July 18, 2018

Emmaus Life Sciences Announces the New England Journal of Medicine has Published the Phase 3 Trial Results of Endari™ (L-Glutamine Oral Powder) in Sickle Cell Disease

Results showed a significant improvement in these measures: lower median number of sickle cell crises, fewer hospitalizations, lower cumulative days in hospital, and lower incidence of acute chest syndrome

TORRANCE, Calif.--(BUSINESS WIRE)-- **VIEW E-MEDIA KIT** – Emmaus Life Sciences, Inc. (Emmaus) announced today that the *New England Journal of Medicine* (NEJM) has published the results of its 48-week phase 3 clinical trial of Endari™ (L-glutamine oral powder) which supported the FDA approval in July 2017 to reduce the acute complications of sickle cell disease in adult and pediatric patients 5 years of age and older.

The article reports results that showed significantly fewer sickle cell crises in those receiving Endari compared to placebo by 25 percent; p=0.005 (median 3 vs. median 4) and significantly fewer hospitalizations by 33 percent; p=0.005 (median 2 vs. median 3). Additional findings showed lower cumulative days in hospital of 41 percent; p=0.02 (median 6.5 days vs. median 11 days) and a lower incidence of acute chest syndrome (ACS) by more than 60 percent; p=0.003 (13 of 152 patients [8.6%] had at least 1 ACS compared with 18 of 78 in the placebo group [23.1%]). The most common adverse reactions, occurring in greater than 10 percent, of the clinical study were constipation, nausea, headache, abdominal pain, cough, pain in extremity, back pain, and chest pain.

“Endari is the first approved treatment for sickle cell disease in pediatric patients 5 years of age and older and the first in nearly 20 years for adults. Our hope in sharing the results of this data from the *New England Journal of Medicine*, a publication with worldwide reach and significance, is to aid in increasing the awareness of sickle cell disease, a lifelong hereditary blood disorder which commonly affects those of African descent, as well as those from Central and South America and people of Middle Eastern, Asian, Indian and Mediterranean descent,” said co-author Yutaka Niihara, MD, CEO and founder of Emmaus.

“Sickle cell disease affects thousands of people in the United States,” said Beverley Francis-Gibson, President and CEO of The Sickle Cell Disease Association of America, Inc. “While there is no universal cure for this life-threatening disease, patient awareness and education on treatment options remain important factors for the sickle cell community.”

Additional Information About the Phase 3 Clinical Study

The randomized, double-blind, placebo-controlled, multicenter Phase 3 trial evaluated the efficacy and safety of Endari (0.3 gram per kilogram of body weight per dose) administered twice daily by mouth, as compared with placebo. The study included patients at least 5 years of age with sickle cell anemia or sickle β^0-thalassemia, with a history of two or more pain crises during the previous year. Patients who were receiving hydroxyurea at a dose that had been stable for at least 3 months before screening and who continued such therapy during the 48-week treatment period were eligible. A total of 230 patients (age range, 5 to 58 years; 53.9% female) were randomly assigned, in a 2:1 ratio, to receive L-glutamine (152 patients) or placebo (78 patients).

About Sickle Cell Disease
Sickle cell disease is an inherited blood disorder characterized by the production of an altered form of hemoglobin which polymerizes and becomes fibrous, causing red blood cells to become rigid and change form so they appear sickle shaped instead of soft and rounded. Patients with sickle cell disease suffer from debilitating episodes of sickle cell crises, which occur when the rigid, adhesive and inflexible red blood cells occlude blood vessels. Sickle cell crises cause excruciating pain as a result of insufficient oxygen being delivered to tissue, referred to as tissue ischemia, and inflammation. These events may lead to a variety of other adverse outcomes such as acute chest syndrome that requires hospitalization. Sickle cell disease is an orphan disease, affecting approximately 100,000 patients in the U.S. and millions worldwide, with significant unmet medical needs.

About Endari
Endari (L-glutamine oral powder) is a prescription oral treatment approved by the U.S. Food and Drug Administration (FDA) to reduce the acute complications of sickle cell disease in adult and pediatric patients 5 years of age and older. Endari was approved in July 2017 and was the first treatment in nearly 20 years shown to reduce the acute complications of sickle cell disease in adults when used as directed. Endari is also the first treatment approved to reduce the acute complications of sickle cell disease in children five years and older when used as directed. Endari has received Orphan Drug designation in the U.S., and Orphan Medicinal Product designation in the EU.

Important Safety Information
The most common adverse reactions (incidence >10 percent) in clinical studies were constipation, nausea, headache, abdominal pain, cough, pain in extremities, back pain and chest pain.

Adverse reactions leading to treatment discontinuation included one case each of hypersplenism, abdominal pain, dyspepsia, burning sensation and hot flash.

The safety and efficacy of Endari in pediatric patients with sickle cell disease younger than five years of age has not been established.

For more information, please see full Prescribing Information of Endari at: www.ENDARIrx.com/PI

About Emmaus Life Sciences, Inc.
Emmaus Life Sciences, Inc. is a biopharmaceutical company engaged in the discovery, development and commercialization of innovative treatments and therapies primarily for rare and orphan disease. Its lead product, Endari, demonstrated positive clinical results in the

completed Phase 3 clinical trial for sickle cell anemia and sickle ß0-thalassemia and has received FDA approval. Visit: http://www.emmausmedical.com/.

Forward-Looking Statements
This press release contains forward-looking statements as that term is defined in the Private Securities Litigation Reform Act of 1995, regarding the research, development and potential commercialization of pharmaceutical products. Such forward-looking statements are based on current expectations and involve inherent risks and uncertainties, including factors that could delay, divert or change any of them, and could cause actual outcomes and results to differ materially from current expectations. Additional risks and uncertainties are described in reports filed by Emmaus Life Sciences, Inc., with the U.S. Securities and Exchange Commission, including its Annual Report on Form 10-K and Quarterly Reports on Form 10-Q. Emmaus is providing this information as of the date of this press release and does not undertake any obligation to update any forward-looking statements as a result of new information, future events or otherwise.

Article Reference

Niihara, Y, *et al.* A Phase 3 Trial of L-Glutamine in Sickle Cell Disease. *NEJM to* be published 19 July 2018

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