Editas Medicine Reports Data Demonstrating the Potential of CRISPR/Cas9 to Enable Gene Repair Through a New Mechanism

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Oral presentation at Keystone Symposium for Genomic Instability and DNA Repair Also Highlights Potential for Use in Sickle Cell Anemia

Cambridge, Mass., March 2, 2015 -- Editas Medicine, a leading genome editing company, today presented data from *in vitro* experiments demonstrating a novel CRISPR/Cas9 mechanism for genome editing in the hemoglobin beta (HBB) gene. This novel mechanism achieved gene editing through gene conversion and importantly did not require the use of an external donor DNA source. These data suggest a potential therapeutic approach in which gene conversion using the hemoglobin delta (HBD) gene may correct the sickle cell mutation in the HBB gene. These data were presented today in an oral presentation at the Keystone Symposium for Genomic Instability and DNA Repair in Whistler, British Columbia, Canada.

"These data suggest gene conversion as a possible new approach to genomic repair for certain kinds of genetic mutations," said Katrine Bosley, chief executive officer, Editas Medicine. "While the results are early and further work is needed to see if this approach could be used therapeutically, the data exemplify Editas' commitment to explore and develop the full potential of genome editing to treat a broad range of genetically driven diseases."

The oral presentation is entitled "Gene targeting of the HBB locus by CRISPR/Cas9 to investigate repair pathway choice in response to different types of DNA lesions." Editas researchers targeted the HBB gene using CRISPR/Cas9 *in vitro* at the position that is most frequently mutated in sickle cell disease. They constructed and tested several different versions of a Cas9 nuclease (wild type Cas9 nuclease and Cas9 nickases D10A and N863A) and assessed each one's ability to achieve DNA repair of the HBB gene with and without donor DNA. The experiments demonstrated that the D10A nickase edited approximately 30% of the HBB loci in cells, including when no donor DNA was provided. Data showed that this repair was achieved through gene conversion of the HBB gene by the HBD gene.

Gene conversion is a process through which a cell replaces all of a part of one gene with the sequence of a different but closely related gene. One gene is used by the cell as a template with which to repair a second gene that has been cut, in this case by Cas9 cleavage. As a result, the second gene is partially or completely "converted."

About Genome Editing

Genome editing is a method to perform sequence-targeted modifications at the DNA level. Recent advances in the field have made it possible to modify, in a targeted way, almost any gene in the human body with the ability to directly turn on, turn off or edit disease-causing genes and has the potential to address diseases that have previously been intractable to traditional gene therapy, gene knock-down or other genome modification techniques.

The CRISPR/Cas9 (clustered, regularly interspaced short palindromic repeats)/Cas9 (CRISPR associated protein 9) system, the newest genome editing approach, uses a protein-RNA complex composed of an enzyme known as Cas9 bound to a guide RNA molecule that has been designed to recognize a particular DNA sequence. The RNA molecules guide the Cas9 complex to the location in the genome that requires repair. CRISPR/Cas9 uniquely enables highly efficient knock-out, knock-down or selective editing of defective genes in the context of their natural promoters, unlocking the potential to treat the root cause of a broad range of diseases.

About Editas Medicine

Editas Medicine is a leading genome editing company and part of a transformational new area of health care – genomic medicine. The company was founded by pioneers and world leaders in genome editing bringing specific expertise in CRISPR/Cas9 and TALENs technologies. The company's mission is to translate its proprietary technology into novel

solutions to treat a broad range of genetically driven diseases. For more information, visit www.editasmedicine.com.

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