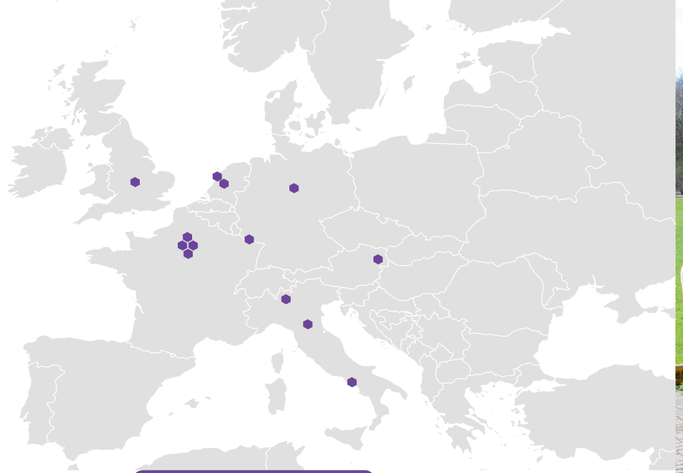

CureCN Consortium

Genethon **France**
Assistance Publique – Hôpitaux de Paris **France**
Amsterdam UMC, Universitair Medische Centra **Netherlands**
Medizinische Hochschule Hannover **Germany**
Azienda Ospedaliera Papa Giovanni XXIII **Italy**
Università Degli Studi di Napoli Federico II. **Italy**
Genosafe SAS **France**
Crigler-Najjar Patient Organisations **France, Italy, Netherlands**
MC Toxicology Consulting GmbH **Austria**
University of Leicester **United Kingdom**
Eurice – European Research and Project Office GmbH **Germany**

CureCN

Developing a curative gene therapy for Crigler-Najjar syndrome

www.curecn.eu

Coordination
Dr Federico Mingozzi
Genethon

fmingozzi@genethon.fr

Management
Melanie Haupenthal
Eurice – European Research and Project Office GmbH
m.haupenthal@eurice.eu

Patient Organisations
AFCN / CiamiOnlus / DeNajjarStichting
crigler-najjar@9online.fr
segreteria@ciami.it
secretariaat@najjar.nl

The CureCN project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement No 755225.

1.1.2018–31.12.2022

6.25 M €

11 Partners from 6 Countries

CureCN

Adeno-Associated Virus Vector-Mediated Liver Gene Therapy for Crigler-Najjar Syndrome

About Crigler-Najjar

Crigler-Najjar (CN) is an extremely rare, life-threatening liver disease that occurs directly after birth. The disorder is caused by the deficiency of a liver-specific enzyme resulting in the accumulation of toxic unconjugated bilirubin in all body tissues. Untreated, CN causes irreversible neurological damage in the brain and eventually leads to death. The only curative treatment currently available is a liver transplant that implies a high risk of complications. Currently, patients are treated with phototherapy – a treatment with blue light – which reduces symptoms but does not eliminate the risk of life-threatening spikes of bilirubin.

"Adeno-Associated Virus (AAV) vector-mediated gene therapy has shown promising results in preclinical trials and we strongly believe that we will be able to provide a healing treatment for very young CN patients, as well as for older CN patients with a pre-existing immunity to AAV. If validated in our clinical trial, AAV-mediated gene therapy could not just cure CN but also heal other inherited liver diseases."

Dr Federico Mingozzi
CureCN coordinator

CureCN Mission

The overall mission of the European research project CureCN is to develop a gene therapy approach for Crigler-Najjar. In order to achieve this goal, the project includes a clinical trial to prove the safety and efficacy of an innovative gene therapy based on liver gene transfer with an adeno-associated virus (AAV). Additionally, CureCN aims at addressing important limitations of the AAV technology such as vector readministration and pre-existing humoral immunity against the vector capsid.

Watch our video

Involving CN patients

As a patient-driven initiative, the CureCN consortium comprises all active Crigler-Najjar patient organisations in Europe. They are a direct link to patients and their families enabling a vivid exchange of information to ensure that those affected are directly involved in the research process. Furthermore, CureCN aims at establishing the first global Crigler-Najjar patient registry. The compiled data will be analysed to allow conclusions on the natural course of the disease and to compare existing treatment modalities and strategies.