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F.D.A. Approves First Gene-Altering Leukemia Treatment, Costing $475,000

By Denise Grady
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The Food and Drug Administration on Wednesday approved the first-ever treatment that genetically alters a patient's own cells to fight cancer, a milestone that is expected to transform treatment in the coming years.

The new therapy turns a patient's cells into a "living drug," and trains them to recognize and attack the disease. It is part of the rapidly growing field of immunotherapy that bolsters the immune system through drugs and other therapies and has, in some cases, led to long remissions and possibly even cures.

The therapy, marketed as Kymriah and made by Novartis, was approved for children and young adults for an aggressive type of leukemia — B-cell acute lymphoblastic leukemia — that has resisted standard treatment or relapsed. The F.D.A. called the disease "devastating and deadly" and said the new treatment fills an "unmet need."

Novartis and other companies have been racing to develop gene therapies for other types of cancers, and experts expect more approvals in the near future. Dr. Scott Gottlieb, the F.D.A. commissioner, said that more than 550 types of experimental gene therapy were being studied.

There are drawbacks to the approach. Because Kymriah can have life-threatening side effects, including dangerous drops in blood pressure, the F.D.A. is requiring that hospitals and doctors be specially trained and certified to administer it, and that they stock a certain drug needed to quell severe reactions.

Kymriah, which will be given to patients just once and must be made individually for each, will cost $475,000. Novartis said that if a patient does not respond within the first month after treatment, there will be no charge. The company also said it would provide financial help to families who were uninsured or underinsured.

Emily Whitehead, shown here in May, was near death at age 6 from leukemia and became the first pediatric patient to receive the experimental gene therapy. She is now 12 and has been in remission for more than five years. Children's Hospital of Philadelphia, via Associated Press

Discussing the high price during a telephone news conference, a Novartis official noted that bone-marrow transplants, which can cure some cases of leukemia, cost even more, from $540,000 to $800,000.

About 600 children and young adults a year in the United States would be candidates for the new treatment.

The approval was based largely on a trial in 63 severely ill children and young adults who had a remission rate of 83 percent within three months — a high rate, given that relapsed or treatment-resistant disease is often quickly fatal.

The first child to receive the therapy was Emily Whitehead, who was 6 and near death from leukemia in 2012 when she was treated, at the Children's Hospital of Philadelphia. Now 12, she has been free of leukemia for more than five years.

To customize Kymriah for individual patients, white blood cells called T cells will be removed from a patient's bloodstream at an approved medical center, frozen, shipped to Novartis in Morris Plains, N.J., for genetic engineering and multiplying, frozen again and shipped back to the medical center to be dripped into the patient. That processing is expected to take 22 days.

Novartis said the treatment would be available at an initial network of 20 approved medical centers to be certified within a month, a number that would be expanded to 32 by the end of the year. Five centers will be ready to start extracting T cells from patients within three to five days, the company said.

An intravenous bag of Kymriah, which must be customized for individual patients. It is expected to cost $475,000 and can have potentially fatal side effects. Novartis, via Associated Press

Certification is being required because the revved-up T cells can touch off an intense reaction, sometimes called a cytokine storm, that can cause high fever, low blood pressure, lung congestion, neurological problems and other life-threatening complications. Medical staff members need training to manage these reactions, and hospitals are being told that before giving Kymriah to patients, they must be sure that they have the drug needed to treat the problems, tocilizumab, also called Actemra.

Dr. Kevin J. Curran, a pediatric oncologist at Memorial Sloan Kettering Cancer Center in Manhattan, said his hospital was "99 percent" of the way through the certification process, and would soon be offering Kymriah.

"This is a big paradigm shift, using this living drug," Dr. Curran said. "It will provide a lot of hope. This is the beginning."

He said he expected that eventually this type of treatment would work for other, more common types of cancer, not just for leukemia.

The F.D.A.'s approval of Kymriah ushers in "a new approach to the treatment of cancer and other serious and life-threatening diseases," the agency said in a statement, noting that the new therapy is "the first gene therapy available in the United States."

Dr. Carl June, a leader in developing the treatment at the University of Pennsylvania, recalled that in 2010, when tests showed that the first patient was leukemia-free a month after being treated, he and his colleagues did not believe it. They ordered another biopsy to be sure.

"Now, I have to keep pinching myself to see that this happened," Dr. June said, his voice breaking with emotion. "It was so improbable that this would ever be a commercially approved therapy, and now it's the first gene therapy approved in the United States. It's so different from all the pharmaceutical models. I think the cancer world is forever changed."

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