The CRISPR/Cas9 gene editing system has emerged as an exciting new tool for a wide range of applications, including customized genetic engineering, gene therapy, and treating viral infections. The FDA has approved CRISPR/Cas9 for use in human clinical trials, and several companies are working toward commercialization. But these efforts may hit a snag from off-target gene changes and a general lack of external control. To make CRISPR/Cas9 safer and more effective, we modified the system to be light-activated so that users can unleash this powerful gene editing tool precisely when and where it is needed.

**Technology Description**

To achieve spatial and temporal control, we genetically engineered the Cas9 protein to be light-activated. The addition of a caged lysine residue keeps Cas9 dormant by default, but in the presence of light the lysine caging group falls off and Cas9 springs into action. Unlike standard CRISPR/Cas9, which has full reign over the body, our light-activated system offers precise spatial and temporal control over genetic modifications, thereby reducing the chance of off-target effects and providing exclusive targeting of disease tissue.

**Advantages**
- Precise spatial and temporal control of CRISPR/Cas9 activity
- Reduced toxicity from off-target mutations

**Applications**
- Gene therapy
- Genome engineering
- Treating persistent viral infections
- Generating novel cell lines
- Functional genomic screening

**Light-Activated CRISPR/Cas9: Precise Control over Gene Editing**

**Featured Innovators:** Alex Deiters, PhD

Light exposure removes the caging group to activate the Cas9 protein. Once active, Cas9 works as an enzyme to facilitate sequence-specific DNA editing with CRISPR.

**Stage of Development**

*In vitro* mammalian cell culture data

**IP Status**
PCT/US2016/026753 patent in prosecution

**Notable Mentions**

Alex Deiters, PhD
Professor
Department of Chemistry
University of Pittsburgh

Dr. Deiters has published over one hundred peer-reviewed papers, 11 review articles, and five book chapters. He has presented over one hundred research seminars and has consulted for several pharmaceutical companies.

His research has won many accolades, including a Basil O’Connor Starter Scholar Award from the March of Dimes Foundation, a Sigma Xi Research Faculty Award, a Cottrell Scholar Award, a Beckman Young Investigator Award, a National Science Foundation CAREER Award, a Teva USA Scholars Grant from the American Chemical Society, a Thieme Chemistry Journal Award, an American Cancer Society Research Scholar Grant, Bill & Melinda Gates Foundation Grand Challenges Explorations Grant, an NCSU Alumni Association Outstanding Research Award, and a Charles E. Kaufman Foundation New Initiative Research Award.

Education
Postdoctorate
The Scripps Research Institute

Postdoctorate
The University of Texas at Austin

PhD in Chemistry
University of Münster

BS in Chemistry
University of Münster

Selected Publications


