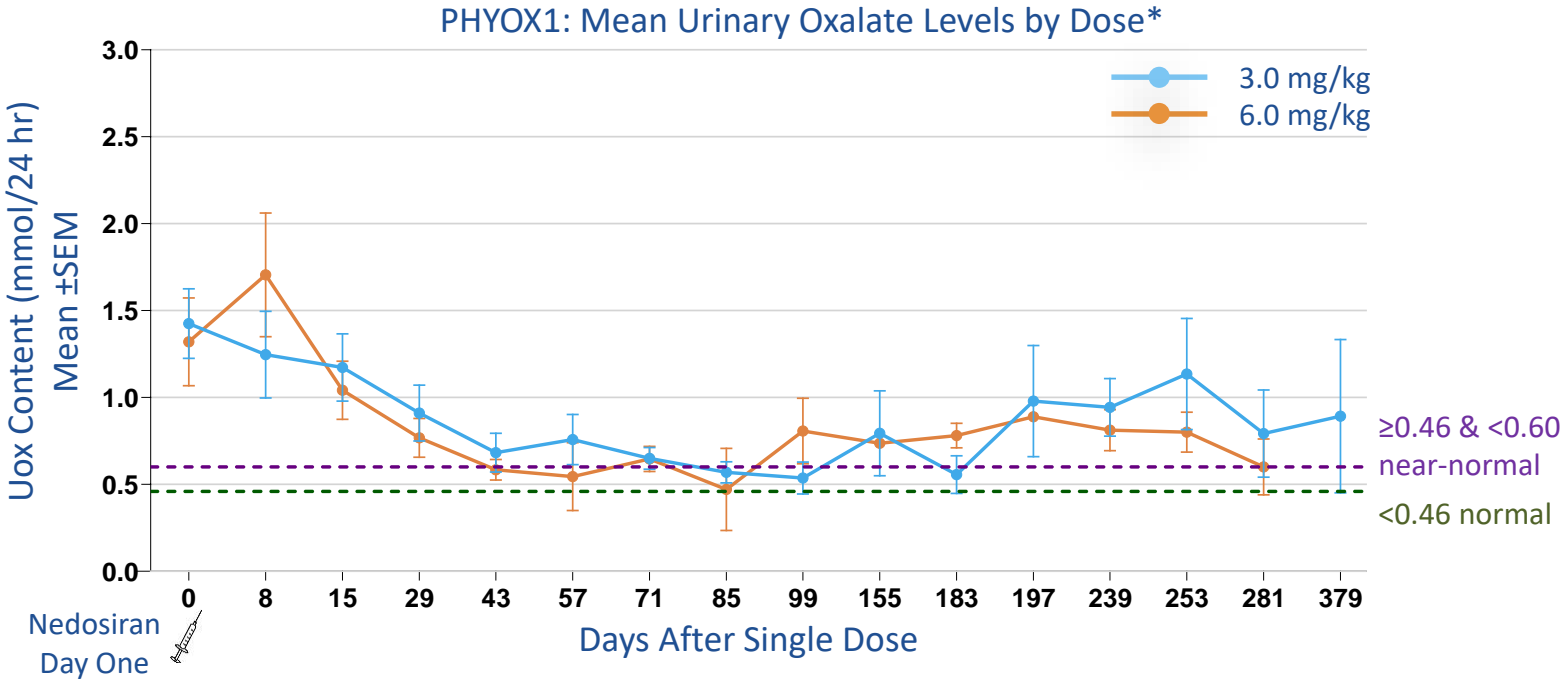
- Three known types of PH, each resulting from a mutation in one of three different genes, cause enzyme deficiencies manifesting in overproduction of oxalate
- Abnormal production and accumulation of oxalate leads to:
 - Recurrent kidney stones
 - Nephrocalcinosis (deposition of calcium in the kidney)
 - Chronic kidney disease that may progress to end-stage renal disease, requiring regular dialysis and transplant (dual liver-kidney or kidney)
 - Systemic oxalosis, which also impacts the heart, skin, eyes, bones
- Dicerna's **nedosiran** silences *LDHA*, the ultimate step in the oxalate production pathway



PHYOX1: Phase 1 Single-Dose Study of Nedosiran in PH1 and PH2 Participants

14/18 participants achieved normalization or near-normalization

- PHYOX1 open-label study included:
 - 18 participants dosed: PH1 (n=15) and PH2 (n=3)
 - Genetically confirmed diagnosis
 - Uox ≥ 0.7 mmol/24hr
 - eGFR ≥ 30 mL/min/1.73m²
 - 1.5, 3.0, 6.0 mg/kg doses delivered subcutaneously
- Normalization or near-normalization in 60%, 83% and 100% of PH1 patients at doses of 1.5, 3.0 and 6.0 mg/kg, respectively
- 2 of 3 PH2 patients with normalization or near-normalization
- The only drug-related AEs were mild to moderate injection-site reactions
- No drug-related SAEs were observed



PH Type	Dose (mg/kg)	Pts. Reaching Normalization or Near-Normalization (%)	Max Reduction Uox (%) Mean (range)
PH1	1.5 (n=5)	3 (60)	51 (28-72)
	3.0 (n=6)	5 (83)	72 (62-80)
	6.0 (n=4)	4 (100)	72 (35-100)
PH2	1.5 (n=1)	0 (0)	39
	3.0 (n=2)	2 (100)	54 (42-66)

