FDA Approves New Prep Regimen for Bone Marrow Transplant

Jan. 23, 2025 – The FDA has approved a new treatment to help people get ready for a bone marrow transplant. It is for adults and children 1 year and older with certain blood cancers, like acute myeloid leukemia (AML) and myelodysplastic syndromes (MDS).

Sold under the name Grafapex (treosulfan), it is used with another drug called fludarabine as a pretreatment in patients undergoing a donor bone marrow transplant.

A donor bone marrow transplant, also called allogeneic hematopoietic stem cell transplantation (alloHSCT), replaces unhealthy stem cells with healthy stem cells and is crucial for treating AML and MDS. Before the transplant, patients receive chemotherapy, with or without radiation, to destroy cancer cells and suppress the immune system, improving the chances of success. This process, called conditioning, can impact outcomes as factors like graft rejection, infections, and treatment side effects affect survival.

Treosulfan with fludarabine forms a conditioning regimen that damages a cancer cell's DNA functions, causing the cell to die. While treosulfan injection has now received its first FDA approval, fludarabine was approved in 1991. Medexus, the maker of Grafapex, says it can help people live longer while reducing side effects and has announced in a press release that it will launch the medication in the first half of 2025.

The FDA approved the treatment after reviewing a study of 570 patients who had AML or MDS. The study showed that people who got the new drug combination lived longer than those who got the older treatment before their bone marrow transplant.

Overall, patients who got the new treatment were 33% less likely to die compared to those who got the older treatment.

The most common side effects of the medication are muscle and bone pain, mouth sores, fever, nausea, swelling, infections, and vomiting.

News Source:

https://www.webmd.com/cancer/lymphoma/news/20250123/fda-approves-regimen-bone-marrow-transplant-grafapex