FDA rejects muscular dystrophy drug, says it doesn't work

October 25 2017, by Linda A. Johnson

This Oct. 14, 2015, file photo, shows the U.S. Food & Drug Administration campus in Silver Spring, Md. On Wednesday, Oct. 25, 2017, the FDA rejected an experimental drug for a common type of muscular dystrophy. The drug from PTC Therapeutics was intended for Duchenne muscular dystrophy patients with a certain genetic mutation. The muscle-destroying disorder affects 1 in 3,500 to 5,000 boys in the U.S. (AP Photo/Andrew Harnik, File)

The U.S. Food and Drug Administration has rejected an experimental

drug for a common type of muscular dystrophy.

The FDA's decision Wednesday was expected because the agency had said the drug didn't work in two key patient tests. Last month, FDA advisers voted 10-1 against recommending approval.

The drug from PTC Therapeutics was intended for Duchenne muscular dystrophy patients with a certain genetic mutation. The muscle-destroying disorder affects 1 in 3,500 to 5,000 boys in the U.S.

The South Plainfield, New Jersey company said it will appeal. The FDA had twice refused to review the company's applications. The company used a rare maneuver to force a review.

Last year, the FDA approved the first drug for Duchenne's, for a different group of patients, despite inconclusive evidence it worked.