*Days with at least two values
ClinicalTrials.gov: NCT03392896

Nedosiran Clinical Trial Program to NDA Filing

Coordinated program of clinical trials to support a broad label in PH

Pivotal Study Package

Trial	Description/Details	Status	PH Type
PHYOX TM 2	Study 201: Pivotal, double-blind, randomized, placebo-controlled trial (2:1 randomization) Monthly fixed-dose, enabling prefilled syringes at launch	Enrolling n=~36	1,2
PHYOX TM 3	Study 301: Long-term, multi-dose, open-label extension; rollover study open to all patients in PHYOX trials	Enrolling	1,2,3

Additional Supportive Studies

PHYOX TM 4	Study 104: Single-dose open-label study in patients with primary hyperoxaluria type 3 (PH3)	Expected to initiate: 1H 2020*	3
PHYOX TM 7	Study 204: Multi-dose trial in patients (birth to adult) with PH and end-stage renal disease (ESRD)	Expected to initiate: 1H 2020*	1,2
PHYOX TM 8	Study 203: Open-label study in children 2-5 yrs	Expected to initiate: 2H 2020*	1,2

** Subject to regulatory approvals*

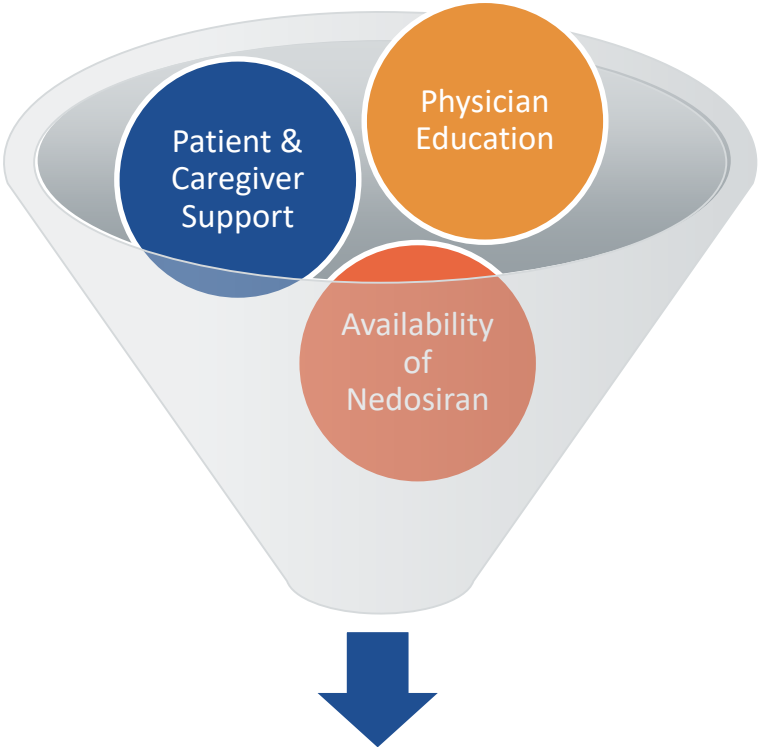
Nedosiran: The Only RNAi Drug Candidate in Development for All PH Types

High unmet medical need across all PH types can be addressed

Genetics of PH

	PH1	PH2	PH3	All Known PH Types
Genetic prevalence (per million)	8.23	5.08	12.58	25.89
US	2,681	1,655	4,098	8,434
EU	2,607	1,609	3,986	8,202
Estimated Prevalent Population (U.S. + EU)	5,288	3,264	8,084	16,636

