

Evaluating pediatric leukemia treatment

12 April 2018, by Anna Williams

A drug called dasatinib was found to be safe and effective for children with chronic myeloid leukemia, according to a clinical trial published in the *Journal of Clinical Oncology*.

Based on the findings, the drug has been approved by the FDA and is now available for use in some pediatric patients.

The study was co-authored by Nobuko Hijiya, MD, professor of Pediatrics in the Division of Hematology, Oncology, and Stem Cell Transplantation.

Chronic myeloid leukemia (CML) is a cancer of the [white blood cells](#), and though it is rare in children, the disease is also generally more aggressive in younger patients.

Previously, a drug called imatinib was the only [tyrosine kinase inhibitor](#) approved for first-line treatment of pediatric patients with CML in chronic phase (CP), a relatively early stage. But there were no approved therapies to treat children with chronic-phase CML who were resistant or intolerant to imatinib, the standard treatment.

Dasatinib, a tyrosine kinase inhibitor, was previously approved for adult patients with CML that is Philadelphia chromosome-positive, a genetic abnormality in the disease.

In the current trial, the investigators evaluated [dasatinib](#) in a group of 130 patients younger than 18 with Philadelphia chromosome-positive CML.

The phase II, non-randomized trial included participants at medical centers across the world, and is the largest clinical trial of pediatric patients with chronic-phase CML to date.

The investigators found that dasatinib was associated with a high response rate in such patients, with deep molecular responses. Further, the therapy was found to be safe, with few serious adverse events reported.

"Although we did not see significant acute toxicities—including pleural effusion or cardiovascular events that have been seen in adults—we will need to follow the children on dasatinib for a much longer period to see if there are other long-term side effects like growth delay, as children need much longer treatment compared to adults because of longer life expectancy," Hijiya explained.

In November, the FDA expanded its approval of dasatinib to include [pediatric patients](#) with Philadelphia chromosome-positive CML who are newly diagnosed or unresponsive to imatinib.

More information: Lia Gore et al. Dasatinib in Pediatric Patients With Chronic Myeloid Leukemia in Chronic Phase: Results From a Phase II Trial, *Journal of Clinical Oncology* (2018). [DOI: 10.1200/JCO.2017.75.9597](#)

Provided by Northwestern University

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