

'Breakthrough' leukemia drug also portends 'quantum leap' in cost

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Credit: National Cancer Institute

When doctors talk about a new leukemia drug from Novartis, they ooze enthusiasm, using words like "breakthrough," "revolutionary" and "a watershed moment."

But when they think about how much the [therapy](#) is likely to cost, their tone turns alarmist.

"It's going to cost a fortune," said Dr. Ivan Borrello at Johns Hopkins Sidney Kimmel Comprehensive Cancer Center in Baltimore.

"From what we're hearing, this will be a quantum leap more expensive than other [cancer drugs](#)," said Leonard Saltz, chief of gastrointestinal oncology at Memorial Sloan Kettering Cancer Center in New York.

Switzerland-based Novartis hasn't announced a price for the medicine, but British health authorities have said a price of \$649,000 for a one-time treatment would be justified given the significant benefits.

The [cancer](#) therapy was unanimously approved by a Food and Drug Administration advisory committee in July, and its approval seems all but certain.

The treatment, CTL019, belongs to a new class of medications called CAR T-cell therapies, which involve harvesting patients' immune cells and genetically altering them to kill cancer. It's been tested in patients whose leukemia has relapsed in spite of the best chemotherapy or a bone-marrow transplant.

The prognosis for these patients is normally bleak. But in a clinical trial, 83 percent of those treated with CAR T-cell therapy - described as a "living drug" because it derives from a patient's own cells - have gone into remission.

CAR T cells have been successful only in a limited number of cancers, however, and are being suggested for use as a last resort when all else has failed. As a result, only a few hundred patients a year would be eligible for them, at least initially, said Dr. J. Leonard Lichtenfeld, deputy chief medical officer for the American Cancer Society.

The FDA is scheduled to decide on approval by Oct. 3. The agency is

also considering a CAR T-cell therapy from Kite Pharma.

A third company, Juno Therapeutics, halted the development of one of its CAR T-cell therapies after five patients died from complications of the treatment.

Rather than wait for Novartis to announce a price, an advocacy group called Patients for Affordable Drugs has launched a pre-emptive strike, asking to meet with company officials to discuss a "fair" price for the therapy. The Novartis drug has the potential to be one of the most expensive drugs ever sold, said David Mitchell, the patients group's president, who has been treated for multiple myeloma, a blood cancer, since 2010. (The Laura and John Arnold Foundation, which provides some funding for Kaiser Health News, supports Patients for Affordable Drugs.)

"Many people with cancer look forward with great hope to the potential of your new drug," Mitchell wrote in a letter to Novartis. "But drugs don't work if patients can't afford them."

Cancer drugs today routinely cost more than \$100,000 a year. A combination therapy for melanoma sells for \$250,000. Such prices are particularly outrageous, given that taxpayers fund many drugs' early research, Mitchell said.

The federal government spent more than \$200 million over two decades to support the basic research into CAR T-cell therapy, long before Novartis bought the rights.

The patients group urged Novartis to charge no more for the drug in the U.S. than in other developed countries.

Novartis has agreed to meet with the patients group. In a statement,

Novartis said the company is "carefully considering the appropriate price for CTL019, taking into consideration the value that this treatment represents for patients, society and the healthcare system, both near-term and long-term."

Novartis made a significant investment in CAR T-cell therapy, according to the statement.

"We employ hundreds of people around the world who work on CAR-Ts, we are conducting ongoing U.S. and global clinical trials, and have developed a sophisticated, FDA-validated manufacturing site and process for this personalized therapy."

Soaring prices for cancer drugs have led many patients to cut back on treatment or skip pills, a recent Kaiser Health News analysis showed.

The effect of CAR T-cell therapies on overall health costs would initially be relatively small, because it would be used by relatively few people, Lichtenfeld said.

Health systems and insurers may struggle to pay for the treatment, however, if the FDA approves it for wider use, Lichtenfeld said. Researchers are studying CAR T-cells in a number of cancers. So far, the technology seems more effective in blood cancers, such as leukemias and lymphomas.

Hidden costs could further add to patients' financial burdens, Borrello said.

Beyond the cost of the procedure, patients would need to pay for traditional chemotherapy, which is given before CAR T cell therapy to improve its odds of success. They would also have to foot the bill for travel and lodging to one of the 30 to 35 hospitals in the country

equipped to provide the high-tech treatment, said Dr. Prakash Satwani, a pediatric hematologist at New York-Presbyterian/Columbia University Medical Center, which plans to offer the therapy.

Because patients can develop life-threatening side effects weeks after the procedure, doctors will ask patients to stay within two hours of the hospital for up to a month. In New York, even budget hotels cost more than \$200 a night - an expense not typically covered by insurance. Patients who develop a dangerous complication, in which the immune system overreacts and attacks vital organs, might need coverage for emergency room care, as well as lengthy stays in the intensive care unit, Satwani said.

Doctors don't yet know what the full range of long-term side effects will be. CAR T-cell therapies can damage healthy immune cells, including the cells that produce the antibodies that fight disease. Some patients will need long-term treatments with a product called intravenous immunoglobulin, which provides the antibodies that patients need to prevent infection, Lichtenfeld said.

Saltz, an oncologist who has long spoken out about high drug prices, said he applauded the patients group's efforts. But he said he doubts their efforts will persuade Novartis to set a reasonably affordable price.

"I'm not optimistic that this will have much effect on the company," Saltz said. "There's no market pressure for the company to respond to."

High [drug](#) prices don't just hurt [patients](#), Saltz said. They also drive up insurance premiums for everyone.

"They affect each and every one of us," he said, "because these costs will be paid by anyone who has any kind of insurance coverage."

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