New therapies for rare diseases: New EU project MYOCURE advances treatment of rare hereditary muscle disorders

Contributed by: **EURICE – European Research and Project Office GmbH**

Vrije Universiteit Brussel (VUB) coordinates new Horizon2020 research initiative on the development of novel therapies for patients suffering from rare muscle diseases. MYOCURE is funded with €6 million by the European Commission and involves 8 European partner institutions. The official project kick-off meeting will take place in Belgium from February 8 to 9, 2016.

The new collaborative research project MYOCURE aims at advancing the development of new therapies for patients with rare muscle diseases. In particular, MYOCURE will impact on an estimated 20,000 people in the EU suffering from myotubular myopathy (MTM) and glycogen storage disorder (GSD) type II (Pompe's disease) as well as impact on other muscle disorders. MTM and GSD II are quintessential hereditary muscle diseases for which there is no definitive cure. They are attractive diseases for gene therapy as they comprise a diverse family of rare genetic diseases typically caused by single gene defects, and often provoke significant morbidity and mortality due to skeletal muscle, cardiac and/or diaphragm dysfunction. The costs for treatment and care taking of GSD II patients, amount to €400,000 to €700,000 per year per patient. In the EU, this corresponds to an estimated cost of €4 billion to €7 billion annually. Thus, advancing research in this field is a societal need and a crucial commitment to improve patient care and relieve the healthcare-system.

In this context, MYOCURE was launched to overcome current scientific and societal bottlenecks, and to develop an innovative and clinically applicable gene therapy platform to cure rare hereditary muscle disorders. Equipped with a budget of about €6 million, the novel approaches that will be developed in MYOCURE shall form the basis for a one-time treatment, which is expected to provide enhanced patient treatment and significantly reduce healthcare costs.

Professor Marinee Chuah from the Vrije Universiteit Brussels and Coordinator of the project stresses the importance of the social and economic impact of the project: "Establishing such a clinically translatable one-time treatment platform will contribute significantly to a higher patients' quality of life. Moreover, the social impact stretches out to the families and relatives of the affected persons, as the need for home or hospital care is decreased, hence reducing the overall social and economic costs". She further emphasizes that one of the unique strengths of MYOCURE is the combination of most recent innovations in gene therapy with the latest insights in immune control to maximize efficacy and safety. Moreover, MYOCURE will enhance expertise in the field of orphan and rare diseases as well as strengthen the competitiveness and growth of companies in the EU by developing innovations meeting the needs of European and global markets.

MYOCURE brings together leading experts in science and industry from eight European partner institutions to reach these goals and successfully implement the project's ambitious work plan.

The partners in MYOCURE at a glance:

Belgium
Vrije Universiteit Brussel (Prof. Marinee K.L. Chuah, Prof. Thierry VandenDriessche, Dr Kelvin Y.C. Chai)

France
Université Pierre et Marie Curie (Prof. Federico Mingozzi)
Association Généthon (Dr Ana Buj Bello, Dr Matthias Hebben)

Germany
Universitaetsklinikum Heidelberg (Dr Dirk Grimm)
European Research and Project Office GmbH (Dr Birte Kretschmer)

Spain
Universitat Autònoma de Barcelona (Prof. Fatima Bosch)
Asphalion SL (Ms Anna Graupera)

United Kingdom
Envigo CRS Limited (contact information on request)

Contact

Coordinator
Prof. Marinee Chuah
Vrije Universiteit Brussel (VUB)
E-mail: marinee.chuah@vub.ac.be
Phone: +32 473 215849

Project Management
European Research and Project Office GmbH
Dr Birte Kretschmer
Phone: +49 30 374415-833
Email: b.kretschmer@eurice.eu

Contributor

Organisation
EURICE – European Research and Project Office GmbH
Science Park 1
66123 Saarbrücken
Germany
Website

Contact
Stephanie Weber
E-mail
See more news from this contributor

Related information

Programmes H2020-EU.3.1.

Countries Belgium, Germany, Spain, France, United Kingdom

Subjects

Medical biotechnology - Medicine and Health - Scientific Research

Keywords

Rare diseases; Hereditary Muscle Disorders; Myotubular Myopathy; MTM; Glycogen Storage Disorder; GSD; Gene Therapy

Last updated on 2016-02-02

Category: New products and technologies
Provider: WIRE
Revision: 0

Retrieved on 2016-04-07

Permalink: http://cordis.europa.eu/news/rcn/130654_en.html