Information from journals and other sources

PEXIDARTINIB RECEIVES CATEGORY 1 RECOMMENDATION FROM NCCN

Pexidartinib, the newly approved agent to treat patients with tenosynovial giant cell tumor (TGCT), received a category 1 recommendation from the National Comprehensive Cancer Network (NCCN) in the recent update of its Clinical Practice Guidelines in Oncology, Soft Tissue Sarcoma (Version 4.2019). A category 1 recommendation is based on a high level of evidence with uniform consensus that the intervention is appropriate.

The NCCN based its recommendation on the randomized, placebo-controlled phase 3 ENLIVEN study (NCT02371369) published in *The Lancet* (Tap WD, Gelderblom H, Palmerini E, et al. *Lancet*. 2019;394:478-487). The placebo-controlled portion of the study showed that patients treated with pexidartinib achieved a significantly higher overall response than patients in the placebo arm, 39% compared to none, respectively. The investigators identified mixed or cholestatic hepatotoxicity to be a risk of systemic therapy with the agent. Nevertheless, the "robust tumour response," they wrote, "with improved patient symptoms and functional outcomes" establish pexidartinib as a potential treatment for TGCT in cases not amenable to improvement with surgery.

FB SUPPORT GROUPS ENABLE RAPID ACCESS TO LARGE NUMBERS OF PATIENTS WITH RARE DISEASES

Investigators conducted a survey study of 214 patients with dermatofibrosarcoma protuberans (DFSP) or their family members using in part existing Facebook patient support groups (FBSG) to recruit respondents. They found the approach provides a "powerful" tool to collect relevant disease information from large numbers of patients with rare diseases.

A team of medical practitioners and patients developed the multiple-choice survey, and after testing the survey twice, posted a survey announcement on FBSGs for DFSP. The survey was live for

3 weeks in 2015. The investigators rapidly collected disease statistics, including information on recurrence, metastasis, surgical outcomes, diagnostic delay, and more, suggesting that FBSGs are useful medical research tools.

One hundred ninety-nine respondents were patients and 15 were family members. The respondents reported a median of 4 years to receive a correct diagnosis after noticing a lesion, ranging from less than 1 year to 42 years. About half the patients (52.3%) believed they received a misdiagnosis at some point, either from a dermatologist, primary care clinician, or another type of physician. Patients first noticed DFSP at a median age of 29.6 years. Many of their lesions appeared initially as flat plaques that eventually became protuberant. Because of this disconnect between the disease name and its clinical presentation, the investigators proposed the alternative term, dermatofibrosarcoma, often protuberant, be adopted. The investigators concluded that "FBSGs appear to be powerful tools to synergize effective and rapid research collaborations with large numbers of international patients with rare disease." **TSJ**

Source: David MP, Funderburg A, Selig JP, et al. Perspectives of patients with dermatofibrosarcoma protuberans on diagnostic delays, surgical outcomes, and nonprotuberance. *JAMA Network Open*. 2019;2(8):e1910413.

› "FBSGs APPEAR TO BE POWERFUL TOOLS TO SYNERGIZE EFFECTIVE AND RAPID RESEARCH COLLABORATIONS WITH LARGE NUMBERS OF INTERNATIONAL PATIENTS WITH RARE DISEASE."