



Organovo Announces Target Validation Ahead of Schedule, Accelerating Timeline for First Crohn's Disease Drug Candidates

November 15, 2022

SAN DIEGO, Nov. 15, 2022 (GLOBE NEWSWIRE) -- Organovo Holdings, Inc. (Nasdaq: ONVO), a three-dimensional biology (3D biology) company focused on delivering scientific and medical breakthroughs using novel technologies including 3D bioprinting, today announced that it has successfully advanced the use of its first inflammatory bowel disease (IBD) model and has achieved the next milestone, target validation. The company announced in May 2022 that it had achieved a successful Crohn's disease model that demonstrates key aspects of patient biology that differ from a non-diseased state and that the model would be used to find and characterize therapeutics. The Crohn's disease model has since been utilized to understand the biology of disease and identify specific gene targets. Treating some of those targets, by introducing a compound directly to the 3D tissue model that affects the target, has now been shown to reproducibly reduce disease. Multiple targets are now considered validated, and they represent specific druggable opportunities that the company plans to leverage. Organovo plans to advance at least one target program to medicinal chemistry by the end of 2022 to build a proprietary new drug for Crohn's disease.

"The Organovo team has successfully identified disease regulated genes from our Crohn's disease model and importantly, identified a subset that are disease-driver genes," said Jeff Miner, Ph.D., Organovo's Chief Scientific Officer. "These disease-drivers directly affect the disease when modulated and represent superb therapeutic targets" he added.

Organovo Executive Chairman Keith Murphy commented, "Organovo's progress towards our goal has been driven by a strong, dedicated scientific team that has continued to rapidly advance our programs. We are pleased to have this step completed earlier than expected, and plan to continue to meet or exceed stockholder expectations as we move therapeutics to the clinic to meet unmet medical needs."

In September 2020, Organovo announced that it had updated its business model to capitalize on its human 3D tissue technologies in drug discovery, building disease models to find effective therapies that are more likely to succeed in the clinic than drugs discovered using animal models. We have focused on the creation of three-dimensional intestinal disease models to discover therapeutic opportunities that leverage the insights possible from the truer human biology achievable in 3D human disease models.

More than 1 million people in North America suffer from Crohn's and its prevalence is increasing globally. Because of its progressive nature, more than 70% will require at least one surgical intervention to relieve symptoms. Current drugs can slow progression in about 50% of patients, but none are curative. Defects in the intestinal lining and inflammation can lead to abdominal pain, severe diarrhea, fatigue, weight loss, malnutrition, and lower quality of life. Chronic inflammation leads to ulcers, fistulas and bowel obstructions caused by fibrosis. Organovo believes that its 3D tissue technologies are well suited to drug and target discovery in IBD because they contain a functional intestinal epithelium that is affected by disease and a stromal layer demonstrating disease-dependent fibrosis, key targets for therapy.

The drug discovery process using 3D tissues consists of several stages. Two early steps that are uniquely important in Organovo's approach to drug discovery are creation of a disease model followed by target discovery and validation. The steps involved in each require groundbreaking science that involves the application of tools specific to our approach. We believe the achievement of these goals represents a strong step forward and lowering of risk. The remaining steps after validation of a target are typical pharma development steps to advance a drug to Phase 1 clinical trials. The next phase will be medicinal chemistry, the creation and evaluation of new chemical entities to pick the best drug candidate. Organovo plans to do this work through an outside contract research organization. Organovo is on track to have its medicinal chemistry program result in an investigational new drug (IND) application by 2025 in alignment with our previously communicated objective to have multiple INDs by that time.

Medicinal chemistry, preclinical safety studies, and other common tasks that follow consist of common practices across pharmaceutical development, and are not unique to 3D drug discovery, but can benefit greatly from the additional insights that can be provided by testing candidates in human tissue 3D models as the candidates are screened and developed. Since these steps are more typical, the risk profile is similar to traditional pharmaceutical development at these stages. However, upon entering the clinic and seeking evidence of efficacy in humans, we believe that our use of human 3D disease models to select drug candidates will result in significantly higher probability of success.

Organovo is working on additional IBD disease models representing different aspects of disease, each of which can represent a significant advancement over previous tools. We will also seek to leverage validated targets and scientific knowledge from these disease models into business development deals and partnerships with pharmaceutical companies.

About Organovo

Organovo is an early-stage biotechnology company that is developing and utilizing highly customized 3D human tissues as dynamic models of healthy and diseased human biology for drug development. The company's proprietary technology is being used to build functional 3D human tissues that mimic key aspects of native human tissue composition, architecture, function and disease. Organovo's advances include cell type-specific compartments, prevalent intercellular tight junctions, and the formation of microvascular structures. Management believes these attributes can enable critical complex, multicellular disease models that can be used to develop clinically effective drugs for selected therapeutic areas.

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 June 10, 2022, as such risk factors are updated in its most recently filed Quarterly Report on Form 10-Q filed with the SEC on November 10, 2022. 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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Source: Organovo, Inc.