

Pubertal development of transfusion-dependent thalassemia patients in the era of oral chelation with deferasirox: results from the French registry

Authors

Mathilde Veneziano Broccia,^{1,2} Julia Vergier,³ Audrey Benoit,⁴ Yoann Huguenin,⁵ Anne Lambilliotte,⁶ Marie Pierre Castex,⁷ Stephanie Gourdon,⁸ Ghislaine Ithier,⁹ Kamila Kebaili,¹⁰ Pierre Rohrlich,¹¹ Corinne Pondarre,¹² Abdourahim Chamouine,¹³ Pauline Simon,¹⁴ Kokou Placide Agbo Kpati,¹⁵ Slimane Allali,¹⁶ Sandrine Baron-Joly,¹⁷ Sophie Bayart,¹⁸ Nicolas Billaud,¹⁹ Valentine Brousse,²⁰ Cecile Dumesnil,²¹ Nathalie Garnier,¹⁰ Isabelle Guichard,²² Laure Joseph,²³ Annie Kamdem,²⁴ Julie Maitre,²⁵ Catherine Mathey,¹ Catherine Paillard,²⁶ Aurelie Phulpin,²⁷ Cecile Renard,¹⁰ Cecile Stoven,²⁸ Mohamed Touati,²⁹ Capucine Trochu,³⁰ Suzanne Mathieu Nafissi,³¹ Catherine Badens,⁴ Sarah Szepetowski^{1,2} and Isabelle Thuret^{1,2}

¹Service d'Hematologie, Immunologie et Oncologie Pediatrique, Hopital La Timone Enfants, AP-HM, Marseille; ²Centre de Reference MCGRE, Service d'Hematologie, Immunologie et Oncologie Pediatrique, Hopital La Timone Enfants, AP-HM, Marseille; ³Service de Pediatrie Multidisciplinaire, Hopital de la Timone Enfants, AP-HM, Marseille; ⁴National Thalassemia Registry (NaThalY), Service de Genetique, Hopital La Timone Enfants, AP-HM, Marseille; ⁵Service d'Oncologie et d'Hematologie Pediatrique, Hopital Pellegrin, CHU de Bordeaux, Bordeaux; ⁶Service d'Hematologie et d'Oncologie Pediatrique, Centre Hospitalo-Universitaire de Lille, Lille; ⁷Service d'Hemato-Oncologie Pediatrique, Hopital des Enfants, Toulouse; ⁸Service d'Oncologie et Hematologie Pediatrique, Centre Hospitalier Universitaire de La Reunion, Saint Denis; ⁹Centre de Reference MCGRE, Unite d'Hematologie, Hopital Robert Debre, Assistance Publique-Hopitaux de Paris (AP-HP), Paris; ¹⁰Institut d'Hematologie et d'Oncologie Pediatrique, Hospices Civils de Lyon, Lyon; ¹¹Service d'Hematologie Pediatrique, CHU de Nice, Nice; ¹²Centre de

Reference MCGRE, Service de Pediatrie, Centre Hospitalier Intercommunal de Creteil, Creteil, Inserm U955, Universite Paris-XII, Creteil; ¹³Centre de Reference MCGRE, Service de Pediatrie, Centre Hospitalier de Mayotte, Mamoudzou, Mayotte; ¹⁴Service d'Hematologie Pediatrique, CHU de Besançon, Besançon; ¹⁵Service de Pediatrie-Neonatalogie-Medecine de l'Adolescent, GHEF-Site de Marne La Vallee, Jossigny; ¹⁶Centre de Reference MCGRE, Service de Pediatrie Generale, Hopital Necker-Enfants Malades, AP-HP, Paris; ¹⁷Service de Pediatrie, CHU de Nimes, Nimes; ¹⁸Service d'Hematologie Pediatrique, CHU de Rennes, Rennes; ¹⁹Service de Pediatrie, CHR Metz-Thionville, Metz; ²⁰Centre de Réference MCGRE, Service d'Hematologie-Immunologie, Hopital Robert Debre, AP-HP, Paris; ²¹Service d'Hematologie Pediatrique, CHU de Rouen, Rouen; ²²Service de Medecine Interne, Hopital Nord, Saint-Etienne; ²³Centre de Reference MCGRE, CIC Biotherapie, Hopital Necker-Enfants Malades, AP-HP, Paris; ²⁴Centre de Reference MCGRE, Service de Pediatrie, Centre Hospitalier Intercommunal de Creteil (CHIC), Creteil; ²⁵Service de Pediatrie, CHR d'Orleans, Orleans; ²⁶Service d'Hematologie et d'Oncologie Pediatrique, Hopital HautePierre, Strasbourg; ²⁷Service d'Onco-Hematologie Pediatrique, CHRU Nancy, Vandoeuvre les Nancy; ²⁸Service de Pediatrie, CHU La Reunion, Groupe Hospitalier Sud Reunion, Saint Pierre; ²⁹Service d'Hematologie Clinique, CHU Limoges, Limoges; ³⁰Service de Pediatrie, Hopital d'Enfants, Roubaix and ³¹Hopitaux de Brabois, CHRU de Nancy, Vandoeuvre les Nancy, France

