HEALTH

As parents clamor for a treatment touted for autism, doctors hesitate to prescribe it

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Swathi Balantrapu has been searching for a treatment for her 10-year-old son with autism.

She first tried applied behavior analysis therapy, or ABA therapy — an evidence-based approach. She later pursued a $7,000 movement and stretching program. Like many parents of autistic kids, she also turned to alternative options, including a stem cell therapy treatment in Germany, costing $40,000 per year, repeated over the past three years.

After a press conference last fall, where federal health officials touted a new treatment for autism, Balantrapu found a new reason to hope her son's condition could be cured.

"I would do anything for my child," she said. "Maybe there is something to it. It just feels like one last stone unturned."

The treatment in question is leucovorin, a derivative of vitamin B9 — also called folinic acid — that is commonly used during cancer treatment. Federal officials said it could help treat a rare brain condition called cerebral folate deficiency and that it could benefit "hundreds of thousands" of kids with autism.

SCIENCE

Can the prescription drug leucovorin treat autism? History says, probably not

The announcement triggered a surge of interest among parents seeking the medication, with social media groups popping up with tens of thousands of members sharing doctors' referrals and notes. It also led to pushback from major medical groups advising against prescribing it routinely.

This has put pressure on clinicians and led to a divide between providers and parents.

"All of a sudden, there's this onslaught of information being disseminated that we now have to explain, disentangle and interpret," said Dr. Shafali Jeste, an autism researcher at the University of California, Los Angeles. "It's a big burden, and we're all willing to bear it … but I think that a lot of it ends up becoming a distraction."

Using leucovorin to treat patients with cerebral folate deficiency is not controversial, experts told NPR — and it's been done for decades. But they say that the Trump administration's messaging was misleading.

Dr. Shafali Jeste
Alex Beattie

And in fact, last week, the administration walked back its earlier claim that the drug could treat autism, expanding approval only for a subset of patients with cerebral folate deficiency — those with *FOLR1*-related cerebral folate transport deficiency, an extremely rare condition with fewer than 50 cases identified worldwide.

"Right now we don't have sufficient data to say that we could establish efficacy for autism more broadly," a senior FDA official said in a briefing. "It'll be up to patients to talk with their physicians to see if that might be right for them."

PUBLIC HEALTH

Trump blames Tylenol for autism. Science doesn't back him up

It's too early to tell if the new guidance will dampen interest in the drug among families of children with autism, but many doctors expect demand to persist.

The damage to public perception has already been done, Jeste said, and it will take a "really long time" for enthusiasm around the drug to subside. Just on Monday, she saw nine patients. All but one family asked about the drug.

Clamoring for a new cure

After the initial press conference last fall, many parents came away with the impression that leucovorin was a proven treatment for autism, said Dr. Frederick Bassal, a child neurologist at the University of California, Davis. Others assumed that a substantial proportion of children with autism have cerebral folate deficiency.

This claim, based on a small study, isn't backed by enough evidence, experts said. Dr. Alice Kuo, a pediatrician at the University of California, Los Angeles, said the study was too small to conclude that a correlation exists.

Still demand for the drug spiked, fueled by online discussion and growing interest among parents. When her son's pediatrician, who works at a big health system in Palo Alto, Calif., would not prescribe the drug, Balantrapu turned to Facebook.

Keith Joyce (left), who is raising a 5-year-old with autism, ran a Facebook group for parents interested in leucovorin as a treatment for their children with autism.

Joyce family

She wasn't the only one. Around 80,000 other users joined a Facebook group moderated by Keith Joyce (Facebook deleted the group in late October, writing it had violated its community standards "multiple times." Joyce attempted to build a similar community on Telegram).

Hundreds of posts poured in every day, said Joyce, 60. Most came from parents attempting to find a doctor who would prescribe leucovorin for their child. Many believe the drug could help nonverbal children begin to speak.

Joyce himself cares for a 5-year-old with autism and said the child began speaking after starting the medication.

"Parents were very frustrated," Joyce recalled, likening the situation to opening Pandora's box. "Many doctors were saying, 'I'm not going to prescribe it simply because there's not enough information.'"

Interest remains high. There are several other groups on social media, including a WhatsApp group with dozens of messages a day, sharing prescribers' names, queries and tips for how parents should administer the drug. In all, Joyce said many parents — even those who did receive a script from their doctor — are largely left to navigate treatment on their own.

Prescriptions for leucovorin increased by 71% in the last quarter of 2025, according to a paper published this month. The rise in prescriptions shows "the apparent power that public authority figures have to drive sudden changes in health care practices," the authors wrote, referring to the Trump administration press conference last fall.

