

## Gene therapy delivered directly to the brain treats extremely rare disease in children

When Rylae-Ann Poulin was a year old, she didn't crawl or babble like other kids her age. A rare genetic disorder kept her from even lifting her head. Her parents took turns holding her upright at night just so she could breathe comfortably and sleep.

Then, months later, doctors delivered gene therapy directly to her brain.

Now the 4-year-old is walking, running, swimming, reading and riding horses — “just doing so many amazing things that doctors once said were impossible,” said her mother, Judy Wei.

Rylae-Ann, who lives with her family in Bangkok, was among the first to benefit from a new way of delivering gene therapy — attacking diseases inside the brain — that experts believe holds great promise for treating a host of brain disorders.

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Her treatment recently became the first brain-delivered gene therapy after its approval in Europe and the United Kingdom for AADC deficiency, a disorder that interferes with the way cells in the nervous system communicate. New Jersey drugmaker PTC Therapeutics plans to seek U.S. approval this year.

Meanwhile, about 30 U.S. studies testing gene therapy to the brain for various disorders are ongoing, according to the National Institutes of Health. One, led by Dr. Krystof Bankiewicz at Ohio State University, also targets AADC deficiency. Others test treatments for disorders such as Alzheimer's, Parkinson's and Huntington's.

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