CureCN
Project ID: 755225
Funded under: H2020-EU.3.1.3. - Treating and managing
disease

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

From 2018-01-01 to 2022-12-31, ongoing project | CureCN Website

Project details

<table>
<thead>
<tr>
<th>Total cost:</th>
<th>Topic(s):</th>
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<tr>
<td>EUR 6 249 103,75</td>
<td>SC1-PM-08-2017 - New therapies for rare diseases</td>
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<th>EU contribution:</th>
<th>Call for proposal:</th>
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<td>EUR 6 249 103,75</td>
<td>H2020-SC1-2017-Two-Stage-RTD</td>
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<th>Coordinated in:</th>
<th>Funding scheme:</th>
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<tr>
<td>France</td>
<td>RIA - Research and Innovation action</td>
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Objective

Crigler-Najjar syndrome (CN) is a rare recessive disorder caused by mutations in the uridine diphosphate glucuronosyltransferase 1A1 (UGT1A1) gene. CN is a life-threatening disease with no cure which constitutes a severe burden for the patients, their families, and the society. CureCN has the objective of developing a curative gene therapy for CN syndrome based on liver gene transfer with and adeno-associated virus (AAV) vector expressing the UGT1A1 transgene. Additional goals of CureCN are to develop strategies to allow for vector re-administration and to address the issue of pre-existing anti-AAV neutralizing antibodies (NAbs), which prevent large proportion of seropositive patients from receiving AAV mediated gene therapy. Proof-of-concept studies of AAV8-UGT1A1 gene transfer provide a strong rationale for the safety and efficacy of gene therapy for CN. CureCN proposes to carry out an open-label, multicenter clinical trial of AAV8-UGT1A1 gene transfer to prove the safety and efficacy of the therapy in severe CN patients, and file for marketing authorization in Europe at the end of the study. CureCN will also produce enabling data for the clinical translation of a groundbreaking immunomodulatory strategy to allow for vector administration. Additionally, a technology for the selective removal of anti-AAV NAbs from the bloodstream of seropositive patients will be developed. The goal of these studies is to ultimately allow all CN patients to access AAV8-UGT1A1 gene therapy. CureCN is a patient-driven initiative that gathers top clinicians and scientists; it also includes small medium enterprises in its partners, to foster economic growth and valorization of intellectual property. CureCN sets itself in the ambitious goal set by the IRDiRC by 2020 by developing a curative treatment for CN syndrome. Importantly, it validates technologies that will broaden the scope of gene therapy, thus will have an impact on the development of treatments for several other rare diseases.

Related information

News

- EU Research Project CureCN Releases Video Clip
- New Research Project CureCN Aims to Cure the Ultra-Rare Crigler-Najjar Syndrome
### Coordinator

<table>
<thead>
<tr>
<th>Organisation</th>
<th>Address</th>
<th>City</th>
<th>Country</th>
<th>EU contribution:</th>
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<tbody>
<tr>
<td>ASSOCIATION GENETHON</td>
<td>RUE DE L INTERNATIONALE 1 BIS 91002 EVRY</td>
<td>France</td>
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</tr>
<tr>
<td>ASSISTANCE PUBLIQUE - HOPITAUX DE PARIS</td>
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<td>France</td>
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<td>MEDIZINISCHE HOCHSCHULE HANNOVER</td>
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<td>Germany</td>
<td>EUR 149 000</td>
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<tr>
<td>AZIENDA OSPEDALIERA PAPA GIOVANNI XXIII</td>
<td>PIAZZA OMS 1 24127 BERGAMO</td>
<td>Italy</td>
<td>EUR 147 500</td>
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### Activity type: Higher or Secondary Education Establishments

Contact the organisation

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### Participants

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<th>City</th>
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<tr>
<td>Academisch Medisch Centrum bij de Universiteit van Amsterdam</td>
<td>MEIBERGDOREEF 9 1105AZ AMSTERDAM</td>
<td>Netherlands</td>
<td>EUR 526 718,75</td>
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Contact the organisation

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Contact the organisation
UNIVERSITA DEGLI STUDI DI NAPOLI FEDERICO II
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**Activity type:** Higher or Secondary Education Establishments

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EU contribution: EUR 147 125

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GENOSAFE SAS
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**Activity type:** Other

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EU contribution: EUR 207 017,50

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ASSOCIATION FRANCAISE DE CRIGLER NAJJAR
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**Activity type:** Higher or Secondary Education Establishments

Contact the organisation

EU contribution: EUR 92 000

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MC TOXICOLOGY CONSULTING GMBH
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**Activity type:** Other

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EU contribution: EUR 156 872,50

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United Kingdom
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**Activity type:** Higher or Secondary Education Establishments

Contact the organisation

EU contribution: EUR 396 182,50