

Late effects after hematopoietic stem cell transplantation for β -thalassemia major: the French national experience

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Online Supplement

Supplementary Methods:

Details on endpoints definitions

Overweight in adults was defined as a Body Mass Index (BMI) over 25 kg/m².

Puberty delay was defined as lack of puberty onset in boys aged 14 or more and in girls aged 13 or more. Hypogonadism was defined in females as low estradiol levels or long-term sex hormone replacement therapy and in males as low levels of testosterone or long-term sex hormone replacement therapy. According follicle stimulating hormone (FSH) and luteinising hormone (LH) basal values, hypogonadism was classified as hypergonadotropic or hypogonadotropic.

Hypothyroidism was defined as elevated thyroid-stimulating hormone (TSH) concentration with normal (subclinical) or decreased (overt) levels of serum free thyroxin (FT4) concentration.

Cardiac function was impaired when the echocardiographic ejection fraction (EF) was reduced (EF≤50%). Diabetes was diagnosed through blood tests (fasting glucose test >7mmol/l or glycosylated haemoglobin test HbA_{1c}>6.5%) and insulin or oral antidiabetic medication requirement.

Supplementary Table1

Table S1: Multivariate longitudinal analysis of factors influencing height and weight SDS evolution after HSCT

Characteristics	Coefficient (β)	95% CI	p-value
Height			
Age at transplantation (per year)	-0.12	-0.15 to -0.1	<0.001
Serum ferritin level before HSCT (per 100 $\mu\text{g/L}$)	-0.007	-0.01 to -0.003	<0.001
Follow-up duration (per year)	-0.02	-0.03 to -0.01	<0.001
Weight			
Age at transplantation (per year)	-0.24	-0.28 to -0.2	<0.001
Sex			
- Males vs females	-0.42	-0.7 to -0.14	0.003
Serum ferritin level before HSCT (per 100 $\mu\text{g/L}$)	-0.01	-0.01 to -0.005	<0.001
Follow-up duration (per year)	0.05	0.03 to 0.07	<0.001

HSCT: Hematopoietic Stem Cell Transplantation; CI: confidence interval

Supplementary Table2

Table S2: Multivariate longitudinal analysis of factors influencing serum ferritin levels evolution after HSCT

Characteristics	Coefficient (β)	95% CI	<i>p</i>-value
Phlebotomy/chelation therapy - Yes vs No	-327.9	-577. to -78.8	0.011
Follow-up duration (per year)	-78	-90 to -66	<0.001
Serum Ferritin level at HSCT ($\mu\text{g/L}$)	0.15	0.056 to 0.261	<0.001
Age at transplantation (per year)	35.8	15.6 to 56	0.001

HSCT: Hematopoietic Stem Cell Transplantation; CI: confidence interval