Drug shows some benefit for kids with autism

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Study found no gains in lethargy, social withdrawal, but those on the drug improved in other ways.

(HealthDay)—An experimental drug for autism did not improve levels of lethargy and social withdrawal in children who took it, but it did show some other benefits, a new study finds.

Children on arbaclofen did improve on an overall measure of autism severity when compared to kids taking an inactive placebo, said lead researcher Dr. Jeremy Veenstra-VanderWeele, an associate professor of psychiatry, pediatrics and pharmacology at Vanderbilt University.

He is to present the findings Thursday at the International Meeting for Autism Research (IMFAR) in Spain.

One of 88 children in the United States is now diagnosed with an autism spectrum disorder, the umbrella term for complex brain development disorders marked by problems in social interaction and communication.

Veenstra-VanderWeele focused on evaluating the social improvement with the drug because earlier research had suggested it could help. However, one of the earlier studies did not compare the drug to a placebo, but simply measured improvement in those who took the drug.

In the new study, Veenstra-VanderWeele and his team assigned 150 people with autism, aged 5 to 21, to take the medicine or a placebo, without knowing which group they were in, for eight weeks. The participants had been diagnosed with autistic disorder, Asperger's syndrome or another related condition known as pervasive developmental disorder.

In all, 130 finished the study. When no differences were found in social withdrawal or lethargy between the two groups, the researchers looked at a scale that measures severity and improvement of autism with treatment.

Those on the drug improved more on that scale. A child, for instance, who began the study evaluated as having marked severity might be described as moderate by the study's end, Veenstra-VanderWeele said.

"This is the sort of improvement that would motivate us to start a medicine," he said.

The drug is believed to work, Veenstra-VanderWeele said, by increasing inhibition, improving social functioning and interactions.

Right now, Veenstra-VanderWeele said, "there is no medication that has clear evidence to improve social function in autism."

Those on the drug did report side effects, including suicidal thoughts reported by one patient on the drug and one on the placebo. Some patients on the drug became upset more easily; others reported sleepiness.

The next phase of trials of the drug are in the planning stages, Veenstra-VanderWeele said.

But more research is needed, said Dr. Andrew Adesman, chief of developmental and behavioral pediatrics at the Steven and Alexandra Cohen Children's Medical Center of New York.

Even though the expected benefit did not materialize, Adesman sees a reason to continue to study the medication. "There is [still] some suggestion of benefit from the medicine," Adesman said. "It just didn't quite show up where they expected."

The drug may offer benefit to some children with autism, Adesman said. "But it's unclear which children may be the best candidates."

The trial received funding from the drug's maker, Seaside Therapeutics. The medication is not currently approved by the U.S. Food and Drug Administration.

The data and conclusions of research presented at medical meetings should be viewed as preliminary until published in a peer-reviewed journal.

More information: To learn more about autism, visit the U.S. Centers for Disease Control and Prevention.

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