Hematology-Related Research Advances

Approval of CAR-T therapy for hematologic malignancies:
The approval of chimeric antigen receptor T-cell (CAR-T) therapy by the Food and Drug Administration in August 2017 marked an important shift in the blood cancer treatment paradigm. CAR-T therapy is an innovative new treatment for certain patients with leukemia and lymphoma. We now have proof that it is possible to eradicate cancer by harnessing the power of a patient’s own immune system. This is a potentially curative therapy in patients who have typically exhausted all other treatment options, including chemotherapy, radiation, or stem cell transplant, and represents the latest milestone in the shift away from chemotherapy toward precision medicine. The FDA’s approval of this groundbreaking therapy was the result of over a decade of hematology research, including research funded by the NIH.

Targeted therapy for CML:
In the 1950s the only treatment for chronic myelogenous leukemia (CML) was radiation of the spleen, granting patients about 30 months of survival. The analysis of the CML-specific chromosomal translocation allowed the development of Gleevec (imatinib), a gene-targeting drug that is the paradigm of a new generation of “smart” drugs that allow disease-specific therapy. Using this non-toxic oral drug, more than 75% of patients diagnosed with CML achieve a durable complete cytogenetic remission.

Development of antithrombotic therapy:
Diseases such as heart attacks and strokes arise from clots in our blood vessels. These disorders are of some the most common causes of death in developed countries. However, developments in antithrombotic therapy have had a huge impact on our health. New antithrombotic treatments have lowered the risk of blood clots in leg veins by more than 70 percent. Deaths from heart attacks have been reduced by around 50 percent.

Development of diagnostic techniques to prevent stroke in sickle cell disease:
Despite progress in understanding the causes of sickle cell disease, the health of patients with this disease was largely ignored until the 1970s. During the 1980s and 1990s, several studies worked to improve the quality of life for these patients. In 1998, transcranial screening allowed doctors to identify sickle cell patients at risk for stroke and treat them with blood transfusions to prevent stroke.

请求:
认识到生物医学研究的价值，向 NIH 提供至少 490.048 亿美元用于财政年度 (FY) 2023。