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Tonix Pharmaceuticals Announces FDA Orphan-Drug Designation for TNX-2900 for the Treatment of Prader-Willi Syndrome

CHATHAM, N.J., March 03, 2022 (GLOBE NEWSWIRE) -- Tonix Pharmaceuticals Holding Corp. (Nasdaq: TNXP) (Tonix or the Company), a clinical-stage biopharmaceutical company, today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan-Drug Designation for TNX-2900* (intranasal potentiated oxytocin) for the treatment of Prader-Willi syndrome.

“Orphan-Drug Designation by the FDA is an important milestone and further validates our efforts to investigate the utility of TNX-2900 for Prader-Willi syndrome,” said Seth Lederman, M.D., Chief Executive Officer of Tonix Pharmaceuticals. “It underscores the urgent, unmet medical need for patients diagnosed with this disease, and will benefit us as we continue to advance our program.”

As recently announced, Tonix entered into a sponsored research agreement with Inserm Transfert, the private subsidiary of Inserm, on behalf of Inserm (the French National Institute of Health and Medical Research) and Aix-Marseille Université to study oxytocin in the genetically engineered mouse model of Prader-Willi syndrome, a rare genetic disorder that causes distinct, but related pathological eating disorders in adults and newborns. In adults, Prader-Willi causes hyperphagia, or pathological over-eating, which leads to obesity and other complications associated with significant mortality. In newborns, Prader-Willi causes a deficiency in suckling, which has been shown to be normalized by oxytocin treatment.

The FDA’s Office of Orphan Drug Products grants orphan status to the active moiety of drugs and biologics that demonstrate promise for the treatment of diseases or conditions affecting fewer than 200,000 people in the United States. Orphan drug designation provides Tonix Pharmaceuticals with certain development incentives, including tax credits for qualified clinical testing, exemptions from certain FDA application fees, and potential market exclusivity for seven years, if approved.

**TNX-2900 is an investigational new drug and has not been approved for any indication.*

About Prader-Willi Syndrome

Prader-Willi syndrome is recognized as the most common genetic cause of life-threatening childhood obesity[1] and affects males and females with equal frequency and all races and ethnicities. The hallmarks of Prader-Willi syndrome are lack of suckling in infants and, in children and adults, severe hyperphagia, an overriding physiological drive to eat, leading to severe obesity and other complications associated with significant mortality. There is currently no approved treatment for either the suckling deficit in babies or the obesity and

hyperphagia in older children associated with Prader-Willi syndrome.

[1]*Foundation for Prader-Willi Research (fpwr.org).*

About TNX-2900 and Tonix's Potentiated Oxytocin Platform

TNX-2900 is based on Tonix's patented intranasal potentiated oxytocin formulation intended for use by adults and adolescents. Tonix's patented potentiated oxytocin formulation is believed to increase specificity for oxytocin receptors relative to vasopressin receptors as well as to enhance the potency of oxytocin. Tonix is also developing a different intranasal formulation and device, designated TNX-1900, for prophylaxis of chronic migraine and for the treatment of insulin resistance and related conditions. Oxytocin is a naturally occurring human hormone that acts as a neurotransmitter in the brain. It was originally approved by the U.S. Food and Drug Administration as Pitocin®*, an intravenous infusion or intramuscular injection drug, for use in pregnant women to induce labor. An intranasal form of oxytocin was marketed in the U.S. by Novartis to assist in the production of breast milk as Syntocinon®** (oxytocin nasal 40 units/ml), but the product was discontinued, and the New Drug Application was withdrawn.

**Pitocin® is a trademark of Par Pharmaceutical, Inc.*

***Syntocinon® is a trademark of BGP Products Operations GmbH.*

About Tonix Pharmaceuticals Holding Corp.

Tonix is a clinical-stage biopharmaceutical company focused on discovering, licensing, acquiring and developing therapeutics and diagnostics to treat and prevent human disease and alleviate suffering. Tonix's portfolio is composed of immunology, infectious disease, and central nervous system (CNS) product candidates. Tonix's immunology portfolio includes biologics to address organ transplant rejection, autoimmunity and cancer, including TNX-1500[1] which is a humanized monoclonal antibody targeting CD40 ligand being developed for the prevention of allograft rejection treatment of autoimmune diseases. A Phase 1 study of TNX-1500 is expected to be initiated in the second half of 2022. Tonix's infectious disease pipeline includes next-generation vaccines to prevent COVID-19, an antiviral to treat COVID-19, and a potential treatment for Long COVID. The pipeline also includes a vaccine in development to prevent smallpox. Tonix's lead vaccine candidate for COVID-19, TNX-1800[2], is a live virus vaccine based on Tonix's recombinant pox vaccine (RPV) platform. TNX-3500[3] (sangivamycin, *i.v.* solution) is a small molecule antiviral drug to treat acute COVID-19 and is in the pre-IND stage of development. TNX-102 SL[4] , (cyclobenzaprine HCl sublingual tablets), is a small molecule drug being developed to treat Long COVID, a chronic post-COVID condition. Tonix expects to initiate a Phase 2 study in Long COVID in the first half of 2022. The Company's CNS portfolio includes both small molecules and biologics to treat pain, neurologic, psychiatric and addiction conditions. Tonix's lead CNS candidate, TNX-102 SL, is in mid-Phase 3 development for the management of fibromyalgia with a new Phase 3 study expected to start in the first half of 2022. Finally, TNX-1300[5] is a biologic designed to treat cocaine intoxication that is expected to start a Phase 2 trial in the first quarter of 2022.

[1]*TNX-1500 is an investigational new biologic and has not been approved for any indication.*

[2]TNX-1800 is an investigational new biologic and has not been approved for any indication. TNX-1800 is based on TNX-801, live horsepox virus vaccine for percutaneous administration, which is in development to protect against smallpox and monkeypox. TNX-801 is an investigational new biologic and has not been approved for any indication.

[3]TNX-3500 is an investigational new drug at the pre-IND stage of development and has not been approved for any indication.

[4]TNX-102 SL is an investigational new drug and has not been approved for any indication.

[5]TNX-1300 is an investigational new biologic and has not been approved for any indication.

This press release and further information about Tonix can be found at www.tonixpharma.com.

Forward-Looking Statements

Certain statements in this press release are forward-looking within the meaning of the Private Securities Litigation Reform Act of 1995. These statements may be identified by the use of forward-looking words such as “anticipate,” “believe,” “forecast,” “estimate,” “expect,” and “intend,” among others. These forward-looking statements are based on Tonix's current expectations and actual results could differ materially. There are a number of factors that could cause actual events to differ materially from those indicated by such forward-looking statements. These factors include, but are not limited to, risks related to the development of TNX-2900, the failure to obtain FDA clearances or approvals and noncompliance with FDA regulations; delays and uncertainties caused by the global COVID-19 pandemic; risks related to the timing and progress of clinical development of our product candidates; our need for additional financing; uncertainties of patent protection and litigation; uncertainties of government or third party payor reimbursement; limited research and development efforts and dependence upon third parties; and substantial competition. As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. Tonix does not undertake an obligation to update or revise any forward-looking statement. Investors should read the risk factors set forth in the Annual Report on Form 10-K for the year ended December 31, 2020, as filed with the Securities and Exchange Commission (the “SEC”) on March 15, 2021, and periodic reports filed with the SEC on or after the date thereof. All of Tonix's forward-looking statements are expressly qualified by all such risk factors and other cautionary statements. The information set forth herein speaks only as of the date thereof.

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