Understanding genetic basis for heart disease opens up opportunity for gene editing solution

Later this year, Verve Therapeutics of Cambridge, Ma., will initiate Phase 1 clinical trials to test VERVE-101, a new medication that, if successful, will employ gene editing to significantly reduce low-density lipoprotein cholesterol, or LDL.

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Verve’s Founder and CEO, Sekar Kathiresan, spent two decades studying the genetic basis for heart attacks while serving as a professor of medicine at Harvard Medical School. His research led to two critical insights.

“One is that there are some people that are naturally resistant to heart attack and have lifelong, low levels of LDL,” the cardiologist says. “Second, there are some genes that can be switched off that lead to very low LDL cholesterol, and individuals with those genes switched off are resistant to heart attacks.”

... 

“Imagine a future where somebody gets a one-time treatment at the time of their heart attack or before as a preventive measure,” says Kathiresan.

The medication is targeted specifically for patients who have a genetic form of high cholesterol known as heterozygous familial hypercholesterolemia, or FH, caused by expression of a gene called PCSK9. Verve also plans to develop a program to silence a gene called ANGPTL3 for patients with FH and possibly those with or at risk of atherosclerotic cardiovascular disease.

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