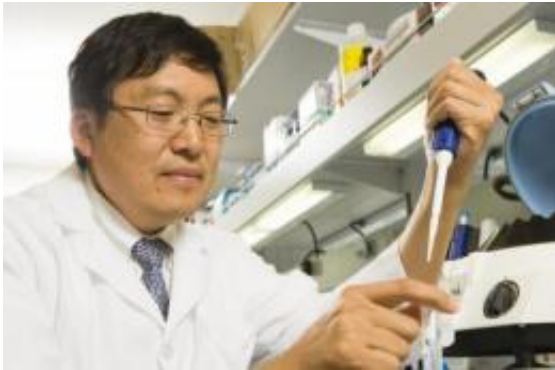


# Sickle cell treatment enters Phase 3 clinical trial

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Dr. Yutaka Niihara, LA BioMed researcher, developed a promising new treatment for sickle cell disease. Credit: Dana Maione

An experimental treatment for sickle cell disease developed at the Los Angeles Biomedical Research Institute (LA BioMed) has entered Phase 3 clinical trials, David I. Meyer, PhD, LA BioMed president and CEO announced today.

Researchers throughout the U.S. have begun administering the sickle cell treatment developed by investigators led by Yutaka Niihara, MD, MPH, at LA BioMed and licensed to Emmaus Medical, Inc. The patented drug treatment involves the oral administration of L-glutamine, which is the most common amino acid in the body. This is one of a very few experimental treatments for sickle cell disease to reach the Phase 3 clinical trial stage.

Sickle cell disease is an inherited disorder that causes [red blood cells](#) to become oxidized, sticky and sickle shaped instead of smooth, pliable and round. Sickle cell disease leads to anemia, organ damage, chronic and acute pain and a host of other problems.

"This is exciting news in the potential development of a novel new treatment for the millions of people who suffer from the painful effects of sickle cell disease," said Dr. Meyer. "We are proud of the dedication of Dr. Niihara and the team of researchers who first developed this treatment. They demonstrate the pioneering spirit that has kept LA BioMed at the forefront of translating discoveries into treatments that can transform lives."

Phase 3 clinical trials are large, randomized studies conducted at multiple sites to determine the safety and efficacy of a potential treatment. They are usually the last clinical trials conducted before the [Food and Drug Administration](#) gives its approval for a treatment to be made widely available to the patient population.

"As a physician I have seen firsthand the severe pain in patients with sickle cell disease, so I am very pleased we have reached this stage in our development of this potential treatment," said Dr. Niihara. "In the Phase 2 clinical trial, we observed an excellent safety profile and positive trends in decreasing the number of crises as well as reducing the frequency of hospitalizations in [sickle cell disease](#) patients. We look forward to the findings from the much larger group of research volunteers we will be seeking in the Phase 3 clinical trial."

In the Phase 3 clinical trial, researchers at 20 to 25 sites around the country will be seeking up to 225 research volunteers, age 5 years and older, with a diagnosis of sickle cell anemia or sickle beta O-thalassemia who have a history of at least two episodes of painful crisis during the past 12 months. The trial is a 53-week study requiring monthly visits to

the research facility. It is funded by Emmaus Medical.

Provided by University of California Los Angeles

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