Vivet Therapeutics et Pfizer Inc. annoncent l'autorisation de la FDA de commencer l'étude de Phase 1/2 du VTX-801, GATEWAY, la thérapie génique expérimentale de Vivet pour la maladie de Wilson

PARIS, France et NEW YORK, NY - 18 novembre 2020 - Vivet Therapeutics («Vivet»), une société privée de biotechnologie de thérapie génique spécialisée dans le développement de traitements pour des maladies hépatiques héréditaires aux besoins médicaux élevés non satisfaits, et Pfizer Inc. (NYSE: PFE) ont annoncé aujourd'hui que la US Food and Drug Administration (FDA) a autorisé la demande d'IND de Vivet pour l'étude GATEWAY, un essai clinique de Phase 1/2 évaluant la thérapie génique expérimentale propriétaire de Vivet, VTX-801, pour le traitement potentiel de la maladie de Wilson (WD), une maladie hépatique rare et potentiellement mortelle. L'essai devrait commencer au début de 2021.

« Nous sommes heureux d'annoncer la première autorisation d'essai thérapeutique de Vivet par la FDA, pour notre étude de Phase 1/2 GATEWAY pour le VTX-801 », a déclaré Jean-Philippe Combal, Président et cofondateur de Vivet. « Il s'agit d'une étape très importante pour la communauté de la maladie de Wilson pour qui le VTX-801 pourrait potentiellement apporter un bénéfice thérapeutique significatif. Le VTX-801 vise à restaurer le métabolisme naturel du cuivre et l'essai GATEWAY mesurera des biomarqueurs pertinents pour évaluer la restauration physiologique de l'élimination et du transport du cuivre chez les patients. Nous sommes impatients de faire entrer le VTX-801 en clinique début 2021. »

VTX-801 est un nouveau traitement expérimental de thérapie génique utilisant un virus adéno-associé (AAV) conçu pour délivrer un transgène ATP7B miniaturisé codant pour une protéine fonctionnelle, qui a démontré la restauration de l'homéostasie du cuivre, l'amélioration de la pathologie hépatique et la réduction du cuivre accumulé dans le cerveau chez un modèle murin de maladie de Wilson. Le sérotype d'AAV du VTX-801 a été sélectionné en fonction de son tropisme démontré pour la transduction des cellules hépatiques humaines.

En mars 2019, les 2 sociétés ont annoncé que Pfizer avait acquis une participation minoritaire dans Vivet et obtenu une option exclusive pour l'acquisition du capital restant. En septembre 2020, Vivet et Pfizer ont annoncé la signature d'un accord pour la fabrication par Pfizer du vecteur VTX-801 pour l'étude GATEWAY.

« L'acceptation par la FDA de l'IND de Vivet marque une étape importante pour le programme VTX-801, qui, selon nous, a le potentiel de devenir une thérapie transformationnelle pour les personnes atteintes de la maladie de Wilson », a déclaré Seng Cheng, directeur scientifique de l'unité de recherche sur les maladies rares de Pfizer. « Pfizer a commencé à fabriquer le matériel clinique pour l'étude GATEWAY et attend avec impatience le début de l'étude. »

« Cet IND est une reconnaissance de l'expertise de l'équipe de recherche de Vivet dirigée par notre Directrice scientifique et cofondatrice, le Dr Gloria González-Aseguinolaza, de nos collaborations de recherche, notamment avec la Fundación para la Investigación Médica Aplicada (FIMA), et notre équipe de développement expérimentée. Nous pensons que notre expertise globale en développement, ainsi que notre collaboration avec Pfizer, nous permettront d'avancer rapidement et d'apporter aux patients avec des besoins médicaux importants non satisfaits, cette thérapie potentiellement transformative », a ajouté Jean-Philippe Combal.

Le texte du communiqué issu d'une traduction ne doit d'aucune manière être considéré comme officiel. La seule version du communiqué qui fasse foi est celle du communiqué dans sa langue d'origine. La traduction devra toujours être confrontée au texte source, qui fera jurisprudence.

About GATEWAY - Phase 1/2 Clinical Trial of VTX-801 in Wilson disease

The GATEWAY trial is a multi-center, non-randomized, open-label, Phase 1/2 clinical trial designed to assess the safety, tolerability and pharmacological activity of a single intravenous infusion of VTX-801 in adult patients with Wilson disease, prior to and following background WD therapy withdrawal.

