AUTOLOGOUS HEMATOPOIETIC STEM-CELL TRANSPLANTATION AS A THERAPEUTIC OPTION FOR SYSTEMIC SCLEROSIS IN CHILDREN: A REPORT CASE.
Case Report

• A 3-year-old Brazilian girl presented with skin lesions in limbs, trunk and abdomen over a 18-month-period, with a worsening condition after one year having the disease.

• In addition, she had febrile episodes, Raynaud's phenomenon, dysphagia, diarrhea and failure to thrive.

• Physical examination showed diffuse sclerotic skin plaques over her trunk, abdomen, and limbs (Rodnan score 7).

• Lab exams were normal (Hb, CBC, acute phase markers, urine, renal and hepatic function).

• Thorax CT scan: normal.
Case Report

- Cutaneous biopsy confirmed the hypothesis of scleroderma and videodeglutogram showed decreased esophageal motility.

- Treatment was initiated with prednisone (1mg/kg/d), methotrexate 15mg/m2/week and nifedipine.

- After 6 months, patient’s condition worsened, with long periods of Raynaud's phenomenon associated with digital ulcers, frequent vomiting and intense abdominal pain episodes with pallor.

- Her dysphagia worsened, she was not able to eat and was undernourished (weight and height below P3 for age).

- Intestinal radiological evaluation showed decreased motility and evidence of vasculitis.
What would you do?
In your opinion, what treatment should be done?
What we did...

• The rapid progression of symptoms despite conventional treatment, led us to decide, with family consent, to use HSCT as an option for the disease control.

• Prior to infusion of stem cells, the conditioning phase was performed with anti-lymphocyte immunoglobulin, methylprednisolone and cyclophosphamide.

• Patient evolution after HSCT was good with apparent complete recovery and absence of previous symptoms.

• After being free of any drugs for 3 months (following the standard one year medication protocol) the skin lesions returned but not Raynaud's phenomenon neither gastrointestinal symptoms.
The follow up:

Figure 1: Patient’s weight for age chart. Red marking: before hematopoietic stem cell transplantation. Blue marking: after hematopoietic stem cell transplantation.

Figure 2: Patient’s height for age chart. Red marking: before hematopoietic stem cell transplantation. Blue marking: after hematopoietic stem cell transplantation.
Question:

• What other treatment would you do?

• What would be the best treatment option for the post-transplantion reactivation?

• What is your experience with HSCT?
Thank you!

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