New startup looking to cure genetic diseases by editing genes in new way

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(Medical Xpress)—A new startup company called Editas Medicine (with $43 million in funding) is looking to expand on research that has already led to a system called Clustered Regularly Interspaced Short Palindromic Repeats/Cas (the Cas part is the name of a protein that is carried via RNA to the desired location in a DNA base pair) shortened to CRISPR/Cas. Such research, the team believes, will lead to a new type of gene therapy that could perhaps lead to cures for such diseases as Huntington's, sickle-cell anemia or cystic fibrosis.

Traditional gene therapy uses a technique to apply a healthy gene to a part of the DNA. That healthy gene can than override an unhealthy one and thus cure some diseases. Unfortunately, the technique doesn't work if the unhealthy gene causes disease by producing toxic proteins or if it leads to mutations that can override the healthy gene. CRISPR/Cas is better, the researchers at the new company say, because it can actually be used to replace unhealthy genes with healthy ones.

The system works by mimicking a process researchers discovered only a few years ago—some microbes they noted, use RNA to direct proteins to parts of the DNA to defend themselves. With CRISPR/Cas, the Cas9 protein is directed to a DNA base pair by RNA. Once there it sets to work editing the base pair or it can even work on larger segments. The editing process can result in repairs, an override or complete replacement of a base pair. It's that last option that sets the system ahead of the others, team members note—totally removing base pairs that cause disease and replacing them with ones that do not, could prove

revolutionary to both gene therapy and medicine in general.

Officials with the new startup are being coy about which diseases they will focus on first, though it appears likely they will start with those like Huntington's which are caused by a problem with a single base pair, rather than multiples.

Of course, they still have to make it all work in the way intended first. Currently, the RNA sometimes delivers the Cas9 to the wrong base pair, which is unacceptable, of course. There is also the problem of how to have the genetic edits realized throughout all of the cells in the body at the same time.

More information: www.editasmedicine.com/ www.editasmedicine.com/documen … edicine-LaunchPR.pdf