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Costly Vertex Drug Is Denied, and Medicaid Patients Sue

Kalydeco, a \$300,000-a-Year Cystic Fibrosis Treatment, Sparks Legal Battle in Arkansas and Shows Dilemma States Face

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By **JOSEPH WALKER** **CONNECT**

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Chloe Jones, a 14-year-old plaintiff in the Arkansas suit, wears an inflatable vest three times a day to help clear mucus from her lungs. *Karen E. Segrave for The Wall Street Journal*

LITTLE ROCK, Ark.— [Vertex Pharmaceuticals Inc.](#) **VRTX -1.95%**'s \$300,000-a-year cystic-fibrosis drug has sparked a legal battle here, where the state's Medicaid program is restricting access to the expensive therapy.

In a lawsuit filed in Arkansas federal court last month, three people suffering from the fatal lung disease allege Medicaid officials have for two years denied them access to Kalydeco because of its cost. The plaintiffs allege state officials have violated their civil rights under federal law governing Medicaid, the government-run insurance plan for the poor.

The patients all meet the eligibility criteria established by the Food and Drug Administration when it approved Kalydeco in 2012, including the presence of a rare genetic mutation it is designed to correct. But Arkansas officials have said the patients must prove their disease has failed to benefit from older, less-expensive therapies, a policy their doctors say contradicts treatment guidelines.

Arkansas officials declined to comment on specific allegations but said they are mainly restricting access because existing data don't support the drug's use as a first option. Cost also appears to be a factor: Emails obtained by the patients' attorneys show officials discussing Kalydeco's cost, and their worries about the expense of future cystic fibrosis drugs.

The legal flap is the latest example of the pressure expensive new drugs are putting on cash-strapped government insurance programs. State prison systems and some

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Medicaid programs in recent months have limited use of another expensive new drug— [Gilead Sciences Inc.](#) ([GILD +0.86%](#))'s hepatitis C pill, Sovaldi, which has a wholesale price of \$84,000 for a full course of treatment—to all but the sickest patients. The American Society of Clinical Oncology, meanwhile, recently said it would begin publishing a cost-benefit guide to cancer drugs.



The Costs of Rare Diseases
The most expensive treatments per prescription with at least \$20 million or more in total Medicaid spending in 2013

Drug Company	Disease	Average cost per prescription	Total paid for Medicaid beneficiaries
Vertex Pharmaceuticals	Kalydeco	\$311,000	\$1.1M
Amgen Inc.	Enbrel	\$1,300	\$1.1M
Novartis AG	Humira	\$1,300	\$1.1M
Amgen Inc.	Enbrel	\$1,300	\$1.1M
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Novartis AG	Humira	\$1,300	\$1.1M

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And more of these types of expensive, niche drugs are on the way. A recent report by insurer [UnitedHealth Group Inc.](#) ([UNH +0.70%](#)) estimated specialty-drug spending in the U.S. could more than quadruple to about \$400 billion in 2020, up from \$87 billion in 2012.

That creates a dilemma for state Medicaid agencies with limited budgets. New high-cost treatments like Kalydeco and Gilead's Sovaldi are likely just the "tip of the iceberg" for high-price therapies, said Matt Salo, executive director of the National Association of Medicaid Directors, a professional association.

"We have this public health mentality that all people have to be cured no matter what the cost, and also let the innovators charge whatever they want," said Mr. Salo. "Those are fine theories independently, but when you combine them together in a finite budget environment, it's not sustainable."

A state spokeswoman said of Arkansas's Kalydeco policy: "Cost alone was not the determining factor, but how we will pay for it is something we must consider in advance as we are a state agency with limited funds."

Doctors, patient advocates and Vertex said Arkansas is the only state they know of that has denied Kalydeco to patients who meet the FDA criteria, and that the state's policy appears to be unique.

Medicaid benefits cover doctor visits, prescription drugs and other medical services for more than 70 million low-income people. Unlike Medicare, the health program for the elderly that is fully funded by the federal government, Medicaid programs are administered by the states and, on average, received an estimated 57% of their Medicaid budgets from federal funds, according to the National Association of State Budget Officers.

