FRAXA, Healx Collaboration Identifies Therapies that Could Be Repurposed for Fragile X

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A collaboration between startup company Healx and nonprofit FRAXA Research Foundation is moving forward with preclinical testing after identifying a list of existing therapies that hold the most promise for treating fragile X syndrome.

In late 2017, Healx, based at the University of Cambridge in England, received a £435,000 grant from FRAXA to identify new fragile X therapies based on in vitro systems already approved by the U.S. Food and Drug Administration for other disorders, such as diabetes. This method, called drug repurposing, can be a quick and inexpensive way to develop new therapies.

To accomplish this, researchers used front-line drug-matching technologies developed at the University of Cambridge involving machine learning algorithms and computational biology to establish links between existing therapies and rare diseases.

This process identified eight therapies that hold the most potential for treating fragile X, including topiramate (anti-epileptic, autistic spectrum disorder), phenformin (linked to memory improvement), quinapril (potent with anti-inflammatory properties), penbutolol (used to treat high blood pressure), zardaverine (a bronchodilator agent), disulfiram (used for chronic alcoholism), sulindac (non-steroidal anti-inflammatory), and metoprolol (beta-blocker used in asthma treatment).

FRAXA has now granted Healx another $47,500 to continue with a new round of studies.

With the most promising compounds selected, the Fragile X Syndrome Drug Validation Initiative (FRAXA-DVI), which provides quick, cost-effective, objective testing of potential therapies, will determine which compounds are the best candidates to advance to clinical trials.

Researchers expect to finish the preclinical research within the next two years, after which therapies that hold the most promise for treating fragile X syndrome will be moving forward with preclinical testing after identifying a list of existing therapies that hold the most promise for treating fragile X syndrome.

Increased Protein Synthesis Is a Biomarker of Fragile X

Fragile X News Today

A collaboration between FRAXA and Healx identifies promising new potential therapies for Fragile X syndrome.

Be Repurposed for Fragile X

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