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

We read with great interest the randomised, open-label, multicentre, phase 2 study by Maud Toulmonde and colleagues[1] reporting the activity of pazopanib for adult patients with desmoid tumours. The cohort of this study was limited to patients with progressive tumours according to the Response Evaluation Criteria in Solid Tumors, which is the major strength of this study. The enigmatic features of desmoid tumours, including occasional spontaneous regression, and its very rare incidence preclude the gathering of an identical cohort, particularly with progressive status. Similarly, another excellent randomised study[2] of sorafenib for desmoid tumours could not gather a cohort with progressive status either.

The results of this study provide useful information not only on pazopanib, but also on methotrexate and vinblastine combination therapy for desmoid tumours. However, several points require clarification to accurately interpret these results. First, the cohort did not include paediatric or adolescent patients, and the median age of the pazopanib cohort was 35 years (range 18–78). Physicians treating patients with desmoid tumours have occasional difficulty, particularly with aggressive disease in children. Conversely, for methotrexate and vinblastine combination use, not only the effect but also the safety for paediatric and adolescent patients has already been reported.[3] Second, in Toulmonde and colleagues' paper, figures 2A and 2B might indicate the difference in desmoid tumour behaviour after treatment discontinuation. After the timepoint of 12 months, the pazopanib cohort showed worse survival compared with that of the methotrexate and vinblastine cohort. The sustained effect of treatment is important for the patient, and additionally, pazopanib is more expensive. Third, dose reduction was required in more than 70% of patients in both cohorts, meaning that the adequate dose-intensity needs to be determined. For instance, administration of methotrexate and vinblastine every other week could reduce the proportion of adverse effects without decreasing the proportion of patients achieving a response.[4] Fourth, quality of life, including pain, should be evaluated carefully. The results of this study showed a favourable effect for pain mitigation in the pazopanib cohort in comparison to the methotrexate and vinblastine cohort. However, only six patients were evaluated in the methotrexate and vinblastine cohort after treatment, making precise conclusions difficult to draw. Previous studies have showed pain relief with methotrexate and vinblastine treatment.[4,5]

The results of this randomised study provide very useful information for physicians and patients, particularly regarding pazopanib. However, several clinical issues remain to be resolved including use in paediatric patients, duration of effects, and patient financial burden.

We declare no competing interests.

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