EU H2020 grant for unique HIT-CYSTIC FIBROSIS project

The EU has granted 6.7 M€ from the Horizon 2020 research program to a broad collaborative group of researchers, doctors, pharmaceutical companies and patient representatives.

The aim is to develop '**personalized treatments'** for Cystic Fibrosis (CF) patients with uncommon genetic profiles throughout Europe. These patients with extremely rare gene mutations can otherwise never get access to possibly life-saving treatment. Once this concept has been proven, the methodology can extend to all patients with CF and other rare genetic diseases, so that HIT-CF will impact the entire field of orphan diseases.

Approach

Adults with CF and a rare CF genotype reported to the European Cystic Fibrosis Society Patient Registry (ECFSPR), will be approached by their doctors and encouraged by the patient organizations (united in CF Europe) to participate in the studies within the scope of this project.

Firstly, stem cells from the patients' own intestine will be grown into "mini guts" (termed organoids) by the foundation Hubrecht Organoid Technology and distributed to laboratories in the Netherlands (Utrecht), Belgium (Leuven) and Portugal (Lisboa). This requires taking a few millimeters of tissue via a tiny biopsy, a painless procedure.
CF drug candidates from Galapagos, Flatley Discovery Lab and possibly other companies will be tested in these organoids to check for their efficacy in the tissue of the individual patient.

Secondly, based on the effects in organoids, selected patients will be invited to participate in drug trials (organized within one of 43 CF-centers of the ECFS Clinical Trial Network and assisted by Julius Clinical) to evaluate the real life benefit of these compounds for these patients.
HIT-CF aims to enable access to the most relevant drugs in development, and each trial group will test a drug candidate from one of the pharmaceutical consortium partners.

In parallel with this project, the pharmaceutical partners will work towards market approval of their drug candidates for the larger group of patients with more common genetic profiles.

The ultimate goal of this project is to develop a path for access to therapies for patient groups or individuals who show positive response to the therapy in an organoid test. One of the major impacts of this project will be the innovative methodologies used to acquire approval (and reimbursement) for current and future "off-label" treatments of people with CF. This will represent a new era in CF treatment as it implements a new type of personalized medicine based on organoids, by shifting therapeutic trials from patients to the laboratory.

See this short movie

The Website www.HITCF.org will be launched soon for more information

About CF

Cystic Fibrosis (CF) is the most common life-threatening inherited disease in Europe. Sticky mucus blocks the respiratory and digestive systems. CF is yet an incurable disease. But early diagnosis, regular follow-up by a multidisciplinary team in a specialized CF clinic, and correct, timely treatment of symptoms can prolong and save lives and improve the quality of life.
A new era of drugs, targeting the basic defect in the cells of people with CF, is arising and will have a big impact on the lives of people with CF if they get access to these drugs.

About the consortium partners:

University Medical Centre Utrecht (The Netherlands, coordinating center)
Care, research and education are the three mainstays of UMC Utrecht. These mainstays are inextricably intertwined in their ongoing efforts to improve people's health. Leading scientific research, groundbreaking innovation and collaboration with patients and other interested parties form the basis of their first-rate healthcare. UMC Utrecht aims for responsible innovation and is eager to put improvements into practice.
The CF Centre Utrecht is taking care of 460 patients with CF

***Cystic Fibrosis Europe* (CFE)**
CFE is the federation of national CF Associations in Europe. CFE represents persons with CF and their families in Europe. Currently, national CF Associations from 39 European countries are member of CF Europe. CFE works in close collaboration with other international organizations and is an active partner in several European projects.

European Cystic Fibrosis Society (ECFS)
ECFS is an international community of scientific and clinical professionals committed to improving survival and quality of life for people with CF by promoting high quality research, education and care.
The Clinical Trial Network (ECFS-CTN) provides access to 43 CF centers experienced in clinical research and spread over 15 different countries throughout Europe. The aim of this network is to intensify clinical research in CF and to bring new medicines to the patients as quickly as possible.

University Hospital Leuven (Belgium)
The University Hospital of Leuven, embedded in the Catholic University of Leuven (KU Leuven), provides high-quality, comprehensive health care, including specialized tertiary care based on scientific expertise and social responsibility. In doing so it strives towards optimum accessibility and respect for all patients. Working at all times for better, safer patient care is the essence of the hospital's philosophy. The hospital has developed a leading CF reference center taking care of 360 patients with CF.

BioISI – Biosystems & Integrative Sciences Institute, Faculty of Sciences, University of Lisboa (Portugal)
The mission of BioISI is to pursue research of excellence in biological systems and Integrative sciences and that of the Faculty of Sciences of the University of Lisboa is to expand the limits of science and technology, transfer scientific knowledge into society, and promote research-based student education.

Flatley Discovery Lab (FDL)
Flatley Discovery Lab has a focus – helping those who live with Cystic Fibrosis. They have friends, family and staff who live with CF and they are reminded daily how this affects quality of life. Their singular mission "is to identify and develop superior therapeutics to treat CF and delivery these to patients in the most efficient manner possible."

Galapagos

Galapagos is a clinical-stage biotechnology company specialized in the discovery and development of small molecule medicines with novel modes of action. Their pipeline comprises Phase 3, 2, 1, pre-clinical studies and discovery small-molecule and antibody programs in Cystic Fibrosis, inflammation, and other indications.

Hubrecht Organoid Technology (HUB)
Hub is a not-for-profit organization founded by the Hubrecht Institute, KNAW and University Medical Center Utrecht, The Netherlands. The HUB is founded on the pioneering work of Prof. Dr. Hans Clevers who discovered methods to grow stem cell-derived human epithelial 'mini-organs' (organoids) from tissues of patients with various diseases including cancer and cystic fibrosis, representing an in vitro platform for preclinical drug discovery and validation and a tool for precision medicine.

Julius Clinical
Julius Clinical was founded as a spin-off from the University Medical Center (UMC) Utrecht, the Netherlands. Julius Clinical manages global clinical drug trials that will have a major impact on medicine and make a real difference to people's lives around the world. The unique combination of scientific leadership and operational excellence ensures that the data obtained from the trials is of the highest quality.

Patergrus - Biotechsubsidy
Biotechsubsidy is the commercial name of the legal entity Patergrus. They are founded in 2007 by Dr. Marc Van de Craen and located in Belgium. Patergrus combines business and marketing services with grant management services. By mains of their solid scientific background and in-depth knowledge of the biotech industry, they go far beyond their peers in these areas.

Program leader: Prof C.K. van der Ent, UMC Utrecht (NL).

Contact details for more information:
Arjen Zeegers
press@umcutrecht.nl
+31 (0)648211804