Jim Januzzi KOL Video 3: Addressing Common Patient Questions on CRISPR/Cas9 Transcript

00:10 – 00:25
Patients will actually ask me, “I've read about CRISPR/Cas9. Is this something that's new?” It's actually not new. It was originally described in 1987 and in fact is being used now in agriculture and farming on an everyday basis.

00:25 - 00:36
Now, of course, the applications of CRISPR/Cas9 in medicine are more recent, but studies are being done to carefully evaluate the safety of this therapy.

00:41 – 01:00
In order to allow CRISPR/Cas9 to recognize where to apply gene editing, it requires a guide RNA. I like to think of it as a GPS for locating the correct location in the DNA, where CRISPR/Cas9 can then go to work and do what it has to do.

01:00 - 01:13
In order to ensure the precision and accuracy of a guide RNA, studies are done to extensively evaluate different guides in order to maximize the likelihood for precision.

01:18 - 01:22
We sometimes get asked about what double stranded breaks in the DNA are.

01:22 - 01:43
I can understand why concern would exist about double stranded breaks, because patients may hear this and think there's something damaging the DNA. It turns out that double stranded breaks are very, very common. They're up to 10 to 50 double stranded breaks per cell in the body on an everyday basis.

01:43 - 01:56
They tend to happen quite randomly throughout the DNA. In contrast, CRISPR introduces a double stranded break in a very precise location because it uses a guide RNA in order to bind a very specific location that it's targeting.

01:56 - 02:04
With normal double stranded breaks, there's a repair process that is the very same repair process that CRISPR/Cas9 utilizes.

02:09 - 02:17
One question that I get asked is whether CRISPR/Cas9 will be passed on to children from someone that has been treated.

02:17 - 02:25

The Intellia CRISPR/Cas9 approach for treatment of disease was not designed to be passed on from a treated individual to their child.

02:30 – 02:42
Intellia's CRISPR-based therapies are intended to be administered as a one-time infusion with the intent to durably, and possibly permanently reduce protein levels.

02:47 - 03:01
It's understandable that someone may be worried that this therapy might stay in their body forever, but it's actually been shown in preclinical studies that Intellia's CRISPR-based technology is out of the body within just a few days.

03:06 - 03:10
Intellia did three things in the design of their CRISPR/Cas9 therapeutics.

03:10 - 03:19
The first was the development of a lipid nanoparticle delivery system to efficiently deliver the CRISPR/Cas9 therapeutic.

03:19 - 03:26
Second, through extensive preclinical studies, a very accurate guide RNA was developed.

03:26 - 03:33
Third was transient expression of the CRISPR/Cas9 components typically leaving the body within five days.

03:33 – 03:44
With those three aspects in mind, Intellia is now evaluating the safety and efficacy of its CRISPR/Cas9 therapeutics in clinical trials.