

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



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# 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/m<sup>2</sup>/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 P<sub>3</sub> 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:

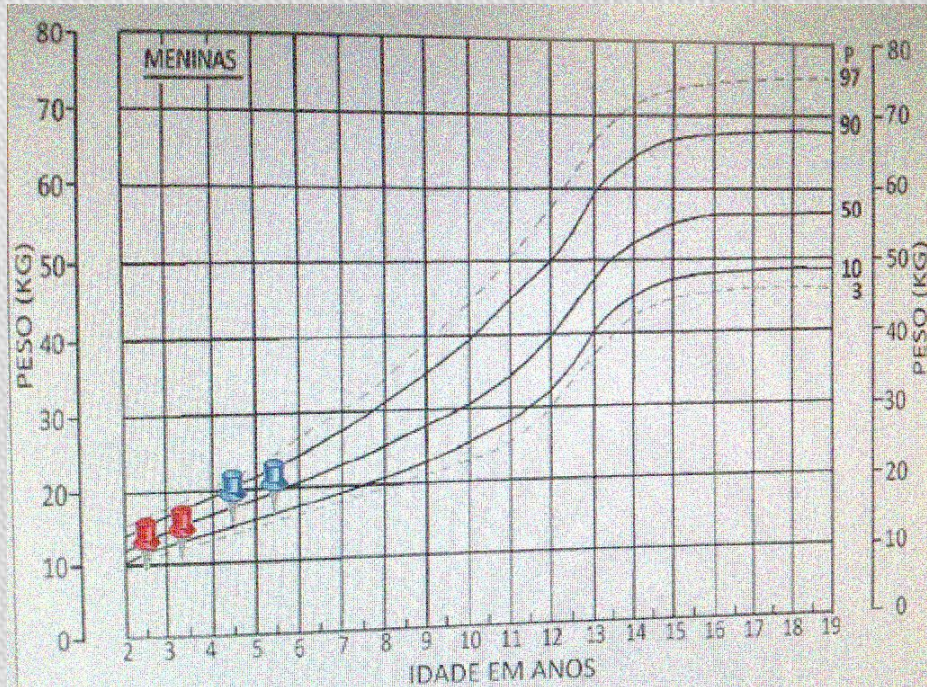


Figure 1: Patient's weight for age chart.  
Red marking: before hematopoietic stem cell transplantation.  
Blue marking: after hematopoietic stem cell transplantation .

