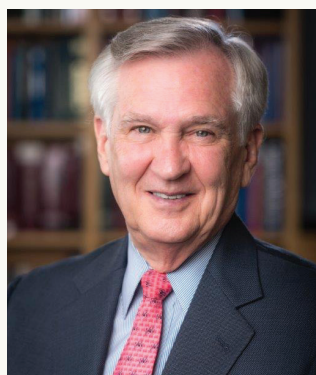


CURE SICKLE CELL.

A Catalyst for Cures

Dr. Edward J. Benz, Jr.



It's time to rewrite the story of sickle cell disease.

The Cure Sickle Cell Initiative, launched by NIH, will help speed the development of cures for the disease, which is known to affect at least 100,000 people in the United States and 20 million worldwide. I have the privilege of leading this important new initiative.

In receiving many questions over the past several months, I thought ASH's annual meeting – would be a great forum to share some of these.

WHAT WAS THE GENESIS OF THE CURE SICKLE CELL INITIATIVE?

A brainstorming retreat convened by Dr. Francis Collins, director of the NIH revealed that the traditional NIH mechanisms are structurally too slow for what is needed for late-stage translational research. The hope was to build a program where federal funding could be available in days or weeks.

WHAT IS THE GOAL?

The core aim is to utilize a funding mechanism that is more nimble and flexible than the traditional funding paradigm. It will build on the legacy of research that has greatly improved clinical care of individuals who have sickle cell disease.

WHAT DOES THIS MEAN FOR ASH?

Our researchers and investigators will take advantage of the growing number of technological advances and latest discoveries to address the gene that causes sickle cell disease. As Dr. Gary

H. Gibbons, director of NIH's National Heart, Lung, and Blood Institute, said, "We are now ready to use these tools to speed up our quest for a cure."

ASH has committed to improving the lives of sickle cell patients by supporting a Clinical Trials Network, registry, and several other important elements. We are very excited to have developed a MOU with ASH in which we will work together to accelerate curative therapies.

The Cure Sickle Cell Initiative offers a novel funding opportunity for ASH members and the entire research community. Patients will work alongside researchers in developing and recruiting for clinical trials.

WHO WILL BE INVOLVED IN THE INITIATIVE?

This will truly be a collaborative effort, involving patients, researchers, the private sector, clinicians, advocacy groups, NIH, and ASH.

We will establish a national data warehouse of genetic therapies for sickle cell disease and conduct comparative analyses of therapeutic approaches to assess clinical and cost effectiveness. We'll also establish national networks to make it easier for patients and providers to learn and engage with the research, clinical trials, and other activities.

WHY DID YOU SIGN ON AS EXECUTIVE DIRECTOR?

One reason is the collaboration. Another is the opportunity to accelerate the development of genetic therapies to transform the lives of those living with the disease. In short, hope for a cure.

WHAT'S NEXT FOR THE INITIATIVE?

We're using our website, www.curesickle.org, to share progress. Please visit the site and add your voice.

Dr. Benz is Executive Director of the Cure Sickle Cell Initiative and the President and CEO Emeritus at Dana-Farber Cancer Institute.