Whiteboard Animation: Understanding CRISPR/Cas9 Gene Editing and Intellia Therapeutics' Approach Transcript

0:00:00 – 0:00:05

Understanding CRISPR/Cas9 Gene Editing and Intellia Therapeutics' Approach

0:00:06 – 0:00:10

Genes are sections of the DNA that contain instructions for producing proteins.

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Sometimes, those DNA instructions contain errors and proteins are incorrectly produced, which can lead to disease.

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Other times, although the instructions are correct, a correctly produced protein acts differently than it is supposed to, also leading to disease.

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CRISPR/Cas9 is a precision DNA-editing technology that has been studied by scientists for its…

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…potential medical implications and uses since its discovery in 1987.

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For over a decade, Intellia Therapeutics has been exploring the therapeutic potential of their Nobel Prize-winning CRISPR/Cas9 technology…

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…to address the underlying mechanisms of a variety of human diseases at the genetic level, aiming to treat or potentially cure them.

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This groundbreaking technology is made up of two components: a “molecular GPS” called a guide RNA,

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…and a Cas9 enzyme that is responsible for DNA editing.

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Using the guide RNA as a sort of “molecular GPS,” the CRISPR/Cas9 complex can precisely target…

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…a specific section of DNA by recognizing a short, predefined location in a gene, which it marks as the target.

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Once the location is found, the CRISPR/Cas9 complex unwinds the double-stranded DNA…

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… and verifies that the “molecular GPS” has identified the intended target and that it matches the DNA strand.

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Then, the CRISPR/Cas9 complex edits each strand at a precise, predefined location.

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The cell's natural DNA repair mechanism immediately recognizes the cut ends of DNA and rejoins them.

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During this process, the cell's DNA repair machinery can insert or delete pieces of DNA.

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These small DNA insertions and deletions, also known as indels…

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…change the way the gene is read by the cell, which can result in the knockout or inactivation of the target gene.

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The edits made to the DNA in the target gene result in reduced production of the disease-causing protein.

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Intellia has developed an approach to deliver the gene-editing CRISPR/Cas9 complex…

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…to the specific cell types that produce the protein underlying the disease…

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…by packaging the CRISPR/Cas9 system within a rigorously tested and proprietary, nonviral lipid nanoparticle delivery system…

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…that enters the body through a one-time treatment via IV infusion.

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In animal models, Intellia's investigational CRISPR/Cas9 therapies have been shown to remain in circulation for as little as five days,

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…yet they are intended to have a permanent effect on disease.

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Intellia has conducted over a decade of rigorous research and preclinical testing…

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…to ensure its investigational CRISPR/Cas9 gene-editing therapies target the intended gene with the highest possible level of precision and accuracy.

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Clinical trials, including late-stage clinical trials, are ongoing to evaluate the safety and efficacy…

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…of Intellia's investigational *in vivo* CRISPR/Cas9 therapies in patients.

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By bringing CRISPR/Cas9 gene editing therapies to life, Intellia is at the forefront of changing medicine…

0:03:47 – 0:03:51

…to potentially treat or cure a variety of human diseases.