



Spero Therapeutics Receives QIDP Designation from the U.S. FDA for the Development of SPR720

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Phase 1 top-line data readout for oral SPR720 expected in second half of 2019

CAMBRIDGE, Mass., Feb. 26, 2019 (GLOBE NEWSWIRE) -- Spero Therapeutics, Inc. (Nasdaq:SPRO), a multi-asset clinical-stage biopharmaceutical company focused on identifying, developing and commercializing novel treatments for multidrug-resistant bacterial infections, announced today that SPR720, an orally administered antimicrobial agent being developed for the treatment of non-tuberculous mycobacterial (NTM) infections, has been granted Qualified Infectious Disease Product (QIDP) designation by the U.S. Food and Drug Administration for the treatment of lung infections caused by non-tuberculous mycobacteria and lung infections caused by *Mycobacterium tuberculosis* (TB).

"We are pleased that the FDA has acknowledged the promise of SPR720 for the treatment of NTM and TB, by granting QIDP status for the compound," said Ankit Mahadevia, M.D., CEO of Spero Therapeutics. "SPR720 has the potential to become the first approved oral treatment for NTM infections, an area of high unmet need that requires prolonged therapy and where no specifically approved orally administered treatments exist."

The QIDP designation was created by the Generating Antibiotic Incentives Now (GAIN) Act and creates incentives for the development of certain antibiotics that treat serious or life-threatening infections. The primary incentives are an additional five-year extension of Hatch-Waxman Act exclusivity, as well as priority FDA review of the first marketing application or efficacy supplement for SPR720 and the indication for which QIDP designation was granted, and the opportunity to request Fast Track designation for SPR720.

SPR720 is currently being evaluated in a Phase 1 double-blind, placebo-controlled clinical trial designed to assess the safety, tolerability and pharmacokinetics of SPR720 in healthy volunteers. The advancement of SPR720 into this first-in-human clinical trial was based on cumulative data from pre-clinical safety, toxicology and ADME (absorption, distribution, metabolism and excretion) studies as well as nonclinical efficacy studies demonstrating potent activity for SPR720 *in vitro* and *in vivo* against TB and clinically important NTM species, including *Mycobacterium avium* complex and *Mycobacterium abscessus*. The collective pre-clinical data to date suggest that SPR720 has an acceptable safety profile, with encouraging activity against the target NTM and TB pathogens, drug distribution to key sites of infection, such as the lung, and a wide therapeutic margin. Spero expects to receive top-line data from the Phase 1 clinical trial in the second half of 2019.

SPR720 Research Support

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About SPR720

SPR720 represents a novel class of antibacterial agents that target enzymes essential for bacterial DNA replication. SPR720 was in-licensed from Vertex and is under development as an oral therapy for the treatment of non-tuberculous mycobacterial (NTM) infections. NTM are ubiquitous environmental pathogens that can cause progressive lung damage and respiratory failure, particularly in patients with compromised immune systems or underlying pulmonary disorders. Although rare, the incidence of pulmonary NTM infections is increasing worldwide. Treatment of pulmonary NTM infections requires prolonged therapy (continuing for approximately 12 to 24 months) with a combination regimen and is frequently complicated by tolerability and/or toxicity issues. Additionally, there are currently no oral antibiotics specifically approved for use to treat pulmonary NTM infections. Thus, if successfully developed, SPR720 has the potential to address an important unmet need as the first oral antibiotic approved for the treatment of this debilitating disease. SPR720 is currently in a Phase 1 clinical trial in healthy subjects and Spero expects to receive top-line data from the trial in the second half of 2019. Pre-clinical *in vitro* and *in vivo* studies have demonstrated the potency of SPR720 against a range of bacteria that cause TB and pulmonary NTM infections, including *Mycobacterium avium* complex and *Mycobacterium abscessus*, a highly resistant species responsible for high mortality. Spero believes that its intellectual property portfolio for SPR720 will provide protection globally, including in the United States and Europe, through 2033.

About Spero Therapeutics

Spero Therapeutics, Inc. is a multi-asset, clinical-stage biopharmaceutical company focused on identifying, developing and commercializing novel treatments for MDR bacterial infections.

Spero's lead product candidate, SPR994, is designed to be the first oral carbapenem-class antibiotic for use in adults to treat MDR Gram-negative infections.

Spero also has a platform technology known as its Potentiator Platform that it believes will enable it to develop drugs that will expand the spectrum and potency of existing antibiotics, including formerly inactive antibiotics, against Gram-negative bacteria. Spero's lead product candidates generated from its Potentiator Platform are two IV-administered agents, SPR206 and SPR741, designed to treat MDR Gram-negative infections in the hospital setting.

Spero is also advancing SPR720, its novel oral therapy product candidate designed for the treatment of non-tuberculous mycobacterial (NTM) infections.

For more information, visit <https://sperotherapeutics.com>.

Forward-Looking Statements

This press release may contain forward-looking statements. These statements include, but are not limited to, statements about the initiation, timing, progress and results of Spero's preclinical studies and clinical trials and its research and development programs, including statements regarding management's assessment of the results of the SPR720 preclinical studies and management's belief that SPR720 may demonstrate favorable clinical results and may be able to address an unmet medical need, the therapeutic potential of SPR720, the timing of clinical data, including the availability of interim data from the Phase 1 clinical trial of SPR720, Spero's cash forecast and anticipated expenses, the sufficiency of its cash resources and the availability of additional non-dilutive funding from governmental agencies beyond any initially funded awards. In some cases, forward-looking statements can be identified by terms such as "may," "will," "should," "expect," "plan," "aim," "anticipate," "could," "intent," "target," "project," "contemplate," "believe," "estimate," "predict," "potential" or "continue" or the negative of these terms or other similar expressions. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including whether results obtained in the SPR720 preclinical studies will be indicative of results obtained in future clinical trials; whether SPR720 will advance through the preclinical development and clinical trial process on a timely basis, or at all, taking into account the effects of possible regulatory delays, slower than anticipated patient enrollment, manufacturing challenges, clinical trial design and clinical outcomes; whether the results of such trials will warrant submission for approval from the U.S. Food and Drug Administration or equivalent foreign regulatory agencies; whether Spero's cash resources will be sufficient to fund its continuing operations for the periods and/or trials anticipated; Spero's ability to continue obtaining and maintaining intellectual property protection for its product candidates; and other factors discussed in the "Risk Factors" set forth in filings that Spero periodically makes with the U.S. Securities Exchange Commission. The forward-looking statements included in this press release represent Spero's views as of the date of this press release. Spero anticipates that subsequent events and developments will cause its views to change. However, while Spero may elect to update these forward-looking statements at some point in the future, it specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing Spero's views as of any date subsequent to the date of this press release.

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