British startup Healx has secured FDA approval for a phase 2a clinical trial of an AI-discovered compound that could help manage the symptoms of the genetic disorder Fragile X syndrome.

Cambridge, England-based startup Healx will begin a year-long trial with around 70 patients to test if HLX-0201, a drug compound that was originally developed as an anti-inflammatory treatment, can be repurposed to help manage symptoms caused by a genetic disorder that affects around 1 in 4,000 boys.

The start of the trial marks another milestone in the use of artificial intelligence to help find new applications for existing drugs by mining patient records and research databases. This promises to accelerate the years-long process, and $1.3 billion average cost, of developing and securing approval for new drugs. It could also mean new treatments for patients with rare conditions that have been overlooked by the pharmaceutical industry if AI can find multiple uses for existing medications.

Fragile X is caused by a single gene on the X chromosome malfunctioning. For those impacted, it can cause symptoms like autism, intellectual disabilities and behavioral
disorders. There is no cure for the genetic disorder, while a wide variety of medications are prescribed to treat behavioral symptoms like anxiety, aggression and attention-deficit disorder, but all carry serious side effects.

Tim Guilliams, CEO and cofounder of Healx, says that his team discovered the potential new application of the anti-inflammatory drug in a 24-month project that mined existing research, patient insights from FRAXA Research Foundation and Healx’s AI, which matched the neurological pathway targeted by the drug with the gene expression of the disease. The nearly year-long clinical trial will run as a combination study to see if the drug can help manage some of the behavioral symptoms of the genetic disorder.

“We are excited about the potential of HLX-0201: a proven drug with a long track record of effectiveness. It never would have occurred to us to investigate the compound for Fragile X without Healx’s innovative technology pointing us in the right direction, but the preclinical studies have shown remarkable effectiveness,” says Dr. Mike Tranfaglia, medical director and chief scientific officer at the Fraxa Research Foundation, which worked with the Healx team to uncover the drug.

Founded in 2014, by Guilliams and David Brown, co-inventor of Viagra, Healx has raised a total of $66 million in a Series A and B round. It is one of a crop of British startups in the AI drug discovery space like Exscientia, which listed on the Nasdaq with a $2.3 billion valuation earlier this month, and BenevolentAI, which used its AI to find ways to repurpose existing drugs to fight Covid,
culminating in Lilly obtaining emergency authorizations to use its rheumatoid arthritis drug baricitinib for hospitalized Covid patients. The space has attracted billions in funding from investors and big pharma but so far only a few AI-picked drugs have entered clinical trials.

“There is a lot of work in the AI drug discovery space but there are very few companies who have been able to go to clinical trials,” says Guilliams. “This is a key moment in our space and not just for our company.”

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