Kaposiform hemangioendothelioma: Could sirolimus replace vincristine as the first-choice treatment ?

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BACKGROUND: Kaposiform hemangioendothelioma (KHE) with or without Kasabach–Merritt phenomenon (KMP) is a rare tumor with inherent diagnostic and treatment challenges. First-choice treatment has up to recently been steroids, and vincristine for steroid-resistant KHE. The m–TOR inhibitors, sirolimus and everolimus have shown efficacy in cases of non-response to vincristine or regrowth after cessation of treatment. Could Sirolimus/everolimus replace vincristine as the first-choice treatment of KHE ? Pros and cons for vincristine vs sirolimus include way of administration, acute side effects as well as long term side effects and cost of treatment.

METHODS AND RESULTS: For unknown reasons, an unusually high number of cases of KHE were diagnosed and treated at our institution the last 3 years. One case of multifocal subcutaneous KHE in the fronto-temporal area in a 6 year old boy and 4 cases in infants, aged from 1 day to 4 months; one tumor localized on the neck, 2 on the trunk and one on the lower extremity. KMP was present in 3 of the infants, although mild in 2 of these. All were treated with vincristin with excellent response and no relapse so far.

We review : Mode of administration of vincristine vs sirolimus in infants and very young children, the cost of treatment, acute and long term side effects, the existing literature and experience concerning vincristine and m-TOR inhibitors in young children.

CONCLUSION: Since KHE is most common in infants or very young children and there is not yet enough experience concerning the side effects of m-TOR inhibitors in this group, vincristine should so far be considered the first-choice treatment of KHE and sirolimus/everolimus reserved to refractory cases or relapses.

Drolet BA et al. Journal of Pediatrics, 2013;163: 285-291. Consensus-Derived Practice Standards Plan for Complicated Kaposiform Hemangioendothelioma

Wang Z et al. Pediatr Blood Cancer 2015;62: 577–580. Steroid-Resistant Kaposiform Hemangioendothelioma: A Retrospective Study of 37 Patients Treated With Vincristine and Long-Term Follow-up

Kai. L. J Cancer Res Clin Oncol. 2014;140: 471-6. Sirolimus, a promising treatment for refractory Kaposiform hemangioendothelioma.

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