Repurposing available drugs for neurodevelopmental disorders: The fragile X experience

Tranfaglia, Michael R.

Thibodeaux, Clare

Mason, Daniel J.

Brown, David

Roberts, Ian

Smith, Richard

Guilliams, Tim

Cogram, Patricia

Many available drugs have been repurposed as treatments for neurodevelopmental disorders. In the specific case of fragile X syndrome, many clinical trials of available drugs have been conducted with the goal of disease modification. In some cases, detailed understanding of basic disease mechanisms has guided the choice of drugs for clinical trials, and several notable successes in fragile X clinical trials have led to common use of drugs such as minocycline in routine medical practice. Newer technologies like Disease-Gene Expression Matching (DGEM) may allow for more rapid identification of promising repurposing candidates. A DGEM study predicted that sulindac could be therapeutic for fragile X, and subsequent preclinical validation studies have shown promising results. The use of combinations of available drugs and nutraceuticals has the potential to greatly expand the options for repurposing, and may even be a viable business strategy. This article is part of the S