Providing the three people with Kalydeco, which has an annual wholesale cost of \$311,000, would have a small impact on Arkansas Medicaid's prescription drug spending, which totaled \$351.28 million in 2013 and represented 7.5% of the state's total Medicaid spending. But the prospect of paying for similarly priced drugs for cystic fibrosis and other diseases in the future appears to have been a factor in the state's policy.

Chloe Jones, a 14-year-old plaintiff in the Arkansas suit, spends several hours each day undergoing treatment and taking medications. One step requires her to spend 45 minutes, three times each day in an inflatable vest that puts pressure on the chest and loosens mucus in the lungs.

William Golden, medical director of Arkansas Medicaid, said that since Chloe's lung function was normal at the time her doctors prescribed Kalydeco, the state couldn't justify approving Kalydeco.

The state Medicaid agency denied her doctor's request for Kalydeco in June 2012, stating Chloe hadn't met the requirement of taking older medications for 12 months, according to a letter Medicaid sent her doctor. The state denied Chloe subsequent appeals in July and September of 2012, and in August 2013, according to attorneys at Sufian & Passamano LLP, a Houston law firm representing Chloe.

"They just don't want to pay for it," Chloe said during an interview in Little Rock, where she travels for treatment from her home in Walnut Ridge, Ark. "I feel like they don't care about what's wrong with me, that I'm not as important as everybody else."

Chloe's physician, Dennis Schellhase, says Chloe has now been taking all of the medications required by the state for close to a year and the hospital will soon submit

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another application for the drug with the state Medicaid program.

(Arkansas has approved the drug for two patients, Dr. Golden said, but one left Medicaid and the other hasn't filled the prescription.)

Roughly 27% of Arkansas's population lives in poverty or near poverty, the second highest rate in the U.S. after Mississippi, according to the most recent U.S. Census data. Federal funds accounted for more than two-thirds (70%) of Arkansas's \$4.79 billion Medicaid budget in 2013, according to a state report.

In exchange for having their drugs covered by Medicaid programs, drug makers are required to provide rebates that are split between the states and Washington. Last year, Arkansas received rebates totaling \$142.97 million, or about 41% of its total drug spending, of which it kept 22%, with the rest accruing to the federal government.

Some expensive new drugs, including Kalydeco, are more effective than older therapies because they target specific genes that help cause a disease, doctors say. Companies say the high price tags reflect the costs and effort of developing the drugs, as well as their benefit to patients. Rare disease drugs are priced higher to compensate for the lack of a large patient population, industry officials say.

Kalydeco is approved in the U.S. for patients aged six years or older with one of several genetic mutations carried by about 1,100 people in North America. Globally, about 2,150 people are eligible for the drug, or about 3% of the 70,000 patients world-wide who have cystic fibrosis. Patients take the pill twice a day for life.

The mutations cause the disease by inhibiting the work of a protein that is responsible for transporting water and salt through the lungs and other organs. The disease causes thick mucus to build up in the lungs, which can eventually lead to respiratory failure. There are more than 1,800 genetic mutations that can cause cystic fibrosis.

Kalydeco is designed to restore function of the protein, which many doctors expect will halt or substantially diminish lung damage and extend patients' lifespan, though long-term studies haven't yet been completed to prove that, said Robert Giusti, a cystic-fibrosis specialist at NYU Langone Medical Center in New York.

"It would be the standard of care to offer Kalydeco to all patients who are within that mutation panel," Dr. Giusti said. Older drugs alleviate symptoms of the disease, mainly by clearing mucus from the lungs, but don't treat its underlying cause.

Chloe and the other Arkansas plaintiffs, Elizabeth West and Catherine Kiger, both 21 years old, also sought financial assistance from Vertex, which says it provides Kalydeco free to patients without insurance or whose insurance doesn't cover the drug. The company informed her hospital nurses that Chloe would have to exhaust the Medicaid coverage appeals process before Vertex would provide the drug, according to her lawyers and her doctor. But the company later said the assistance program specifically excluded Medicaid patients, her doctor said.