- Dicerna estimates nedosiran peak sales between \$500M and \$1B

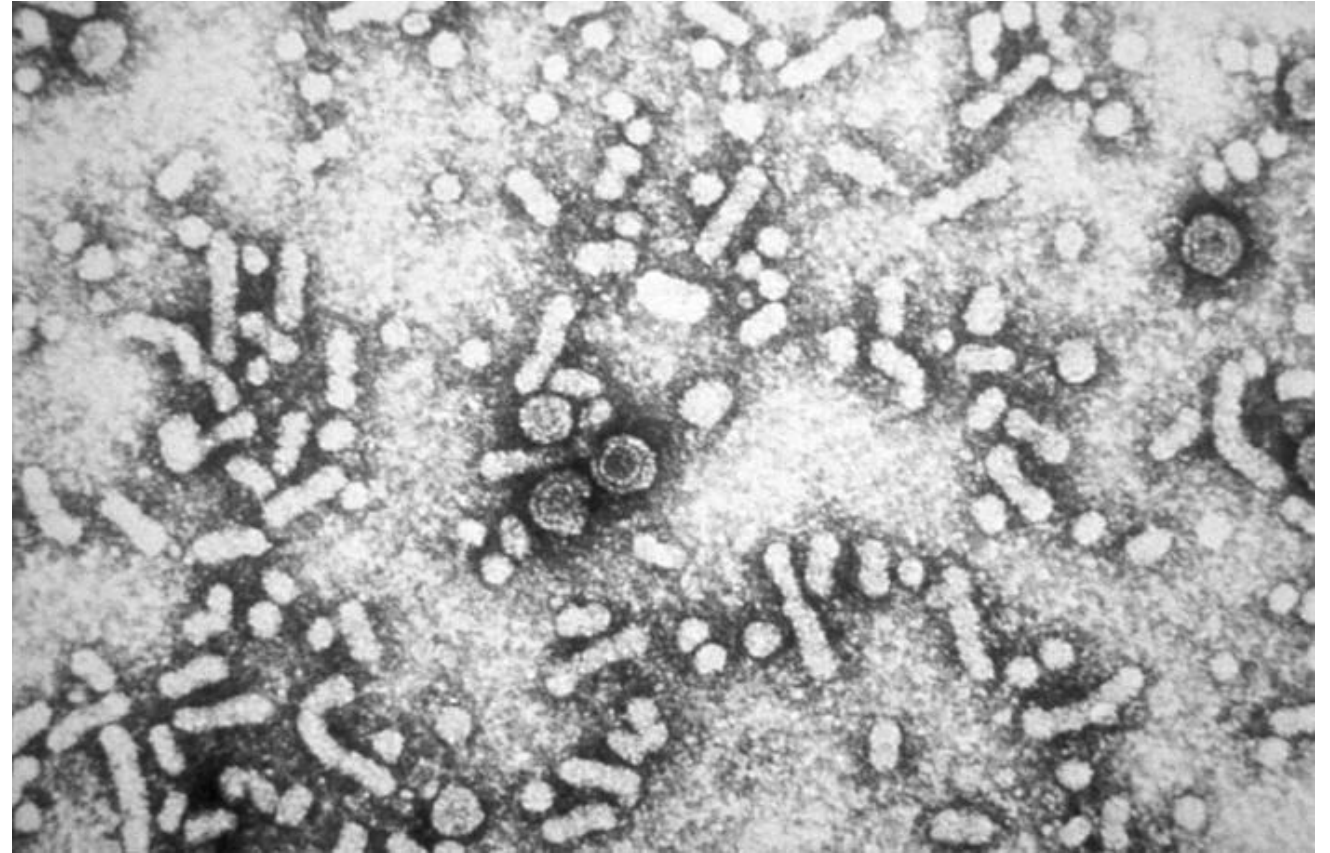


Pre- and post-launch activities will improve diagnosis across all PH types

Chronic Hepatitis B Virus Infection

Hepatitis B: A Severe, Global Unmet Medical Need

- Significant worldwide prevalence: ~292 million infected
- Causes more than 887,000 deaths per year
- Current treatments are rarely effective in achieving functional cures
- Dicerna is collaborating with Roche to develop **RG6346 (DCR-HBVS)** and potentially other agents for the treatment of HBV



Electron micrograph of HBV showing infectious viral particles (~42 nm) and non-infectious sub-viral “decoy” particles (~22 nm) and filaments

Sources: Global prevalence, treatment, and prevention of hepatitis B virus infection in 2016: a modelling study. *The Lancet Gastroenterology and Hepatology*. Volume 3, Issue 6, June 2018, Pages 383-403. World Health Organization. Finding a cure for hepatitis B: are we close? <https://www.who.int/hepatitis/news-events/hbv-cure-overview/en/>. Accessed Dec. 30, 2019.

GalXC RNAi May Play a Key Role in Establishing a Functional HBV Cure

Organization of the HBV genome enables effective RNAi targeting of multiple viral functions

