Editas has become the first of the group not only to attract crossover backers, but to begin discussing the diseases that its targeting. Its first program, Bosley says, is a potential treatment for a form of leber congenital amaurosis (LCA), a genetically driven blindness. It's a different form of the LCA than the gene therapy company Spark Therapeutics (NASDAQ: ONCE) is targeting; Bosley says the one Editas is going after can't be solved by gene therapy.

Beyond that, and Editas's ongoing immuno-oncology work with Juno, Editas has done some very early work in Duchenne muscular dystrophy and is exploring ways to repair a mutant hemoglobin gene—something that could have an impact in a range of bleeding disorders. That doesn't mean this is where Editas will ultimately focus—Bosley notes, for instance, that there are big technical challenges of producing an effective CRISPR-Cas9 therapy for Duchenne—but it's a glimpse into the company's thinking.

Bosley won't estimate how long it'll be before the first Editas therapy begins human testing, noting that the company is in the midst of preclinical work, testing its technology in patient cells. "We have a little bit more work to do before we can really be explicit about a specific timeline," she says.

Does that mean a few years? "I don't think it'll take that long," she says. "We'll move sooner than that if we have a construct that's good enough."

For those new to the story, CRISPR-Cas9 is a two-part system derived from a defense mechanism that bacteria use to fend off viruses. Think of it as a pair of molecular scissors (an enzyme called CRISPR-associated protein 9, or Cas9) being carried into a cell's nucleus by a strand of RNA that serves as a guide (clustered, regularly interspaced short palindromic repeats, aka CRISPR). Once there, the scissors may be able to snip out a defective gene, and perhaps replace it with a new, functioning one. In the case of Editas's LCA program, for instance, Bosley says the company aims to make two specific cuts in two different DNA sites to eliminate the genetic mutation causing the disease.

This isn't the first gene editing technique to emerge; Sangamo Biosciences (NASDAQ: SGMO) and its zinc finger nuclease platform have the most advanced gene editing candidate, a potential therapy for HIV in Phase 2 testing. But CRISPR-Cas9 has taken the medical world by storm because of how easy it is to use, and the broad potential it may have. CRISPR technology has already been used to modify the genomes of plants and animals, but that ease of use has also led to some serious ethical questions. One of the field's pioneers, UC Berkeley's Jennifer Doudna, and several others have called for a moratorium on using CRISPR-Cas9 to edit the human germline—making changes to sperm, eggs, and embryos that would then be passed along to future generations—and have warned against altering humans for non-medical reasons.

There are also some practical concerns when it comes to using CRISPR-Cas9 for therapeutics. How can a therapy using this technology be delivered into the body effectively? Will it safely do its work, or cut DNA in the wrong places? One wrong snip—a so-called off-target effect—could cause serious unintended consequences, and ensuring that this doesn't happen is just one of the technical challenges that have to be faced before a CRISPR-Cas9 drug begins human testing. One need only