CRISPR’s ability to treat blood disorders illustrates potential as medical therapy

[T]he jury’s still out on whether Crispr will be as transformative as a medical therapy as it has been as lab tool. Plenty of gene-editing techniques have been attempted as therapies, but few have made significant impacts—especially when it comes to diseases as complex as cancer. A better place to start testing gene therapies is with inherited blood disorders, like sickle cell anemia….
These diseases are a good comparison point because they’re relatively easy to treat…Researchers have thrown a number of gene-editing techniques at these diseases…[b]ut if you ask experts in the field, the smart money’s on Crispr.

…

With Crispr, since cells are being brought outside the body and treated in the lab, it’s easier to ensure that it’s the actual stem cells that are being edited. And if a Crispr team can get a higher fraction of edited stem cells to persist in bone marrow, a one-time treatment could permanently alleviate a blood disorder.

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A huge advantage of the technique is what led to its mass adoption in the lab—the gene-editing systems are simple and easy to make. PNAs, on the other hand, involve complex chemistry reminiscent of zinc finger nucleases (ZFNs)….

The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion, and analysis. Read full, original post: Blood Diseases Could Show CRISPR’s Potential as Therapy