Personalised Treatment For Cystic Fibrosis Patients With Ultra-rare CFTR Mutations (and beyond)

From 2018-01-01 to 2022-12-31, ongoing project

Project details

<table>
<thead>
<tr>
<th>Total cost:</th>
<th>Topic(s):</th>
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<tr>
<td>EUR 8 753 615</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 6 701 365</td>
<td>H2020-SC1-2017-Two-Stage-RTD</td>
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<tr>
<th>Coordinated in:</th>
<th>Funding scheme:</th>
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<tbody>
<tr>
<td>Netherlands</td>
<td>RIA - Research and Innovation action</td>
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Objective

In our HIT-CF project, we aim to bring personalised disease modifying therapies to cystic fibrosis (CF) patients with ultra-rare CFTR mutations, who could otherwise never get access to such treatment. Once we have proven our unique concept, the CF community can easily extend our state-of-the-art methodology to all CF patients such that HIT-CF will impact the entire CF field.

We will achieve our goals by means of a randomised, double-blind, placebo-controlled, repeated-crossover, three-armed platform trial with prospectively defined meta-analysis to evaluate efficacy at group and individual level. HIT-CF is designed to enable access to the most relevant global drug products, and each trial arm will test a drug product candidate (a single compound or a compound combination) from one of our pharmaceutical consortium partners. The patients will be assigned to the specific trial based on the effect of the drug product candidates on cultured intestinal miniature organs (termed organoids) grown from rectal biopsies, instead of based on typical genotyping only.

In parallel with this H2020 project, our pharmaceutical partners will obtain market approval of their drug product candidates for common (F508del or gating) mutations in the CFTR gene. Ultimately, our project will enable ‘managed’ off-label access to these therapies towards patient groups or individuals who show response to the therapy in a prospective intestinal organoid test.

One of the major impacts of this project will be the innovative methodologies to acquire reimbursement for current and future off-label treatments of people with CFTR mutations. This will represent a real paradigm shift in CF treatment as it implements a new type of personalized medicine paradigm based on organoids, by shifting therapeutic trials from patients to the laboratory.
Coordinator

UNIVERSITAIR MEDISCH CENTRUM UTRECHT
3584 CX UTRECHT
Netherlands

EU contribution: EUR 1,434,417,50

Activity type: Higher or Secondary Education Establishments

See on map

Participants

EUROPEAN CYSTIC FIBROSIS SOCIETY
KASTANIEPARKEN 7
7470 KARUP
Denmark

EU contribution: EUR 334,740

Activity type: Other

See on map

PATERGRUS BVBA
SPARHOEKDREEF 57
9880 AALTER
Belgium

EU contribution: EUR 302,075

Activity type: Private for-profit entities (excluding Higher or Secondary Education Establishments)

See on map

STICHTING HUBRECHT ORGANOID TECHNOLOGY
YALELAAN 62
3584 CM UTRECHT
Netherlands

EU contribution: EUR 1,088,050

Activity type: Other

See on map

CYSTIC FIBROSIS EUROPE EV
IN DEN DAUEN 6
53117 BONN
Germany

EU contribution: EUR 141,312,50

Activity type: Other

See on map

Contact the organisation
<table>
<thead>
<tr>
<th>Organisation</th>
<th>Country</th>
<th>Address</th>
<th>EU contribution</th>
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<td>FCIENCIAS.ID - ASSOCIACAO PARA A INVESTIGACAO E DESENVOLVIMENTO DE CIENCIAS</td>
<td>Portugal</td>
<td>CAMPO GRANDE, EDIFICIO C1, PISO 3</td>
<td>EUR 257,367.50</td>
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<td>KATHOLIEKE UNIVERSITEIT LEUVEN</td>
<td>Belgium</td>
<td>Oude Markt 13</td>
<td>EUR 386 250</td>
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<td>FLATLEY DISCOVERY LAB LLC</td>
<td>United States</td>
<td>35 BRAINTREE HILL OFFICE PARK, SUITE 200</td>
<td>EUR 27 000</td>
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<td>JULIUS CLINICAL RESEARCH BV</td>
<td>Netherlands</td>
<td>BROEDERPLEIN 41-43</td>
<td>EUR 2 703 152.50</td>
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<td>GALAPAGOS</td>
<td>Belgium</td>
<td>GENERAAL DE WITTELAAN L11 A3</td>
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**Activity type:**
- Research Organisations
- Higher or Secondary Education Establishments
- Other
- Private for-profit entities (excluding Higher or Secondary Education Establishments)

**Last updated on** 2018-01-23  
**Retrieved on** 2018-10-09

**Permalink:** [https://cordis.europa.eu/project/rcn/212684_en.html](https://cordis.europa.eu/project/rcn/212684_en.html)