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Emmaus Life Sciences Presents Positive Transfusion Data from a Post-Hoc Analysis of its Phase 3 Clinical Study of Endari® in Patients With Sickle Cell Disease at the 63rd American Society of Hematology (ASH) Annual Meeting

Analysis Indicates that Patients Requiring Blood Transfusions and Receiving Endari Required ~43% Fewer Units of Red Blood Cells

TORRANCE, Calif., Dec. 14, 2021 /PRNewswire/ --**Emmaus Life Sciences, Inc.** (OTCQX: EMMA), a commercial-stage biopharmaceutical company and leader in the treatment of sickle cell disease, today announced positive transfusion data from a post-hoc analysis of its phase 3 clinical study of Endari®, the company's prescription L-glutamine oral powder, in patients with sickle cell disease (SCD). The data was introduced in a poster presentation on Monday, December 13, 2021 at the 63rd American Society of Hematology (ASH) Annual Meeting and Exhibition at the Georgia World Congress Center in Atlanta, Georgia, and virtually.

The multicenter phase 3 trial enrolled a total of 230 patients, randomized 2:1, to receive Endari (152 patients) or placebo (78 patients). Following 48 weeks of therapy, patients in the Endari group had significantly fewer pain crises and fewer hospitalizations than those in the placebo group. Two thirds of the patients in both trial groups received concomitant hydroxyurea. The multicenter study of hydroxyurea showed that the treatment group differed from the placebo group both in the number of units of packed red blood cells (RBC) transfused and in the number of patients receiving transfusions. Since an evaluation of transfusions was not pre-specified in the Endari study, a post-hoc analysis was performed on the number of RBCs transfused and on the number of transfusions that took place during the study.

Results showed that there was a significant difference in the number of units of RBCs transfused in the Endari treatment arm than in the placebo arm; 2.86 units per patient-year in the treatment group vs. 5.38 units per patient-year in the placebo group. There was a lower trend in the mean cumulative number of RBC transfusion episodes in the treatment arm than the placebo arm during the 48-week treatment period; 1.702 RBC transfusion episodes per patient-year in the Endari arm compared to 2.659 RBC transfusion episodes per patient-year in the placebo arm.

Overall, the post-hoc analyses of the Endari phase 3 clinical study in SCD indicated that, of patients requiring RBC transfusions, those assigned to Endari required approximately 43% fewer units of RBCs compared to those assigned to placebo over the 48-week period. The recurrent event-time analysis also showed a favorable trend in the fewer number of RBC transfusion episodes for those receiving Endari as compared to placebo.

"This post-hoc analysis confirms that Endari meaningfully reduces both pain crises and hospitalizations for patients with sickle cell disease," stated Yutaka Niihara, M.D., M.P.H., Chairman and Chief Executive Officer of Emmaus. "Importantly, for patients requiring blood transfusions, those treated with Endari required approximately 43% fewer units of red blood cells, which is important considering the fact that two-thirds of participants in both arms of this study were already on hydroxyurea therapy. This serves to illustrate that Endari can safely be used in combination with other therapeutics to provide additional benefits to sickle cell disease patients in need."

Title: *The Evaluation of Transfusion Data from the Phase 3 Clinical Study of L-Glutamine in Sickle Cell Disease*
Presenter: Hung Lam, PhD[1]
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Program: Oral and Poster Abstracts
Session: 114. Hemoglobinopathies, Excluding Thalassemia: Clinical and Epidemiological: Poster III

The abstract is also accessible on the "Research Publications" page of the Emmaus website at: https://www.emmausmedical.com/content/pipeline/researchpub/research-publications-210

About Emmaus Life Sciences
Emmaus Life Sciences, Inc. is a commercial-stage biopharmaceutical company and leader in the treatment of sickle cell disease. The company currently markets U.S. Food and Drug Administration approved Endari® (L-glutamine oral powder) indicated to reduce the acute complications of sickle cell disease in adults and children 5 years and older. The company is also engaged in the discovery and development of innovative treatments and therapies for certain rare and orphan diseases as well as those affecting larger populations, such as diverticulosis. For more information, please visit www.emmausmedical.com.

About Endari® (prescription grade L-glutamine oral powder)
Endari®, Emmaus' prescription grade L-glutamine oral powder, was approved by the FDA in

July 2017 for treating sickle cell disease in adult and pediatric patients five years of age and older. Sales of Endari® began in the United States in 2018.

Indication
Endari® is indicated to reduce the acute complications of sickle cell disease in adult and pediatric patients five years of age and older.

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 Sickle Cell Disease
There are approximately 100,000 people living with sickle cell disease (SCD) in the United States and millions more globally. The sickle gene is found in every ethnic group, not just among those of African descent; and in the United States an estimated 1-in-365 African Americans and 1-in-16,300 Hispanic Americans are born with SCD.[1] The genetic mutation responsible for SCD causes an individual's red blood cells to distort into a "C" or a sickle shape, reducing their ability to transport oxygen throughout the body. These sickled red blood cells break down rapidly, become very sticky, and develop a propensity to clump together, which causes them to become stuck and cause damage within blood vessels. The result is reduced blood flow to distal organs, which leads to physical symptoms of incapacitating pain, tissue and organ damage, and early death.[2]

[1]Source: Data & Statistics on Sickle Cell Disease – National Center on Birth Defects and Developmental Disabilities, Centers for Disease Control and Prevention, December 2020.
[2]Source: Committee on Addressing Sickle Cell Disease – A Strategic Plan and Blueprint for Action -- National Academy of Sciences Press, 2020.

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SOURCE Emmaus Life Sciences, Inc.