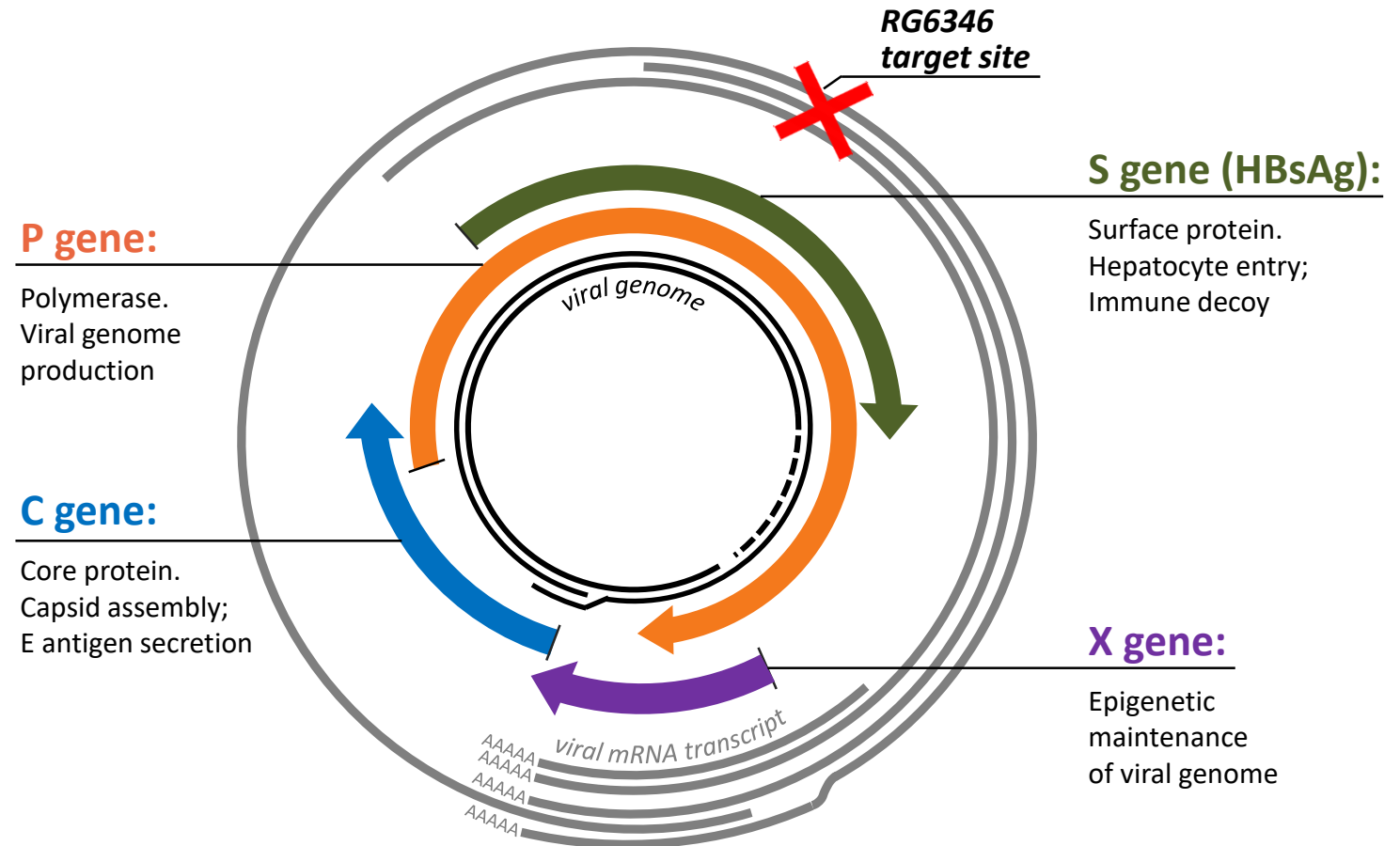
The Promise of RNAi for HBV

- RNAi can simultaneously inhibit multiple viral activities due to overlapping transcripts
- RNAi can target all viral transcripts from cccDNA and integrated genomes

Current HBV Therapies Are Inadequate

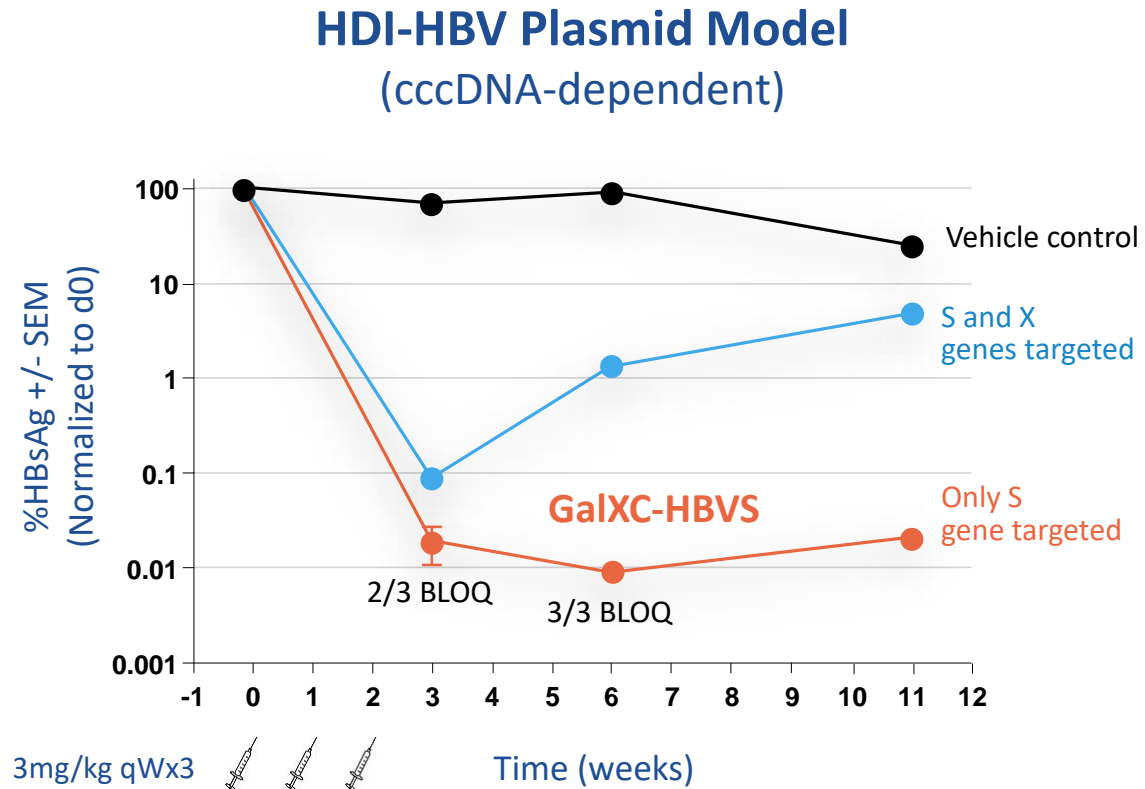
- Functional cure of chronic HBV would be the best treatment outcome
 - Defined by the lack of detectable HBsAg in serum (often associated with seroconversion to anti-HBsAg+)
- Interferons and NUCs are the only approved therapies, but offer very low functional cure rates

Overlapping mRNAs and protein-coding regions enable targeting multiple HBV genes and proteins with a single GalXC trigger



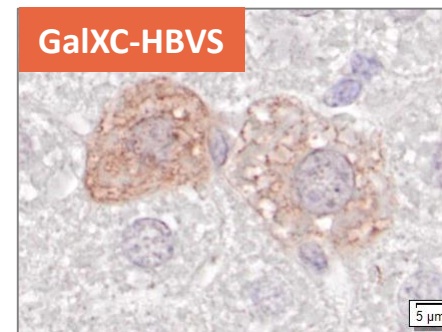
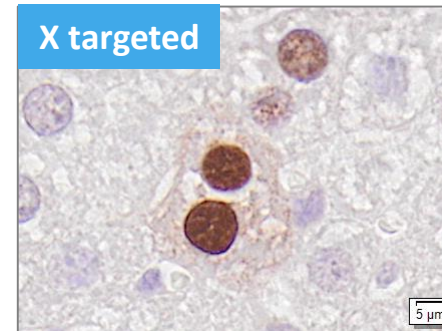
Single-Dose GalXC-HBVS Reduced HBsAg to Below Lower Level of Detection

