Traws Pharma Announces Positive Topline Phase 1 Results for COVID Candidate, Ratutrelvir, an Oral Mpro Inhibitor

Sep 30, 2024

Ratutrelvir was well-tolerated for 10 days and achieved consistent plasma levels in the predicted therapeutic window, without the need for co-administration of ritonavir

Phase 2a study expected to begin in H1 2025 in patients with COVID

Improving COVID care is an ongoing need, with approximately 50,000 US deaths in 2023

NEWTOWN, Pa., Sept. 30, 2024 (GLOBE NEWSWIRE) -- Traws Pharma, Inc. (NASDAQ: TRAW) ("Traws Pharma", "Traws" or "the Company"), a clinical-stage biopharmaceutical company developing potential oral small molecule therapies for the treatment of respiratory viral diseases, today announced positive topline Phase 1 results for its potential best-in-class COVID (SARS-CoV-2) candidate, ratutrelvir, an oral inhibitor of the Main protease (Mpro).

"Topline data indicate that administration of ratutrelvir, our product candidate for COVID, as monotherapy, for 10 days to healthy volunteers, showed no treatment related adverse events and demonstrated consistent plasma drug levels in the predicted therapeutic window. We are especially pleased by ratutrelvir's ability to achieve plasma concentrations that are considerably above the EC_{90} against a comprehensive panel of SARS-CoV-2 viruses, without the need for ritonavir co-administration that can be a source of drug-drug interactions and potential severe side effects," said **Werner Cautreels, PhD, Chief Executive Officer** of Traws Pharma. "These data provide us with further indication that ratutrelvir has the potential to be a potent, best-in-class, once-a-day, single-dose, 10-day antiviral therapy for COVID. This profile contrasts with Paxlovid ™, an approved Mpro inhibitor, which requires co-administration of ritonavir, a metabolic inhibitor. We believe that ratutrelvir's ritonavir-free regimen has the potential to reduce the burden of treatment, especially for patients with underlying medical conditions. Based on the Phase 1 data, we have selected the dose for our Phase 2a study, expected to begin in H1 2025."

"As we near the fifth anniversary of the pandemic, it is notable that, despite the wide availability of approved antiviral therapies, COVID was a major cause of mortality in the US in 2023, with approximately 50,000 deaths[1]," 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). "We believe it is important for new COVID therapies to have a simple treatment regimen that can be used broadly, especially in patients who are at higher risk of serious symptoms or who are over 65 years of age with underlying medical conditions including heart disease, kidney disease, and chronic lung disease[2]. In addition, we consider activity against resistant viruses and low risk of clinical rebound to be important features of potential new COVID therapies. Our enthusiasm for ratutrelvir's potential to meet the need for improved COVID treatment has been enhanced by the Phase 1 data."

"Our goal for ratutrelvir is to effectively treat COVID, limit the symptoms of infection, and lower the risk for clinical rebound," said **C. David Pauza, PhD, Chief Scientific Officer** for Traws Pharma. "Preclinical studies, presented at the annual International Conference on Antiviral Research in 2024 (ICAR2024), showed that ratutrelvir monotherapy has differentiated activity compared to nirmatrelvir against a range of drug-resistant viruses. Also, preclinical testing in animal models showed that levels of ratutrelvir in the lung were higher than in plasma. Phase 1 data show that ratutrelvir achieved consistent plasma levels in the predicted therapeutic window, with a pharmacokinetic profile that may reduce the likelihood of clinical rebound."

Topline Phase 1 Results

The Phase 1 trial (NCT06402136, conducted in Australia) was designed as a randomized, double-blind, placebo-controlled study to assess the safety, tolerability, pharmacokinetics (PK) and pharmacodynamics (PD) of single- and multiple ascending doses (SAD and MAD) of ratutrelvir, administered as a capsule formulation in 56 healthy, COVID-negative, adult volunteers, randomized three-to-one (eight subjects per dosing group). The SAD portion of the study evaluated five ratutrelvir dose levels; the MAD segment evaluated two ratutrelvir dose levels, administered once daily for 10 days.

This first-in-human study showed no treatment related adverse events reported up to the highest dose. Topline data also showed that once-daily administration of ratutrelvir maintained plasma drug levels within the predicted therapeutic window. Preclinical studies, presented at the annual International Conference on Antiviral Research in 2024 (ICAR2024), showed that ratutrelvir demonstrated differentiated activity compared to nirmatrelvir against a range of SARS-CoV-2 variants, based on a comparison of EC_{50} values.

About Ratutrelvir

Industry data indicate that COVID treatment represents a potential multi-billion dollar market opportunity. Ratutrelvir (also previously known as 83-0060 or TRX-01) was designed as an inhibitor of the SARS-CoV-2 Main protease (Mpro or 3CL protease). It has demonstrated *in vitro* activity against the original strain of the virus as well as the delta and omicron variants, and is more active than nirmatrelvir (Pfizer's Mpro inhibitor, co-packaged with ritonavir as PAXLOVID ™) in preclinical studies. Based on preclinical studies, ratutrelvir did not require co-administration with a metabolic inhibitor, such as ritonavir, which inhibits human cytochrome P450 (CYP) 3A4. Because of this, ratutrelvir is expected to avoid ritonavir-associated drug-drug interactions and potential resulting severe side effects, which may permit wider patient use. The drug candidate's pharmacokinetic (PK) profile, including the ability to achieve plasma levels within the predicted therapeutic window, as demonstrated in the Phase 1 study, may also enable a once daily treatment regimen and reduce the likelihood of clinical rebound.

Source information:

1. https://www.nbcnews.com/health/health-news/covid-fell-10th-leading-cause-death-last-year-4th-rcna165775
2. https://www.mayoclinic.org/diseases-conditions/coronavirus/in-depth/coronavirus-who-is-at-risk/art-20483301

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 Phase 1 potentially best-in-class oral small molecules in development: tivoxavir marboxil, a novel oral antiviral drug candidate for influenza and avian flu, targeting the influenza cap-dependent endonuclease, and ratutrelvir, targeting Mpro (3CL protease) for COVID.

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 and cancer.

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 Traws regulatory plans for ratutrelvir. 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, merger integration, 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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