

Jim Januzzi KOL Video 2: Communicating the Potential Outcomes of Genetic Modification to Patients Transcript

00:00:05:00 - 00:00:19:02

So what I would tell my patients about the potential effects of CRISPR/Cas9 includes in the short term, to start with, that, the gene editing might be expected to reduce protein production dramatically.

00:00:19:04 - 00:00:25:06

So if a disease is being driven by the production of an abnormal protein, for example,

00:00:25:06 - 00:00:56:14

that we can treat with CRISPR/Cas9 as opposed to a lifetime of medical therapies, doctor visits, hospitalizations, and progressive disease. In theory, through reduction, dramatic reduction in protein expression, we might be able to alter the course of that disease quite substantially, reducing the need for medical therapies, reducing the need for hospitalizations, improving quality of life, and perhaps lengthening quantity of life as well.

00:00:56:14 - 00:01:36:15

So the theory that CRISPR/Cas9 can alter the trajectory of a disease is being examined in a number of medical conditions presently. It's actually been studied now in diseases of the blood, such as thalassemia and sickle cell disease, where there seems to be an impact of CRISPR/Cas9. It's being studied in other diseases, including transthyretin amyloidosis, a diagnosis based in the liver, where long term studies are now ongoing to examine whether CRISPR/Cas9 editing of the transthyretin gene could be expected to alter disease progression in the diagnosis of transthyretin amyloidosis.