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Scientists identify treatments that may restore brain function to autism patients

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Scientists have identified a pair of treatments that may restore brain function to autism patients who lack a gene critical to maintaining connections between neurons, according to a study from the Peter O'Donnell Jr. Brain Institute at UT Southwestern Medical Center.

Although this gene has been linked to abnormal brain size, the research in mice demonstrates the gene has no such role and instead is needed to regulate a protein capable of inhibiting the ability of neurons to communicate with each other. Furthermore, the study found that brain connections lost due to absence of the gene can be fully restored within hours by using drugs that block the protein.

"The deletion of this gene impairs brain function in a major way, and we found a way to repair the damage. But we have more work to do before we try these treatments on people. The findings give us a clue as to what pathways are altered and where to look," said Dr. Craig Powell, Director of Preclinical Research, Director of the Erma Lowe Center for Alzheimer's Research, and Section Chief of Developmental Brain Disorders in the Department of Neurology & Neurotherapeutics.

The study published in *Nature* comes amid several recent and ongoing efforts to improve early diagnosis of autism spectrum disorder (ASD) by shifting focus to biological measurements instead of behavioral symptoms. But little is understood about what genes may be effective targets for treatment after a diagnosis is made.

Dr. Powell's research focused on *KCTD13*, one of 29 genes in an area of chromosome 16 that is strongly linked to autism, developmental delay, and intellectual disability.

By deleting the gene in mice and measuring various effects, Dr. Powell's team disproved previous research that indicated *KCTD13* deletion caused brain overgrowth commonly seen in people affected by mutations in this chromosomal region. Instead of altering brain size, *Kctd13*'s absence reduced

by half the amount of synaptic connections through which neurons communicate with each other.

Scientists traced the root of the problem to the RhoA protein, which accumulates when *Kctd13* is missing. By administering RhoA-inhibiting drugs – either Rhosin or Exoenzyme C3 – Dr. Powell's laboratory restored brain function in less than four hours.

Exoenzyme C3 is already in human clinical trials for spinal-cord injury – a necessary first step that could speed the process for clinical trials involving autism.

However, Dr. Powell said scientists must first investigate *KCTD13*'s role in the broader gene pool and study whether improving brain connections can reverse behavioral changes.

"This is an important step, but there is a long road ahead. Now we need to better understand the function of other genes in this chromosomal region and how these may lead to brain dysfunction and the behavioral changes we call autism," said Dr. Powell, Professor of Neurology & Neurotherapeutics, Neuroscience, and Psychiatry, and holder of the Ed and Sue Rose Distinguished Professorship in Neurology.

Source:

http://www.utsouthwestern.edu/newsroom/articles/year-2017/autism-gene-powell.html

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