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Intramyocardial injection of autologous bone marrow stem cells in children with Hypoplastic Left Heart Syndrome: the THABY Phase I trial

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INTRODUCTION AND OBJECTIVES

Treatment of heart failure in Hypoplastic Left Heart Syndrome (HLHS) remains one of the biggest challenges among congenital heart diseases. Cell therapy has shown encouraging results in the field of heart failure. The aim of this trial was to determine the feasibility and safety of intramyocardial injection of mononuclear stem cells (MSC) in children with HLHS.

METHODS

We designed a nonrandomized trial to prospectively include 9 patients with diagnosis of HLHS in Norwood stage I or II. At the time of subsequent palliative surgery we performed intramyocardial injection of autologous bone marrow MSC derived under GMP conditions.

Primary end point was to examine the feasibility and safety of the procedure. Security was defined as absence of clinical events, arrhythmias on Holter ECG or focal lesions on echocardiography and cardiac MRI. As secondary end point we tried to assess improvement on cardiac function evaluated by MRI.

RESULTS

Between November 2013 and April 2015, 9 patients (3-9 years) at Norwood stage II received MSC (339x106 cells ± 191x106) during Fontan surgery. Bone marrow extraction, cell processing and delivery were feasible and did no interfere with surgery. There were no incidences related with the injection (no more bleeding, no arrhythmias) We found no adverse effects on a 12 months follow-up. Cardiac function improved, although this did no reach statistical significance (FE 46%±5% vs 54%±13%; p=0,253)

CONCLUSIONS

Intramyocardial injection of autologous bone marrow MSC is feasible and safe in children with HLHS during Fontan surgery. Phase 2 trials are needed to determine the potential benefits on cardiac function and clinical outcomes.