

Examining outcomes in children with Down syndrome and acute lymphoblastic leukemia

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Children with Down syndrome have an increased risk of developing acute lymphoblastic leukemia (ALL) and experience higher rates of relapse and treatment toxicity. In recent decades, new therapies have

improved outcomes for the general population, but outcomes for children with Down syndrome have lagged and have not been reported for contemporary treatment regimens.

A new report published in the [*Journal of Clinical Oncology*](#), led by Baylor College of Medicine and Texas Children's Cancer Center, reviewed results of Children's Oncology Group [clinical trials](#) from 2003-2019 and found that children with Down [syndrome](#) and ALL still experience an increased rate of relapse and treatment-related mortality on contemporary treatment protocols, compared to children without Down syndrome.

Researchers conclude that use of novel, less toxic therapies will be necessary to improve outcomes in this patient group.

The study examined outcomes from 743 patients with Down syndrome and ALL, the largest cohort reported to date, compared to more than 20,000 ALL patients without Down syndrome. Five-year overall survival for patients with Down syndrome was approximately 7% lower than other patients.

Relapse and treatment-related mortality have always been the main causes of outcome disparity for children with Down syndrome. In this study, researchers found that the increased risk of relapse has narrowed with use of modern therapies, but the risk of treatment-related mortality remains high.

"Outcomes in childhood ALL have improved in large part because treatment has intensified, which prevents relapse," said Dr. Karen Rabin, corresponding author of the study, professor of pediatric hematology and oncology at Baylor and director of the Leukemia Program at Texas Children's Cancer Center. "However, children with Down syndrome aren't benefiting as much as other children because they experience a

higher risk of life-threatening infection."

Low white blood cell counts during treatment make patients more vulnerable to infection. Children with Down syndrome also experience a higher frequency of mouth sores, increased blood sugar levels and seizures related to the chemotherapy. Researchers say this indicates a need for evaluating different treatment strategies.

"Increased use of immunotherapeutic strategies, which are less toxic than chemotherapy, has been a revolutionary advance for all children with ALL, and [children](#) with Down syndrome especially stand to gain from this less toxic approach to treatment," Rabin said.

More information: Karen R. Rabin et al, Outcomes in Children, Adolescents, and Young Adults With Down Syndrome and ALL: A Report From the Children's Oncology Group, *Journal of Clinical Oncology* (2023). [DOI: 10.1200/JCO.23.00389](https://doi.org/10.1200/JCO.23.00389)

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