Dr. Danny Crohn Video #3: Intellia's Approach to Responsible Innovation in CRISPR/Cas 9 Transcript

00:04 – 00:40

When innovating in medicine, it's important to do so responsibly.

Over a decade of rigorous research and preclinical testing has been conducted to help ensure investigational CRISPR-based therapies target the intended gene with the highest possible level of both precision and accuracy, two vital therapeutic attributes.

To help maximize precision and accuracy, a comprehensive guide RNA specificity assessment is conducted prior to the development of the CRISPR-based therapy, which is done not only to preemptively uncover potential off-target edits, but also to assess their potential biological impact.

00:41 – 00:57

This assessment includes an off-target characterization workflow. Although the risk of unintended, off-target editing cannot be ruled out, this workflow helps ensure investigational CRISPR-based therapies target the gene of interest with high specificity while avoiding off-target effects.

00:58 – 01:21

First, genomic loci with the potential to knock out the target gene, if edited, are identified.

Second, guide RNA candidates directing to those loci are characterized for off-target activity across the human genome.

Finally, guide RNAs with highly selective activity to the target DNA are verified, and then moved forward as candidates for potential therapeutic applications.

01:22 – 01:31

Based on that background research and testing, Intellia is evaluating the safety and efficacy of its investigational *in vivo* CRISPR-based therapies in clinical trials.