

Innovative Gene Editing Approach to Sickle Cell Disease Wins National Clinical Research Award

The Top 10 Clinical Research Achievement Awards showcase the immense value of our nation's investment in clinical and translational research

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*Dr. Harry Selker, Chair,
Clinical Research Forum*

/EINPresswire.com/ -- [The Clinical Research \(CR\) Forum](#), comprised of top clinical research experts and thought leaders from the nation's leading academic health centers, awarded its most prestigious honor, The Herbert Pardes Clinical Research Excellence Award, to [Dr. James LaBelle of the University of Chicago](#). Dr. LaBelle's study demonstrated that a gene-editing approach has powerful potential to offer a lifelong cure to every patient with sickle cell disease. This hereditary disease affects more than 100,000 Americans; about 1 in 13 Black or African American babies are born with the sickle cell trait and may develop the disease. The average lifespan of an individual with sickle-cell disease is only 43 years.

This study was recognized at CR Forum's annual [Top Ten Clinical Research Achievement Awards](#) on April 2nd at the Paris Hotel in Las Vegas, NV. The Top Ten Awards highlight outstanding research advances that benefit the health and welfare of all Americans. A complete list of the 2024 Top Ten Award Winners is available on the website.

"This year's award winners demonstrate the immense value of our nation's investment in clinical research, and the direct impact of that work on the health of millions of people in the United States," said Harry P. Selker, MD, MSPH, CR Forum Board Chair and Dean of the Clinical and Translational Science Institute at Tufts University.

In addition to the award, named after CR Forum's Founding Vice Chair Dr. Herbert Pardes, Dr. LaBelle and his team at the University of Chicago also received a \$7,500 cash prize.

Sickle cell disease is the most common inherited blood disorder in the United States. The only cure is bone marrow transplantation from a close genetically matched donor, something available to only 20% of patients. Dr. LaBelle's study, CRISPR-Cas9 Editing of the HBG1 and HBG2 Promoters to Treat Sickle Cell Disease, used an innovative genetic approach to increase fetal hemoglobin in the blood, a proven treatment for sickle-cell disease. Gene-edited cells from the patients themselves were used in a study involving three patients with severe disease. The transplantation of gene-edited stem cells resulted in clinical improvement in disease severity over 6 to 18 months of follow-up. Because the patient's own stem cells could be used, thus ensuring a genetic match, this treatment could be potentially available to everyone with the disease. The study was published in the New England Journal of Medicine in August 2023.



From left to right, Dr. Harry Selker, Chair of Clinical Research Forum, and Dr. James LaBelle, Winner of the Herbert Pardes Clinical Research Excellence Award

Two other studies received Distinguished Clinical Research Achievement Awards, and a \$5,000 prize:

- Azithromycin to Prevent Sepsis or Death in Women Planning a Vaginal Birth, nominated by the University of Alabama-Birmingham and published in the New England Journal of Medicine in March 2023, demonstrated that a single oral dose of azithromycin, a common antibiotic used to treat infections, reduced the risk of maternal sepsis or death by 33% in women planning a vaginal birth. The study enrolled 29,000 women in seven low-income and middle-income countries and proved the feasibility of using this low-cost intervention at scale. Dr. Waldemar Carlo and Dr. Alan Tita, the first back-to-back two-time Top 10 winner, led the international research team.
- Prevent Clot: Aspirin or Low-Molecular Weight Heparin for Thromboprophylaxis after a Fracture, nominated by the University of Maryland and published in the New England Journal of Medicine in January 2023, demonstrated that aspirin was just as effective as an injectable blood thinner in preventing pulmonary embolism in patients with traumatic fractures (such as from a car accident). The multi-center randomized clinical trial, which included more than 12,000 patients at 21 trauma centers in the U.S. and Canada, is the largest trial ever conducted with orthopedic trauma patients. Many patients with fractures will likely take a daily aspirin instead of receiving injections based on this study, at a much lower cost and less inconvenience to patients.

The 2024 Top Ten Clinical Research Achievement Awards showcase clinical and translational research advances that benefit the health and welfare of all Americans, and reflect the influential work being done by investigators at 68 research institutions and hospitals across the United States, as well as partner institutions around the world.

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