Striking pharmacodynamic differences between targeting in the S alone vs. S and X ORFs in preclinical model



- GalXC-HBVS: ≥ 3.9 log reduction, *long duration of activity*
- X gene targeted: 3.0 log reduction, *shorter duration of activity*

HDI-HBV



- Immunohistochemical staining of mouse liver sections for HBV Core Protein reveals **differential subcellular localization** in the HDI-HBV plasmid model
- Silencing of X gene leads to nuclear localized Core Protein likely driving additional S expression
- These results have been reproduced using alternative guide strand sequences (i.e., different mRNA binding sites) for both GalXC-HBVS and GalXC-HBVX

RG6346 (DCR-HBVS) Clinical Program for Proof of Concept

Includes placebo-controlled studies in both NUC-naïve and NUC-experienced patients

Three-Part Study in Healthy Volunteers and Patients With Chronic HBV Infection

Group A	Placebo-controlled, single-ascending-dose study in healthy volunteers	Completed n=30	RG6346 dose cohorts: 0.1, 1.5, 3.0, 6.0, 12.0 mg/kg
Group B	Placebo-controlled, single-dose study in patients with no prior use of nucleoside or nucleotide analogue (NUC) therapy (NUC-naïve) with chronic HBV infection	Dosing n=8	RG6346 dose cohort: 3.0 mg/kg (NUCs initiated after 12 wks)
Group C	Placebo-controlled, multiple-ascending-dose study in NUC-experienced patients with chronic HBV infection	Dosing n=18 Enrolling 6.0 mg/kg cohort	RG6346 dose cohorts: 1.5, 3.0, 6.0 mg/kg; 4 monthly doses

- Patients with ≥ 1 log HBsAg suppression will continue in observational follow-up after 12 weeks in Group B, 16 weeks in Group C. Groups B and C are now represented in extended follow-up phase.
- Data expected to be presented at R&D Day event in August 2020

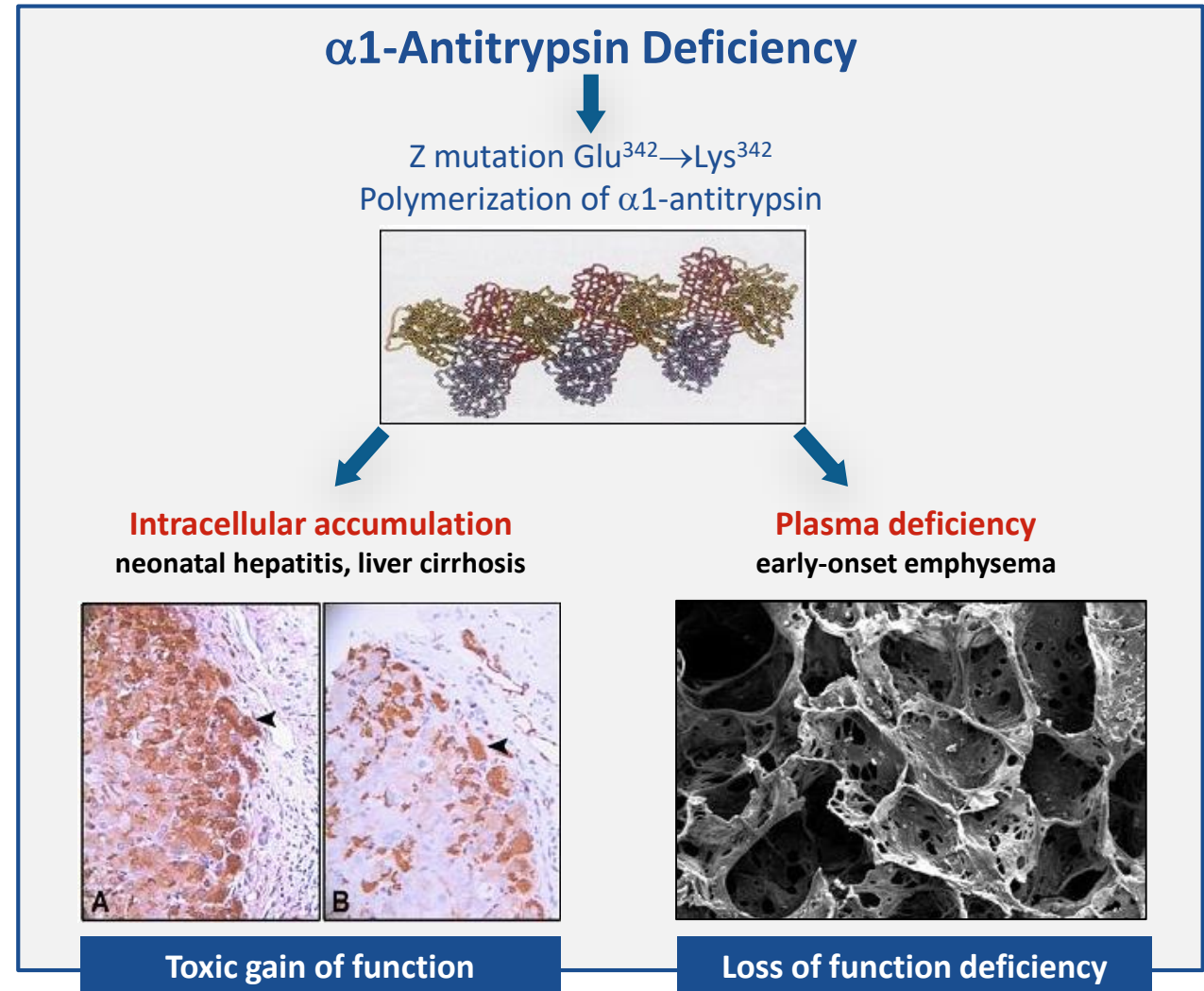


Alpha-1 Antitrypsin Deficiency- Associated Liver Disease

Alpha-1 Antitrypsin Deficiency-Associated Liver Disease

Most disease is caused by a single missense allele that forms aggregates in hepatocytes

