Press Release

EU Research Project CureCN Releases Video Clip

July 2018 – After its successful start at the beginning of 2018, the EU-funded research project CureCN project has published a video on its aims and activities. In the clip, different scientists and the patient organisations explain the importance of the project that aims at finding a curative gene therapy for the so far incurable Crigler-Najjar (CN) syndrome.

Watch the video

Crigler-Najjar is a life-threatening liver disease which affects one in a million individuals at birth. Caused by the deficiency of a liver-specific substance, toxic unconjugated bilirubin is accumulated in serum and body tissue leading to irreversible neurological damage in the brain. Currently, there is no curative treatment available apart from a liver transplant. So far, the treatment with phototherapy – a treatment with blue light – reduces the bilirubin levels to keep the patients alive. But the treatment needs a lot of time and does not heal the disorder.

The European research project CureCN now aims at developing a curative gene therapy for CN. The multidisciplinary consortium has the goal to validate the innovative gene therapy based on liver gene transfer with an adeno-associated virus (AAV) in a clinical trial.

"We are very proud and happy having started the programme and are very grateful for the support of the scientists and the European Union," states Marylène Beinat, coordinator of the three Crigler-Najjar patients associations from France, Italy and the Netherlands implied in CureCN. "With the AAV gene therapy that is currently being tested in the clinical trial, we hope to provide a curative treatment for patients who until now depend upon the treatment with phototherapy which constitutes a severe burden for them and their families."

The CureCN consortium brings together 11 partners from six European countries who will be working on the project for five years.

Project facts

Project acronym:	**CureCN**
Duration:	01.01.2018-31.12.2022
Budget:	€ 6.25 million
Coordination:	Genethon (France), Dr Federico Mingozzi

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