

A Phase III Randomized Trial of Gemtuzumab Ozogamicin (Mylotarg®) Combined with Conventional Chemotherapy for De Novo Acute Myeloid Leukemia (AML) in Children, Adolescents, and Young Adults (AAML0531)

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**Synopsis:**

The overall goal of this study is to improve the cure rate of Acute Myeloid Leukemia (AML) without increasing the side effects of therapy. In this randomized study, a new chemotherapy drug, Gemtuzumab (GMTZ), will be added to the standard chemotherapy for AML. The objectives are:

**Primary Objective**

- 1- To compare the event free survival (EFS) and overall survival (OS) of AML patients randomized between the best current chemotherapy with or without gemtuzumab ozogamicin (GMTZ)

**Secondary Objectives:**

- 1- To compare the remission induction rates after two courses of standard chemotherapy with or without GMTZ
- 2- For those patients who are eligible for a HLA-matched family donor (MFD) stem cell transplant (SCT), to compare disease free survival and overall survival between patients assigned to SCT if a matched family donor is available, or to chemotherapy if a MFD is not available.
- 3- To determine the outcome of patients with Down syndrome who are 4 years of age or older at diagnosis and treated on this regimen without GMTZ
- 4- To compare the the EFS and OS of AML patients censoring MFD SCT recipients

**Laboratory correlates:**

- 5- To identify the optimal cutoff of FLT3 internal tandem duplications allelic ratios to predict patients at high risk for relapse.
- 6- To assess the ability of a second generation flow cytometric assay to predict patients at high risk for relapse
- 7- To examine whether GMTZ improves EFS and OS in patients with higher CD33 concentrations.
- 8- To examine whether GMTZ improves outcome in each of the cytogenetic risk groups
- 9- To utilize FISH (florescence in situ hybridization) analysis to identify variant patterns among subgroups of patients who demonstrate the same type of chromosomal abnormality.
- 10- To examine the impact of complex karyotypes upon OS and EFS in Intermediate Risk patients