- Alpha-1 antitrypsin (A1AT): predominantly produced in liver and secreted into blood
- Pi*ZZ genotype of the *SERPINA1* gene affects 95% of patients with A1AT deficiency
- The Z-allele of the *SERPINA1* gene produces an abnormal form of the protein, which can form pathological aggregates in hepatocytes
 - Abnormal A1AT protein aggregates accumulate in liver, triggering injury cascade that can lead to liver disease
 - Lack of normal A1AT protein can lead to lung disease, especially in smokers

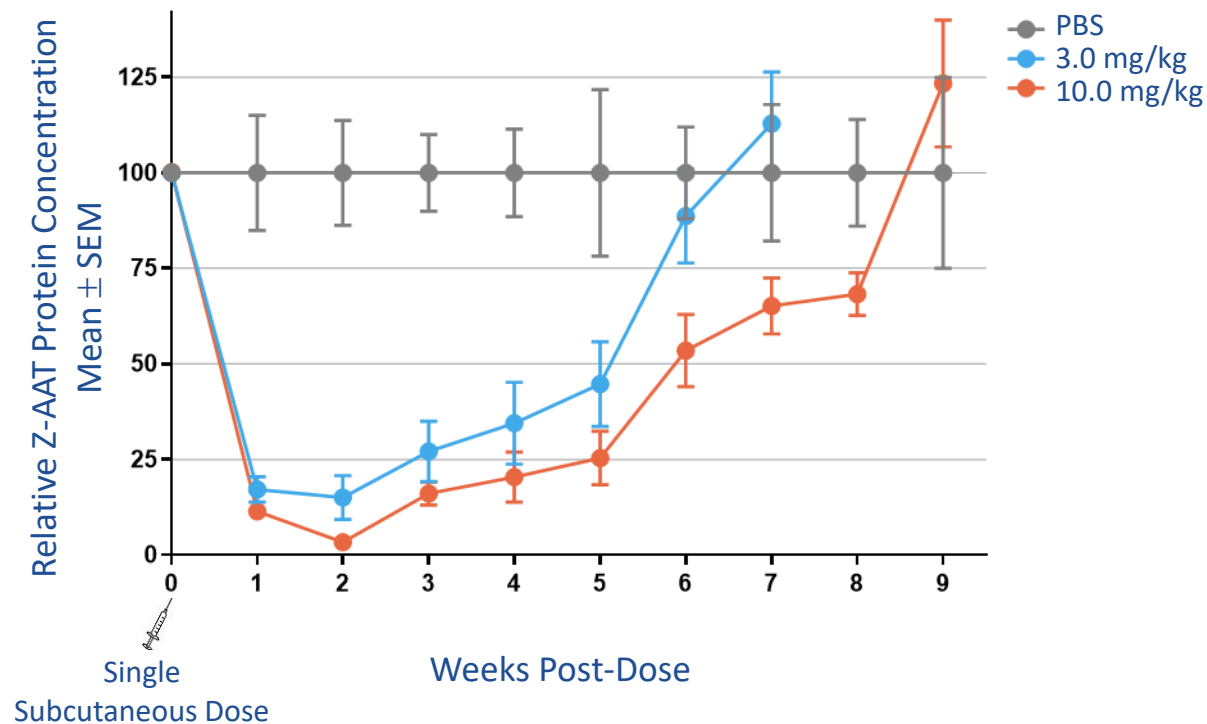


Janciauskiene et al. 2013: Acute Phase Proteins

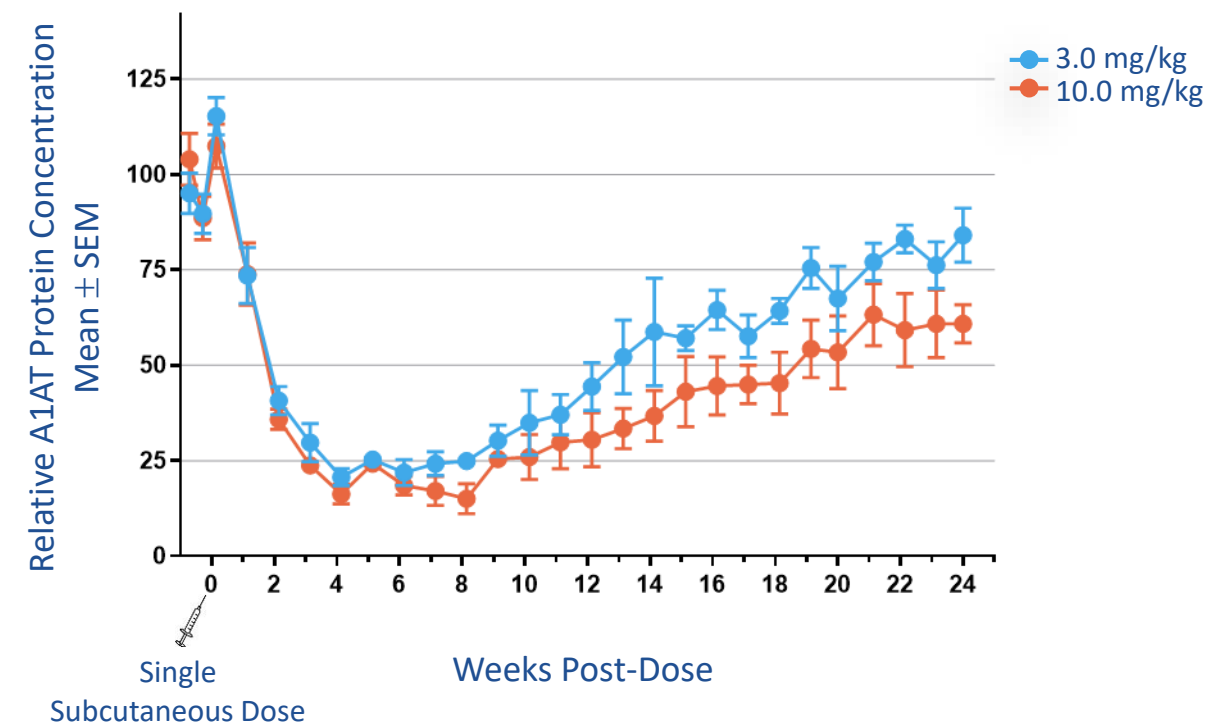
Activity of DCR-A1AT in Mice and Non-Human Primates

