Growth Hormone Eases Autism: Study

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A growth hormone has been shown to markedly improve the social deficits that typically affect people who have autism spectrum disorder (ASD).

A new study conducted at the Icahn School of Medicine at Mount Sinai found people with autism and a related genetic condition — known as Phelan-McDermid syndrome (PMS) — benefit significantly from the use of insulin-like growth factor-1 (IGF-1).

The findings, the first to examine IGF-1 treatment in PMS, were published on Pub Med, a public database of biomedical topics maintained by the National Institutes of Health, and in the journal Molecular Autism.

"Ours is the first controlled trial of any treatment for [PMS]," said Alexander Kolevzon, M.D., clinical director of the Seaver Autism Center at the Icahn School of Medicine. "Because different genetic causes of ASD converge on common underlying chemical signaling pathways, the findings of this study may have implications for many forms of ASD."

IGF-1 strengthens communication between brain cells, in response to increases or decreases in their activity. It is also approved by the Food and Drug Administration for the treatment of short stature.

PMS is a disorder linked to genetic defects on the human chromosome 22. Most people with PMS also have autism, and face developmental and language delays, and motor skill deficits.

The Mount Sinai study is the first to suggest that IGF-1 is safe, tolerable and associated with significant improvement in both social impairment and restrictive behaviors (fascination with one subject or activity; strong attachment to one specific object; preoccupation with part[s] of an object rather than the whole object; preoccupation with movement or things that move) in people with Phelan-McDermid syndrome, said the study authors.

For the new study, researchers tracked nine children aged 5-15 years diagnosed with PMS given three months of treatment with IGF-1 and three months of a placebo, in random order.

The IGF-1 was found to produce significant improvements in social and restrictive behaviors, based on standard assessments of autism symptoms, compared to the placebo.

"This clinical trial is part of a paradigm shift to develop targeted, disease modifying medicines specifically to treat the core symptoms of ASD," said Joseph Buxbaum, director of the Seaver Autism Center and Professor of Psychiatry, Genetics and Genomic Sciences and Neuroscience at Mount Sinai. "Results from this pilot trial will facilitate larger studies that more definitively
inform efficacy and better targeted therapeutic treatments."

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