"It's been like a yo-yo, and it's devastating every time," said Chloe's mother, Amie Ledman.

(Kalydeco would be covered by the Medicare drug program if patients lived into their 60s, but most cystic fibrosis patients die by the age of 40.)

Vertex Chief Commercial Officer Stuart Arbuckle said the company has never provided free Kalydeco to Medicaid patients. If Vertex gives Kalydeco free to the Arkansas Medicaid patients, it could lead other states to establish similar eligibility criteria, Mr. Arbuckle said. The patient-assistance program, he said, "isn't there to subsidize Medicaid, which is there to provide medical care to poor families."

State Medicaid programs are required to pay for most FDA-approved drugs, regardless of their price, unless there are equivalent therapies available. For drugs like Kalydeco that have no equivalent, states can require physicians to prove the drugs are being used in a medically accepted way.





Amie Ledman, left, prepares medication for her daughter, Chloe Jones, 14, in a hotel room in downtown Little Rock, Ark. *Karen E. Segrave for The Wall Street Journal*

One of the main studies Vertex conducted to gain marketing approval for Kalydeco tested the drug against placebo. Patients in one arm of the study took Kalydeco in addition to standard cystic fibrosis therapies; the other group took placebo in addition to standard therapies. Patients taking Kalydeco had an average lung function improvement of 10.1% after about 11 months, compared with a decline of 0.4% in patients taking placebo, according to data published in the *New England Journal of Medicine* in 2011.

Dr. Golden said the study doesn't prove Kalydeco is more effective because patients weren't taking hypertonic saline, a salt water mist that is one of several treatments patients take to help clear mucus from their lungs. The treatment is recommended by the Cystic Fibrosis Foundation and is estimated to have a moderate net benefit. The Foundation, a nonprofit advocacy organization also responsible for issuing treatment guidelines and accrediting medical facilities, also funds pharmaceutical research—including to Vertex—and receives royalty payments from Vertex on sales of Kalydeco.

In a series of 2012 emails obtained by Chloe's attorneys and reviewed by *The Wall Street Journal*, Arkansas Medicaid officials discussed Kalydeco's cost. In an email to colleagues discussing a review board's deliberations about Kalydeco, a pharmacist named Pamela Ford wrote, "the consensus of the physicians on the board was that none of the prescribers would have a clue that this will cost AR Medicaid \$303,408 per patient per year."

She also noted in a separate email that Vertex was working on a new product that would combine Kalydeco with another drug, which could treat the majority of cystic fibrosis patients. The new therapy would be "likely even more expensive" and a "budget-breaker!" Ms. Ford wrote. "So we will be very strict in these reviews knowing it will come back even worse the next go-round."

Vertex is developing other therapies for cystic fibrosis that it hopes will eventually treat all genetic mutations.

An Arkansas state spokeswoman said Ms. Ford was unavailable to comment due to department policy.

Cystic fibrosis experts say Arkansas's criteria conflict with standard treatment approaches for patients with the genetic mutation. In 2012, Chloe's lung function was relatively stable at about 90% of what would be expected in a healthy person, but cystic fibrosis is thought to damage the lungs even when patients don't have symptoms, said Dr. Schellhase, Chloe's physician. Since 2013, Chloe has been hospitalized four times for roughly two weeks each stay after her condition worsened, and her lung function sank as low as 71%, Dr. Schellhase said. Arkansas Children's Hospital declined to specify the costs of Chloe's hospitalizations but said the average annual cost of hospital stays is about \$109,000 for a child with cystic fibrosis.

If Chloe had been given Kalydeco in 2012, "we probably would've avoided most of the hospitalizations, if not all of them," said Dr. Schellhase, who doesn't receive any

money from Vertex.

Brian O'Sullivan, a cystic-fibrosis specialist at University of Massachusetts Medical School, said the consensus among doctors is that all patients with the genetic defect should receive Kalydeco. Dr. O'Sullivan and other doctors criticized Vertex's pricing of the drug in 2012, saying the company was "leveraging pain and suffering into huge financial gain." But he called Arkansas's policy "unconscionable."

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