Potency and duration of action

Z-AAT Protein Knockdown in PiZ Mice



A1AT Protein Knockdown in Monkeys



- Sustained, dose-dependent knockdown of A1AT protein in both mice and monkeys

DCR-A1AT Clinical Program for Proof of Concept

Part of larger clinical plan to achieve a rapid path to approval

Two-part study: Single-ascending-dose (SAD) in healthy volunteers (HVs) and multiple-ascending-dose (MAD) in patients with A1AT deficiency-associated liver disease

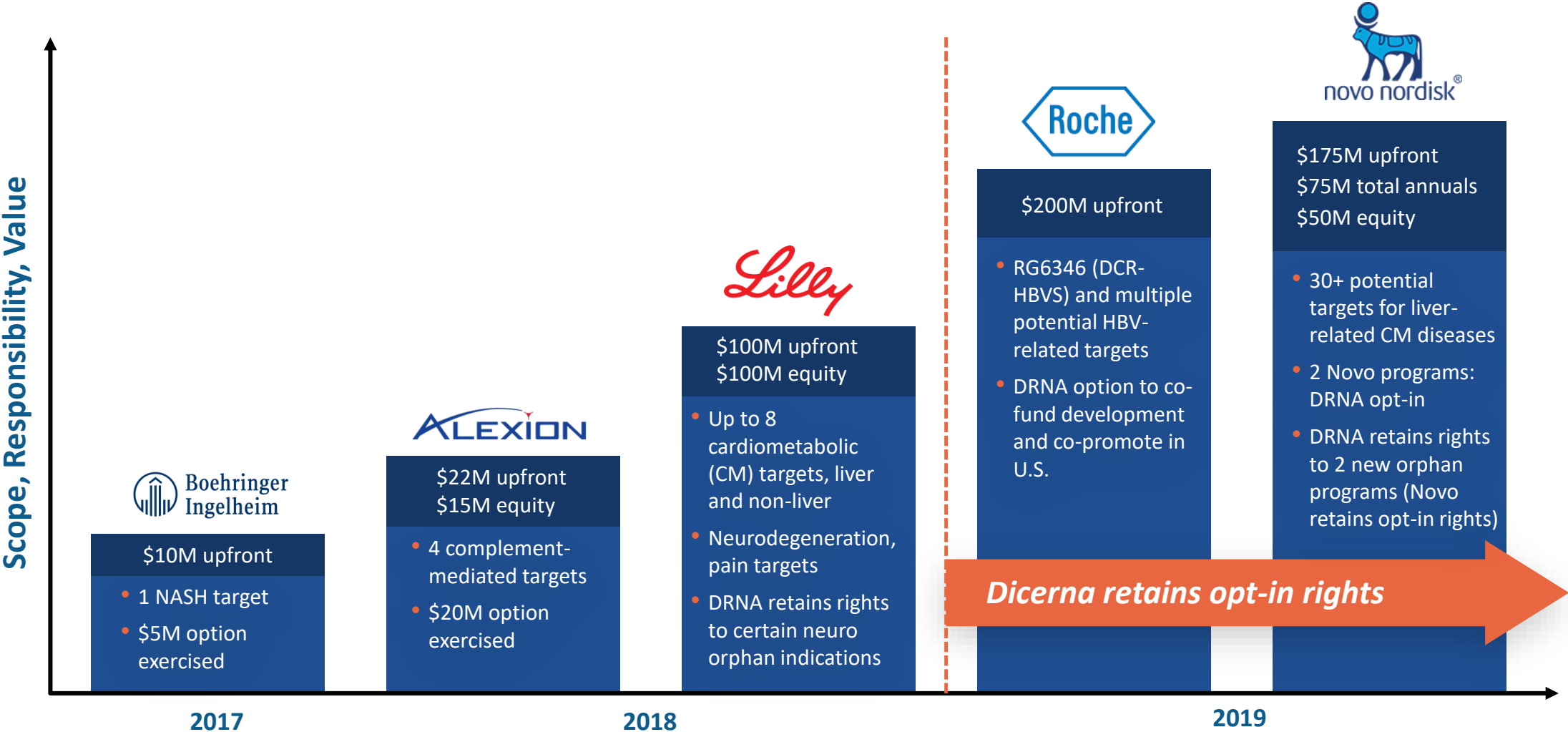
- **HVs:** SAD study, placebo-controlled, up to 36 participants
 - 5 dose cohorts: 0.1, 1.0, 3.0, 6.0, 12.0 mg/kg, with a potential additional cohort
 - Overlapping cohorts
 - PK/PD data will be used to determine MAD regimen
- **Patients with A1AT deficiency-associated liver disease:** MAD study, up to 24 patients
 - 2-3 dose cohorts: dose levels dependent on PK/PD data from HVs
 - 2-4 doses to be administered within a 13-week period
- Currently dosing healthy volunteers
- Expect to dose first patient in 2H 2020

