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HAEMATOLOGICAL CANCER

Feasible mutation-targeted therapy

Approximately one-third of patients with acute myeloid leukaemia (AML) harbour mutations in *FLT3*, which encodes receptor-type tyrosine-protein kinase FLT3. Now, the therapeutic potential of the multikinase inhibitor midostaurin has been explored in this patient population in a study led by Richard Stone, with promising results published.

The researchers screened 3,277 patients with newly diagnosed AML for *FLT3* mutations; 717 patients with positive results were randomly assigned to receive induction therapy with daunorubicin and cytarabine supplemented with either midostaurin (n = 360) or placebo (n = 357). The median overall survival duration was longer for patients treated with midostaurin than for those who received placebo (74.7 months versus 25.6 months). The median event-free survival durations were 8.2 months and 3 months, respectively. Over the course of the disease, 57% of patients in the intention-to-treat population underwent transplantation. In these patients, although the data were not mature at the time of publication, overall survival durations were also longer with midostaurin (69.8 months–not reached versus 21.8 months–not reached).

On the basis of these results, the FDA has approved midostaurin for the treatment of patients with newly diagnosed, *FLT3*-mutated AML. Stone is confident this agent could become the standard-of-care therapy in this setting. In his words, "our study shows that mutation-targeted therapies are feasible and beneficial for patients with AML. We now want to explore agents targeting other actionable mutations in this disease, such as those affecting *IDH3*." This is not the only future direction that Stone and co-workers contemplate. He explains "we need to improve therapies for patients with other malignancies harbouring *FLT3* mutations. The potential benefit of including midostaurin as a backbone of such therapies should be studied."

Diana Romero

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