

P1-P166: Safety and effectiveness of growth hormone treatment in patients with Prader-Willi syndrome under 2 years of age in a reference Hospital

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Introduction

Growth hormone (GH) treatment was approved by FDA (2000) and EMEA (2001) for patients with Prader-Willi syndrome (PWS) to improve body composition.

Two fatal events led to initiate it from 2 years of age arbitrarily (real start: 4-6 years). GH does not seem to be a risk factor for mortality, so the 2013 guideline recommends starting it as soon as possible (from 4-6 months of age), preferably below 2 years of age.

Objective

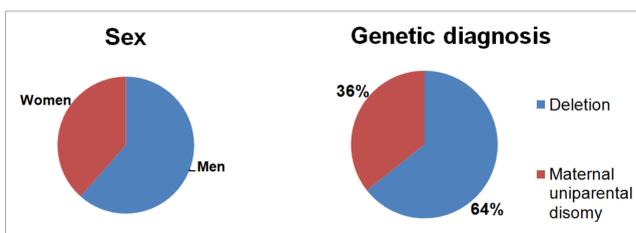
To test the safety and effectiveness of GH treatment in patients with PWS less than 2 years of age (Clinical Trial Govcode: NCT02205450).

Methods

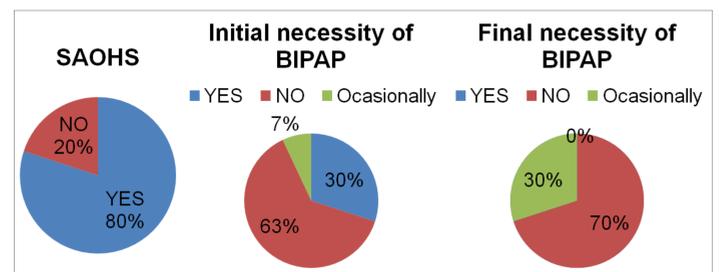
Two-year longitudinal prospective study of children with PWS controlled in our Reference Unit, who started GH treatment before 2 years of age

Results

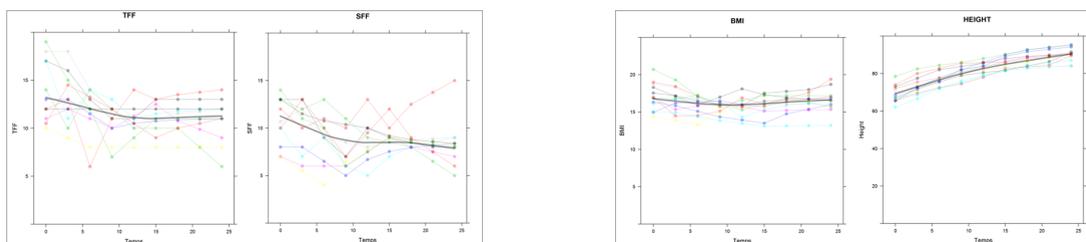
We analyzed the data of 13 patients. Mean age of onset of GH: 12.3 months of age (range of 9-20 months)



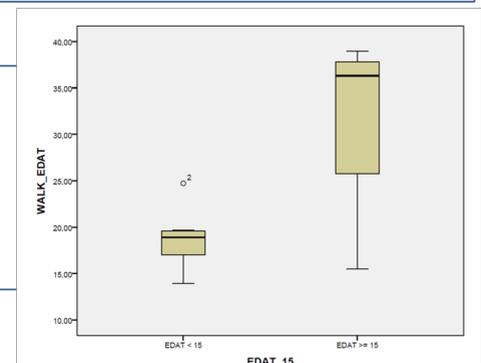
NO fatal adverse effects. One patient had a serious adverse effect not attributable to GH (published in Am J Case Rep 2017 Gastric Dilatat. Blat et al.)



We observed a significant ↓ in the subscapular and triceps folds ($p < 0.0001$), an upward trend in height and a ↓ in body mass index



They **started walking and speaking** at median age 19.47 and 16.93 months, respectively, (clearly lower in those who started GH <15 months and before what was classically described)



Conclusions:

- Growth hormone therapy is safe in PWS toddlers, improves their body composition and psychomotor development

Disclosure statement: nothing to declare