

Novel therapy helps ease pain and suffering for sickle cell patients

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Chronic, debilitating pain and potential organ failure are what approximately 100,000 sickle cell patients in the United States live with each day. Yutaka Niihara, M.D., M.P.H. - lead investigator at The Los Angeles Biomedical Research Institute at Harbor-UCLA Medical Center (LA BioMed) and co-founder of Emmaus Medical, Inc., an LA BioMed spin-off company - is developing a low-cost, noninvasive treatment that helps provide relief for patients suffering from the debilitating effects of sickle cell disease.

Dr. Niihara and his team of investigators discovered that L-glutamine – which is the most common free-form amino acid in the body – can help alleviate many of the painful symptoms associated with sickle cell when used in powder form and added to liquid.

Sickle cell is an inherited disease that causes red blood cells to become oxidized, sticky, and crescent or sickle-shaped instead of smooth, pliable, and round. It can lead to anemia, [organ damage](#), chronic and [acute pain](#), and a host of other problems. L-glutamine, in its powder form, helps to reduce the oxidation of [red blood cells](#).

Dr. Niihara first began seeking [alternative treatments](#) for sickle cell nearly 20 years ago, after years of seeing patients suffer from this debilitating disease. He was encouraged by Dr. Charles Zerez, an LA BioMed investigator in the 1980s and 1990s, to pursue glutamine research. Of the approximately 80 medications for sickle cell that have entered into clinical trials, this therapy is one of only three experimental

treatments to reach Phase III trials, which are usually the last trials conducted before the [Food and Drug Administration](#) approves a treatment to be made widely available to the patient population.

"As a physician, I have seen firsthand the [severe pain](#) suffered by patients with sickle cell disease," said Dr. Niihara. "We are pleased to have reached this stage in our development of this potential treatment, and hope to bring relief to the millions of people suffering from this disorder."

According to the Centers for Disease Control (CDC), there are approximately 100,000 people in the United States living with sickle cell disease, and approximately 3 million people carrying the sickle cell trait. With Dr. Niihara's treatment in [Phase III](#) clinical trials, this should come as a welcome relief to millions of people worldwide, as other treatment options such as pain medication, hydration, or bone marrow transplants are either not well tolerated, are too costly, or have limited availability.

"We are excited about the potential development of a novel treatment for the millions of people who suffer from the effects of sickle cell disease," said David I. Meyer, Ph.D., president and CEO of LA BioMed. "The achievements of Dr. Niihara and his team of researchers demonstrate the pioneering spirit that has kept LA BioMed at the forefront of medical discoveries that have helped transform the lives of so many."

Dr. Niihara's L-glutamine treatment recently received an Orphan Medicinal Product designation from the European Commission (EC). This designation follows a recommendation announced in May from the European Medicines Agency's (EMA) Committee for Orphan Medicinal Products. The therapy currently has orphan drug designation and "fast track" status in the United States, which is a process designed to facilitate the development and expedite the review of drugs to treat

serious diseases and fill an unmet medical need.

Provided by Los Angeles Biomedical Research Institute at Harbor

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