

Moderna's therapeutics: Crigler-Najjar Syndrome Type 1 (CN-1)

Last program update: September 9, 2021

| Modality | Program | ID # | Preclinical development | Phase 1 | Phase 2 | Phase 3 | Commercial | Moderna rights |
|--|---|-----------|--|---------|---------|---------|------------|---|
|  Systemic secreted & cell surface therapeutics | Antibody against Chikungunya virus | mRNA-1944 | [Progress bar: Preclinical development to Phase 1] | | | | | Worldwide DARPA funded |
| | IL-2 Autoimmune disorders | mRNA-6231 | [Progress bar: Preclinical development to Phase 1] | | | | | Worldwide |
| | Relaxin Heart failure | mRNA-0184 | [Progress bar: Preclinical development] | | | | | Worldwide |
| | PD-L1 Autoimmune hepatitis | mRNA-6981 | [Progress bar: Preclinical development] | | | | | Worldwide |
|  Cancer vaccines | Personalized cancer vaccine (PCV) | mRNA-4157 | [Progress bar: Preclinical development to Phase 2] | | | | | 50-50 global profit sharing with Merck |
| | KRAS vaccine | mRNA-5671 | [Progress bar: Preclinical development to Phase 1] | | | | | 50-50 global profit sharing with Merck |
|  Intratumoral Immunology | OX40L/IL-23/IL-36γ (Triplet) Solid tumors/Lymphoma | mRNA-2752 | [Progress bar: Preclinical development to Phase 1] | | | | | Worldwide |
| | IL-12 Solid tumors | MEDI1191 | [Progress bar: Preclinical development to Phase 1] | | | | | 50-50 U.S. profit sharing; AZ to pay royalties on ex-U.S. sales |
|  Localized Regenerative Therapeutics | VEGF-A Myocardial ischemia | AZD8601 | [Progress bar: Preclinical development to Phase 2] | | | | | AZ to pay milestones and royalties |
| | Propionic Acidemia (PA) | mRNA-3927 | [Progress bar: Preclinical development to Phase 1] | | | | | Worldwide |
| | Methylmalonic Acidemia (MMA) | mRNA-3705 | [Progress bar: Preclinical development to Phase 1] | | | | | Worldwide |
|  Systemic Intracellular Therapeutics | Glycogen Storage Disease Type 1a (GSD1a) | mRNA-3745 | [Progress bar: Preclinical development to Phase 1, labeled 'Open IND'] | | | | | Worldwide |
| | Phenylketonuria (PKU) | mRNA-3283 | [Progress bar: Preclinical development] | | | | | Worldwide |
| | Crigler-Najjar Syndrome Type 1 (CN-1) | mRNA-3351 | [Progress bar: Preclinical development] | | | | | Provided to ILCM free of charge |

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

- On September 7, Moderna **announced a new collaboration to develop a new mRNA therapeutic (mRNA-3351) for Crigler-Najjar Syndrome Type 1 (CN-1), an ultra-rare disease**
 - It is estimated that there **are only approximately 70-100 known cases of CN-1 in the world**
 - CN-1 is an ultra-rare genetically inherited disorder caused by the mutation in the UGT1A1 gene in which bilirubin, a substance made by the liver cannot be broken down. The syndrome occurs when the protein that normally converts bilirubin into a form that can be easily removed from the body does not work properly
 - 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
 - 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
- 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**
- ILCM plans to **initiate clinical studies of mRNA-3351 in 2022**

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