

# World registry of Crigler-Najjar patients

## AIM:

In most countries treatment and monitoring of patients suffering from Crigler-Najjar syndrome is at local hospitals or single clinician or general practitioner. As a result, the data on the natural course of the disease and current treatments are scattered and not easy to retrieve.

The goal of this registry of CN patients from all over the world is to make these data accessible to all patients and treating clinicians to learn more on the natural course of this severe disorder and to help MDs, patients and families to find the best treatment.

## THE REGISTRY:

The registry has been setup according to the recommendations by the European Platform for Rare Disease Registries, the European Union Committee of Experts on Rare Diseases and the European Medicines Agency. A secure online fully Good Clinical Practice compliant platform is used that allows easy access and data entry and ensures optimal protection of privacy of each patient.

## GOVERNANCE:

To decide on access to the data in the registry, a governance board has been installed. The board is formed by clinicians treating CN patients, complemented with an ethical and legal advisor, and representatives of the CN patient associations.

The board will ensure i) that robust operational procedures and processes are in place, and easy to access for everyone involved in data collection, ii) transparency in operations, iii) decision making, iv) reporting of results via publications and on the website, and v) access for third parties.

## PARTICIPANTS:

To establish this world registry, centers and clinicians treating CN patients known to us have been contacted directly, or via the CureCN network. More than 14 centers and or clinicians from different countries are already participating (see map). Data from more centers and countries is needed for world coverage.

## TO PARTICIPATE:

If you are a patient and would like to participate in this initiative please ask the doctor treating you to contact us by email ([p.j.bosma@amc.uva.nl](mailto:p.j.bosma@amc.uva.nl)). If you are a clinical treating CN patients please contact us by mail so we can help you with the details on how to participate.

## TO REQUEST INFORMATION:

Requests for access to registered data should be done by email to [p.j.bosma@amc.uva.nl](mailto:p.j.bosma@amc.uva.nl)

**Countries with centers participating in the World Registry of Crigler-Najjar patients**

