Enanta Pharmaceuticals Announces Topline Results Showing EDP-938 Achieved its Primary and Secondary Endpoints in its Phase 2a Human Challenge Study in Healthy Adults Infected with Respiratory Syncytial Virus (RSV)

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- *EDP-938 achieved highly statistically significant (p<0.001) reductions in viral load and in resolution of clinical symptoms compared to placebo*
- *Conference call and webcast to discuss the data at 8:30 a.m. ET today*

WATERTOWN, Mass.--(BUSINESS WIRE)-- Enanta Pharmaceuticals, Inc. (NASDAQ:ENTA), a research and development-focused biotechnology company dedicated to creating small molecule drugs for viral infections and liver diseases, today announced topline results that demonstrated that EDP-938 achieved highly statistically significant (p<0.001) reductions in viral load and in resolution of clinical symptoms compared to placebo in its Phase 2a human challenge study of EDP-938 in healthy adults infected with respiratory syncytial virus (RSV).

“RSV is a serious unmet medical need with no therapeutic treatment currently available,” stated Jay R. Luly, Ph.D., President and Chief Executive Officer. “Based on today’s positive data for EDP-938, the only N-inhibitor in clinical development, our goal is to initiate our first Phase 2b study by the end of calendar 2019 in adult outpatients with confirmed RSV infections.”

EDP-938 Phase 2a Challenge Study Topline Results
The Phase 2a study was a randomized, double-blind, placebo-controlled, human challenge study in healthy adult subjects inoculated with RSV.

Subjects were randomized into 1 of 2 dosing arms or a placebo arm and received either a once-daily (QD) 600 mg dose, a single 500 mg loading dose (LD) followed by a 300 mg twice daily (BID) dose, or placebo for 5 days.

A total of 115 subjects were enrolled and inoculated with the challenge virus. Once RSV infection was confirmed, participants were then randomized: 38 in the placebo arm, 39 in the 600 mg QD arm (one subject was randomized, but never dosed), and 38 in the 500mg LD plus 300mg BID arm.

A highly statistically significant reduction was observed for the primary efficacy endpoint, the area under the curve (AUC) for viral load in the intent-to-treat-infected population (ITT-I: all randomized subjects receiving challenge virus and at least one dose of study drug with confirmed RSV infection) for each of the EDP-938 dosing groups as compared with placebo. Specifically, EDP-938 lowered viral load AUC to 203.95 ± 173.50 hours x Log_{10} copies/mL in the QD arm and 217.71 ± 217.55 hours x Log_{10} copies/mL in the BID arm, compared to 790.15 ± 408.80 hours x Log_{10} copies/mL in the placebo arm(p<0.001 for each of the EDP-938 groups compared to placebo). There was no statistically significant difference between the two EDP-938 dosing groups.

For the key secondary efficacy endpoint, the AUC for total symptom score (TSS), a highly statistically significant reduction was observed in the ITT-I population for each of the EDP-938 dosing groups (124.47 hours x score ± 115.60 for the QD arm and 181.75 ± 248.42 hours x score for the BID arm, compared to 478.75 ± 422.29 hours x score in the placebo arm (p<0.001 for each of the EDP-938 groups compared to placebo). There was no statistically significant difference between the two EDP-938 dosing groups.

EDP-938 demonstrated good pharmacokinetics, and mean trough levels of drug were maintained at approximately 20-40x above the *in vitro* EC90 for RSV-infected human cells.

Overall, EDP-938 was generally safe and well tolerated. EDP-938 demonstrated a favorable safety profile over 5 days of dosing through Day 28 of follow-up, comparable to placebo for both dosing groups. There were no serious adverse events and no discontinuation of study drug.

The study will be presented at a future medical conference.

