Clinical trial of gene therapy for MPS VI - a severe lysosomal storage disorder

Fact Sheet

Project Information

MEUSIX
Grant agreement ID: 304999
Status
Closed project
Start date 1 December 2012
End date 30 November 2017

Funded under
FP7-HEALTH
Overall budget € 7 876 955
EU contribution € 5 995 041

Coordinated by
FONDAZIONE TELETHON
Italy

Objective

"Mucopolysaccharidosis VI (MPS VI, or Maroteaux-Lamy syndrome; OMIM #253200) is a rare lysosomal storage disease caused by deficient activity of arylsulfatase B (ARSB). MPS VI is characterized by growth retardation, corneal clouding, cardiac valve disease, organomegaly, skeletal dysplasia, without central nervous system involvement. Thus, systemic therapies targeting peripheral organs have the potential to fully correct the MPS VI phenotype. Enzyme replacement therapy, the current treatment for MPS VI, requires weekly infusions of a costly enzyme and has limited efficacy on bone and corneal disease. Based on the encouraging preclinical results generated by our group, gene therapy based on a single intravascular administration of adeno-associated viral (AAV) vectors targeting liver has the potential to provide a lifelong source of ARSB. The MeuSIX consortium plans to conduct a multicenter phase 1/2 clinical trial to investigate the safety and efficacy of AAV-mediated gene therapy in patients with MPS VI. An orphan drug designation (ODD) has been obtained from both the European Medicinal Agency and the US Food and Drug Administration for the MPS VI therapeutic AAV vector. The objective of this trial is to test whether MEUSIX can be safely administered to patients with MPS VI.
results from this clinical trial proposed by the MeuSIX consortium has the potential to have a tremendous impact on the natural history of MPSVI and to significantly improve the quality of life of the affected patients. Moreover, the approach developed may facilitate the development of similar approaches for other inborn errors of metabolism."

Field of science

/medical and health sciences/medical biotechnology/genetic engineering/gene therapy
/medical and health sciences/clinical medicine/ophthalmology
/humanities/history and archaeology/history
/natural sciences/biological sciences/biochemistry/biomolecules/proteins/enzymes

Programme(s)

Topic(s)

Call for proposal

FP7-HEALTH-2012-INNOVATION-1

Funding Scheme

CP-FP - Small or medium-scale focused research project

Coordinator

FONDAZIONE TELETHON

Address
Via Varese 16/B
00185 Roma
Italy

Website
Contact the organisation

Administrative Contact
Raffaella Pettorruso (Mrs.)

Participants (8)

REGENXBIO INC

United States
<table>
<thead>
<tr>
<th>EU contribution</th>
<th>Activity type</th>
</tr>
</thead>
<tbody>
<tr>
<td>€ 1 709 809</td>
<td>Private for-profit entities (excluding Higher or Secondary Education Establishments)</td>
</tr>
</tbody>
</table>

**GENOSAFE SAS**  
France  
EU contribution € 777 274  
Address Rue De L'internationale 1 91000 Evry  
Activity type Private for-profit entities (excluding Higher or Secondary Education Establishments)  
Website  
Administrative Contact Severine Pouillot (Dr.)

**UNIVERSITA DEGLI STUDI DI NAPOLI FEDERICO II**  
Italy  
EU contribution € 819 120  
Address Corso Umberto I, 40 80138 Napoli  
Activity type Higher or Secondary Education Establishments  
Website  
Administrative Contact Gaetana Errico (Mrs.)

**HACETTEPE UNIVERSITESI**  
Turkey  
EU contribution € 240 000  
Website  
Administrative Contact
Hacettepe Universitesi
Beytepe Kampusu Rektorluk
Binasi
06800 Cankaya Ankara

Website  
Contact the organisation

Administrative Contact
Hatice Serap Sivri (Prof.)

ERASMUS UNIVERSITAIR MEDISCH CENTRUM ROTTERDAM

Netherlands
EU contribution
€ 241 278

Address
Dr Molewaterplein 40
3015 GD Rotterdam

Website  
Contact the organisation

Administrative Contact
Ans Van Der Ploeg (Prof.)

UNIVERSITA’ DEGLI STUDI DI MILANO-BICOCCA

Italy
EU contribution
€ 117 000

Address
Piazza Dell’ateneo Nuovo 1
20126 Milano

Website  
Contact the organisation

Administrative Contact
Paola Di Rienzo (Dr.)

INFORMA SRL

Italy
EU contribution
€ 94 479.20

Address
Via Luigi Rava 43
00149 Roma

Activity type
Private for-profit entities
(excluding Higher or Secondary Education)
INFORMAPRO - SOCIETA' A RESPONSABILITA' LIMITATA

Italy

EU contribution
€ 205 520,80

Address
Via Luigi Rava 43
00149 Roma

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

Website
Contact the organisation

Administrative Contact
Francesca Incardona (Dr.)

Administrative Contact
Dario Corsini (Dr.)

Last update: 2 August 2019
Record number: 106240

Permalink: https://cordis.europa.eu/project/id/304999/

© European Union, 2020