A new Orphan Drug Designation to Sirolimus

Milan (Italy), June 30th, 2016 – **Rare Partners** Srl Impresa Sociale announces that U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to Sirolimus (Rapamycin) for the treatment of beta thalassaemia.

The decision has been communicated by the Agency on June 21 and follows the positive opinion issued by the Committee for Orphan Medicinal Products (COMP) of the European Medicines Agency (EMA) on 12 November 2015, recommending the designation of the medicinal product containing Sirolimus as an orphan medicinal product for the indication: treatment of beta thalassaemia intermedia and major.

Marco Prosdocimi, Managing Director of **Rare Partners**, said that "*This important result further confirm that the collaboration we established since 2011 with Roberto Gambari (University of Ferrara) and his collaborators is extremely fruitful. Their research, supported for many years by AVLT (Associazione Veneta per la Lotta alla Talassemia) has obtained outstanding results, with potential application in patients within a short time frame. A collaborative grant, obtained by Rare Partners from Wellcome Trust (UK) within the Pathfinder Award scheme, allowed completion of fundamental preclinical work and preparation of the application to FDA. We are now ready to start clinical trials aimed at demonstrating preliminary clinical efficacy*"

Roberto Gambari, professor of Biochemistry at the Department of Life Sciences and Biotechnology, Ferrara University, said that: "*This significant achievement in the development of possible therapeutic protocols for beta-thalassemia was obtained also thanks to the activity performed within the project THALAMOSS (THALAssaemia MOdular Stratification System for personalized therapy of beta-thalassemia), funded by UE within the FP7 Program. The research on thalassemia was also supported by Fondazione CARIPARO and Telethon.*

.RarePartners Srl Corso Magenta 31 - 20123 Milano, Italy - **tel** +39 02.45074463 - **fax** +39 02.87152906
skype RarePartners – **mail** info@rarepartners.org - **web** www.rareparters.org

The FDA granted the possible use of Sirolimus for the treatment of beta-thalassemia, which allows possible clinical studies on a broad range of beta-thalassemia patients independently from their genotype, phenotype and DNA polymorphisms. The results we obtained on erythroid cells from beta thalassemia patients demonstrated not only that Sirolimus was active in inducing fetal hemoglobin, but also that it was in some cases more effective than hydroxyurea, a molecule extensively used in beta thalassemia intermedia to induce fetal hemoglobin. Now Sirolimus can be employed for clinical trials on beta-thalassemia patients selected on the basis of the in vitro response of their erythroid precursors to this treatment.

Combined treatments using both Sirolimus and hydroxyurea might also be considered as an effective therapeutic option in selected patients".

Orphan Drug Designation is granted to novel drugs or biologics that treat a rare disease or condition affecting fewer than 200,000 patients in the U.S. The designation provides developers with a seven-year period of U.S. marketing exclusivity upon marketing approval for the designated indication, as well as with tax credits for clinical research costs, the ability to apply annually for grant funding, clinical research trial design assistance and the waiver of Prescription Drug User Fee Act (PDUFA) filing fees.

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About Thalassemia

Beta-thalassemias are a group of inherited blood disorders. They are caused by reduced or absent synthesis of the beta chains of hemoglobin that results in variable outcomes, ranging from severe anemia to clinically asymptomatic individuals.

Treatment is symptomatic and it is generally accepted that beta-thalassemia treatment can still be considered a major unmet medical need, being thalassemia a disease without an adequate treatment. Prevalence of the disease is estimated to be 0.4 to 1 in 10,000 people in the European Union (EU) and close to 0.1 in 10,000 people in the US.

About Sirolimus application in Thalassemia

It is known that an increase of fetal hemoglobin in thalassemic patients may result in a relevant clinical improvement. Sirolimus, already used as an immunosuppressant in transplanted patients, should act in thalassemia patients by inducing erythroid differentiation and expression of fetal hemoglobin, thus reducing the need of frequent blood transfusions. This new use of the drug has been patented by professor Gambari and coworkers.

Rare Partners finalized an agreement with the patent's inventors and assignees (University of Ferrara and Associazione Veneta per la Lotta alla Talassemia), aimed at completing the preclinical studies and proceeding with Orphan Drug Designation and clinical development in thalassemic patients.

About University of Ferrara

The University of Ferrara, established in 1391 is one of the oldest universities in Italy, counting with 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

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University of Ferrara. At present, Roberto Gambari is Director of the Department of Life Sciences and Biotechnology, Ferrara University, and coordinator of the EU FP7 Project THALAMOSS.

About Rare Partners

Rare Partners is a non profit biopharmaceutical company devoted to the development of new therapies and diagnostics in the field of rare diseases. The company was founded in Milan on March 2010 and registered in Italy as “Impresa Sociale”. The basic idea of **Rare Partners** is to match non profit financial resources (public and private) with industrial drug development expertise, provided by the company’s organization together with a strong network of consultants.

For further information, please contact:

Marco Prosdocimi, Managing Director: m.prosdocimi@rarepartners.org

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skype RarePartners – **mail** info@rarepartners.org - **web** www.rareparters.org