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Title **The Effectiveness and Cost-effectiveness of Imatinib in Chronic Myeloid Leukemia: A Systematic Review**

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Aim

To systematically review the efficacy and cost effectiveness of imatinib in treating chronic myeloid leukemia (CML) in the chronic, accelerated, and blast phases, and compare it to existing drug regimes.

Conclusions and results

Only 3 unpublished Phase II studies of imatinib, one in each phase of CML, were available for inclusion. Due to limited data, information relating to existing treatments was examined to allow indirect comparison. Eleven RCTs (10 chronic phase CML and 1 accelerated/ blast phase) comparing hydroxyurea, busulphan, interferon-a, and other chemotherapy were included. Also, 40 case series studies of existing treatments (27 chronic phase and 13 accelerated/ blast phases) were included. No economic or quality of life studies were found. The imatinib studies had not been peer reviewed. There were important differences in patient characteristics, treatment, and doses between trials. The RCTs were of moderate quality. The case series studies were often small and quality varied widely. Indirect comparisons between case series (as was necessary in this review) are susceptible to confounding and should be interpreted with caution.

In the chronic phase, imatinib shows similar 1-year survival to other treatments, but higher complete HR and CR rates. No information on survival beyond 1 year with imatinib treatment was available. In the accelerated phase, survival with imatinib appears to be longer than with other drugs, but this relies on indirect comparisons of case series. In the blast phase, imatinib appears to show limited longer survival compared to other reports, and complete CR and HR rates for imatinib are within the range of other studies. Patients enrolled in these other studies are not well described, making conclusions difficult. Few studies are published, and study populations are small. Absence of control groups limits the reliability of analysis.

Recommendations

Based on limited evidence, imatinib appears to offer an alternative treatment for CML in the accelerated and blast phases. Information about imatinib in the chronic phase was insufficient to draw firm conclusions about survival. Cost-utility estimates for imatinib are particularly sensitive to assumptions about long-term survival, and may be extremely high.

Methods

Randomized controlled trials (RCTs), cohort studies, case series of first- and second-line drug treatments (minimum of 20 participants), economic analyses, and quality of life studies were included. Novartis provided pre-publication reports of 3 Phase II studies. The report represents a narrative summary - no formal statistical synthesis of results was undertaken.

Further research/reviews required

More research into imatinib for CML is needed. Key areas include: 1) efficacy of imatinib in chronic phase CML in the long term; 2) RCTs to establish the effectiveness of imatinib in all phases of CML compared to IFN-a, hydroxyurea and other chemotherapy; 3) further elucidation of the relationship between response rates (HR and CR) and long-term survival with different treatments in all phases of CML.

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