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# Emmaus Life Sciences Reinforces Positive Outcomes of Endari ® Clinical Trials, Including Efficacy Findings That Led to FDA Approval

TORRANCE, Calif., Sept. 24, 2019 /PRNewswire/ --**Emmaus Life Sciences, Inc.** (OTC: EMMA), a leader in sickle cell disease (SCD) treatment, today released the following summary review of the clinical trials and efficacy findings for Endari®.

"In light of our decision to withdraw our marketing authorization application (MAA) to the European Medicines Agency (EMA) for Xyndari™ (glutamine), there is some confusion relating to the previous clinical trials and efficacy findings that led to Endari's approval by the FDA in 2017," said Yutaka Niihara, M.D., M.P.H., Chairman and Chief Executive Officer of Emmaus. "We appreciate the opportunity to provide the following summary to our shareholders and the SCD community."

**Endari Clinical Trial Results**

The data supporting Endari® is straightforward, and the clinical trials leading to the therapeutic's approval by the FDA were a resounding success. The Phase 2 study results were a clear indicator of the efficacy of Endari® in preventing vaso-occlusive crises in sickle cell patients.

Based on the Phase 2 study data, Emmaus designed the pivotal Phase 3 study. The results of the Phase 3 study clearly demonstrated the efficacy of Endari® in both the primary and secondary endpoints. Below is a high-level summary of the data, which was published in the *New England Journal of Medicine* in 2018[1].

Additionally, please visit the "CEO Letters" section of the Emmaus Life Sciences website to stay informed of corporate updates and remarks:
https://www.emmausmedical.com/content/ceoletters/ceo-letter-sep-10-2019-193

**Endari® Phase 3 Study Data**

The Phase 3 randomized, double blinded, placebo-controlled study demonstrated the following, among other positive findings:

1. 25% less frequency of painful crises in treatment group (p=0.005)
2. 33% less frequency of hospitalization in treatment group (p=0.005)
3. More than 60% decrease in the frequency of Acute Chest Syndrome, one of the most devastating complications of sickle cell disease, among patients treated with Endari®,

(p=0.003)

Additionally, patients administered both hydroxyurea and Endari® experienced improved health outcomes compared to those administered hydroxyurea and a placebo.

Patients administered Endari® also experienced improved health outcomes compared to those administered the placebo. The safety profile of Endari® was similar to that of the placebo.

The design of the Phase 3 study was sound and agreed upon by the FDA. Also, the imputation of missing data was appropriate as pre-specified. The design of the study prevented hydroxyurea use from influencing either treatment group.

The outcomes of both the Phase 2 and Phase 3 studies are consistent and support the mechanism of action for Endari® in preventing oxidative damage to sickle red blood cells.

**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 that 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 organ damage, stroke, pulmonary complications, skin ulceration, infection and a variety of other adverse outcomes. Sickle cell disease is a significant unmet medical need, affecting approximately one hundred thousand patients in the U.S. and millions worldwide, the majority of which are of African descent. An estimated 1-in-365 African-American children is born with sickle cell disease.

**About Endari®**

**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 in clinical studies include constipation, nausea, headache, and abdominal 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: http://www.ENDARIrx.com/PI

**About Emmaus Life Sciences**

Emmaus Life Sciences, Inc. is a commercial-stage biopharmaceutical company engaged in the discovery, development, marketing and sale of innovative treatments and therapies, including those in the rare and orphan disease categories. For more information, please visit www.emmauslifesciences.com.

**Forward-looking Statements**

Except for the historical information contained herein, the matters discussed in this press release are forward-looking statements made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995, as amended. These forward-looking statements are subject to numerous assumptions, risks and uncertainties which change over time. Forward-looking statements speak only as of the date they are made, and Emmaus assumes no duty to update forward-looking statements. Factors previously disclosed in Emmaus' reports filed with the Securities and Exchange Commission, among others, could cause actual results to differ materially from forward-looking statements.

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[1] Niihara, Yutaka, et al. "A Phase 3 Trial of l-Glutamine in Sickle Cell Disease."*New England Journal of Medicine*, vol. 379, no. 3, 2018, pp. 226–235., doi:10.1056/nejmoa1715971.