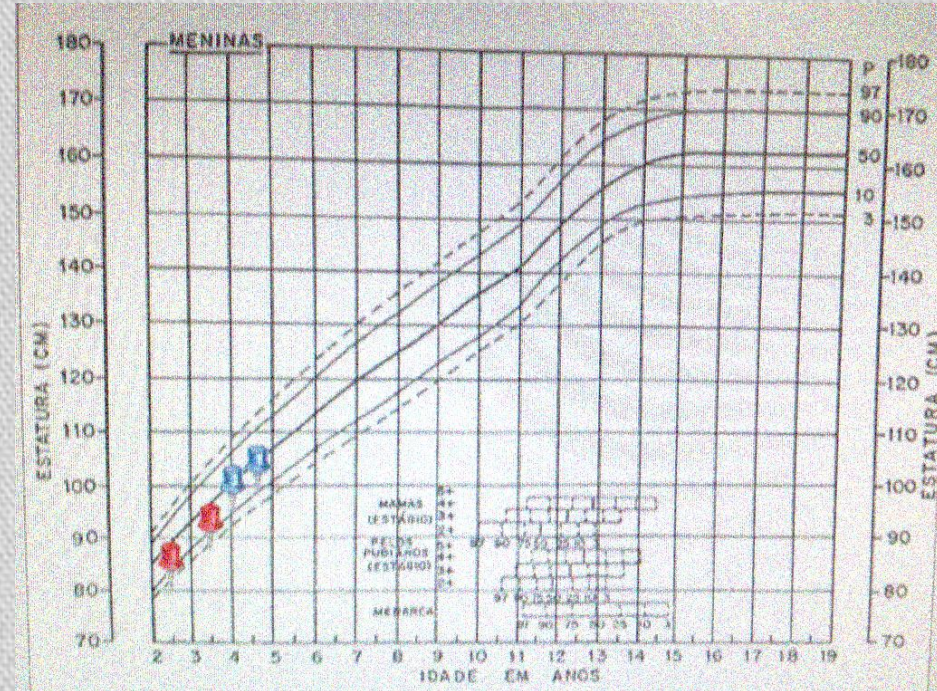
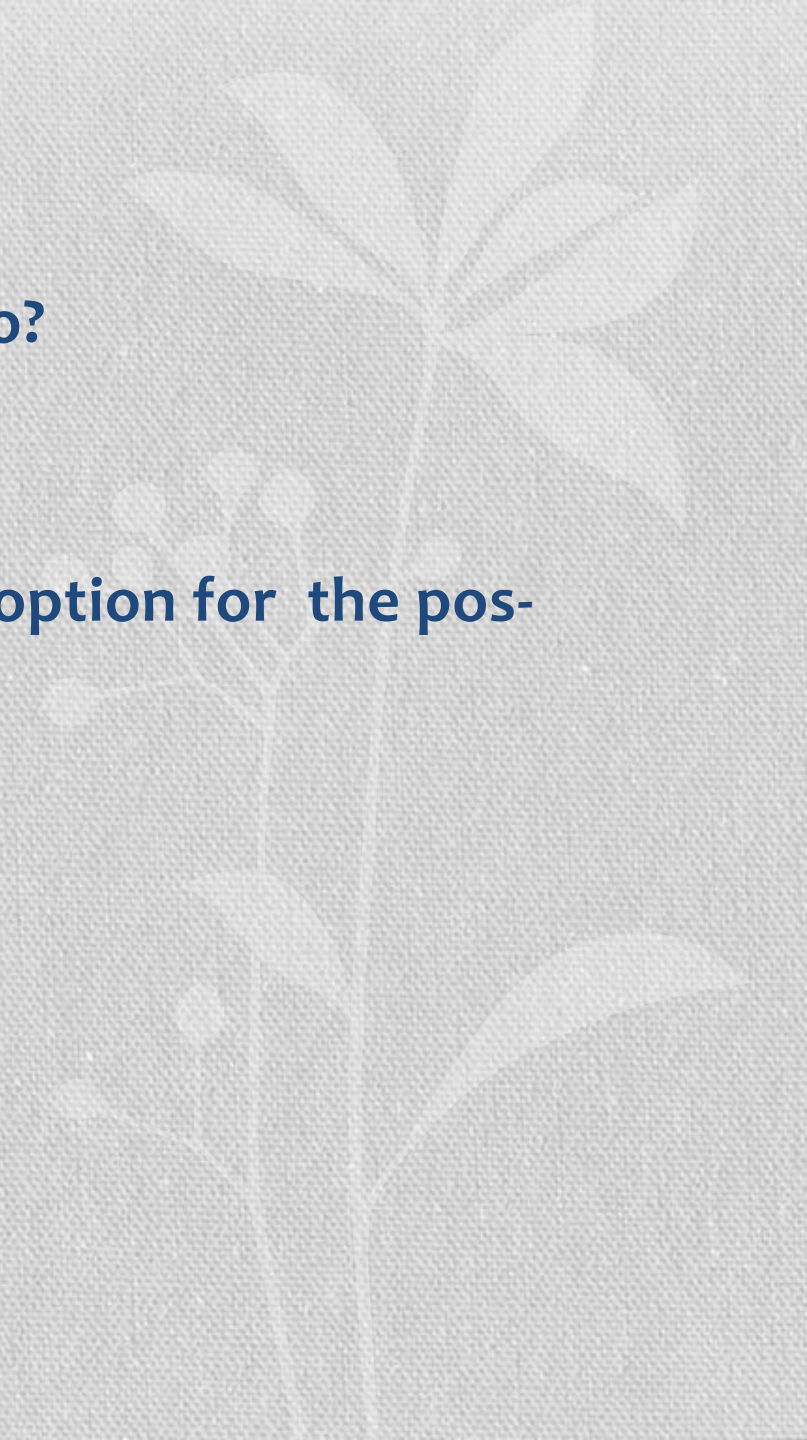


Figure 2: Patient's height for age chart.  
Red marking: before hematopoietic stem cell transplantation.  
Blue marking: after hematopoietic stem cell transplantation .

# Question:

- **What other treatment would you do?**
  - **What would be the best treatment option for the post-transplantation reactivation?**
  - **What is your experience with HSCT?**
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# Thank you!

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