look at the up and down history of gene therapy and RNA interference drugs to see the roller coaster likely ahead as researchers try to figure out how to use CRISPR-Cas9 for therapeutics.

While Bosley acknowledges the challenges to come, she notes that part of the excitement surrounding CRISPR-Cas9 is that it's come at a time when "our knowledge of the genome is just at a fundamentally different place" than it was many years ago. She adds that a lot of progress has been made to combat potential off-target effects, like figuring out the exact right size of the RNA guides and which types of Cas9 enzymes to use. Meanwhile, Editas co-founder Keith Joung has developed a tool called "Guide-Seq" to track instances of unintended DNA cuts.

And as for delivering these treatments, Editas "isn't trying to reinvent the wheel," Bosley says. Rather, it's looking to proven delivery methods—at least initially. For the LCA program, it's delivering a CRISPR/Cas9 using adeno-associated virus, a delivery vector that has been used by a number of gene therapy companies. It could use other established delivery technologies, like lipid nanoparticles (often used to shepherd RNA interference drugs into the body) or electroporation (in which an electric pulse creates tiny holes in cells that allow drugs to gain entry).

Still, delivery is "a critical challenge in this field, there's no question about that," she says.

A patent battle between Editas and Doudna's group at UC Berkeley is also part of the mix. The U.S. Patent and Trademark Office awarded the first CRISPR-related patent in April 2014 to the Broad Institute of MIT and Harvard for work led by the Broad's Feng Zhang (an Editas co-founder). The Berkeley group is fighting the patent, claiming it made the invention first. Doudna's work is licensed to Caribou, which in turn has licensed use of its technology for human therapies to Intellia. The work of Doudna's co-inventor, Emmanuelle Charpentier, is licensed to CRISPR. And Doudna herself was an Editas co-founder, but as MIT Technology Review first reported, later cut ties with the company. When asked about the patent case, Bosley didn't give an update directly, but said that the company has a "broad portfolio of IP" that it's licensed in, and that it's developing patent applications from its own internal work as well.

All of which is why the progress of Editas and its rivals will be so closely watched, and why the financing today marks such a noteworthy step for the technology. Crossover backers have been increasingly active during the biotech boom, joining up with early stage companies to lay the foundation for a number of public offerings. Editas has become the first of the CRISPR-Cas9 group to amass that kind of support, but deciding when to take the leap to the public markets is critical, particularly for a company with a new and unproven technology. Moderna executives, for instance, contended that they were not thinking of an IPO in the short term when they raised $450 million.

Bosley also brushed off thoughts of an IPO, at least in the short term. While Editas will almost certainly have to tap Wall Street at some point to build the broad type of company it hopes to be, there's much work to be done first. That means adding a significant number to its roughly 40-person staff, refining its strategy, and using some of that $120 million to bring several programs to clinical testing.