Joyce called last week's announcement from the FDA limiting approval to a narrow subset of patients, "maddening," and said it would now be "even more difficult" to get a prescription.

TREATMENTS

It's the 'gold standard' in autism care. Why are states reining it in?

"It actually makes me angry," Joyce said. Joyce has done multiple interviews with the press about the 5-year-old's story. "I put him out there and all this to promote the effect. Now they are basically saying the opposite."

Mainstream medicine's reaction

Many doctors struggle with what to do about the demand for a drug once far outside the orbit of autism care, says Dr. Sarah Mohiuddin, who directs the Multidisciplinary Autism Program at Michigan Medicine.

Uncertainty has lingered after last fall's announcement, even after major medical associations like the American Academy of Pediatrics and the Coalition of Autism

Scientists said they do not recommend the drug for autism, citing insufficient evidence to support its use.

Most health systems issued similar guidance. Others like Kaiser Permanente and Oregon Health and Science University have stated that their providers will not prescribe the drug until more research is done on its safety and effectiveness.

Leucovorin is generally considered a safe medication. And Mohiuddin said if patients use it briefly, it "most likely" won't cause harm. But the drug's safety profile hasn't been established in children with autism. And no data exists on long-term use of the drug for longer than six months in that patient population, Mohiuddin noted.

Dosing is a mystery as well. Those who prescribe the drug typically follow a protocol created in 2013 — which has been replicated by only a handful of studies — and can be up to 100 times higher than the minimum recommended daily intake of folate, a derivative of leucovorin's active ingredient. Current formulations of the drug also persist in the bloodstream for long periods, which can have unintended consequences.

The two double-blinded, placebo-controlled trials that tested the drug in kids with autism didn't test it as a standalone treatment either, Mohiuddin said. That means ABA therapy and other early intervention treatments remain important.

One of those studies was retracted by the European Journal of Pediatrics on Jan. 29 because of "errors in the results" and "concerns with the statistical analyses performed." Only two of the six authors responded to and agreed with the journal's retraction.

Still despite concerns with the research, the Trump administration appointed at least one doctor who regularly prescribes it to the federal autism advisory board. Another new member referred to a physician who prescribes the drug as one of her "favorite" specialists.

UCLA's Jeste said attention on the drug has created a rift between doctors and patients. But she argues that prescribing the drug simply because parents are clamoring for it would set a slippery precedent.

"If we say, 'Yes,' to leucovorin because this is what's received the most attention from our government and from media, then we may as well say, 'Yes,' to other treatments that have been poorly studied," Jeste said.

Nonetheless, some doctors at large health systems are prescribing the drug selectively. Most declined to speak with NPR, fearing backlash from colleagues who may view them as prioritizing patient satisfaction over evidence. Dr. Antonio Hardan, a child psychiatrist at Stanford Medicine, and Mohiuddin said some of their colleagues have prescribed the medication in unique clinical scenarios.

Another concern raised by experts is the unintended consequences of prescribing the medication, which is commonly used during cancer treatment. Beyond safety risks, oncologists like Dr. Amer Karam, who practices at Stanford Medicine, worry that the growing demand for its use in autism could result in shortages for cancer patients who rely on it.

On Jan. 12, the FDA announced it was moving to address a "shortfall" of the drug's oral form by allowing imports of a version sold in Canada. The American Society of Health-System Pharmacists reported the medication has been in shortage since November.

Joyce, the guardian of the 5-year-old boy, has struggled to find a steady supply of the drug since December, bouncing between two pharmacies to fill the prescription.

Researchers warn that shortages are a "recurring" problem and rising demand could drive up prices.

Some providers say yes to leucovorin

While many doctors at major health centers won't prescribe it, some parents have found physicians — often in private practice — who will.

Many of these providers advertise their services online. That's how Balantrapu found one of them: Dr. Richard Frye.

Dr. Richard Frye

Richard Frye

Frye is a vocal proponent of leucovorin, and his research on the drug was cited by federal health officials in September. His interest in leucovorin is linked to the unproven theory that autism stems from mitochondrial dysfunction, which is intensified by vaccines. He has publicly expressed skepticism about vaccines.

Frye practiced in large academic centers until he left when they questioned his research.

He now works in private practice at Rossignol Medical Center and estimates about 80% of his autism patients get a prescription for leucovorin. He said he believes in the treatment and is puzzled by mainstream medicine's skepticism of it.

"Refusing to acknowledge [the benefits of leucovorin] and refusing to at least try it is really very confusing to me," Frye said. "I'm all about trying to improve the lives of children in a safe manner with safe treatments."

