



Childhood AML Research Summaries

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Title

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Acute Myelogenous Leukemia (AML): Three Research Approaches to Improve Treatments

Personalized Therapy for AML.

In the 1970's children with AML had very little hope of being cured. In 2009, about 60% of children with AML can be cured with chemotherapy. This remarkable improvement in treatment has been achieved through organized clinical trials. In clinical trials, all the children get the same treatment so we can start to figure out which drugs work best. This is excellent progress. However, while 60% of children with AML are cured, still in 2009, 25% of children with AML will have a relapse, meaning the leukemia comes back, so these children didn't get enough chemotherapy to be cured. On the other hand, about 15% of children get severe side effects from the chemotherapy that they die from, so those children got too much therapy for them. This happens even though the children all get the same dose of chemotherapy, so what determines whether the child is cured or not must be differences in the children themselves. . We believe that the way to move forward to our goal of curing all children is to start understanding what is different in the genes of the children that changes their response to the chemotherapy drugs. Once we understand this we can test the children at the start of treatment and adjust the drug doses so each child gets just the right amount of chemotherapy for them instead of using the same dose for every child.

Improving Bone Marrow Transplantation; Picking the Very Best Transplant Donor

Some children with very severe AML, for example, those who have had a relapse, those with no response to chemotherapy, or those who have a genetic abnormality called monosomy-7 in their leukemia cells need a bone marrow

transplant to be cured. We are studying a new blood test called KIR-typing to help us pick out the best unrelated donors for transplant. We believe that healthy new donor cells can help children fight the leukemia and allow the child to be cured. However some donor cells are better at doing this than others. This study is being conducted in partnership with Dr. Wing Leung at St. Jude Children's Hospital. This study is open to children throughout the USA, through the Children's Oncology Group and over 20 children have been enrolled and are being treated. We hope to study almost 200 children to find out if the test will work.

Looking Back into the Past, Children with Fanconi anemia and AML

Parents are very distressed when their child is diagnosed with leukemia and will often ask "when did this happen", and "How long has this been in his body?". In most children we are not able to answer these questions because we cannot look into the past. Fanconi anemia is a genetic disorder that makes children have a very high chance of getting AML. Children with Fanconi anemia have a 40% chance of getting AML and when they do the leukemia cells often have the genetic abnormality monosomy-7. We are currently collecting, studying and storing marrow over time from children with Fanconi anemia. When these children develop problems with their bone marrow or leukemia we will be able to take the samples from the bank, some of them stored years ago, and be able to "look back in time" and see when the very earliest signs developed and start to answer the question "How long does it take for cells to grow into a leukemia." This study will give us important information about the biology of monosomy-7 in children with Fanconi anemia, and children who do not have Fanconi anemia, to allow us to develop better therapy for this particularly difficult kind of leukemia.