The European Commission grants Orphan Drug Designation to Sirolimus (Rapamycin) for the treatment of beta thalassaemia intermedia and major

Milan (Italy), January 6, 2016 –Rare Partners Srl Impresa Sociale announces that the European Commission has granted Orphan Drug Designation to Sirolimus (Rapamycin) for the treatment of beta thalassaemia intermedia and major. The decision has been taken by the Commission on December 14, 2015, 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 was made possible by the collaboration we established since 2011 with Roberto Gambari (University of Ferrara) and his collaborators. 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 EMA. We are now ready to start clinical trials and establish a partnership with companies interested in the therapy of this severe disease.*

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 results we obtained on erythroid cells from beta thalassemia patients demonstrated not only that Sirolimus was active in inducing fetal hemoglobin, but*

.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

also that it was in some cases more effective than hydroxyurea, a molecule extensively used in beta thalassemia intermedia to induce fetal hemoglobin. Moreover, our results indicate that combined treatments using both Sirolimus and hydroxyurea might be considered as an effective therapeutic option in selected patients. Since it is formally demonstrated that in vitro erythroid culture are predictive of hydroxyurea response in vivo, the screening of beta thalassemia patients expected not to be responders to hydroxyurea treatment is feasible. These cohort of patients might be considered for the first pilot clinical trials using Sirolimus as fetal hemoglobin inducer.

The EMA grants orphan medicinal product designations to advance the development of drugs intended to treat, prevent or diagnose life-threatening or very serious conditions that are rare and affect not more than 5 in 10,000 persons in the E.U. "*With this designation"*, added Dr. Prosdocimi, *"Rare Partners will have market exclusivity in the E.U. for ten years in the event that Sirolimus receives marketing approval as a treatment for thalassemia".* This designation also gives rights to special benefits, including research support, eligibility for protocol assistance, and possible exemptions or reductions in some regulatory fees during development or at the time of application for market authorization.

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.

.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

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 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.

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 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.

For further information, please contact:

Marco Prosdocimi, Managing Director

m.prosdocimi@rarepartners.org

.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