



Vivet's Second Gene Therapy Product, VTX-803 for PFIC3, Receives US and European Orphan Drug Designation.

PARIS, France June 1st, 2020, Vivet Therapeutics announced today that both the Food and Drug Administration (FDA) and the European Commission (EC) have granted Orphan Drug Designation (ODD) for Vivet's second gene therapy product, VTX-803, for the treatment of Progressive Familial Intrahepatic Cholestasis type 3 (PFIC3). PFIC3 is a rare life-threatening and chronically debilitating condition due to progressive severe liver dysfunction, accompanied by jaundice, portal hypertension, hepatosplenomegaly and failure to thrive. The symptoms of the disease usually appear first in childhood with progressive cholestasis, evolving to hepatic failure, cirrhosis and need for liver transplantation.

"We are very pleased to receive these designations which further validate Vivet's efforts to treat rare inherited metabolic disorders. The FDA and EC have both recognized the unmet need for a safe and effective treatment of PFIC3 and the potential of VTX-803 to address such need. The field of Progressive Familial Intrahepatic Cholestasis has been gaining increased attention in the medical field with numerous ongoing clinical trials. There is a high unmet medical need, especially in the paediatric segment, for this condition, for which liver transplantation remains the only truly curative option today" stated Jean-Philippe Combal, co-founder & CEO of Vivet Therapeutics.

VTX-803 is Vivet's second gene therapy product to be granted Orphan Drug Designation. This is an important value driver for Vivet which would provide 10 & 7 years of market exclusivity in the EU & US respectively if VTX-803 is approved for the treatment of PFIC3. VTX-803 is currently under IND-enabling studies and initiation of clinical development is planned for the second part of 2021 in both the US and the EU.

"We are very happy about this recognition of our scientific achievement for a complex liver disease and further validation of our strategic collaboration with Fundación para la Investigación Médica Aplicada (FIMA). This program could be a game-changer in the gene therapy field for PFIC disorders" added Dr. Gloria González-Aseguinolaza, Vivet Therapeutics co-founder & CSO and deputy director at CIMA.

VTX-803 was recently the subject of an oral presentation at 2019 ASGCT (American Society of Gene and Cell Therapy), a lecture at the 2019 ESGCT (European Society of Gene and Cell Therapy) annual meetings and a publication in Nature Communications (Weber et al. Nature Communications, 2019, 10(1):5694).



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About Vivet Therapeutics

Vivet Therapeutics is an emerging biotechnology company developing novel gene therapy treatments for rare, inherited metabolic diseases.

Vivet is building a diversified gene therapy pipeline based on novel adeno-associated virus (AAV) technologies developed through its partnerships with, and exclusive licenses from, the Fundación para la Investigación Médica Aplicada (FIMA), a not-for-profit foundation at the Centro de Investigación Médica Aplicada, University of Navarra based in Pamplona, Spain.

Vivet's lead program, VTX-801, is a novel investigational gene therapy for Wilson disease which has been granted Orphan Drug Designation by the Food and Drug Administration and the European Commission.

Vivet is supported by international life science investors. For more information please visit us on www.vivet-therapeutics.com and follow us on Twitter at @Vivet_tx and LinkedIn.