CRISPR’s potential in gene therapy shadowed by concerns over ethical use

Tracy Antonelli and her three daughters suffer from thalassemia, a blood disorder that saps their strength, leaves them anemic, and requires them to visit Boston Children’s Hospital every three weeks for transfusions. “We’re lucky we have a treatment regimen that’s available to us, but it’s cumbersome,” Antonelli says.

A technology in development at several drug companies offers some hope for a more effective and convenient treatment for the Antonellis, and patients with other serious genetic conditions, such as sickle cell anemia. The technique is called Crispr, which stands for clustered regularly interspaced short palindromic repeats. Crispr, a method for editing the human genome—the complete set of an individual’s genetic material present in any of her cells—allows scientists to cut out faulty sections of DNA that can lead to serious illnesses and replace them with healthy ones.

The expectations raised by Crispr have roused ethical questions about the technique and its potential uses. In March, MIT Technology Review reported that OvaScience, a biotechnology company that develops fertility treatments, plans to use Crispr to correct gene disorders in human eggs. In April, researchers at Sun Yat-Sen University in Guangzhou, China, said they had used Crispr to try to repair a flawed gene in human embryos that’s responsible for a form of thalassemia. The researchers attempted the procedure in more than 80 embryos that were never viable. A handful successfully adopted the replacement gene; unexpected mutations occurred in others.

The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion and analysis. Read full, original post: The Promise and Perile of Crispr