Prader Willi Syndrome and growth hormone therapy: valuable effects and adverse events.

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Introduction

Prader - Willi Syndrome (PWS) is a complex genetic psychosomatic disorder caused by deletion or maternal disomy within the long arm of chromosome 15.
Incidence of PWS in Polish population is **1:10 000 – 1:25 000** of live births, which equals 20 – 25 born children each year.
PWS is characterized by hypothalamic- pituary dysfunction, which lead to short statue, abnormal body composition (increased fat mass and reduced lean tissue mass), hypogonadism and even morbidly obesity.
The treatment of choice of PWS is the therapy with **recombinant human growth hormone (rhGH)** – since 2006 in Poland**.**

Aims

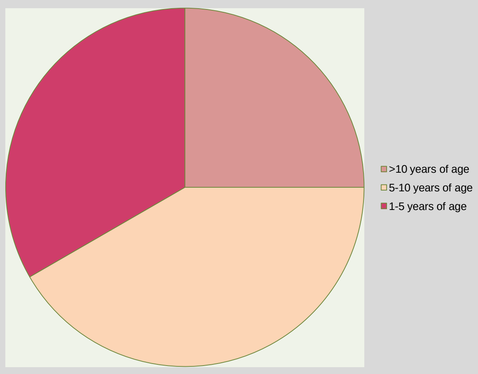
The objective of this study is to evaluate effects of rhGH treatment in children with Prader Willi Syndrome:

- to investigate the evolution of:
  - obesity on basis of BMI-SDS,
  - growth velocity,
  - weight;
- to estimate glucose metabolism;
- to assess complication of treatment.

Methods

Patients treated in Department of Paediatric and Adolescent Endocrinology in Polish American Institute of Paediatric in Cracow in 2013. The study was conducted on a basis of data from patients medical records. Mean dose od rhGH **– 0,62 IU/kg/week.** Treatment duration was **5.9±2.62 yrs.**

Patients' age during the study	
Median	13,4 (± 5,64)
Max	17
Min	5
Number of patients	
Boys	7 (58,3%)
Girls	5 (41,7%)
Together	12 (100%)

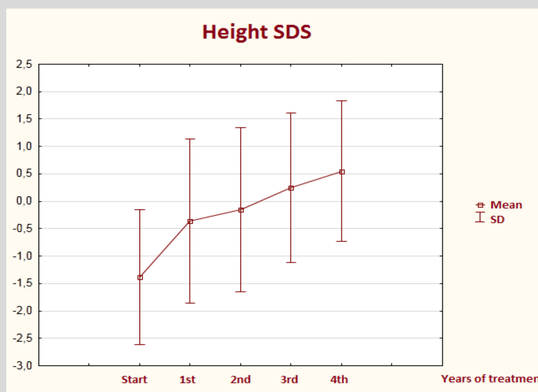


Discussion

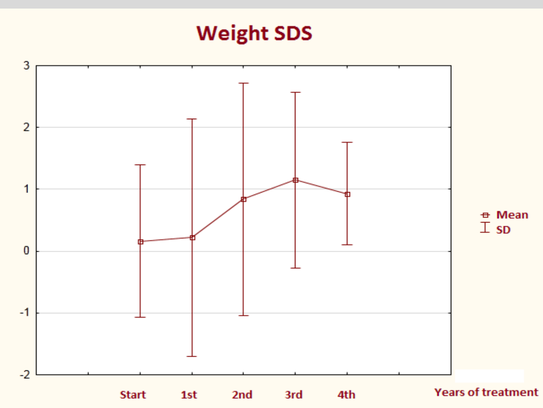
- Referring to the program's objectives of growth hormone treatment in Prader- Willi syndrome, 5 children achieved **normalization of growth** above the 10th percentile, and 6 increases in the appropriate channel or slightly higher MPH channel. In comparison, children not treated with GH reached height 155cm for males and 148cm for females.
- **Body weight** increase should be appropriate to the increase of height.
- Compared to population norms, **BMI** remained at a similar level during 4 years of treatment, but this is variable between individuals, because some children exhibit a very high levels of obesity.
- BMI is insensitive surrogate measure for changes in body composition, particularly if lean tissue mass increased in parallel with decreased fat mass.
- Increased IGF-I level can lead to lymphoid tissue growth in children, which increases the risk of **sleep apnea**. Therefore laryngological consultation should be performed before and during the therapy.
- Careful attention should be given to glucose metabolism. GH decreases insulin sensitivity which may lead to **type 2 diabetes** mellitus, particularly in obese patients or patients with positive family history.
- **Scoliosis** progression was observed in one pt, although according to the recent literature it is no longer considered to be an effect of rhGH therapy.

