年輕急性骨髓性白血病的治療進展

Treatment Advances in Young AML

葉士芃

中國醫藥大學附設醫院血液腫瘤科

This first phase of treatment is aimed at quickly getting rid of as many leukemia cells as possible. How intense the treatment is can depend on a person's age and health. For young and fit patients, "3+7" induction chemotherapy followed by intermediate or high dose Ara-C consolidation or allogeneic stem cell transplantation remains the gold standard of AML treatment. Nevertheless, many novel agents developed in the recent years have changed the landscape of AML treatment dramatically.

Adding **Gemtuzumab ozogamicin (GO)**, a monoclonal antibody to CD33, to "3+7" induction and intermediate dose Ara-C consolidation chemotherapy significantly reduces the relapse rate and improves the overall survival for patients with Core-binding factor (CBF)-AML. For patients of therapy-related AML (tAML) or AML with myelodysplasia-related change (MRC-AML), treatment with **CPX-351**, a liposomal formulation of Daunorubicin and Cytarabine, improves the overall survival comparing with the traditional "3+7"-based chemotherapies. For FLT3-ITD or FLT3-TKD mutated AML, adding **Midostaurin**, a FLT3 inhibitor, to "3+7" induction and intermediate dose Ara-C consolidation also reduces the relapse rate and improves the overall survival. In addition, after the completion of induction and consolidation chemotherapy, the use of **oral Azacitidine (CC-486)** maintenance therapy also demonstrates a better relapse free survival and overall survival.

For patients of refractory or relapsed AML, the efficacy of traditional salvage chemotherapy is usually limited and the prognosis remains poor. With the progress of molecular biology, identifying the targetable mutation of leukemic cells offers a new hope for these difficult patients. **Gilteritinib, Ivosidenib, and Enasidenib** are now approved for the treatment of RR-AML harboring FLT3ITD/TKD, IDH1, and IDH2 mutation respectively. For patients who entered 2nd complete remission, allogeneic stem cell transplantation should be considered as it represents the only change for long-term survival.

In this lecture, I will discuss these new agents approved for the treatment of AML within the last 2 years, and will outline the mechanistic features and clinical trials that led to their approvals.