# The clinical trial SIRTHALACLIN for the treatment of transfusion-dependent beta-thalassemia patients has started patient enrolment.

**Milan (Italy), 12th December 2019** – Rare Partners Srl Impresa Sociale announced the start of patient enrolment in the phase 2 clinical trial SIRTHALACLIN (EudraCT n°2018-001942-33) for the treatment of transfusion-dependent beta-thalassemia patients.

The trial is taking place at the "Azienda Ospedaliera-Universitaria S. Anna" of Ferrara under the supervision of Dr. Maria Rita Gamberini, director of the Day Hospital for Thalassemia and Hemoglobinopathies of the same hospital.

The clinical trial is aiming to enroll 20 patients that will be treated for 1 year with sirolimus given by oral route at the starting daily dose of 1 mg and has as primary end point the evaluation of fetal hemoglobin levels at the end of the treatment period.

An increase of fetal hemoglobin is currently one major approach aiming at improving the clinical status and reducing the frequency of blood transfusions in beta-thalassemia patients.

The drug sirolimus has been utilized for more than 20 years as immunosuppressive agent in kidney transplantation and has been recently approved by FDA and EMA for a rare indication, namely Lymphangioleiomyomatosis.

Many research groups, and in particular that of Prof. Roberto Gambari at the Ferrara University, active in the beta-thalassemia field since several decades, have shown the capacity of sirolimus of raising the fetal hemoglobin levels in several pre-clinical models and in ex vivo experiments.

On the basis of such evidences, Rare Partners has obtained the Orphan Drug Designation both from the European Agency EMA (2015) and from the corresponding US Agency FDA (2016).

The first results from the ongoing trial in Ferrara are expected in the first half of 2021. In case they will confirm the sirolimus efficacy observed in pre-clinic, they could pave the way



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to the full development of a therapeutic agent able to improve the quality of life of patients affected by beta-thalassemia, significantly reducing the frequency of transfusions.

EudraCT: 2018-001942-33

ClinicalTrials.gov: NCT03877809

## About Rare Partners

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

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