PAEDIATRIC DERMATOLOGY

SUCCESSFUL SIROLIMUS TREATMENT OF A NEONATE WITH LIFE THREATENING KAPOSIFORM HEMANGIOENDOTHELIOMA THAT FAILED PREDNISOLONE AND VINCRISTINE

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Background : Kaposiform Hemangioendothelioma (KHE) are vascular tumors that causes morbidity primarily from mass effect and Kasabach-Merritt Phenomenon (KMP). Sirolimus, a mammalian target of Rapamycin (mTOR) inhibitor, has anti-angiogenic activity.

Observation : A term baby girl presented with a painful violaceous left chest wall mass at birth. She was diagnosed as KHE with KMP when the lesion expanded rapidly with reducing platelet counts & coagulopathy. MRI+MRA at day 5 of life was supportive of KHE with an overall mass size of 5cm(W) x 6cm(AP) x 8cm(H). She was then treated with Prednisolone 3mg/kg/day & IV Vincristine 0.05mg/kg/week for a total of 22 cycles.
Initial response to first 12 cycles of Vincristine was encouraging with mass shrinkage.
Unfortunately the mass re-enlarged after stopping Vincristine & did not respond to another 10cycles of Vincristine.
Oral Sirolimus was initiated at 0.1mg/kg/day in divided doses, targeted to trough levels of 8-15ng/ml.The mass progressively reduced in size & was no longer tender. Diarrhea, hepatitis & rash was experienced when trough levels exceeded > 5X normal limit but no toxicity was experienced at targeted trough levels. Repeated MRI/MRA after 6 months of Sirolimus showed marked reduction in both the size and number of feeding vessels. Although the absolute size of the mass remained unchanged, there was marked reduction of the relative size of the mass in comparison to the size of the patient.

Key messages : Sirolimus is effective & safe in life threatening KHE who has exhausted all other alternatives.