Novel therapeutic approaches for the treatment of cystic fibrosis based on small molecule transmembrane anion transporters

This project will develop an innovative therapeutic approach for the treatment of Cystic Fibrosis (CF). This condition originates from the defective function of the CFTR protein, a chloride and bicarbonate permeable transmembrane channel. This project will evaluate small molecules capable of facilitating the transmembrane transport of anions such as chloride and bicarbonate and will thus enable CF treatment by replacing the missing CFTR anion permeation activity. This represents an unexplored path in the treatment of CF and a paradigm shift with respect to current strategies searching for a cure for CF. Instead of focusing on the development of mutation-specific treatments, we plan to develop a therapy applicable to CF patients, regardless of the type of mutation they harbor. Thus, this therapeutic approach overcomes the limitation of current mutation-specific treatments and is applicable to CF patients in general.

To achieve this goal we have set up a comprehensive program to validate a research concept and complete the preclinical development of a new lead compound, making it ready for early clinical development. A multidisciplinary team of qualified researchers have been assembled to bring to conclusion a truly translational project from the synthesis of new compounds to validation on animal models.

To conclude, Cystic Fibrosis affects more people than any other rare disease. Therefore, it could be said, at least in quantitative terms, that CF qualifies as the main target of the topic. This project aims to complete the preclinical development of novel, innovative drugs based on a radically new concept in Cystic Fibrosis therapies. This result fully addresses the expected impact set out in the work programme of advancing the development of new therapeutic options for patients living with rare diseases as well as contributing to reach the IRDiRC objective to deliver 200 new therapies for rare diseases by 2020.

Informazioni correlate

Risultato in breve
A mutation-agnostic therapy for cystic fibrosis

Sintesi delle relazioni
Periodic Reporting for period 1 - TAT-CF (Novel therapeutic approaches for the treatment of cystic fibrosis based on small molecule transmembrane anion transporters)
Coordinatore

UNIVERSIDAD DE BURGOS
HOSPITAL DEL REY
09001 BURGOS
Spain
See on map

Activity type: Higher or Secondary Education Establishments
Contact the organisation

Contributo UE: EUR 665 563,75

Partecipanti

STEINBEIS INNOVATION GGMBH
WILLI BLEICHER STRASSE 19
70174 STUTTGART
Germany
See on map

Activity type: Higher or Secondary Education Establishments
Contact the organisation

Contributo UE: EUR 523 953,75

ISTITUTO GIANNINA GASLINI
VIA GEROLAMO GASLINI 5
16147 GENOVA
Italy
See on map

Activity type: Public bodies (excluding Research Organisations and Secondary or Higher Education Establishments)
Contact the organisation

Contributo UE: EUR 390 002,50

CONSIGLIO NAZIONALE DELLE RICERCHE
PIAZZALE ALDO MORO 7
00185 ROMA
Italy
See on map

Activity type: Higher or Secondary Education Establishments
Contact the organisation

Contributo UE: EUR 610 273,75

BIONEER A/S
KOGLE ALLE 2
2970 HOERSHOLM
Denmark
See on map

Activity type: Research Organisations
Contact the organisation

Contributo UE: EUR 557 375
Contributo UE: EUR 591 875

Contributo UE: EUR 732 875

Contributo UE: EUR 519 368,75

Activity type: Other
Contact the organisation

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

Activity type: Higher or Secondary Education Establishments
Contact the organisation

Ultimo aggiornamento 2017-06-10
Recuperato il 2019-06-09

© European Union, 2019