We believe the Phase 1/2 program will enable a pivotal trial without additional studies

Corporate Collaborations

Enhancing Value Through GalXC Collaboration Strategy

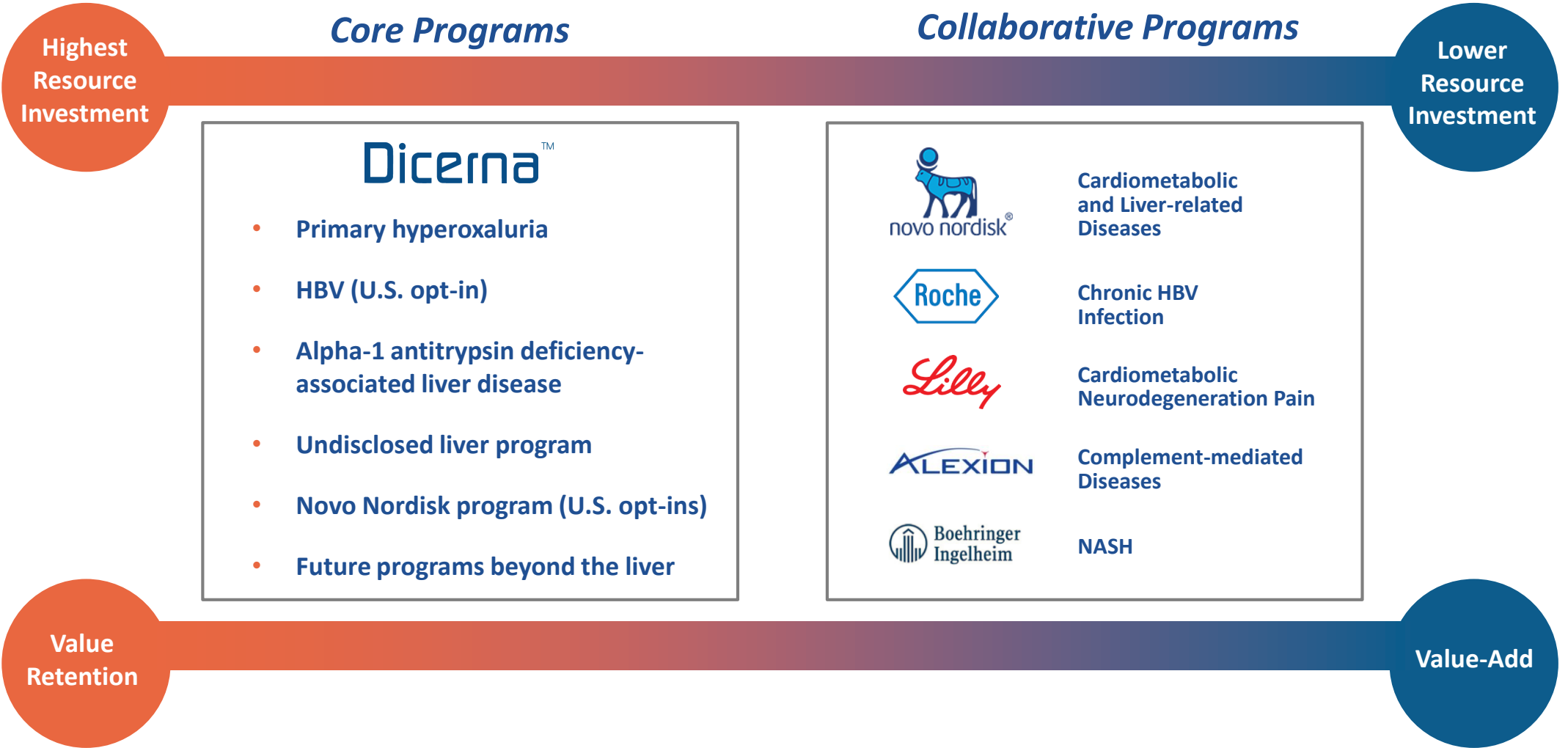
Successive collaborations increasing in scope and value



Summary

Elements of Portfolio Strategy

We seek to generate value across the full spectrum of GalXC clinical applications



Strong Cash Position Provides Runway Through Commercialization

Additional cash upside expected from collaboration milestones and potential royalties on product sales

- Company well capitalized with \$348.9 million in cash (and short-term investments) as of December 31, 2019
- Pro forma cash of more than \$750 million as of January 1, 2020, which also includes:
 - \$200 million upfront payment from Roche, received on January 7, 2020
 - \$175 million upfront payment from Novo Nordisk, received on January 21, 2020
 - \$39 million in net proceeds from sale of common stock to single institutional investor on February 6, 2020
- We expect our current cash and estimated future proceeds from existing collaborations will fund operations into 2023*

**Expectations provided by Dicerna in its Form 10-K dated February 27, 2020 and are current as of this date. Dicerna disclaims any obligation to update or reaffirm expectations and only provides guidance in a Regulation FD compliant manner.*

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DicernaTM

