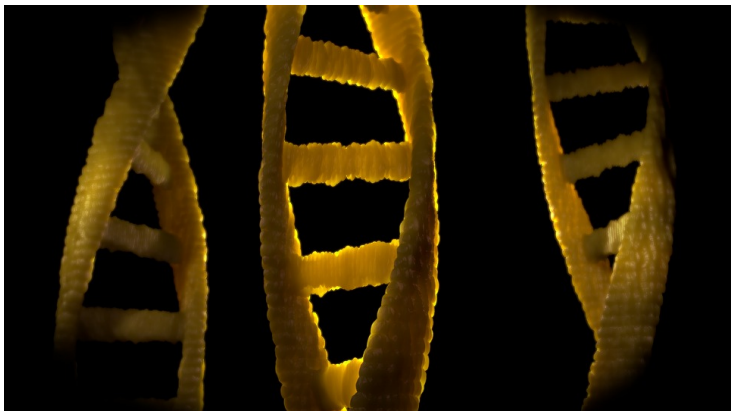


FDA halts CRISPR and Vertex's upcoming gene editing trial

01 June 2018 | News

The companies sought approval in April to begin the early-stage study in humans; it would be one of the first in the U.S. involving gene editing, a method of cutting out and replacing disease-causing snippets of DNA.



Cambridge startup CRISPR Therapeutics and Boston-based Vertex Pharmaceuticals said that the FDA has put a hold on highly anticipated upcoming trial of their experimental gene editing drug for sickle cell disease.

CRISPR and Vertex announced that the FDA had halted the upcoming trial which hasn't started yet. In a brief press release, the companies said that the FDA had placed the hold "pending the resolution of certain questions that will be provided by the FDA as part of its review." A trial of the same drug in Europe, targeting blood disorder Beta thalassemia, is still expected to begin in the second half of this year, reported Boston Business Journal.

The companies sought approval in April to begin the early-stage study in humans; it would be one of the first in the U.S. involving gene editing, a method of cutting out and replacing disease-causing snippets of DNA.

"CRISPR and Vertex expect to obtain additional information on the FDA's questions in the near future and plan to work rapidly with the FDA toward a resolution," the companies said.

CRISPR is one of three publicly-traded startups based in Cambridge that is focused on a particular kind of gene editing technology, called CRISPR/Cas9.