Clinical characteristics and treatment outcome of pediatric patients with chronic myeloid leukemia

In his Letter-to-the- Editor, Dr. Cheuk has raised concerns about the recommendations derived from our paper.[1] He compares the two relapsed patients in the SCT group with the one relapse in the IM group, stating that these data did not support our contention that a role for SCT as first-line therapy for CML needs to be maintained. Our small patient numbers and the very few episodes of disease failure do not allow us to detect any differences in the outcome for the two groups, and at most we can say that there is no significant relapse-free survival benefit for either treatment strategy. Similarly the recurrence of disease in Patient #12 after achieving a "complete" molecular response, on its own, is not the only evidence for achievement of cure following SCT; there is a long track record for this treatment modality that stretches back three decades documenting over 60% disease free survival for patients transplanted in first CHR.

Nonetheless, we agree with Dr. Cheuk that the decision to subject these patients to SCT needs to be taken with great care and deliberation. Certainly discussion with the patients and their families regarding the potential risks and benefits of both modalities is imperative. This should include financial considerations as well, since long-term therapy with IM may in fact be more costly than MSD SCT. As has been noted in the paper, at our institution we "offer MSD SCT to patients with CML in CP1" and "continue IM for the remaining until availability of a suitable donor or further data is generated that would allow changes in practice". Certainly some patients who are "offered" SCT would decide to proceed with IM therapy. This thought process reflects the controversy that persists within the pediatric oncology community at large,[2] and stems from the lack of a sound knowledge base regarding the long-term use of IM in children. In this situation, resorting to SCT, which has a mature track record, seems intuitive.

Certainly there is significant room for further pediatric age-specific research in the treatment of CML with tyrosine kinase inhibitors and direct extrapolation of adult data may not be sufficient. While Dr. Cheuk points out that one of the disadvantages of IM therapy may be the "unknown small risk of long-term adverse effects for children", we have no data to prove that this risk is in fact small. Metabolic effects of IM quoted in the paper could potentially result in significant growth and development adverse effects that will only be evident after decades of follow up for these pediatric patients. These and other questions need to be answered with well-designed, prospective studies.

Our recommendations in the paper were not markedly different from those suggested by Dr. Cheuk. They are, however, different from those prevalent amongst physicians treating adult patients with CML where SCT is no longer a consideration. We believe that all patients in CP1 should be treated with IM to begin with and SCT should be considered for those with a MSD. Those who do not have appropriate donors, or elect not to proceed to SCT, should continue with IM indefinitely.

Asim F. Belgaumi and Ali Al-Shehri

Department of Pediatrics Hematology/Oncology, King Faisal Special, Hospital and Reseach center, Riyadh, Saudi Arabia

Key words: CML, imatinib mesylde, pediatrics.

Correspondence: Asim F Belgaumi, King Faisal Special, Hospital and Reseach center, Riyadh 11211, Saudi Arabia;
E-mail: belgaumi@kfshrc.edu.sa

Citation: Belgaumi AF and Al-Shehri A. Clinical characteristics and treatment outcome of pediatric patients with chronic myeloid leukemia. Haematologica 2010;.95:e4
doi:10.3324/haematol.2010.028290

References

1. Belgaumi AF, Al-Sheri A, Ayas M, Al-Mahr M, Al-Seraihy AS, Al-Ahmari A, El-Solh H. Clinical characteristics and treatment outcome of pediatric patients with chronic myeloid leukemia. Haematologica 2010 Apr 21. [Epub ahead of print]
2. Burke MJ, Willert J, Desai S, Kadota R. The treatment of pediatric Philadelphia positive (Ph+) leukemias in the Imatinib era. Pediatr Blood Cancer. 2009;53(6):992-5.