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Case Report

Pharmacological Treatment Leading to Complete Resolution in Kasabach-Merritt Phenomenon-Case Report

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ABSTRACT

Kasabach-Merritt syndrome is a rare vascular tumor usually seen in infancy. It is locally aggressive and infiltrates the skin, subcutaneous tissue, and muscles. It is characterized by coagulopathy with thrombocytopenia, microangiopathic hemolytic anemia, and raised d-dimer levels. In small infants, this can cause life-threatening bleeding and can be fatal. We report on a two-month-old female child who presented to us with a rapidly enlarging purplish swelling on the right arm. It was also associated with petechial spots all over the body. A clinical diagnosis of hemangioma with Kasabach-Merritt phenomenon was made and was further confirmed by hematological investigations which showed anemia, and thrombocytopenia with hypofibrinogenemia. Imaging of the limb confirmed the diagnosis. After taking parental consent, the baby was started on injections of vincristine weekly with oral prednisolone. There was a significant reduction in the tumor's size and improved blood parameters. After 6 weeks of steroid therapy, the medication was tapered and the child was changed to single agent sirolimus with monitoring of serum levels. There was a remarkable response to sirolimus with complete resolution of the tumour and Kasabach-Merritt phenomenon. Kasabach-Merritt syndrome can be a life-threatening complication in infants. Appropriate pharmacological therapy with stringent monitoring can bring complete resolution.

Keywords: Kasabach-Merritt phenomenon (KMP), kaposiform hemangioendothelioma (KHE), thrombocytopenia, coagulopathy, sirolimus

Introduction

Kasabach-Merritt phenomenon (KMP) is a life-threatening event with the triad of anemia, thrombocytopenia and, coagulopathy in association with vascular tumors (1). Most cases present in infancy and the mortality rate varies from 10-37% in various studies (2). They usually present with a rapidly enlarging firm solitary purpuric, a soft to a firm cutaneous lesion, anemia, thrombocytopenia, and varying degrees of coagulopathy. Complete surgical resection offers the best form of cure; however, in many cases due to extensivity and coagulopathy, surgery is not possible. In such a circumstance, pharmacological therapy is the best option.

Case Report

We report on a 2-month-old female infant who presented with a history of erythematous lesion over the right arm from day 10 of life. It started gradually, initially

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small in size near the posteromedial aspect of the right arm, and progressed rapidly to reach the present size. It was associated with an edematous change of the right upper limb. She was a first-order child with a birth weight of 3 kilogram (kg) and had an uneventful perinatal period.

At admission, the child was conscious, alert, and playful with a weight of 4.6 kg and had stable vitals, with a normal general and systemic examination. Local examination of the right arm showed a tumor of size 8.5 centimeter (cm)×14 cm, warm, non-tender, firm in consistency, associated with edema of the right upper limb with distal pulses clearly palpable. The skin above the lesion was reddish-violaceous with small petechiae without signs of compromise in the blood supply (Figure 1).

Initial investigations including a complete blood count (CBC) revealed a total leukocyte count of 6,700 cells/cu.mm, hemoglobin (Hb) of 6.9 g/dL, total platelet count (TPC) of 4.000/cu.mm (thrombocytopenia) with peripheral smear showing microcytic hypochromic anemia with thrombocytopenia, with prothrombin time: 11.6 seconds (sec), activated partial thromboplastin time: 25.8 secs, internationalized normal ratio: 1.05 (normal), D-dimer: 13.23 ug/mL (high), and serum fibrinogen: <40 mg/dL. (low). Liver and renal functions were normal. Ultrasound examination of the abdomen and pelvis was normal and there was no evidence of hemangiomas elsewhere.

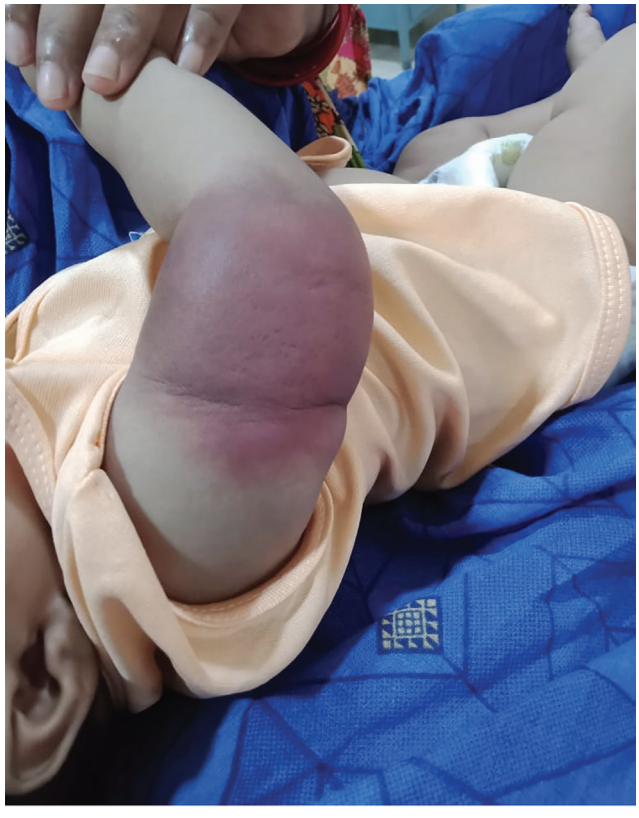

Figure 1. Vascular purplish tumour on right arm at presentation

A Doppler study of the right upper limb showed thickened heterogenous soft tissue over the right arm, and proximal forearm with prominent vessels showing arterial and venous flow with dilated vessels in the subcutaneous plane and intramuscular plane with features suggestive of hemangioma/hemangioendothelioma.

