A milestone in treating cancer

The United States's Food and Drug Administration (FDA) 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 recognise 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 FDA 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 FDA commissioner, said that more than 550 types of experimental gene therapy were being studied.

Drawbacks and cost

There are drawbacks to the approach. Because Kymriah can have life-threatening side effects, including dangerous drops in blood pressure, the FDA 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 patient, will cost $475,000 (approximately 2.8 crore). 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. 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 U.S. 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 high rhemission rate of 83% within three months. The treatment was originally developed by researchers at the University of Pennsylvania and licensed to Novartis. It was identified in previous reports as CAR-T cell therapy, CTL019 or tisagenlecleucel.

The first child to receive the therapy was Emily Whitehead, who was six 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.

Customising Kymriah

To customise Kymriah for individual patients, white blood cells called T cells will be removed from a patient's bloodstream at an approved medical centre, frozen, shipped to Novartis in Morris Plains, New Jersey, for genetic engineering and multiplying, frozen again and shipped back to the medical centre 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 centres

to be certified within a month, a number that would be expanded to 32 by the end of the year. Five centres will be ready to start extracting T cells from patients within three to five days, the company said.

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. NYT

Lifestyle-related risk factors are being cited, compounded by an inadequate number of treatment centres in the region

Without policies to stop the worrying spread of antimicrobial resistance, the mortality rate could be disturbing

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