Most common form of inherited intellectual disability may be treatable

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Advancements over the last 10 years in understanding intellectual disability (ID, formerly mental retardation), have led to the once-unimaginable possibility that ID may be treatable, a review of more than 100 studies on the topic has concluded. It appears in *ACS Chemical Neuroscience*.

Aileen Healy and colleagues explain that people long have viewed intellectual disability as permanent and untreatable, with medical care focusing on relieving some of the symptoms rather than correcting the underlying causes. That includes Fragile X syndrome (FXS), the most common inherited form of intellectual disability. FXS occurs in an array of forms, ranging from mild learning disabilities to more severe intellectual and developmental disabilities. It is the most common known cause of autism or autistic-like behaviors.

Scientists are now beginning to get a handle on the changes that happen to cells and molecules in the body because of a mutation in the Fragile X Mental Retardation 1 gene. That gene contains instructions for making a key protein vital for nerve function in the brain, and does not work properly in FXS. With a better understanding of the biological effects of the mutation, the scientists say that treatments for FXS and similar disorders now seem possible. In addition, several drugs tested in humans seem promising. "In conclusion, the recent clinical introduction of multiple compounds representing a variety of mechanistic approaches to the disorder represents an exciting opportunity to realize the mission of implementing effective treatments of ID," say the researchers.

More information: Fragile X Syndrome: An Update on Developing Treatment Modalities *ACS Chemical Neuroscience*, Article ASAP, DOI: 10.1021/cn200019z

Abstract

Intellectual disability (ID; mental retardation) is considered an immutable condition. Current medical practices are aimed at relieving symptoms and not at altering the underlying cognitive deficits. Scientific advancements from the past decade have led to the exciting possibility that ID may now be treatable. Moreover, pharmaceutical therapies targeting the most common form of inherited ID, Fragile X syndrome (FXS), may become the new benchmark for central nervous system (CNS) drug discovery: seeking cures for neurodevelopmental disorders.

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