

# FDA Approves Tasigna for Some Children With Chronic Myeloid Leukemia

Approval was based on trials enrolling newly diagnosed patients and those with resistant disease.

March 25, 2018 By [Food and Drug Administration \(FDA\)](#)

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FDA approves nilotinib for pediatric patients with newly diagnosed or resistant/intolerant Ph+ CML in chronic phase

On March 22, 2018, the Food and Drug Administration approved nilotinib (TASIGNA, Novartis Pharmaceuticals Corporation) for pediatric patients 1 year of age or older with newly diagnosed Philadelphia chromosome positive chronic myeloid leukemia in chronic phase (Ph+ CML-CP) or Ph+ CML-CP resistant or intolerant to prior tyrosine-kinase inhibitor (TKI) therapy.

Approval was based on results in 69 pediatric patients with Ph+ CML-CP from two open-label, single-arm, multi-center trials: CAMN107A2120 (NCT01077544) in pediatric patients with Ph+ CML-CP resistant or intolerant to imatinib or dasatinib (n=11) and CAMN107A2203 (NCT01844765) in pediatric patients with Ph+ CML-CP resistant or intolerant to imatinib or dasatinib (n=33) and newly diagnosed Ph+ CML-CP (n=25). In both trials, patients received nilotinib 230 mg/m<sup>2</sup> twice daily, rounded to the nearest 50 mg dose (maximum single dose of 400 mg) in 28-day treatment cycles. The median time on treatment was 13.8 months (range: 0.7 to 30.9 months).

In patients with resistant or intolerant Ph+ CML-CP, the major molecular response rate (MMR; BCR-ABL/ABL  $\leq$ 0.1% IS) was 40.9% (18/44, 95% CI: 26.3%, 56.8%) at 12 cycles. In patients with newly diagnosed Ph+ CML-CP, the MMR rate was 60.0% (15/25, 95% CI: 38.7%, 78.9%) at 12 cycles. In patients with resistant or intolerant CML, the cumulative MMR rate was 47.7% (21/44) by cycle 12. In patients with newly diagnosed CML, the cumulative MMR rate was 64.0% (16/25) by cycle 12.

Among patients with resistant or intolerant CML, 4.5% of patients achieved BCR-ABL/ABL  $\leq$ 0.0032% IS (MR4.5) by the cut-off date. Among patients with newly diagnosed CML, the percentage who achieved MR4.5 was 28.0%.

The safety profile in pediatric patients is similar to the known safety profile in adults with Ph+ CML-CP. Common adverse reactions (greater than 20%) were hyperbilirubinemia, thrombocytopenia, rash, neutropenia, lymphopenia, alanine aminotransferase (ALT) increased,

headache, anemia, pyrexia, nausea, upper respiratory tract infection, aspartate aminotransferase increased, and vomiting. The most common grade 3/4 adverse reactions were ALT increased and hyperbilirubinemia. Increase in QTcF greater than 30 msec from baseline was observed in 17 patients (25%). No patient had an absolute QTcF of greater than 500 msec or QTcF increase of greater than 60 msec from baseline.

The recommended pediatric dose is 230 mg/m<sup>2</sup> orally twice daily, rounded to the nearest 50 mg dose (maximum single dose of 400 mg).

Full prescribing information is available

at: [https://www.accessdata.fda.gov/drugsatfda\\_docs/label/2018/022068s027lbl.pdf](https://www.accessdata.fda.gov/drugsatfda_docs/label/2018/022068s027lbl.pdf).

FDA granted this application priority review and orphan drug designation. A description of FDA expedited programs is in the Guidance for Industry: Expedited Programs for Serious Conditions-Drugs and Biologics, available

at: <http://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm358301.pdf>.

Healthcare professionals should report all serious adverse events suspected to be associated with the use of any medicine and device to FDA's MedWatch Reporting System by completing a form online at <http://www.fda.gov/medwatch/report.htm>, by faxing (1-800-FDA-0178) or mailing the postage-paid address form provided online, or by telephone (1-800-FDA-1088).

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This announcement was [originally posted](#) on the FDA's website on March 22, 2018.

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