Search for potential autism treatments turns to ‘trust hormone’

For all of the attention that autism has received, the condition still lacks pharmacological treatments. Faced with this frustrating reality, some researchers have begun exploring whether oxytocin, the so-called ‘trust hormone’, could ease symptoms of the disorder, such as repetitive behaviors and difficulties with social interactions.

Autism spectrum disorders affect an estimated one in every 150 children in the US alone, according to government statistics. So far, the US Food and Drug Administration (FDA) has approved only one drug—Risperdal, which lessens irritability—to treat behaviors associated with autism. But the drug doesn’t help people with autism recognize emotions or eliminate the repetitive, obsessive behaviors linked to the disorder.

“That’s why oxytocin is really exciting,” says Thomas Owley, head of the University of Illinois at Chicago’s Neurodevelopmental Pharmacology Clinic. “It’s really looking at addressing social deficits, for which we have no pharmacological treatment.”

Oxytocin received its nickname of the trust hormone after laboratory experiments demonstrated that it could make people more willing to trust others with their money. Interest in oxytocin intensified in the mid-1990s, partly as a result of animal studies conducted by Larry Young at Emory University in Atlanta, Georgia. Young found that blocking the oxytocin receptors in prairie voles’ brains made these usually social animals less likely to bond (Adv. Exp. Med. Biol. 395, 227–234; 1995).

Young’s findings have led some researchers to suspect that oxytocin might also play a part in autism, especially because children with the disorder seem to have lower amounts of the hormone in their blood plasma (Biol. Psychiatry 43, 270–277; 1998). More recently, scientists have identified mutations in the oxytocin receptor gene in people with autism (Neurosci. Lett. 417, 6–9; 2007; Biol. Psychiatry 58, 74–77; 2005).

Emotional interpretation

Eric Hollander, a researcher at the Mount Sinai School of Medicine in New York, has led two studies in which high-functioning autistic adults received injections of oxytocin. In one trial, the treatment decreased repetitive behaviors such as body rocking (Neuropsychopharmacology 28, 193–198; 2003). In another, those who received the hormone could interpret emotions more accurately than their counterparts who received a placebo. For example, they could discern whether a neutral sentence, such as “The boy went to the store,” was read in an angry, sad, happy or indifferent tone (Biol. Psychiatry 61, 498–503; 2007). “The results are preliminary, but it’s really exciting that you can see this kind of improvement in adults,” says Hollander.

Researchers are now turning to a nasal spray, because they believe that this delivery method will help the hormone reach the brain. Hollander is a consultant for Nastech, a pharmaceutical company in Bothell, Washington, that is investigating carbetocin, a synthetic oxytocin that the company says will last longer in the body. The company hopes to have the product approved within the next five to seven years.

“It would be presumptuous to say this is a cure,” says Nastech president Gordon Brandt. “But at this point, anything would help. We need treatments.” On 8 April, scientists from Nastech, which specializes in nasal drug delivery, will present their potential approach to treating autism at the Experimental Biology 2008 meeting in San Diego.

Other groups are looking at intranasal oxytocin, too. Evdokia Anagnostou, who, like Hollander, is based at the Mount Sinai School of Medicine, currently leads a pilot study to investigate the long-term therapeutic effects of an oxytocin nasal spray. "There's still a lot to figure out," says Anagnostou. She adds that “we need all of the safety and efficacy data to say that it’s safe and effective, and we need to figure out the best way to give it, and how often to give it.” Across the pond, Simon Baron-Cohen, director of the Autism Research Centre at the University of Cambridge in the UK, is currently awaiting approval from the local research ethics committee to start a similar study involving 25 adults.

Hurdles and hopes

In addition to having to prove the treatment’s safety and efficacy, researchers may face regulatory hurdles before testing it in children. Doctors have used oxytocin nasal spray in adults to promote lactation, but FDA regulations present challenges for the investigation of the hormone as a new drug in children.

Another drug in the pipeline could potentially treat the social deficits associated with autism by reducing levels of a protein called metabotropic glutamate receptor 5. A specific genetic defect seen in about 5% of autism cases causes an excess of the protein. Researchers say that the experimental drug, known as STX107, succeeded in stopping the overproduction of the protein in rodents.

Some people with autism have argued that their condition requires greater acceptance and not a medical solution. But others with the disorder, and psychiatrists, say that pharmacological options warrant investigation. “We don’t know whether these approaches will help, but, given the need, I think they’re worth exploring,” says Owley.

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