Real-world efficacy and safety outcomes of imatinib treatment in patients with chronic myeloid leukemia: an Australian experience

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## Abstract

Background: Tyrosine kinase inhibitors (TKI) have revolutionised the treatment of chronic myeloid leukaemia (CML), but patients still experience treatment-limiting toxicities or therapeutic failure. Aims: To investigate real-world use and outcomes of imatinib in patients with CML in Australia. Methods: A retrospective cohort study of patients with CML commencing imatinib (2001-2018) was conducted across two sites. Prescribing patterns, tolerability outcomes, survival and molecular response were evaluated. Results: 86 patients received 89 imatinib treatments. Dose modifications were frequently observed (12-month rate of 58%). At last follow-up, 62 patients (5-year rate of 55%) had permanently discontinued imatinib treatment, of which 44 switched to another TKI (5-year rate of 46%). Within 3 months of starting imatinib, 43% (95% CI, 32–53%) of patients experienced imatinib-related grade [?]3 adverse drug reactions (ADRs). Higher comorbidity score, lower body weight, higher imatinib starting dose, and Middle Eastern or North African ancestry were associated with a higher risk of grade [?] 3 ADR occurrence on multivariable analysis (MVA). Estimated overall survival and event-free survival rates at 3 years were 97% (95% CI, 92–100%) and 81% (95% CI, 72–92%), respectively. Cumulative incidence of major molecular response (MMR) at 3 years was 63% (95% CI, 50–73%). On MVA, imatinib starting dose, ELTS score, BCR-ABL1 transcript type, pre-existing pulmonary disease, and potential drug-drug interactions were predictive of MMR. Conclusion: Imatinib induced deep molecular responses that translated to good survival outcomes in a real-world setting, but was associated with a higher incidence of ADRs, dose modifications and treatment discontinuations than in clinical trials.

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