A Move to Cut Drug Prices Has Patients With Rare Diseases Worried

The antiretroviral pill Genvoya is among the drugs set to undergo an affordability review in Colorado.

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For people with cystic fibrosis, like Sabrina Walker, Trikafta has been a life-changer.

Before she started taking the drug, she would wind up in the hospital for weeks at a time until antibiotics could eliminate the infections in her lungs. Every day, she would wear a vest that shook her body to loosen the mucus buildup.

One particularly bad flare-up, known as a pulmonary exacerbation, had her coughing up blood in 2019, so she was put on the newly approved breakthrough medication.

Within a month, her lung function increased by 20%, she said, and her health improved. Before she started taking Trakafta, she could count on three to four hospitalizations a year. Over the four years on the medication, she has been hospitalized only once.

“I was spending hours a day doing airway clearance and breathing treatments, and that has been significantly reduced,” said the 37-year-old Erie, Colorado, mother. “I’ve gained hours back in my day.”

Now she runs and hikes in the thin Colorado air and works a full-time job. Other patients have seen similar gains with the drug therapy, allowing many to resume regular lives and even take themselves off waiting lists for a lung transplant. Yet Walker and scores of other Colorado patients with cystic fibrosis are worried they could lose access to that transformative medication.

A state board charged with addressing the affordability of the most expensive prescription drugs has chosen Trikafta among its first five drugs to review, and it could move to cut the medication’s average in-state annual price of approximately $200,000, accounting for both insurers’ contributions and patients’ out-of-pocket costs. Drugmakers, including Trikafta’s maker, Vertex Pharmaceuticals, have said payment limits could hurt innovation and limit access, stoking panic among patients that the drug might no longer be sold in Colorado.

Two of the drugs chosen by the state board, the rheumatoid arthritis treatment Enbrel and the

psoriasis medication Stelara, also appear on the initial list of 10 drugs for which Medicare will negotiate prices. Any federally negotiated price reductions won't go into effect until 2026, and it's unclear how that effort will affect the Colorado board's work in the interim.

The Colorado board's choice of drugs to review elucidates one of the thorniest questions the board must wrangle with: Would lowering the price tag for rare-disease medications lead manufacturers to pull out of the state or limit their availability? State officials contend that the high cost of prescription drugs puts them out of reach for some patients, while patients worry that they'll lose access to a life-changing therapy and that fewer dollars will be available to develop breakthrough medications. And with affordability boards in other states poised to undergo similar exercises, what happens in Colorado could have implications nationwide.

"It just puts Trikafta as a whole at risk," Walker said. "It would start here, but it could create a ripple effect."

Cystic fibrosis is a genetic condition that causes the body to produce thick, sticky mucus that clogs the lungs and digestive system, leading to lung damage, infections, and malnutrition. It is a progressive disease that results in irreversible lung damage and a median age of death of 34 years. There is no cure.

The rare disease affects fewer than 40,000 people in the U.S., including about 700 in Colorado. That means research and development costs are spread across a smaller number of patients than for more common conditions, such as the millions of people with heart disease or cancer.

Officials from Vertex Pharmaceuticals declined a request for an interview. But company spokesperson Sarah D'Souza emailed a statement saying that "the price of this medicine reflects its value to patients, the small number of people living with CF, the billions of dollars Vertex has invested to date to develop the first medicines to treat the underlying cause of CF, and the billions more we are investing in CF and other serious diseases."

Setting an upper payment limit, the company said, could hinder access to drugs like Trikafta and curtail investment in scientific innovation and drug discovery.

State officials counter that Vertex and other drugmakers are resorting to fear-mongering to protect their profits.

Colorado Insurance Commissioner Michael Conway said that whenever the state talks about saving people money on health care, the affected entity — be it a hospital, insurance company, or drug manufacturer — cries foul and claims there will be an access problem.

"This is just, from my vantage point, the pharmaceutical industry trying to scare people," he said.

Colorado's Prescription Drug Affordability Board has been working for more than a year to sort through 604 drugs eligible for review, with 17 data points for each, to create a prioritized list. In the end, they decided to focus this year only on drugs that had no brand-name competition or

generic alternatives that could lower costs.

Besides Trikafta, Enbrel, and Stelara, the board will review the affordability of the antiretroviral medication Genvoya, used to treat HIV, and another psoriasis treatment, Cosentyx.

Of those five, Trikafta had the highest average annual costs but the lowest five-year increase in price and the fewest patients taking it.

The board's review of the five drugs will happen over its next three to four meetings this year and early next year, allowing all stakeholders — including patients, pharmacies, suppliers, and manufacturers — to provide feedback on whether the drugs are indeed unaffordable and what a reasonable price should be. Any cost limits wouldn't take effect until next year at the earliest.

The board looked at what patients were paying out-of-pocket for their medicines, using a database that captures all the insurance claims in the state. But that data did not account for patient assistance programs, through which manufacturers reimburse patients for out-of-pocket costs. Such programs boost manufacturer sales of drugs because insurance covers most of the cost, and patients otherwise might not be able to afford them.

Through the first half of the year, Vertex reported profits of $1.6 billion, with 89% of its revenue coming from Trikafta (marketed as Kaftrio in Europe). At the beginning of the year, Vertex decreased copay assistance for people with cystic fibrosis, in what the company said was a response to insurers' limiting patients' ability to apply copay assistance to their deductibles.

Lila Cummings, director of the Colorado board, said its staff could not find any entity that collects data on patient assistance programs, so those figures were not available to the board. Once they begin reviewing the individual medications, board members will dig into what extra financial help patients are getting. Cummings also said the board is hoping manufacturers will convey in good faith what might prompt them to leave the Colorado market.

When Trikafta came up second on the Colorado board's prioritized list of drugs eligible for review, patients and advocacy groups flooded the board with pleas to leave pricing for the medication and other drugs for rare diseases untouched.

"People are scared," Walker said. "If you look at all the drugs out there, it's one that has been so transformational that I think it will go down in history for how positively it's impacted our population as a whole."

According to the Cystic Fibrosis Foundation, lung exacerbations dropped 65% and lung transplants dropped 80% after the drug's approval. More patients have been able to work, attend school, or start a family. Clinicians have reported a baby boom among patients who take Trikafta.

A study published this year showed that two-thirds of people with cystic fibrosis struggled with finances, experiencing debt, food insecurity, or trouble paying for household or health expenses. The survey was conducted in 2019, before the FDA approval of Trikafta.

Years ago, the Cystic Fibrosis Foundation invested in Aurora Biosciences, later acquired by Vertex Pharmaceuticals, to promote development of cystic fibrosis therapies. The foundation completed the sale of its royalty rights in 2020.

Mary Dwight, chief policy and advocacy officer for the Cystic Fibrosis Foundation, said the board should “ensure its review of Trikafta accounts for the overall value this drug has for someone with CF, including the impact on an individual’s long-term health and well-being.”

There is no guarantee that the Colorado board will take action on Trikafta. State officials have stressed that board members are solely focused on improving access and wouldn’t jeopardize the availability of the medication.

“We have a history of being able to save people money on health care that doesn’t lead to access problems,” Conway said. “We’re not talking about these companies losing money at all; we’re talking about making it more affordable so that more Coloradans can get access to the pharmaceutical needs that they have.”

But Walker remains unconvinced.

“They had so much testimony on their call and they still selected Trikafta,” she said. “Everyone was just saying how important this drug is, and it didn’t matter. It still got pushed through.”

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