Wellcome Trust supports the development of sirolimus in beta-thalassemia, a collaborative project between Rare Partners and Ferrara University

Milan (Italy), 2nd May 2018 – **Rare Partners** has received an important grant from the Wellcome Trust (UK), from the "Innovator Award" program, for the clinical development of the drug sirolimus in patients affected by beta-thalassemia.

Rare Partners had already won a Pathfinder Award in 2014 from the Wellcome Trust, that had been utilized for the completion of the preclinical development of the product.

The collaboration between **Rare Partners** and the Department of Life Sciences and Biotechnology of Ferrara University (Italy) for the development of sirolimus in beta-thalassemia began in 2011 and follows the research activities conducted in this field for more than fifteen years by the group of Prof. Roberto Gambari, supported by Ferrara University, AVLT (Associazione Veneta per la Lotta alla Talassemia) and by several Italian and European funding programs.

The funding received from the Wellcome Trust will be used for the first clinical trial in man of sirolimus in the therapeutic indication of beta-thalassemia. The trial will start in the first half of 2018 and follows the granting of Orphan Drug Designation (ODD) from the European agency EMA in 2015 as well as from the US agency FDA in 2016.

The use in beta-thalassemia patients of the drug sirolimus, so far prescribed mainly as immunosuppressant agent in organ transplant, is based on the ability of the molecule to increase the level of fetal haemoglobin (HbF), which has been proven in several preclinical models. Increasing HbF is currently viewed as one of the most promising approaches to improve the quality of life and eventually increase the life expectancy in beta-thalassemia patients.

Marco Prosdocimi, Managing Director of Rare Partners, said: "*Thanks to this important funding granted by the Wellcome Trust we are now ready to start the clinical development*

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aiming to preliminarily demonstrate the efficacy of sirolimus in beta-thalassemia. It is a clear example of "drug repositioning" for a product already on the market that could also be able to improve the quality of life and hopefully reduce the need for blood transfusions in the new rare therapeutic indication of beta-thalassemia.

We are extremely pleased for the positive progress of our collaboration with Ferrara University, one of the worldwide reference centers for the cure and the research in the field of beta-thalassemia".

Roberto Gambari, professor of Biochemistry at the Department of Life Sciences and Biotechnology, Ferrara University, said that: "*Our lab has been interested since the beginning on the induction of fetal hemoglobin (HbF) as a strategy for the treatment of beta-thalassemia, because HbF induction is able to improve the clinical symptoms in patients. We have recently correlated the in vitro response to the inducers of HbF with the genotype of patients affected by beta-thalassemia. Our results are extremely interesting and have led to the identification of a new polymorphism associated with high production of HbF, that could largely explain the intrinsic ability of some beta-thalassemia patients to produce this type of hemoglobin.*

We are convinced that this finding will be of great help for the patient recruitment in clinical trials. In this context, the project financed by the Wellcome Trust will give us the opportunity to verify the efficacy of sirolimus in a first pilot clinical trial, conducted in collaboration with Maria Rita Gamberini, Azienda Ospedaliera Universitaria di Ferrara, and with Ersi Voskaridou, LHGA Hospital of Athens. With respect to the funding that we have received in the past years, we would like to mention the participation of our group to the THALAMOSS Project (THALAssaemia MOdular Stratification System for personalized therapy of beta-thalassemia; Health-2012-INNOVATION-1) and the continuous support of Fondazione Cassa di Risparmio di Padova e Rovigo, which was the major national sponsor of our research activity on hemoglobinopathies.".

About Rare Partners

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Rare Partners Srl, founded in Milan in March 2010 and registered as a Social Enterprise, brings a new approach to finding diagnostic and therapeutic solutions in the field of rare diseases, thanks to the creation of a network of collaborations with research institutes, charities and service organizations.

Rare Partners works as a virtual company that combines the use of non-profit financial resources (public and private) with its expertise in the industrial field, in order to create new opportunities for collaborations between the world of not for profit organizations, the scientific community, and biopharmaceutical companies.

For more information: www.rarepartners.org

About University of Ferrara

The University of Ferrara, established in 1391 is one of the oldest universities in Italy, counting more than 18,000 students and with an outstanding track-record of excellence in scientific research, including life sciences. Professor Roberto Gambari is the founder and Director of the Laboratory for the development of genetic and pharmacogenomic therapy of thalassemia, ThalLab at the University of Ferrara.

For more information: www.unife.it

About Wellcome Trust

The Wellcome Trust is a global charitable foundation dedicated to achieving extraordinary improvements in human and animal health. It supports the brightest minds in biomedical research and the medical humanities. The Trust's breadth of support includes public engagement, education and the application of research to improve health. It is independent of both political and commercial interests.

For more information: www.wellcome.ac.uk

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