Frye founded a pharmaceutical company last year that he said will produce a new formulation of leucovorin. He filed with the Securities and Exchange Commission on Jan. 22, disclosing he had raised $100,000 from one investor. In a statement, Frye called the FDA's narrow expansion of the drug's use last week "meaningful" and of "extreme interest," writing that he is hopeful leucovorin approval will eventually be expanded beyond that.

Tiffany Banks, a naturopathic doctor and nurse practitioner with a hybrid practice in Kentucky, is another provider who openly prescribes the drug. She estimated that about 95% of her patients who seek a leucovorin prescription have received one since she began offering it in June. Calls to Banks' clinic have more than doubled in volume, and she now works longer hours and weekends.

"I just want to help everybody," Banks said. "When a mom sends you an email stating, 'My nonverbal child told me, "I love you," for the first time,' that's pretty special."

Tiffany Banks, right, with her daughter JaynaLee Banks, 18, who has autism. Tiffany described feeling dismissed and unsupported by the medical system early on when JaynaLee was younger.

Banks family

The cost of getting a prescription

If they can find a doctor to prescribe it, parents must next grapple with the fees. While the drug itself is not considered expensive — and is covered by some insurance plans, commonly with a diagnosis of "suspected" cerebral folate deficiency — other costs can add up, according to Robin Feldman, a professor at the University of California College of the Law, San Francisco, who has written about prescription drug prices.

When Balantrapu contacted Frye's clinic, she was shocked by the cost.

According to an email from Katie Pace, an administrative assistant at Rossignol Medical Center, a visit with Frye or his research collaborator, Dr. Dan Rossignol, would cost Balantrapu's family $1,896 plus a nonrefundable $299 deposit, with follow-ups priced at $948. NPR received a copy of the email from Balantrapu.

"It takes time to discover and treat all the underlying medical issues in our kids," Pace wrote, noting the practice doesn't accept insurance.

Frye, who is fully booked until 2028, declined to discuss his fees. He said his income isn't "much more" than what he earned before going into private practice, adding that he's not benefiting from his prescribing practices.

NPR spoke with three other providers who prescribe leucovorin and work in private practice. Their fees range from $150 to $1,500 for an initial visit.

Balantrapu ultimately decided not to go through with an appointment, saying the price and inconvenient office location didn't justify going to great lengths for a treatment with uncertain benefits. She added that she accepts her son's neurodiversity and views it as integral to his identity.

Still, she said, she's sure other parents will try the drug. "Parents are willing to just give any amount of money — even their life — for their children," she said. "We are giving him every resource possible under the planet and waiting for more science to happen."

Consequences of prescribing, or refusing to

NPR spoke with nine doctors for this article. Each varied in their final assessment of leucovorin. Some were more open to trying it briefly, while others refused to prescribe it.

"It's a complicated risk-benefit analysis," Mohiuddin explained. "It requires an individual doctor who understands the state of the science, who's able to articulate that to a particular family and make a specific decision in the context of a particular child."

Doctors who don't prescribe it lose some patients who seek the medication out elsewhere. Others have decided to prescribe it — sometimes to preserve the patient-doctor relationship and prioritize patient satisfaction.

"It's a tricky issue about trust because you want to win patients' trust. You cannot achieve a lot if you don't have their trust," said Gil Eyal, a sociologist at Columbia University who studies how experts gain and lose authority. "But does that mean that you have paid in some kind of currency you didn't want to pay, and you're creating some damage? It's not as simple as, 'Oh yeah, just prescribe it.'"

Clinicians like Frye, though, argue the drug is "extremely safe" and there is "little downside to trying it and incredible upside."

Jeste contends that some patients will get a prescription regardless of her stance, and clinicians need to decide where they set limits.

"If you take the next drug that receives a lot of attention, maybe it's just a tiny bit less safe. Do you decide to prescribe that one, too? And then what if the next one is just slightly less safe than that?"

"At what point do we draw the line?" she asked. "This is not the first time nor the last time that there will be non-evidence-based alternative treatments for autism that are tried by families."

Mohiuddin's verdict is that, in very selective cases, it might be reasonable to prescribe the drug. But nuanced conversations are needed, and future funding should be directed toward evidence-based interventions — like ABA therapy.

"They need more funding and support for day-to-day services and supports that already exist," Mohiuddin said. "All these other services and supports are really not prioritized or talked about."

The balance between maintaining parents' trust and sticking to evidence is delicate — and when it tips, it can lead to unintended consequences.

"Doctors today are faced with the fact that their authority is challenged and often for good reason," Eyal said. "The power of prescriptions is one of the main powers of doctors, and they jealously protect it."

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