Sigilon Therapeutics Receives Orphan Drug Designation for SIG-005 for the Treatment of Mucopolysaccharidosis Type I

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Company plans to initiate a Phase 1/2 trial of SIG-005 in patients with mucopolysaccharidosis type I (MPS-1) in the second half of 2021

CAMBRIDGE, Mass., Dec. 17, 2020 (GLOBE NEWSWIRE) -- Sigilon Therapeutics, Inc. (NASDAQ: SGTX), a biotechnology company that seeks to develop functional cures for chronic diseases through its Shielded Living Therapeutics™ platform, today announced that the United States Food and Drug Administration (FDA) has granted Orphan Drug designation to SIG-005 for the treatment of mucopolysaccharidosis type I (MPS-1), a chronic, progressive lysosomal storage disease.

SIG-005 contains a human cell line genetically modified with a non-viral vector designed to express human a-L-iduronidase (IDUA), an enzyme which is missing or defective in patients with MPS-1. The IDUA enzyme is essential for the breakdown of glycosaminoglycans in the lysosomes of patients with MPS-1, resulting in progressive, multiorgan involvement.

“We are pleased that the FDA has granted Orphan Drug designation to SIG-005 for the treatment of MPS-1, a devastating disease for which treatment options are limited,” said Deya Corzo, M.D., Chief Medical Officer at Sigilon. “Like many lysosomal disorders, MPS-1 is a chronic and progressive disease that affects multiple systems throughout the body creating complications that are challenging to manage optimally.”

Added Rogerio Vivaldi, M.D., President and Chief Executive Officer of Sigilon: “This is the second of what we hope will be multiple Orphan Drug designations for Sigilon as we continue progressing novel therapies for chronic diseases through our pipeline based on our Shielded Living Therapeutics™ platform. We look forward to exploring the potential of SIG-005 in patients with MPS-1 and anticipate initiating a Phase 1/2 clinical trial in this patient population in the second half of 2021.”

Orphan drug designation is granted by the FDA Office of Orphan Products Development to advance the evaluation and development of safe and effective therapies for the treatment of rare diseases or conditions affecting fewer than 200,000 people in the United States. Having received the designation, Sigilon is eligible for various development incentives for SIG-005, including tax credits for qualified clinical trials and seven years of market exclusivity in the United States following commercial approval by the FDA. For more information about orphan designation, please visit the FDA website.

About Sigilon Therapeutics
Sigilon Therapeutics seeks to develop functional cures for chronic diseases through its Shielded Living Therapeutics™ platform. Sigilon’s product candidates consist of novel human cells engineered to produce the crucial proteins, enzymes or factors needed by patients living with chronic diseases such as hemophilia, diabetes and lysosomal storage disorders. The engineered cells are protected by Sigilon’s Afibromer™ biomaterials matrix, which shields them from immune rejection and fibrosis. Sigilon was founded by Flagship Pioneering in conjunction with Daniel Anderson, Ph.D., and Robert Langer, Sc.D., of the Massachusetts Institute of Technology.

Forward-Looking Statements

This press release contains forward-looking statements, including, without limitation, statements regarding the expected number of Orphan Drug designations and the timing of our clinical trials for SIG-005 for the treatment of MPS-1. Investors are cautioned not to place undue reliance on these forward-looking statements. The words “may,” “will,” “could,” “would,” “should,” “expect,” “plan,” “anticipate,” “intend,” “believe,” “estimate,” “predict,” “project,” “potential,” “continue,” “target” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements in this press release are based on management’s current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including, without limitation, those risks and uncertainties related to our product candidates, the initiation and enrollment for our clinical trials and the regulatory filings related thereto and other risks identified in our SEC filings, including our Prospectus filed with the SEC on December 7, 2020 and subsequent filings with the SEC. We caution you not to place undue reliance on any forward-looking statements, which speak only as of the date they are made. We disclaim any obligation to publicly update or revise any such statements to reflect any change in expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements. Any forward-looking statements contained in this press release represent our views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date.

Source: Sigilon Therapeutics, Inc.

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