

Secret funding fosters hope for new drugs for autism

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Funding from an anonymous wealthy family has been the secret to progress, at long last, in developing drugs that show promise for helping millions of people worldwide with Fragile X syndrome, the most common genetic cause of autism. That's the topic of a fascinating article in the current issue of Chemical & Engineering News (C&EN), ACS' weekly newsmagazine.

C&EN Senior Editor Lisa Jarvis notes that until recent interest from big pharmaceutical companies, a small drug company in Cambridge, Mass. named Seaside Therapeutics was virtually the only company trying to develop drugs for [autism](#) and [fragile X syndrome](#). Diagnoses of autism and related conditions termed autism spectrum disorders have increased dramatically since the 1980s, for reasons not yet fully clear. They affect millions of people worldwide. Fragile X syndrome, the most common known genetic cause of autism, results from mutation in a single gene. Its symptoms range from learning impairment to mental retardation. The disease affects about 1 in 4,000 males and 1 in 6,000 to 8,000 females.

The article describes how Seaside, armed with funding from an anonymous wealthy family and new insights into the basic science behind these disorders, is making progress toward treating these much-neglected diseases. Two of the company's potential drugs show promise in clinical trials as treatments for Fragile X syndrome. One appears to improve the behavior of children with severe social impairments. On the heels of Seaside's encouraging results, big pharmaceutical companies that once showed little interest in tackling these diseases are now trying

to develop their own new medications.

More information: "Tackling Fragile X", This story is available at pubs.acs.org/cen/business/88/8837bus3.html

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