Correspondence:

M. VENEZIANO BROCCIA - Mathilde.veneziano-broccia@ap-hm.fr

<https://doi.org/10.3324/haematol.2023.283610>

Supplemental table 1: Hormonal assessment of patients with hypogonadism

Patient	Age at evaluation (years)	Age at menarche (years)	Age at amenorrhea (years)	Diagnosis	Oestradiol (pg/mL) <i>N 25 - 95</i>	LH (UI/L) <i>N 1.9 - 12.5</i>	FSH (UI/L) <i>N 2.5 - 10.2</i> <i>POF > 25</i>	Other informations
1	21	-	-	Primary amenorrhea	18	0.73	1.4	
2	17	12.5	14.5	Secondary amenorrhea (HH)	25	0.9	2.4	Death
3	23	14	21	Secondary amenorrhea (HH)	24	6.4	6.9	Spontaneous pregnancy
4	21	15	18	Secondary amenorrhea (mixed origin)	19	6.9	15.6	Premature ovarian failure

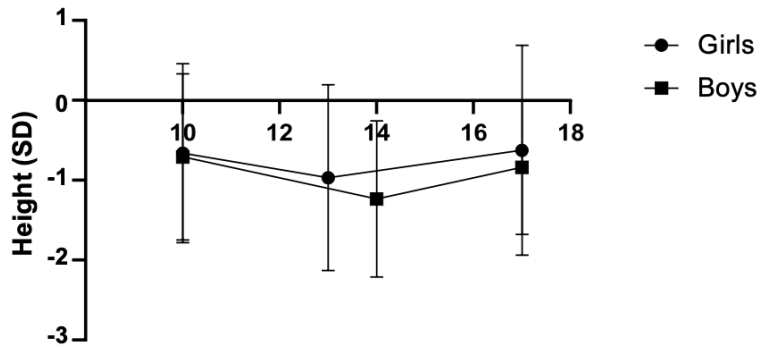
Values at last evaluation

Hypogonadism: decreased synthesis of sexual hormones (estradiol < 25 pg/mL in girls with amenorrhea, testosterone < 0.7 nmol/L in boys)

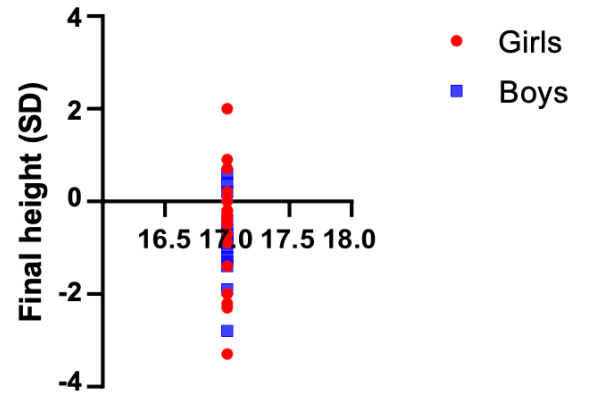
Hypogonadotropic hypogonadism: hypogonadism with normal or low LH and FSH (FSH < 25 UI/L, LH < 12 UI/L)

FSH: Follicle Stimulating Hormone ; HH: Hypogonadotrophic Hypogonadism ; LH: Luteinizing Hormone ; POF : Premature Ovarian Failure

A



B



Supplemental Figure 1: Evolution of patients' heights

A: Evolution of height (standard deviation, SD) according to age (years) and gender

B: Scatterplot of final heights (SD) according to gender