address Corso Magenta 31 - 20123 Milano, Italy - tel&fax +39 02.45074463 - +39 02.87152906 - skype RarePartners - www.rarepartners.org

The European Commission grants Orphan Drug Designation to 4,6,4′–trimethylangelicin for the treatment of Cystic Fibrosis

RP announcement

Milan (Italy), 3rd July 2013 – Rare Partners Srl Impresa Sociale announces that the European Commission has granted Orphan Drug Designation to 4,6,4′–trimethylangelicin (TMA) for the treatment of Cystic Fibrosis.
The decision taken by the Commission on 19 June 2013 follows the positive opinion released by the Committee for Orphan Medicinal Products (COMP) of the European Medicines Agency (EMA), recommending the designation of the medicinal product, containing 4,6,4′–trimethylangelicin, as an orphan medicinal product for the indication: treatment of cystic fibrosis.

4,6,4′–trimethylangelicin is now listed in the Community Register of orphan medicinal products for human use with the code EU/3/13/1137.

Marco Prosdocimi, Managing Director of Rare Partners, said that "*This important result was made possible by the collaboration we established with a network of outstanding Italian scientists: Giulio Cabrini (University Hospital of Verona), Valeria Casavola (University of Bari) and Roberto Gambari (University of Ferrara). These scientists discovered unexpected biological properties for several compounds and established that TMA is of potential help for patients with Cystic Fibrosis. The granting of orphan medicinal product designation by EMA is a significant milestone in the development and eventual future clinical availability of TMA for the treatment of patients affected by cystic fibrosis, and also a validation of* Rare Partners*' business model*".

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 TMA receives marketing approval as a treatment for cystic fibrosis*".

This designation also gives right 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.

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About Cystic Fibrosis

Cystic fibrosis is a life-threatening, genetic disease. In the European Union population its prevalence is estimated between 7 and 13 cases over 100,000, in particular is estimated at 12.6 cases over 100,000 in the latest issue (November 2012) of Orphanet Report Series on the prevalence of rare diseases. A defect in the CFTR gene causes the body to produce abnormally thick, sticky mucus that leads to chronic lung infections and impairs digestion. Lung complications represent the most serious manifestation of the disease and the reason for the high mortality rate amongst patients.

About TMA (4,6,4′–trimethylangelicin)

TMA was originally synthesized in the 80s within an R&D project carried on in the psoriasis area. Recently Giulio Cabrini (Department of Pathology and Diagnostics, University Hospital of Verona), Valeria Casavola (Department of Biosciences, Biotechnologies and Biopharmacetics, University of Bari) and Roberto Gambari (Department of Life Sciences and Biotechnology, University of Ferrara), with their coworkers, discovered that TMA exerts 3 different actions on cells of the respiratory system, each one potentially able to improve clinical status of the patients. First, TMA was shown to inhibit IL-8 gene transcription mainly by intervening on driving the recruitment of activated transcription factors on IL-8 gene promoter. Furthermore, TMA was also tested on CFTR and found to potentiate F508del-CFTR-dependent chloride efflux. Finally, TMA was found to be able to correct F508del-CFTR activity. TMA is expected to provide a relevant benefit to patients, not shared by other treatments, for the peculiarity of this triple action, potentially able to reduce at the same time accumulation of thick and sticky mucus by inducing chloride secretion (activation and correction of CFTR) as well reducing inflammatory status of the respiratory tree (inhibition of IL-8). Roberto Gambari said "*These achievements and activities have been made possible by the key help provided by the Foundation for the Research on Cystic Fibrosis (FFC), who granted financial support to several projects directed by Cabrini, Casavola and me*". Giulio Cabrini said that "T*MA features, namely a triple action, appear to be unique in the field of molecules of potential therapeutic use in cystic fibrosis*." Valeria Casavola pointed out that "*TMA efficacy has been repeatedly observed in different experimental model at very low concentrations, thus clinical development seems particularly promising*". In this respect Rare Partners will perform development activities on TMA, starting with preclinical studies, on the basis of an agreement with Azienda Ospedaliera Universitaria di Verona, Università degli Studi di Bari and Università degli Studi di Ferrara signed in September 2012.

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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. Rare Partners currently has product candidates in the fields of Cystic Fibrosis and of Thalassemia.

For further information, please contact:

m.prosdocimi@rarepartners.org
www.rarepartners.org