Pazopanib for progressive desmoid tumours: children, persistant effects, and cost

Author's reply

We thank Yoshihiro Nishida and colleagues for their interest in the results of the DESMOPAZ study.[1] We agree that inclusion of paediatric patients in clinical trials investigating innovative therapies in mesenchymal tumours should be favoured. However, this inclusion can be particularly challenging in very rare indications, such as desmoid tumours. For instance, in a nationwide survey reporting the activity of the French sarcoma expert network between 2010 and 2013, only 15 (2%) of 861 desmoid tumours were diagnosed in paediatric patients.[2] In our experience, tyrosine kinase inhibitors, such as pazopanib or sorafenib, have meaningful activity in paediatric patients with desmoid tumours that need an active treatment. Regarding, the comparative efficacy or chemotherapy versus pazopanib in desmoid tumours, we highlight the fact that DESMOPAZ was a randomised, phase 2 trial that was not designed to show a superiority of one group versus the other one. Therefore, the fact that the progression-free survival curves cross at one point should not be over-interpreted. Finally, even if the cost of pazopanib is higher than those of methotrexate and vinblastine, the global medico-economic evaluation should also integrate the direct costs related to repeated chemotherapy infusion (eg, facilities and equipment; chemotherapy storage, preparation, and administration; physicians, nurses, secretaries, support staff; and facility maintenance), the indirect costs (eg, loss of earning potential), and non-medical direct costs (eg, transportation, parking, childcare, and meals while making trip for treatment, etc).

The results of the first prospective wait-and-see study in patients with desmoid tumours were recently reported.[3] Only 40 (37%) of 108 patients had a RECIST progression and only 32 (30%) of 108 needed to start an active therapy. Strikingly, 21 (31%) of 69 patients initially experiencing progression had a subsequent spontaneous regression showing that even in the case of substantial disease progression, active therapy is not always necessary and should be guided by the severity of the symptoms. The results of the DESMOPAZ study suggest that when a treatment is needed, pazopanib represent a valid therapeutic option as well as other cytotoxic agents, such as anthracyclines or methotrexate-vinblastine.[4]

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Antoine Italiano
a.italiano@bordeaux.unicancer.fr

Department of Medicine, Institut Bergonié, Early Phase Trials and Sarcoma Units, 33000 Bordeaux, France; University of Bordeaux, Bordeaux, France

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