Conference Call and Webcast Information
Enanta will host a conference call and webcast today at 8:30 a.m. ET. To participate in the live conference call, please dial (855) 840-0595 in the U.S. or (518) 444-4814 for international callers. A replay of the conference call will be available starting at approximately 11:30 a.m. ET on June 14, 2019, through 11:59 p.m. ET on June 16, 2019 by dialing (855) 859-2056 from the U.S. or (404) 537-3406 for international callers. The passcode for both the live call and the replay is 1274559. A live audio webcast of the call and replay can be accessed by visiting the “Events and Presentation” section on the “Investors” page of Enanta’s website at www.enanta.com.

About EDP-938
EDP-938, Enanta’s lead N-protein inhibitor, is being developed for the treatment of RSV infection. Enanta believes EDP-938 is differentiated from fusion inhibitors currently in development by others for RSV because this N-protein inhibitor targets the virus’ replication machinery and has demonstrated high barriers to resistance against the virus *in vitro*. EDP-938 has also been shown to reduce viral load below the level of detection *in vivo*. Additionally, it is possible that N-protein inhibitors may be effective treatments at later stages of infection.

About Respiratory Syncytial Virus

Respiratory syncytial virus (RSV) is a virus that infects the lungs and represents a serious unmet medical need in infants and children. RSV is the most common cause of bronchiolitis (inflammation of the small airways in the lung) and pneumonia in children under 1 year of age in the United States. Also at increased risk of a severe RSV infection are children with compromised (weakened) immune systems due to a medical condition or medical treatment, adults with compromised immune systems and those age 65 and older. Recent estimates suggest that approximately 200,000 hospitalizations in the U.S. and EU occur each year in children under the age of two and approximately 170,000 hospitalizations in these regions occur in each year in adults aged 65 and older. There is currently no safe and effective therapy for already established RSV infection.

About Enanta
Enanta Pharmaceuticals is using its robust, chemistry-driven approach and drug discovery capabilities to become a leader in the discovery and development of small molecule drugs for the treatment of viral infections and liver diseases. Enanta's research and development efforts are currently focused on the following disease targets: respiratory syncytial virus (RSV), non-alcoholic steatohepatitis (NASH)/ primary biliary cholangitis (PBC), and hepatitis B virus (HBV).

Enanta's research and development activities are funded by royalties from HCV products developed under its collaboration with AbbVie. Glecaprevir, a protease inhibitor discovered by Enanta, is now sold by AbbVie in numerous countries as part of its newest treatment for chronic hepatitis C virus (HCV) infection. This leading HCV regimen is sold under the tradenames MAVYRET™ (U.S.) and MAVIRET™ (ex-U.S.) (glecaprevir/pibrentasvir) Please visit www.enanta.com for more information.

Forward Looking Statements Disclaimer
This press release contains forward-looking statements, including statements with respect to the prospects for further development with respect to EDP-938 for RSV. Statements that are not historical facts are based on management's current expectations, estimates, forecasts and projections about Enanta's business and the industry in which it operates and management's beliefs and assumptions. The statements contained in this release are not guarantees of future performance and involve certain risks, uncertainties and assumptions, which are difficult to predict. Therefore, actual outcomes and results may differ materially from what is expressed in such forward-looking statements. Important factors and risks that may affect actual results include: the development risks of early stage discovery efforts in the disease areas in Enanta's research and development pipeline, such as RSV; the impact of development, regulatory and marketing efforts of others with respect to competitive treatments for RSV; Enanta's limited clinical development experience; Enanta's need to attract and retain senior management and key scientific personnel; Enanta's need to obtain and maintain patent protection for its product candidates and avoid potential infringement of the intellectual property rights of others; and other risk factors described or referred to in "Risk Factors" in Enanta's most recent Form 10-Q for the fiscal quarter ended March 31, 2019 and other periodic reports filed more recently with the Securities and Exchange Commission. Enanta cautions investors not to place undue reliance on the forward-looking statements contained in this release. These statements speak only as of the date of this release, and Enanta undertakes no obligation to update or revise these statements, except as may be required by law.

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