Organovo Announces Positive Phase 2 Results for FXR314 in Metabolic Dysfunction-Associated Steatohepatitis (MASH) Showing Both Reduction in Liver Fat Content and Strong Safety and Tolerability Compared to Placebo

April 15, 2024 at 8:05 AM EDT

SAN DIEGO, April 15, 2024 (GLOBE NEWSWIRE) -- Organovo Holdings, Inc. (Nasdaq:ONVO), a clinical stage biotechnology company focused on developing novel treatment approaches based on demonstration of clinical promise in three-dimensional (3D) human tissues, today released the complete details of its 16-week, randomized, placebo-controlled, multi-center Phase 2 study of the non-steroidal, non-bile acid FXR agonist FXR314 for the treatment of metabolic function-associated steatohepatitis (MASH). Study results demonstrated statistically significant reduction in liver fat content from baseline in patients receiving FXR314 compared to placebo.

Study subjects receiving FXR314 achieved statistically significant reduction in liver fat content from baseline, with LS mean percent reduction at end of treatment of 22.8% (p=0.0010) with 3 mg and 17.5% (p=0.0267) with 6 mg doses of FXR314 compared to 6.1% in the placebo group. The proportion of subjects with >30% magnetic resonance imaging-derived proton density fat fraction (MRI-PDFF) reduction was 29.2% (p=0.0023) and 32.2% (p=0.0020) for 3 mg and 6 mg FXR314, respectively, compared to 9.5% with placebo. Investigators observed improvements in hepatocellular damage and liver function based on serological measures, with no evidence of worsening of liver fibrosis.

	FXR314 3 mg	**FXR314 6 mg**	**Placebo**
Liver fat reduction (LS mean reduction from baseline, SE)	22.8 + 3.6% p=0.0010	17.5 + 3.7% p=0.0267	6.1 + 3.5%
Subjects with >30% MRI-PDFF reduction	29.2% p=0.0023	32.2% p=0.0020	9.5%
Pruritus	2.8%	4.2%	2.8%
Pruritus-related treatment discontinuation	0%	0%	0%

FXR314 was also found to be safe and well tolerated. Treatment-emergent adverse events were mostly mild to moderate in severity, with incidence comparable between FXR314 3 mg, 6 mg, and placebo. Drug-related treatment discontinuation was minimal and similar across groups. There was also no evidence with FXR314 of adverse events considered common in the FXR class, including measures of pruritus (3 mg 2.8%, 6 mg 4.2% and placebo 2.8%) and LDL-C levels (change from baseline of 1.5%, 4.5% and -3.6% for 3mg, 6mg, and placebo groups respectively).

“The key findings of this study are that once daily oral FXR314 demonstrated statistically significant liver fat reduction and excellent tolerability. Whereas other FXR agonists have had challenges in providing clear benefit without significant pruritus or other adverse events, or have had lack of efficacy potentially related to lack of sustained exposure, we are pleased to note FXR314’s data demonstrate it clearly rises above previous problems seen with the class,” stated Keith Murphy, Organovo’s Executive Chairman. “Given these exciting findings, we believe the data are supportive of further clinical development of FXR314 in MASH.”

The clinical trial evaluated the safety, tolerability, and pharmacological activity of FXR314, as measured by reductions in liver fat content with magnetic resonance imaging-derived proton density fat fraction (MRI-PDFF), changes in liver enzymes, low-density lipoprotein cholesterol (LDL-C) levels, and incidence of pruritus. The treatment population were MASH patients diagnosed via biopsy, magnetic resonance elastography (MRE), or transient elastography (TE FibroScan), and who had liver fat content ≥ 10% as measured by MRI-PDFF. A total of 214 patients were randomized in a 1:1:1 ratio to either 3 mg or 6 mg of FXR314, or placebo. Treatment was administered orally once daily for 16 weeks. The Company expects that detailed findings of this study (Clinical trial registry NCT047773964) will be presented at an upcoming conference.

About Organovo

Organovo is a clinical stage biotechnology company that is developing drugs that are demonstrated to be effective in three-dimensional (3D) human tissues as candidates for drug development. The company’s lead molecule, FXR314, is on the path for Phase 2 investigation in inflammatory bowel disease and has applications in metabolic liver disease and oncology. The company has proprietary technology used to build 3D human tissues that mimic key aspects of native human tissue composition, architecture, function, and disease. For more information visit Organovo's website at www.organovo.com.

Forward Looking Statements

Any statements contained in this press release that do not describe historical facts constitute forward-looking statements as that term is defined in the Private Securities Litigation Reform Act of 1995. Any forward-looking statements contained herein are based on current expectations but are subject to a number of risks and uncertainties. These risks and uncertainties and other factors are identified and described in more detail in the Company's filings with the SEC, including its Annual Report on Form 10-K filed with the SEC on July 14, 2023, as such risk factors are updated in its most recently filed Quarterly Report on Form 10-Q filed with the SEC on November 9, 2023. You should not place undue reliance on these forward-looking statements, which speak only as of the date that they were made. These cautionary statements should be considered with any written or oral forward-looking statements that the Company may issue in the future. Except as required by applicable law, including the securities laws of the United States, the Company does not intend to update any of the forward-looking statements to conform these statements to reflect actual results, later events, or circumstances or to reflect the occurrence of unanticipated events.

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