

New treatment provided for young patient with rare neurological disorder

by Texas Children's Hospital, USA



Texas Children's neurosurgeon Dr. Daniel Curry leads first-ever FDA-approved gene therapy treatment for AADC deficiency. Credit: Texas Children's Hospital

Texas Children's is pleased to announce that a three-year-old girl has been successfully treated with the first-ever FDA-approved gene therapy treatment for AADC deficiency.

Aromatic l-amino acid decarboxylase (AADC) deficiency is an extremely rare, inherited [neurological disorder](#) that prevents the brain from producing dopamine and serotonin—essential chemicals for controlling movement, mood and basic nervous system functions. The literature reports approximately 350 people with this condition worldwide. Historically, there was no cure or approved treatment for AADC, and the shortened life expectancy was estimated between five and seven years of age.

"Texas Children's was the largest contributor to the clinical trial in the U.S. that led to this drug's approval," said Dr. Daniel J. Curry, who performed the six-hour surgery. Dr. Curry is Director of Functional Neurosurgery and Epilepsy Surgery at Texas Children's Hospital and Professor of Neurological Surgery at Baylor College of Medicine.

"Before now, AADC deficiency was a hopeless diagnosis. With this treatment, we have entered a whole new era where we can deliver solutions to formerly untreatable genetic problems. This is the first step in hopefully many future strides toward the molecular correction of inborn deficits for which there used to be no cure."

Kebilidi (eladocagene exuparvovec-tneq) was approved on November 13, 2024, after a clinical trial involving Texas Children's Hospital and two other testing sites. It is a modified adeno-associated virus serotype 2 (AAV2) vector-based gene therapy administered by a neurosurgeon directly into the brain's putamen area, which is involved in learning and motor control. The procedure is a minimally invasive stereotactic neurosurgery that includes four infusions during one surgical session.

The Texas Children's patient who received the treatment, a 3-year-old girl, first showed signs of developmental delay when she was 7 months old. She had poor muscle control and difficulty holding up her head—one of the common symptoms of AADC deficiency, along with muscle weakness, movement disorders and seizures. Genetic testing confirmed her diagnosis when she was 18 months old, and her local care team has managed her condition with medication and physical therapy until this breakthrough treatment at Texas Children's became available.

"The treatment was successfully administered with no complications," said Dr. Curry. "After a two-week stay in the hospital for follow-up care and monitoring, she and her family are now back home and feeling very optimistic. The therapy is expected to take 2-6 months to show measurable impact, but she is already showing signs of increased energy and happiness."

Dr. Curry helped develop the Texas Children's Intracerebral Gene Therapy Program with investigations into intraputaminal gene therapy for AADC deficiency, as well as investigations into gene therapy for Rett syndrome and NGLY-1 deficiency. Dr. Curry is also Director of the Neuroinfusion Service at Texas Children's, conducting intracerebral enzyme replacement therapy in Batten's disease and other neurodegenerative diseases.

"With these exciting advancements in gene delivery and surgical precision, we can not only treat the root cause of diseases in the brain, but hopefully reverse them," said Dr. Curry. "This will make a life-changing difference for so many patients."

Provided by [Texas Children's Hospital](#)

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