Enanta Pharmaceuticals Announces Publication in The New England Journal of Medicine of Data from the Phase 2a Human Challenge Study of EDP-938 for the Treatment of Respiratory Syncytial Virus (RSV)

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EDP-938 is Currently Being Evaluated in Phase 2 Studies in Pediatric Patients and Hematopoietic Stem Cell Transplant Recipients With RSV

On Track to Report Topline Data from RSVP, a Phase 2b Study in Adults with Community-Acquired RSV Infection, in the Second Quarter of 2022

WATERTOWN, Mass.--(BUSINESS WIRE)--Feb. 17, 2022-- Enanta Pharmaceuticals, Inc. (NASDAQ:ENTA), a clinical-stage biotechnology company dedicated to creating small molecule drugs for viral infections and liver diseases, today announced that data from its Phase 2 human challenge study has been published in *The New England Journal of Medicine* (*NEJM*).

"The publication of positive results from our Phase 2a human challenge study of EDP-938 in *NEJM* demonstrates the significance of our work to develop EDP-938 as a potential treatment option for patients with RSV, a deadly virus affecting children, the elderly and the immune compromised for whom there is no vaccine or therapeutic treatment," said Jay R. Luly, Ph.D., President and Chief Executive Officer of Enanta Pharmaceuticals. "EDP-938 is currently being evaluated in a broad clinical program across multiple patient populations, and we look forward to continuing the development of this important therapy to bring us closer to a treatment for RSV patients."

The Phase 2a study was a randomized, double-blind, placebo-controlled, human challenge study in healthy adult subjects inoculated with RSV. Once RSV infection was confirmed, subjects were randomized to receive either a once-daily (QD) 600 mg dose of EDP-938, a single 500 mg loading dose (LD) followed by a 300 mg twice daily (BID) dose of EDP-938, or placebo for five days. The primary endpoint was change in viral load (determined by RT-qPCR), as measured by the area under the curve (AUC) from initial dose through Day 12 in the intent-to-treat infected population. A key secondary endpoint measured AUC reduction in total symptom score.

The *NEJM* publication highlighted results from the Phase 2 human challenge study in which the primary efficacy endpoint was met with a highly statistically significant reduction ($p<0.001$) in viral load AUC was observed for each of the EDP-938 dosing groups as compared with placebo. 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, a highly statistically significant reduction was observed in total symptom score 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* EC_{90} for RSV-infected human cells.

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

About EDP-938
EDP-938, Enanta's lead N-protein inhibitor, is being developed for the treatment of RSV infection. Granted Fast Track Designation by the U.S. Food and Drug Administration, EDP-938 is differentiated from fusion inhibitors for RSV as 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
RSV is the most common cause of bronchiolitis (inflammation of the small airways in the lung) and pneumonia in children under one year of age in the U.S. and a significant cause of respiratory illness in older adults and immunocompromised individuals.[1] According to the Centers for Disease Control and Prevention, virtually all children in the U.S. get an RSV infection by the time they are 2 years old and one to two out of every 100 children younger than 6 months of age with RSV infection may need to be hospitalized.[2] Globally, there are an estimated 33 million cases of RSV annually in children less than 5 years of age, with about 3 million hospitalized and up to approximately 120,000 dying each year from complications associated with the infection.[3] RSV represents a significant health threat for adults older than 65 years of age, with 177,000 hospitalizations and 14,000 deaths associated with RSV infections estimated annually.[4] In the U.S., compared to influenza, RSV causes more than nine times as many deaths and more than 15 times as many infant hospitalizations.[5,6]

About Enanta Pharmaceuticals, Inc.
Enanta 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 programs include clinical candidates currently in development for the following disease targets: respiratory syncytial virus (RSV), SARS-CoV-2 (COVID-19) and hepatitis B virus (HBV). Enanta is also conducting research in human metapneumovirus (hMPV).

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

FORWARD LOOKING STATEMENTS

This press release contains forward-looking statements, including statements with respect to the prospects for advancement of 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 impact of development, regulatory and marketing efforts of others with respect to competitive treatments for RSV; the discovery and development risks of Enanta's programs in RSV; the competitive impact of development, regulatory and marketing efforts of others in those disease areas; any continuing impact of the COVID-19 pandemic on business operations and clinical trials in RSV; Enanta's lack of clinical development experience; Enanta's need to attract and retain senior management and key research and development 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-K for the fiscal year ended December 31, 2021, and any 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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1. Centers for Disease Control & Prevention – Respiratory Syncytial Virus
2. Centers for Disease Control & Prevention – RSV in Infants and Young Children
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4. Falsey, Ann R et al. "Respiratory syncytial virus infection in elderly and high-risk adults." *The New England Journal of Medicine* vol. 352,17 (2005): 1749-59. doi:10.1056/NEJMoa043951
5. Thompson, William W et al. "Mortality associated with influenza and respiratory syncytial virus in the United States." *JAMA* vol. 289,2 (2003): 179-86. doi:10.1001/jama.289.2.179
6. Zhou, Hong et al. "Hospitalizations associated with influenza and respiratory syncytial virus in the United States, 1993-2008." *Clinical Infectious Diseases* vol. 54,10 (2012): 1427-36. doi:10.1093/cid/cis211

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Media and Investors:
Jennifer Viera
617-744-3848
jviera@enanta.com

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