Six leading centers in the United States and Europe are expected to participate in the GATEWAY Phase 1/2 trial. The trial is expected to enroll up to sixteen adult patients with Wilson disease and will evaluate up to three doses of VTX-801. Patients will participate in a pre-dosing observational period and will be administered a prophylactic steroid regimen.

The primary endpoint of the GATEWAY trial is to assess the safety and tolerability of VTX-801 at 52 weeks after a single infusion. Additional endpoints include changes in disease-related biomarkers, including free serum copper and serum ceruloplasmin activity, as well as radiocopper-related parameters and VTX-801 responder status to allow standard-of-care withdrawal.

Vivet Therapeutics expects to enroll the first patient in early 2021.

More details on:

https://clinicaltrials.gov/ct2/show/NCT04537377?term=VIVET&draw=2&rank=1

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 recombinant adeno-associated virus (rAAV) 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 Medica Aplicada (CIMA), 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 (ODD) by the Food and Drug Administration (FDA) and the European Commission (EC). This rare genetic disorder is caused by mutations in the gene encoding the ATP7B protein, which reduces the ability of the liver and other tissues to regulate copper levels causing severe hepatic damages, neurologic symptoms and potentially death.

Vivet's second gene therapy product, VTX-803 for PFIC3, received US and European Orphan Drug Designation in May 2020.

Vivet is supported by international life science investors including Novartis Venture Fund, Roche Venture Fund, HealthCap, Pfizer Inc., Columbus Venture Partners, Ysios Capital, Kurma Partners and Idinvest Partners.

Please visit us on www.vivet-therapeutics.com and follow us on Twitter at @Vivet_tx and LinkedIn.

About Pfizer: Breakthroughs That Change Patients' Lives

At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development and manufacture of health care products, including innovative medicines and vaccines. Every day, Pfizer colleagues work across developed and emerging markets to advance wellness, prevention, treatments and cures that challenge the most feared diseases of our time. Consistent with our responsibility as one of the world's premier innovative biopharmaceutical companies, we collaborate with health care providers, governments and local communities to support and expand access to reliable, affordable health care around the world. For more than 170 years, we have worked to make a difference for all who rely on us. We routinely post information that may be important to investors on our website at www.Pfizer.com. In addition, to learn more, please visit us on www.Pfizer.com and follow us on Twitter at @Pfizer and @Pfizer News, LinkedIn, YouTube and like us on Facebook at Facebook.com/Pfizer.

Pfizer Disclosure Notice

The information contained in this release is as of November 18, 2020. Pfizer assumes no obligation to update forward-looking statements contained in this release as the result of new information or future events or developments.

This release contains forward-looking information about Vivet Therapeutics' (Vivet) investigational gene therapy, VTX-801, and Pfizer's collaboration with Vivet on the development of VTX-801, including their potential benefits, that involves substantial risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statements. Risks and uncertainties include, among other things, risks related to the ability to realize the anticipated benefits of the collaboration, including the possibility that the expected benefits from the collaboration will not be realized or will not be realized in the expected time; the uncertainties inherent in research and development, including the ability to meet anticipated clinical endpoints, commencement and/or completion dates for our clinical trials, regulatory submission dates, regulatory approval dates and/or launch dates, as well as the possibility of unfavorable new clinical data and further analyses of existing clinical data; the risk that clinical trial data are subject to differing interpretations and assessments by regulatory authorities;

whether regulatory authorities will be satisfied with the design of and results from the clinical studies; whether and when any applications may be filed in any jurisdiction for VTX-801; whether and when any such applications may be approved by regulatory authorities, which will depend on myriad factors, including making a determination as to whether the product's benefits outweigh its known risks and determination of the product's efficacy and, if approved, whether VTX-801 will be commercially successful; decisions by regulatory authorities impacting labeling, manufacturing processes, safety and/or other matters that could affect the availability or commercial potential of VTX-801; uncertainties regarding the impact of COVID-19 on Pfizer's business, operations and financial results; and competitive developments.

A further description of risks and uncertainties can be found in Pfizer's Annual Report on Form 10-K for the fiscal year ended December 31, 2019 and in its subsequent reports on Form 10-Q, including in the sections thereof captioned "Risk Factors" and "Forward-Looking Information and Factors That May Affect Future Results", as well as in its subsequent reports on Form 8-K, all of which are filed with the U.S. Securities and Exchange Commission and available at www.sec.gov and www.pfizer.com.