



Desmoid tumours: a perfect example for making progress in treatment management through international collaboration

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To cite: Kasper B. Desmoid tumours: a perfect example for making progress in treatment management through international collaboration. *ESMO Open* 2019;4:e000636. doi:10.1136/esmoopen-2019-000636

Received 11 November 2019
Accepted 11 November 2019

Desmoid tumour (DT) is a rare disease characterised by a locally aggressive monoclonal, fibroblastic proliferation leading to a variable and often unpredictable clinical course. The incidence is estimated to be 5–6 cases per 1 million of the population per annum with a peak age of 30–40 years and a female predominance.¹ Although a DT is not life-threatening in the vast majority of cases, patients often suffer from pain, functional deficits, psychological problems and a general decrease in quality of life. DT is a clearly understudied disease and until most recently no level I or II evidence did exist; there were only few prospectively conducted studies or meta-analysis. Therefore, an effort has been made in the last 5 years to bring together adult and paediatric sarcoma experts from different disciplines with patients and patient advocates initially from Europe,^{2,3} more recently also from North America and Japan to harmonise DT treatment strategies among clinicians. In addition, FAP-related DTs (familial adenomatous polyposis) have been included into the consensus and recommendation process. In 2018, an evidence-based, joint global consensus guideline approach to the management of this disease has been undertaken focusing on molecular genetics, indications for an active treatment and available systemic therapeutic options. Consensus was reached by (1) An initial evidence-based systematic literature search that was performed by an independent institute involving methodological experts and an analysis of the identified literature according to GRADE (Grading of Recommendations Assessment, Development and Evaluation). (2) This was followed by a consensus meeting held in Milan, Italy, in June 2018 under the auspices of the European Reference Network for rare solid adult cancers (EURACAN) and the European Organisation for Research and

Treatment of Cancer (EORTC) Soft Tissue and Bone Sarcoma Group (STBSG) with the support of the patient advocacy groups ‘sos desmoid’ Germany, Sarcoma Patients EuroNet (SPAEN) and The Desmoid Tumor Research Foundation (DTRF). As an output of this consensus meeting a comprehensive position paper has been written receiving formal EORTC Board approval in 2018; this paper will be published in due course in the *European Journal of Cancer*.⁴

In this context, the published paper in the current issue of *ESMO Open* on ‘FAP-related desmoid tumours treated with low-dose chemotherapy: results from an international, multi-institutional, retrospective analysis’ by Napolitano *et al* is of special interest. The authors retrospectively analysed outcome data from 37 patients with FAP-associated DTs (median age 29 years, range 7–44) treated with weekly methotrexate (MTX) plus vinca alkaloids (vinorelbine or vinblastine) in seven European sarcoma reference centres between 2000 and 2018. According to the Response Evaluation Criteria in Solid Tumours (RECIST), 20/37 (54.1 %) patients achieved partial response, 15/37 (40.5 %) patients had stable disease and 2/37 (5.4 %) patients had progressive disease as best response. The median progression-free survival was 6.5 years (range 0.3–12.1). In this largest series on the activity of low-dose chemotherapy in patients with FAP-related DT, MTX plus vinca alkaloids demonstrated to be an active treatment combination, which was already reported in larger series in patients with sporadic DT.⁵ These results are in line with the updated consensus recommendations: ‘FAP-associated DT (Gardner syndrome) seems to be more aggressive and multifocal and, therefore, tends to be treated more aggressively in terms of medical management. (...) FAP patients should be jointly managed by sarcoma

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specialists and experts in gastrointestinal cancer. (...)’ In principle, the management approach for patients with FAP-associated DT is very similar to that of patients with sporadic DT and should follow the treatment algorithm as recommended in the recent consensus papers by The Desmoid Tumor Working Group.^{3,4}

In conclusion, Napolitano *et al* provided retrospective data supporting the clinical activity of the low-dose chemotherapy combination MTX plus vinca alkaloids in patients with FAP-associated DT. Besides the clinical benefit for our patients, this paper nicely demonstrates the value of international, collaborative (even retrospective) analyses and studies among reference centres to answer relevant clinical questions in rare cancers. One step forward on the way of improving patients’ care in rare cancers in Europe has been the consolidation of EURACAN in 2016 comprising one domain for bone and soft tissue sarcomas (<http://euracan.ern-net.eu/>). This initiative is based on the directives developed by Rare Cancers Europe a few years ago. EURACAN should provide highly specialised care for patients with rare cancer in Europe and should enable second opinions across the European countries. Moreover, EURACAN will promote multidisciplinary advice, develop and implement clinical practice guidelines, disseminate knowledge and support national centres and networks.⁶ In parallel, the European research initiative Joint Action on Rare Cancers (JARC; <https://jointactionrarecancers.eu/>) has been launched by the European Commission closely linked to and forming the basis for the reference network EURACAN. Final recommendations from JARC have just been presented to the European Parliament and summarised in a booklet entitled ‘*Rare Cancer Agenda*

2030 - Ten Recommendations from the EU Joint Action on Rare Cancers’.

Funding The authors have not declared a specific grant for this research from any funding agency in the public, commercial or not-for-profit sectors.

Competing interests None declared.

Patient consent for publication Not required.

Provenance and peer review Commissioned; internally peer reviewed.

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