Vivet's First Gene Therapy Product Receives European and US Orphan Drug Designation VTX 801 for Wilson's Disease.

PARIS, France September 25th, 2017, 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 lead gene therapy product, VTX-801, for the treatment of Wilson's Disease (WD), a chronically debilitating and life-threatening condition if not treated.

« *This designation validates Vivet's efforts to treat the copper metabolism disorder, Wilson's Disease. The FDA and EC have both recognized the unmet need for a safe and effective treatment of Wilson's Disease by physiological restoration of copper homeostasis and the potential of VTX 801 to address such need.* » stated Jean-Philippe Combal, CEO of Vivet Therapeutics.

VTX-801 is Vivet's first gene therapy product to be granted Orphan Drug Designation. This is an important value driver for Vivet, in particular through the 10 & 7-year market exclusivity in the EU & US respectively if VTX-801 is approved for the treatment of Wilson's Disease. Initiation of clinical development is planned for Q4 2018 in both the US and the EU.

"Through our strategic collaboration with Fundación para la Investigación Médica Aplicada (FIMA) at the Centro de Investigación Medica Aplicada (University of Navarra, Spain), we are building an unparalleled platform to address key challenges in gene therapy, notably improving sustainability and immune tolerance, targeting Wilson's Disease and other hepatic rare metabolic genetic disorders. A critical asset to this platform is a new liver targeting AAV-Anc80 serotype, licensed from Massachusetts Eye and Ear (MEE), a teaching hospital of Harvard Medical School, Boston, " added Jean-Philippe Combal.

For more information, visit www.vivet-therapeutics.com

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About VTX-801: VTX-801 is an adeno-associated virus (AAV) containing a gene coding for a functional mini-ATP7B copper transporter, that is intended to restore copper metabolism through liver cell targeting

About Wilson's Disease: Wilson's disease is an inherited orphan disorder affecting approximately 1:40,000 individuals. In this condition, the physiological copper biliary elimination pathway is disrupted, leading to tissue accumulation of toxic copper levels, most notably in liver and CNS. Onset of symptoms typically occurs in teenagers or in young adults and left untreated, the condition uniformly progresses to severely debilitating complications and death. The disease is due to inactivating mutations in the gene encoding the copper transporter ATP7B.

About Vivet Therapeutics: Vivet Therapeutics is a gene therapy biotech company with headquarters in Paris, France, dedicated to the research, development and future commercialization of gene therapy products for inherited liver disorders with high medical need.