

The Clinical Value of Decitabine Monotherapy in Patients with Acute Myeloid Leukemia

V. Santini, M. Lübbert, A. Wierzbowska, G. J. Ossenkoppele

Decitabine (5-aza-2'-deoxy-cytidine) is a hypomethylating agent used to treat **acute myeloid leukemia (AML)**



Decitabine is indicated in the EU for adult patients with newly diagnosed **de novo** or **secondary AML** who are **not** eligible for standard induction chemotherapy

Clinical and real-world data suggest that decitabine is safe and effective in this at-risk population

• DACO-016 clinical trial •

This phase III registration trial examined the effects of decitabine in older patients



Treatment group receiving decitabine

485 patients

Aged 65 or older

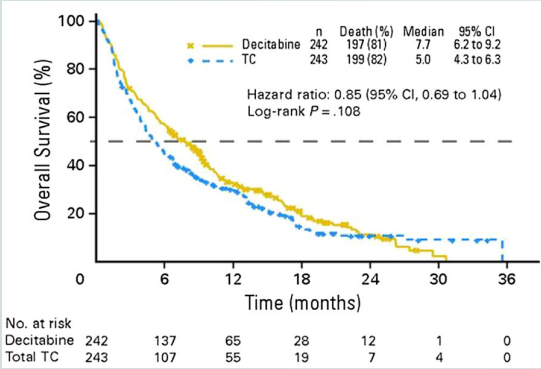
Newly diagnosed or secondary AML

Not eligible for standard chemotherapy

1-hour infusion daily for five consecutive days every 4 weeks



Control group receiving cytarabine or supportive care alone



Results of the trial were favorable for the group receiving decitabine

Event-free survival:

HR 0.75 (95% CI: 0.62-0.91)

Progression-free survival:

HR 0.75 (95% CI: 0.62-0.91)

Clinical response

based on complete remission:

OR 2.5 (95% CI: 1.40-4.78)

• Real-world studies •

United States

Decitabine patient cohort (n=1109):

median OS 8.2 months

Most patients did not receive recommended minimum 4 cycles

Belgium

≥ 4 cycles of decitabine (n = 21):

median OS 17.5 months

< 4 cycles of decitabine (n = 24):
median OS 1.6 months

Italy

Overall patient cohort (n = 306):

median OS 10.0 months

**Achieved CR (n = 71):
median OS 22.1 months**

Safety

- **Toxicity** associated with decitabine is relatively mild
- Adverse events are **manageable**
- **No additional adverse events** reported in real-world vs. clinical studies



Treatment with decitabine may partly overcome the adverse prognostic impact of a **TP53 mutation**, but data are inconclusive

While further studies are needed to identify AML patients likely to benefit from decitabine, clinical and real-world evidence clearly indicate survival improvements are possible among patients who face poor prognoses

AML, acute myeloid leukemia; CR, complete remission; HR, hazard ratio; OR, odds ratio