**Rare disease therapeutics** 

**Program Pack** 

## Crigler-Najjar Syndrome Type 1 (CN-1) (mRNA-3351)

mRNA-3351 encodes for the human UGTA1A1: Designed to restore the missing or dysfunctional proteins that causes CN-1



## Crigler-Najjar Syndrome Type 1 (CN-1) overview

#### Crigler-Najjar syndrome is a severe condition

characterized by high levels of a toxic substance called bilirubin in the blood (hyperbilirubinemia)

- Caused by the mutations in the UGT1A1 gene in which bilirubin, a substance made by the liver, cannot be broken down
- Without this enzyme, bilirubin can build up in the body and lead to jaundice and damage to the brain, muscles and nerves. The symptoms become apparent shortly after birth and can be life-threatening

#### It is estimated that there **are only approximately 70-100 known cases of CN-1 in the world**

 Current standard of care treatments rely on phototherapy treatments of up to 12 hours a day throughout life. The only definitive treatment is liver transplant that is associated with its own set of side effects and risk of death

#### **CN-1** Sequelae

- Kernicterus (potentially life-threatening neurological condition)
- Lethargy
- Moro reflex
- Muscle spasms
- Spasticity (uncontrolled involuntary muscle movements)

# Moderna-Institute For Life Changing Medicines (ILCM) collaboration

The goal of the collaboration is to make an mRNA therapy for the **treatment of CN-1 available at no cost to patients**  Under the terms of the agreement, Moderna will license mRNA-3351 to ILCM with no upfront fees, and without any downstream payments

ILCM will be responsible for the clinical development of mRNA-3351



https://www.lifechangingmedicines.org/

### Forward-looking statement

This presentation contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including regarding the Company's collaboration with the Institute for Life Changing Medicines on the development of an mRNA therapeutic (mRNA-3351) for Crigler-Najjar Syndrome Type 1 and expected market opportunity. In some cases, forward-looking statements can be identified by terminology such as "may," "should," "expects," "intends," "plans," "aims," "anticipates," "believes," "estimates," "predicts," "potential," "continue," or the negative of these terms or other comparable terminology, although not all forward -looking statements contain these words. The forward-looking statements in this presentation are neither promises nor guarantees, and you should not place undue reliance on these forward-looking statements because they involve known and unknown risks, uncertainties and other factors, many of which are beyond Moderna's control and which could cause actual results to differ materially from those expressed or implied by these forward-looking statements. These risks, uncertainties and other factors include those described in Moderna's most recent Annual Report on Form 10-K filed with the U.S. Securities and Exchange Commission (SEC) and in subsequent filings made by Moderna with SEC, which are available on the SEC's website at www.sec.gov. Except as required by law, Moderna disclaims any intention or responsibility for updating or revising any forward-looking statements in this presentation, future developments or otherwise. These forward-looking statements are based on Moderna's current expectations and speak only as of the date referenced on the first page.