DNA

CRISPR-Cas9 Video

SPEAKER: Since a gene is like a page of text, what if gene doctors could fix disease-causing errors letter by letter? A new tool of extraordinary power called CRISPR promises just such precision.

JENNIFER DOUDNA: What CRISPR is, is a technology for changing the sequence of DNA in cells in a precise fashion, like a molecular scalpel. We can design it in the laboratory so that it matches the sequence of, let's say, a mistake in the DNA where we would like to trigger a change, to correct mutations that might otherwise cause disease. It's going to enable a lot of science to be done that was impossible to do in the past. And this is both at the level of research and applications.

DNA

Gene Therapy Case Study: Leber Congenital Amaurosis Video

SPEAKER 1: We think of viruses as the enemy, but they do some things really well. They deliver DNA to cells efficiently.

FREDA LEWIS-HALL: They come in, and they hijack our cellular machinery, if you would, so that they can duplicate themselves. And here we are now hijacking the hijacker to correct these gene mutations, which is absolutely brilliant.

SPEAKER 2: This form of gene therapy simply changes the virus's payload, stripping away its disease-causing genes and inserting a correct copy of the human gene that's flawed. The next step is to inject billions of these viral delivery trucks called vectors near the cells that need the replacement gene. The viruses take it from there, invading those cells and delivering the therapeutic gene.

RNA Splicing

Exon Skipping Case Study: Duchenne Muscular Dystrophy Video

AUSTIN: If I want to do something, I'll generally be able to do it. I like playing power soccer because it gives me the ability to play a sport. Maybe not in the same way, but I still get to be as competitive as everyone else.

NARRATOR: 16-year-old Austin and his 13-year-old brother Max face a lot of challenges. For starters, there's mobility. Max can still get himself around, but Austin lost the ability to walk six years ago. Health aide Patrick Claflin is there to help.

AUSTIN: He's my personal care assistant and part-time friend.

Protein Processing

Small Molecule Drug Case Study: Cystic Fibrosis Video

SPEAKER 1: Kimberly spent a lot of time in hospitals as she grew up.

SPEAKER 2: For 11 years, we spent our every Christmas in the hospital.

KIMBERLY: And some spring breaks too.

SPEAKER 2: There was times where she was like, Mommy, I hate cystic fibrosis. Why me?

SPEAKER 1: As Kimberly turned 12, Nielsen started to consider the treatment of last resort.

SPEAKER 3: We were within six months to a year of calling the transplant team and talking seriously about a lung transplant.

SPEAKER 1: Kimberly got the drug. But would it work well enough to keep her off the transplant list?

KIMBERLY: Right away, things started to change. It felt amazing that this small pill would change everything that I had in my life.

SPEAKER 4: All right, say cheese.

[*chuckles*]

SPEAKER 3: The last two years before we started the drug, she spent 116 days in the hospital. And since we started the drug, she's not been in the hospital one day. And that's now been over two years.