In a phase 3 study in AML patients aged ≥65 years, with previously untreated AML who were candidates for intensive chemotherapy (induction FAB M3); good risk cytogenetics defined as t(15;17), −1q, del(17p), −5, del(5q) or −7/del(7q), and normal karyotype (candidates for intensive chemotherapy, induction chemotherapy, bone marrow, or stem cell transplant) as more effective and better-tolerated treatments, compared to standard care.

**METHODS**

**Study Design**

The phase 2 study evaluated the combination of pracinostat and azacitidine in 50 AML patients aged 66–74 years.

**Patient Population**

- **Age**: 66–74 years
- **ECOG performance status**: 0–1

**Molecular Mutation Analyses**

- **Distribution of mutations**: The mutation profile appeared to be generally typical of an older population with AML. Frequent mutations that are related to secondary AML and clonal evolution were found. The mutation profile was similar to that of younger AML patients, but with certain unique features of elderly AML.

**RESULTS**

- **Efficacy Results by Patient Subsets**
  - Median OS: 13.5 months
  - Median MLFS: 47.6%

**CONCLUSIONS**

- **Mutations in NPM1 and the DNA methylation pathway were common in the study population and associated with a better response to pracinostat and azacitidine, while TP53 mutation was associated with a poor response.**

- **Mutations that respond to pracinostat and azacitidine may be an important factor in the variable OS and CS findings in persistent survival.**

**REFERENCES:**