OPINION

Top of the class

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10 JUNE 2011

Seaside-Article2.jpg

'Tis the season of graduations, even for autism drugs.

About a year ago, a small biotech company announced that one of its compounds **improved social behaviors** in a group of 15 children with fragile X syndrome. Last week, the drug matriculated to a phase III trial — the last and most difficult step on the long road to regulatory approval.

The drug, called arbaclofen, or STX209, was developed by Seaside Therapeutics in Cambridge, Massachusetts. A decade ago, neuroscientist and Seaside founder **Mark Bear** had shown in mice that the cognitive problems of fragile X syndrome are caused by runaway glutamate, a common neurotransmitter.

STX209 acts on this pathway: It activates gamma-amino butyric acid type B (GABA-B) receptors, effectively putting the brakes on glutamate signaling.

In Seaside's new trial, 120 individuals with fragile X will receive either STX209 or placebo for eight weeks. The company plans to enroll participants ranging in age from 12 to 25 years at 20 different clinics across the U.S.

Seaside is also investigating whether the drug can improve social problems of people who have autism but not fragile X syndrome. Many studies have implicated glutamate signaling and **GABA-B glitches** in people with autism.

Last fall, the company's researchers reported that STX209 **quells tantrums, irritability and social withdrawal** in children with autism. That study was 'open-label:' the participants' doctors and parents knew they were taking the drug, making them susceptible to the placebo effect.

The company plans to launch a placebo-controlled trial of STX209 in autism this summer.