Magnetic resonance imaging of the affected limb revealed ill-defined T2 weighted short tau inversion recovery hyperintense soft tissue thickening involving subdermal fat planes in the arm and forearm.

Computed tomography angiogram of the right upper limb showed ill-defined to dense soft tissue thickening, mild homogenous enhancement of skin and subcutaneous plane of the right arm and forearm region on a post-contrast study and retention of contrast in delayed phases with likely hemangioma.

The baby had worsening anemia and received a packed red blood cell transfusion at 10 mL/kg owing to the anemia and thrombocytopenia with hypofibrinogenemia in the presence of a rapidly increasing vascular tumor, the child was diagnosed as a case of KMP. After parental counseling, the child was started on a standard regimen of oral prednisolone at 2 mg/kg/day and IV vincristine 0.05 mg/kg weekly.

After the second dose of vincristine, her CBC parameters improved (Hb: 8.7 gr/dL and TPC: 16,000/cu.mm). No platelet transfusion was given as platelet transfusions have been associated with tumoral bleeding.

On serial monitoring, the edema and the lesion gradually decreased in size, and at the time of discharge after 2 weeks of therapy, the size had reduced to 7 cm×13 cm. The parents were advised to follow up at our outpatient department regularly and were given vincristine and steroids for 4 weeks.

One month after follow-up, because of the persistent considerable size of the tumor, the child was started on sirolimus at 0.8 mg/m^2/dose twice daily along with a tapering dose of the steroids.

The steroids were stopped after 6 weeks and the child continued with sirolimus with regular CBC monitoring. Continuing with sirolimus, the size of the tumor was further reduced and there was no recurrence of thrombocytopenia or anemia. After 4 weeks of continuous sirolimus therapy, it was discontinued and the tumor had completely resolved by then. On follow-up, three months post-stoppage of the

therapy, there was no increase in the size of the tumor or any recurrence of KMP (Figure 2).

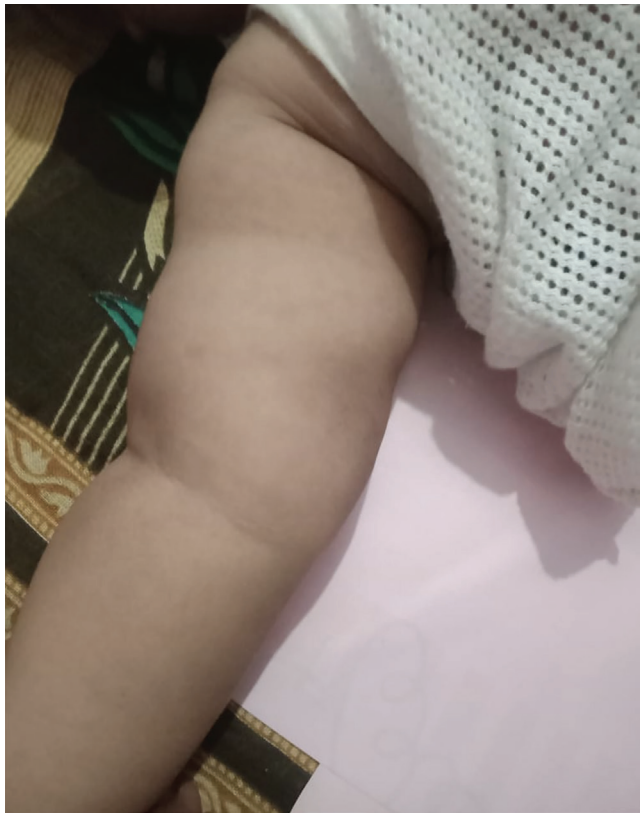

Figure 2. Complete resolution after sirolimus based therapy

Discussion

KMP is a potentially life-threatening disorder and pharmacologic treatment is now considered the first line of management (3). While the North American group recommends daily steroids plus weekly vincristine, European centers recommend a combination of steroids with ticlopidine and sirolimus. The duration of treatment is not well defined with most of the groups recommending vincristine and steroids until clinical response occurs, with sirolimus being recommended for at least 1 year. However, as seen in our case, remission was induced with a much shorter duration of treatment and this needs to be explored further. Sirolimus as a stand-alone treatment can also bring remission and is relatively safe, although serum drug levels have to be measured routinely. However, recent trials have suggested Sirolimus along with steroids to be the best first-line treatment (4).

KMP is a sign of an aggressive vascular tumor and has the propensity to cause severe bleeding which might be life-threatening. While steroids have been considered the first line of therapy, they carry a risk of infections, growth stunting, and developmental delay. Sirolimus is an inhibitor of mammalian target for rapamycin (5). Various study groups have shown sirolimus to be effective even in cases where high-dose corticosteroids have failed to get a response. Sirolimus has been shown to have a pooled odds ratio of 0.91 and minimal side effects (6). We conclude that sirolimus as a stand-alone treatment or with vincristine or steroids is a promising therapy and should be explored more in KMP.

Ethics

Informed Consent: Informed consent was obtained.

Footnotes

Authorship Contributions

Surgical and Medical Practices: M.R.B., N.K.M., S.D., P.D., Concept: J.T., B.A., P.D., Design: M.R.B., N.K.M., S.D., B.A., P.D., Data Collection or Processing: J.T., B.A., Analysis or Interpretation: M.R.B., N.K.M., S.D., P.D., Literature Search: M.R.B., P.D., Writing: J.T., M.R.B., B.A., P.D.

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