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# Emmaus Life Sciences Presented Positive Real-World Data on the Efficacy of Endari® in Preventing Acute Complications from Sickle Cell Disease at the 62nd Annual Scientific Meeting of the British Society for Haematology

*Sickle Cell Patients Treated With Endari® had Statistically Significantly Fewer Vaso-Occlusive Events and Hospitalizations Through 72 Weeks Follow-up, Versus Baseline*

*Adverse Events Among These Patients from French Guiana and Qatar were Few and Mild*

TORRANCE, Calif., April 7, 2022 /PRNewswire/ -- [Emmaus Life Sciences, Inc.](#) (OTCQX: EMMA), a commercial-stage biopharmaceutical company and leader in the treatment of sickle cell disease, today announced real-world data on Endari®, the company's prescription-grade L-glutamine oral powder, in preventing acute complications from sickle cell disease (SCD) and hemolysis in pediatric and adult patients in French Guiana and Qatar. The data was introduced by Dr. Mohamed Yassin and his co-authors at the 62<sup>nd</sup> Annual Meeting of the British Society for Haematology (BSH), which was held April 3-5, 2022 at the Manchester Central in Manchester, England and virtually.



In the observational study conducted from October 2019 through April 2021, 19 patients (4 patients from Qatar and 15 patients from French Guiana) were treated with L-glutamine (Endari) (0.3mg/kg), twice daily. Clinical and laboratory parameters were documented for the year prior to treatment initiation as baseline values and collected at 24, 48, and 72 weeks from treatment initiation.

Compared to baseline, patients had significantly fewer vaso-occlusive crises at 24, 48 and 72 weeks on Endari therapy (median change from 3.0 to 0;  $p < 0.00001$ ) as well as fewer hospitalizations (median change from 3.0 to 0;  $p < 0.00001$ ) and fewer days in hospital

(median change from 15.0 to 0;  $p < 0.00001$ ). Furthermore, as compared to baseline, the number of blood transfusions at 24, 48 and 72 weeks was considerably lower (median, from 3.0 to 0.0;  $p < 0.00001$ ). Following treatment with Endari, the mean hemoglobin level increased significantly from baseline to 72 weeks (8.2 to 8.8 g/dL;  $p < 0.001$ ), with peak mean increase from baseline of 11.2% at 48 weeks. A similar trend was observed for increased hematocrit proportions from baseline to 72 weeks (24% to 27%;  $p < 0.001$ ), with the highest mean improvement from baseline of 15.5% at 48 weeks. Conversely, mean reticulocyte counts and lactate dehydrogenase levels were significantly reduced at 24, 48 and 72 weeks compared to baseline ( $p = 0.003$  and  $p < 0.001$ , respectively).

"Importantly, the observed clinical benefits of Endari coincided with a significant improvement in hemolysis parameters which were sustained over the entire study period of 18 months. In addition, among the 19 study patients, the incidence of acute chest syndrome decreased from 11 events reported the year before initiating treatment with Endari, to only 2 events after 12 months on treatment. Given the deadly implications of acute chest syndrome, this real-world evidence supports the benefits of Endari for sickle cell patients," stated Mohamed Yassin, MD, Senior Consultant Hematologist and Associate Professor of Medicine at CMED QU Hamad Medical Corporation and chief investigator of the study.

"The clinical observations by a leading hematology center in Qatar, in cooperation with French Guiana sickle cell referral centers, demonstrated significant improvements in patient outcomes for the first time in these populations. Moreover, these observations are consistent with clinical data on the effectiveness of Endari in its FDA-approved indication for reducing the acute complications of sickle cell disease in adults and children 5 years and older," stated Yutaka Niihara, M.D., M.P.H., Chairman and Chief Executive Officer of Emmaus.

**Title:** *Real World Data on Efficacy of Pharmaceutical-Grade L-Glutamine in Preventing Sickle Cell Disease-Related Acute Complications and Hemolysis in Pediatric and Adult Patients*

**E-Poster Number:** BSH22- EP78

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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](http://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.ENDARlrx.com/PI](http://www.ENDARlrx.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. Throughout the Middle East North Africa region, Emmaus estimates that there are approximately 225,000 sickle cell disease patients that could potentially be treated with Endari®. 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.<sup>1</sup> 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.<sup>2</sup>

<sup>1</sup>Source: Data & Statistics on Sickle Cell Disease – National Center on Birth Defects and Developmental Disabilities, Centers for Disease Control and Prevention, December 2020.

<sup>2</sup>Source: Committee on Addressing Sickle Cell Disease – A Strategic Plan and Blueprint for Action -- National Academy of Sciences Press, 2020.

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