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Federal officials walked back claims on leucovorin. Here's what families should know now

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By Michal Ruprecht

FDA reverses stance on autism drug

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The drug leucovorin was touted by the Trump administration in September as a potential treatment for thousands of children with autism. Now, some parents and guardians of children with autism say they feel abandoned by the US Food and Drug Administration's **new stance** on leucovorin.

“I bet some probably feel betrayed. I kind of do,” Keith Joyce, 60, said. He is the guardian of a 5-year-old with autism who takes the drug. He added: “I kind of felt vindicated with what I was doing. But now they’re saying, ‘Oh, nope, we’re out, and we were wrong.’ ”

Instead expanding access to leucovorin for “hundreds of thousands” of children with autism, the FDA on Tuesday **made a narrower approval** of the drug for people with a rare genetic disease. Fewer than 50 cases of the condition — cerebral folate deficiency in the folate receptor 1 gene — have been **identified** worldwide.

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Senior FDA officials said this week that there isn't “sufficient data” that leucovorin is a potential treatment for autism.

“It'll be up to patients to talk with their physicians to just see if [leucovorin] might be right for them,” one senior official said.

“We don’t have the evidence now for a broader approval,” another official added.

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Clinicians said the FDA’s decision itself is not controversial. Autism experts told CNN that they applauded the move, describing it as an evidence-based approach to treatment.

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“Today’s decision by the FDA reflects what the science has indicated so far: that evidence for leucovorin as an effective treatment for autism remains limited,” Andy Shih, the chief science officer at the nonprofit advocacy group Autism Speaks wrote in a statement to CNN. “We understand that some families may feel disappointed by this news.”

He added: “Families are always searching for ways to support their child, and early reports about the promise of leucovorin brought hope to many.”

Meagan Johnson, a mom in Pflugerville, Texas, who recently struggled to find the medication for her 3-year-old son Jack, told CNN she feels hurt by the decision after so

much hope was pinned on leucovorin.

“I’m frustrated with the administration,” she said. “I’m tired of them coming out and prematurely saying things without the information to back it.”

What might be next for leucovorin

Health officials endorsed leucovorin last fall — and word spread quickly, spurring interest in the drug. A paper published last week found that prescriptions rose in the last few months of 2025, even though professional societies like the American Academy of Pediatrics recommended against using leucovorin to treat autism.

Leucovorin endorsement
for autism by FDA falls ...

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Bhooshan Sawant, the CEO of ReligenDX — a company that produces the folate receptor autoantibody test, which was popularized in the wake of the White House announcement and is used by many doctors who prescribe leucovorin — also noted a sharp uptick in interest.

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The test is not covered by insurance and costs nearly \$300. The broader research community **does not recommend** the test, calling it inaccurate and experimental, and pointing to evidence that spinal taps are more accurate. Sawant maintains that the test is accurate and reliable but says he would like to see more research.

ReligenDX says that compared with January 2025 averages, it began processing seven times more tests at the height of the frenzy. Now, it says it remained at about 1,600 tests per month: six times higher than monthly averages before leucovorin gained widespread attention.

It's unclear whether all those patients receive a prescription after the test, but some experts said the data and anecdotal evidence suggest that interest in leucovorin is unlikely to wane anytime soon.

“I have days in clinic where every single family is asking about it,” said Dr. Audrey Brumback, a pediatric neurologist at the University of Texas at Austin Dell Medical School. “People know about this and are asking about it.”

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But others like Dr. Helen Tager-Flusberg, an autism researcher at Boston University, are more optimistic that the tide on leucovorin use may turn in the other direction much sooner if more federal officials reinforce the FDA's message.

“My great aspiration is that other parts of Health and Human Services will follow suit,” Tager-Flusberg said. “I hope the FDA continues on the path that they started today.”



Jose Morales-Ortiz, 5, shown with his guardian, Keith Joyce. Morales-Ortiz has autism and was taking leucovorin even before it was touted by the White House in a September briefing. *(Courtesy of Keith Joyce)*

Joyce says 5-year-old Jose Morales-Ortiz started **taking leucovorin** last year. The child was nonverbal before treatment, he said, but slowly improved. Joyce attributes that to the drug and said Morales-Ortiz can now say eight words.

But Joyce now worries that after the FDA's decision, more doctors — including Morales-Ortiz's — may be less willing to prescribe the drug. Many parents are already **struggling** to find providers willing to write a prescription. Drug shortages are also

making it difficult to track down the medication, though FDA officials said the supply remains stable.

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Sawant, the owner of the testing company — and a proponent of leucovorin — fears that some physicians may interpret the FDA's announcement as a reason to stop prescribing leucovorin.

A smaller pool of doctors willing to prescribe the drug can create an opening for unconventional providers to step in and take advantage of families.

Some parents pay thousands to get a prescription — something Dr. Sarah Mohiuddin, a clinician who runs the Multidisciplinary Autism Program at Michigan Medicine, warns about.

“Unfortunately, there are bad-faith players out there who might take advantage of people's situation,” Mohiuddin said. She recently **launched** an initiative to improve communication between clinicians and parents of children with autism. “I think there are opportunities to continue to partner with the medical community.”

Support for families of children with autism

While the drug is considered safe, Brumback warned that doses given to most children can be 100 times **higher** than the recommended daily requirement of folate

— the active ingredient in leucovorin. And much remains unknown about its long-term effects. Parents who opt to pursue leucovorin treatment should work closely with a specialist.

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Guidance **published** on Tuesday by the Child Neurology Society says that improvements parents notice with leucovorin may simply reflect normal development rather than the drug's effects — something a specialist can help determine.

Regardless of their decision about leucovorin, Brumback said, parents should also work with a specialist to find a therapeutic plan that works for their child. For some, that could mean additional genetic testing or finding ways to improve quality of life. Additional supports that help kids develop skills, like applied behavior analysis therapy, are equally important.

“We are all frustrated that we don't have a treatment for autism,” Brumback said.

But she doesn't view additional clinical trials on leucovorin as the path forward, given the limited evidence supporting its use. “If we do a large clinical trial, it won't be motivated by science, it'll be motivated by the need for the public to have a definitive answer for something that wasn't really a question in the first place.

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“Coming to see me in clinic and having us talk about it, I think that that’s the thing that I really want families to walk away with,” Brumback said. “We’re here to help. We’re not the enemy.”

CNN’s Meg Tirrell and Brenda Goodman contributed to this report.



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