Final Adult Height (FAH) in Patients with PROR-1 Gene Mutations during GH Long-Term Therapy

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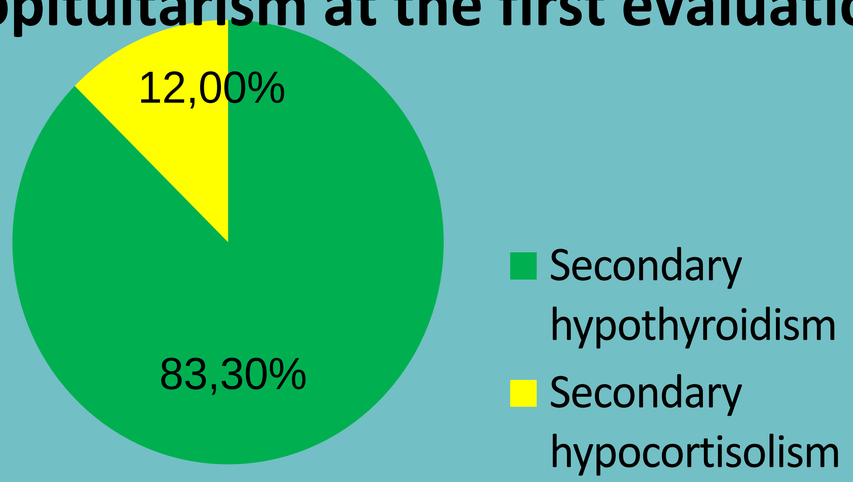
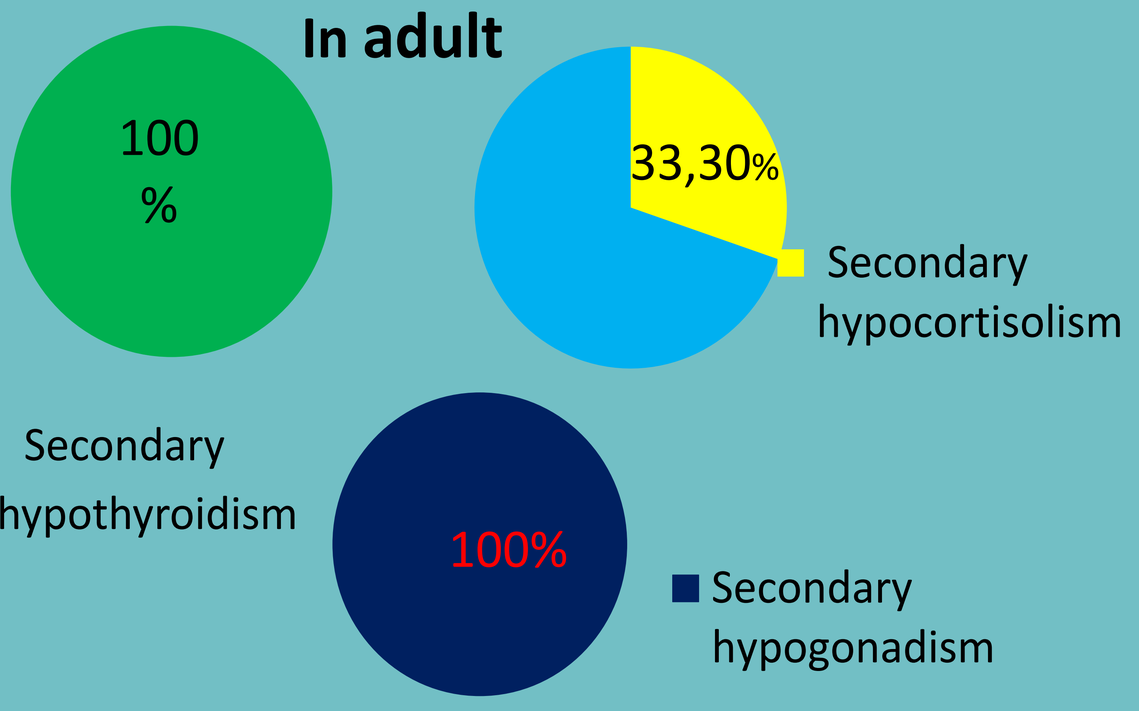
Objective

We performed to evaluate the final adult height (FAH) in a group of patients with a PROP-1 gene mutations. Twenty-five patients (11 males) with a PROP-1 gene mutation, not treated before, were recruited. All the patients had been treated with a fixed rhGH dose (0.033 mg/kg per day) for 10.5 years (7.0–11.45). Fifteen patients (seven males) reachad FAH. All patients received replacement therapy and were compensated all the time during the rhGH treatment.

Additional components of hypopituitarism at the first evaluation



In adult



Design and method:

'Hypopituitarism panel' genes were sequenced using a custom Ion Ampliseq gene panel and PGM semiconductor sequencer (Ion Torrent).

Characteristic	Males (n=11)	Females (n=13)	Total (n=24)
Chronological age at diagnostic (years)	5.0 (5.0 to 6.0)	6.0 (5.0 to 9.0)	6.0 (5.0 to 7.0)
Peak GH level on testing	0.95 (0.2 to 2.5)	0.33 (0.1 to 1.36)	0.5 (0.2 to 1.4)
Height at initiation, SDS	−3.25 (−4.25 to −3.06)	−4.02 (−5.49 to −3.14)	−3.77 (−4.46 to −3.12)

Results:

Characteristic	Males (n=7)	Females (n=8)	P
Height velocity in the first year, SDS	8.98 (6.85 to 11.25)	8.67 (7.35 to 13.02)	
FAH, cm	176.0 (172 to 181.1)	160.5 (158.05 to 166.15)	
HSDS (FAH)	0.24 (−0.41 to 0.98)	−0.25 (−0.67 to 0.69)	0.61
Predicted adult height (PAH), cm	179.0 (174.5 to 190.5)	162.5 (162.5 to 168.5)	
Predicted adult height, SDS	0.65 (−0.03 to 2.38)	0.08 (0.08 to 1.0)	0.62
Δ PAH−FAH	0.00 (−0.26 to 2.14)	0.50 (−0.75 to 1.75)	

Conclusion:

Patients with a PROP-1 gene mutation showed a good response to GH therapy in our study. All patients with a PROP-1 gene mutation reached PAH

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Growth

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