HemAcure
Project ID: 667421
Funded under: H2020-EU.3.1. - SOCIETAL CHALLENGES - Health, demographic change and well-being

Application of combined gene and cell therapy within an implantable therapeutic device for the treatment of severe hemophilia A

From 2015-11-01 to 2018-10-31, closed project | HemAcure Website

Project details

| Total cost: | Topic(s): |
| EUR 5 564 395 | PHC-16-2015 - Tools and technologies for advanced therapies |

| EU contribution: | Call for proposal: |
| EUR 5 564 395 | H2020-PHC-2015-two-stage See other projects for this call |

| Coordinated in: | Funding scheme: |
| Germany | RIA - Research and Innovation action |

Objective

The main objective of the HemAcure project is to develop and refine the tools and technologies for a novel ex vivo prepared cell based therapy to treat the bleeding disease haemophilia A (caused by genetic deficiency in clotting factor VIII (FVIII)) that should ultimately lead to improved quality of life of the patients. The concept is a further development of our approach, established during the FP7 ReLiver project led by Medicyte. From the very beginning, we balance two important goals, maximizing the product’s efficacy and safety profile on one side and minimizing production cost on the other by enhancing the product’s manufacturability. HemAcure relates to the work program as we focus on the refinement of all steps and tools of our ex vivo gene therapy approach. These steps involve 1) isolation and culture of cells from patients’ blood, 2) manipulation of patient cells to repair its genetic defect by ex vivo introducing the correct genetic copy of FVIII (mutations in this gene lead to hemophilia A), 3) automation of cell expansion in a novel and passage-less scalable bioreactor, 4) continuously monitoring of cells during and after expansion with respect to their safety profile and functionality, 5) cell implantation into a worldwide unique medical device for targeted delivery of therapeutic Factor VIII and 6) proof-of-concept and safety studies in appropriate haemophilia A animal models. The aim of adapting the proof-of-concept to GMP requires a risk based approach, by means of a clear understanding of the whole process from design to production of the therapeutic cells and a systematic way to identify and prevent risks that are not acceptable for the patient. All steps will be designed and conducted according to European GMP-regulations to ensure that the product will fully comply to the requirements for quality of the European authorities.

Related information

| Result In Brief | Curative cell-based therapy for haemophilia A |
| Report Summaries | Periodic Reporting for period 1 - HemAcure (Application of combined gene and cell therapy within an implantable therapeutic device for the treatment of severe hemophilia A) |
### Coordinator

UNIVERSITAETS KLINIKUM WUERZBURG - KLINIKUM DER BAYERISCHEN JULIUS-MAXIMILIANS-UNIVERSITAT  
JOSEF-SCHNEIDER-STRASSE 2  
97080 WURZBURG  
Germany  
**EU contribution:** EUR 1,714,513

**Activity type:** Higher or Secondary Education Establishments  
[Contact the organisation](#)

### Participants

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<tr>
<th>Name</th>
<th>Location</th>
<th>Activity type</th>
<th>EU contribution</th>
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<td>GRUNEWALD JURGEN</td>
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<td>KETTELERSTRASSE 30</td>
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<td>Private for-profit entities (excluding Higher or Secondary Education Establishments)</td>
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<td>UNIVERSITA DEGLI STUDI DEL PIEMONTE ORIENTALE AMEDEO AVOGADRO</td>
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<td>Higher or Secondary Education Establishments</td>
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<td>LOUGHBOROUGH UNIVERSITY</td>
<td>United Kingdom</td>
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<td>SERNOVA CORP.</td>
<td>Canada</td>
<td>Private for-profit entities (excluding Higher or Secondary Education Establishments)</td>
<td>EUR 944,177.50</td>
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OSKAR VON MILLER RING 29
80333 MUENCHEN
Germany
See on map

**Activity type:** Other

**EU contribution:** EUR 34 237

GABO: MI GESELLSCHAFT FUR ABLAUFORGANISATION: MILLIARIUM MBH & CO KG

ARTTIC
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75013 PARIS
France
See on map

**Activity type:** Other

**EU contribution:** EUR 150 000

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