Traws Pharma Announces Positive Topline Phase 1 Data for Flu Candidate, Tivoxavir Marboxil

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Investigational agent in development as a one dose treatment or prevention of seasonal and pandemic influenza

A dose ranging, Phase 1 study in healthy volunteers demonstrated positive tolerability results and plasma levels in the predicted therapeutic window, enabling selection of Phase 2 dose

Preclinical data showed potent inhibition of drug-resistant and bird influenza viruses

Phase 2 study expected to begin in H1 2025

Improved therapy is an important need for both seasonal and pandemic flu

NEWTOWN, Pa., Oct. 08, 2024 (GLOBE NEWSWIRE) -- Traws Pharma, Inc. (NASDAQ: TRAW) ("Traws Pharma", "Traws" or "the Company"), a clinical-stage biopharmaceutical company developing oral small molecule therapies for the treatment of respiratory viral diseases, today announced positive topline Phase 1 safety and pharmacokinetic results for its investigational one-dose influenza (flu) therapy, tivoxavir marboxil (tivoxavir). Tivoxavir was designed as a potential best-in-class inhibitor of the highly-conserved influenza protein, CAP-dependent endonuclease (CEN), intended for use across a broad range of flu viruses.

"Topline data from the Phase 1 healthy volunteer study showed good overall tolerability and a pharmacokinetic profile that appears to support tivoxavir's potential use as a one-time treatment for flu, including pandemic flu. We are especially pleased with data showing that a single dose of tivoxavir maintained plasma drug levels above the EC_{90} for greater than 5 days[1]," said **Werner Cautreels, PhD, Chief Executive Officer** of Traws Pharma. "In addition, preclinical data show that tivoxavir is a potent inhibitor of drug-resistant influenza and bird flu viruses[1]. Together, these initial results suggest that tivoxavir has the potential to be developed as a best-in-class agent for influenza. With these data, we plan to advance the program to a Phase 2 study in H1 2025."

"Influenza is a substantial public health burden in the US[2], with a disproportionate impact on older adults and vulnerable populations. Data from the last influenza season showed that flu-related hospitalizations[3] and mortality[4] were highest, by approximately three times, among people 65 years of age or older[5]. Recent bird flu outbreaks, and the risk that this highly pathogenic virus (H5N1) poses for evolving into a pandemic outbreak[6], also signal the need for new antiviral treatments," said **Robert R. Redfield, MD, Chief Medical Officer** for Traws Pharma and former Director of the U.S. Centers for Disease Control and Prevention (CDC). "Our enthusiasm for tivoxavir is based on the data announced today and the need for novel therapeutics, especially as valuable resources in case of an avian flu outbreak or a pandemic, and for potential use to prevent virus spread in households and congregant settings."

"We selected CEN as the target for our flu program because it is conserved among human and avian influenza viruses, including the highly pathogenic H5N1. As a result, we believe tivoxavir has the potential to suppress a wide range of viruses. We are very pleased that tivoxavir demonstrated broad activity in laboratory studies against drug-resistant viruses and highly pathogenic strains such as avian flu[1]. In addition, we believe that preferential uptake in the lung is another important feature of a candidate agent. Preclinical data indicate that a single dose of tivoxavir has more than 15X higher accumulation in the lung compared to plasma[1]," said **C. David Pauza, PhD, Chief Science Officer** for Traws Pharma. "Positive Phase 1 data showing that tivoxavir achieved plasma blood levels in healthy subjects that are consistently above the EC_{90} and within the predicted therapeutic window for more than five days lead to the identification of our Phase 2 dose, supporting further development of the program."

Topline Phase 1 Results

The Phase 1 trial was a randomized, double-blind, placebo-controlled study to assess the safety, tolerability, pharmacokinetics (PK) and pharmacodynamics (PD) of ascending doses of one-time tivoxavir marboxil treatment in healthy, influenza-negative, adult volunteers.

At the identified Phase 2 dose, no treatment related adverse events were reported during the Phase 1 study. Topline data from this study showed that a single dose of tivoxavir marboxil maintained plasma drug levels consistently above the EC_{90} for more than five days and within the predicted therapeutic window. Preclinical studies showed that tivoxavir marboxil demonstrated potent inhibition of drug-resistant influenza viruses, as well as potent inhibition of highly pathogenic bird flu viruses[1].

About Tivoxavir Marboxil

Seasonal influenza is estimated to represent a multi-billion antiviral market opportunity, largely driven by global health organizations, practice guidelines and government tenders[1], with upside potential from pandemic flu outbreaks. Tivoxavir marboxil (also known as 27-5116 or TRX-100) was designed as an inhibitor of the highly conserved influenza protein, CAP-dependent endonuclease (CEN). It has demonstrated potent *in vitro* activity against a range of influenza strains, including the highly pathogenic avian flu, in preclinical studies. The drug candidate's Phase 1 pharmacokinetic (PK) profile in healthy subjects, including the ability to achieve plasma levels that are consistently above the EC_{90} (as determined in laboratory studies), for more than five days and within the predicted therapeutic window, may enable a single dose treatment regimen. These data, combined with good overall tolerability results in healthy subjects, support further development of tivoxavir marboxil as a one-time treatment for influenza.

Source information:

1. TRAW data on file
2. Flu is burdensome
3. Flu hospitalizations
4. Flu mortality
5. Flu in older adults
6. Avian flu

About Traws Pharma, Inc.
Traws Pharma is a clinical stage biopharmaceutical company developing potential oral small molecule therapies for the treatment of respiratory viral diseases and cancer. The viral respiratory disease program includes two oral, novel, Phase 1, potentially best-in-class, small molecule drug candidates: tivoxavir marboxil, in development for flu and pandemic flu, targeting the influenza cap-dependent endonuclease (CEN); and ratutrelvir, in development as a COVID treatment, targeting the Mpro (3CL protease), without the need for co-administration of ritonavir.

In the cancer program, Traws is utilizing a partnering strategy, supported by investigator sponsored studies, to advance two novel proprietary multi-kinase inhibitors, narazaciclib, targeting CDK4+, and rigosertib, targeting cell cycle proteins including PLK-1.

Traws is committed to delivering novel compounds for unmet medical needs using state-of-the-art drug development technology. With a focus on product safety and a commitment to patients in need or that are specifically vulnerable, we aim to build solutions for important medical challenges and alleviate the burden of viral infections.

Forward-Looking Statements

Some of the statements in this release are forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, Section 21E of the Securities Exchange Act of 1934, as amended, and the Private Securities Litigation Reform Act of 1995, and involve risks and uncertainties including statements regarding the Company, its business and product candidates, including the potential opportunity, benefits and the regulatory plans for tivoxavir marboxil. The Company has attempted to identify forward-looking statements by terminology including “believes”, “estimates”, “anticipates”, “expects”, “plans”, “intends”, “may”, “could”, “might”, “will”, “should”, “preliminary”, “encouraging”, “approximately” or other words that convey uncertainty of future events or outcomes. Although Traws believes that the expectations reflected in such forward-looking statements are reasonable as of the date made, expectations may prove to have been materially different from the results expressed or implied by such forward looking statements. These statements are only predictions and involve known and unknown risks, uncertainties, and other factors, including the success and timing of Traws’ clinical trials, collaborations, market conditions and those discussed under the heading “Risk Factors” in Traws’ filings with the U.S. Securities and Exchange Commission (SEC). Any forward-looking statements contained in this release speak only as of its date. Traws undertakes no obligation to update any forward-looking statements contained in this release to reflect events or circumstances occurring after its date or to reflect the occurrence of unanticipated events.

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