Organovo Receives Orphan Designation From U.S. FDA for 3D Bioprinted Therapeutic Liver Tissue Treatment of Alpha-1 Antitrypsin Deficiency

December 26, 2017

SAN DIEGO, Dec. 26, 2017 (GLOBE NEWSWIRE) -- Organovo Holdings, Inc. (NASDAQ:ONVO) ("Organovo") today announced that the U.S. Food and Drug Administration ("FDA") granted orphan drug designation for the Company's treatment of alpha-1 antitrypsin deficiency ("A1AT") with its 3D bioprinted liver therapeutic tissue.

"We are extremely pleased to receive orphan designation for our NovoTissues® treatment of A1AT," said Taylor J. Crouch, CEO, Organovo. "The FDA's rapid action recognizes the importance of developing regenerative medicine therapeutic applications, and mirrors our own urgency in addressing this devastating disease. With tens of thousands of patients being treated for inborn errors of metabolism ("IEMs") in the U.S., and an annual cost per patient that exceeds $250,000 for drug therapy alone, these patient populations are in desperate need of new treatment options."

Crouch concluded, "This is a critical milestone that supports our ongoing development of 3D bioprinted tissues for therapeutic use. We remain on track for filing an Investigational New Drug ("IND") application with the FDA in calendar-year 2020, as we continue to conduct safety and dosing investigations in small animal disease models and move to defining and scoping IND enabling studies."

The FDA Orphan Drug designation program provides incentives to sponsors that are developing therapies for rare diseases which affect fewer than 200,000 people in the United States. Organovo is now qualified to receive significant benefits throughout its orphan drug development program including more frequent FDA interactions, protocol assistance, and tax credits for clinical research costs. The designation also includes a waiver of certain fees and a seven-year term of market exclusivity upon FDA approval of the orphan drug, and can provide for a more streamlined and cost-effective path through to commercialization.

About Organovo Holdings, Inc.

Organovo designs and creates functional, three-dimensional human tissues for use in drug discovery, clinical development, and therapeutic applications. The Company develops 3D human tissue systems through internal research programs and in collaboration with pharmaceutical, academic and other partners. Organovo's 3D human tissues have the potential to transform the drug discovery process, enabling treatments to be developed more effectively and with greater relevance to performance in human trials and commercialization. The Company's ExViveTM Human Liver and Kidney Tissues are used in high-value drug profiling, including compound screening in disease models, toxicology, target and marker discovery/validation, and other drug testing. The Company is also advancing a preclinical program to develop its NovoTissues® liver therapeutic tissues for critical unmet medical needs, including certain life-threatening pediatric diseases. In addition to numerous scientific publications, the Company's technology has been featured in The Wall Street Journal, Time Magazine, The Economist, Forbes, and numerous other media outlets. Organovo is changing the shape of life science research and transforming medical care. Learn more 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. The factors that could cause the Company's actual future results to differ materially from current expectations include, but are not limited to, risks and uncertainties relating to the Company's ability to develop, market and sell products and services based on its technology; the expected benefits and efficacy of the Company's products, services and technology; the Company's ability to successfully complete studies and provide the technical information required to support market acceptance of its products, services and technology, on a timely basis or at all; the Company's business, research, product development, regulatory approval, marketing and distribution plans and strategies, including its use of third party distributors; the Company's ability to secure additional contracted collaborative relationships; the final results of the Company's preclinical studies may be different from the Company's studies or interim preclinical data results and may not support further clinical development of its therapeutic tissues; the Company may not successfully complete the required preclinical and clinical trials required to obtain regulatory approval for its therapeutic tissues on a timely basis or at all; the Company's ability to control the costs and to achieve the expected operational benefits and long-term cost savings of its previously announced restructuring plan; and the Company's ability to meet its fiscal year 2018 outlook. These 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 June 7, 2017. 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.

Investor Contact:
Steve Kunszabo
Organovo Holdings, Inc.
+1 (858) 224-1092
skunszabo@organovo.com

Press Contact:
Jessica Yingling

Little Dog Communications
+1 (858) 344-8091
jessica@litldog.com

Organovo, Inc.