Results

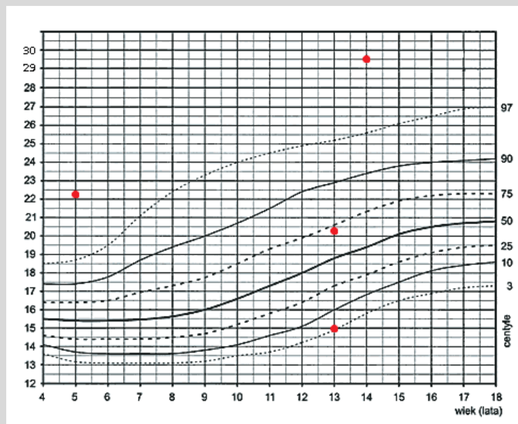
Increase in growth during rhGH treatment



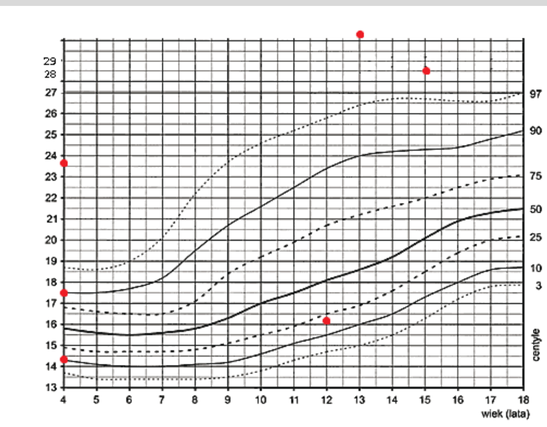
Changes in weight during rhGH treatment



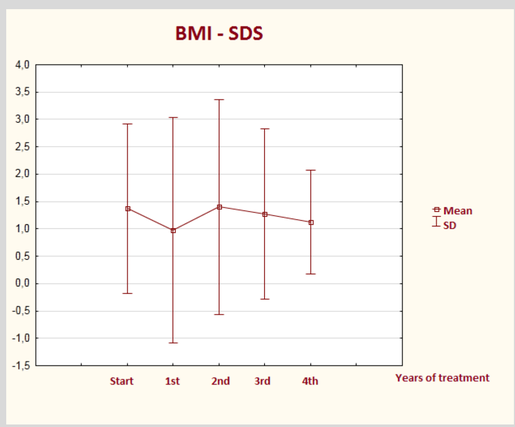
Girls' BMI at the time of study



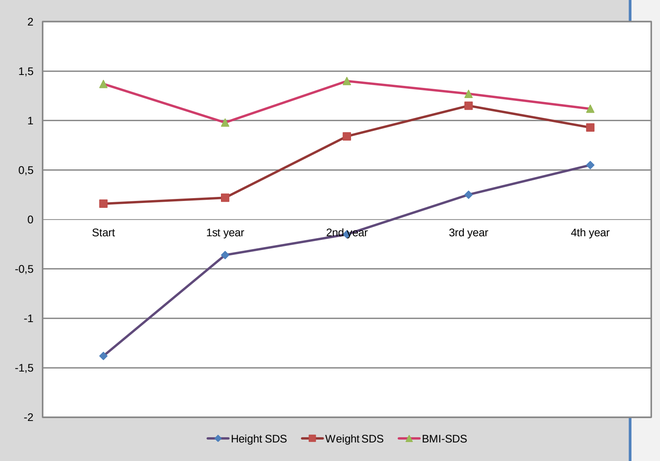
Boys' BMI at the time of study



Changes in BMI SDS during rhGH treatment

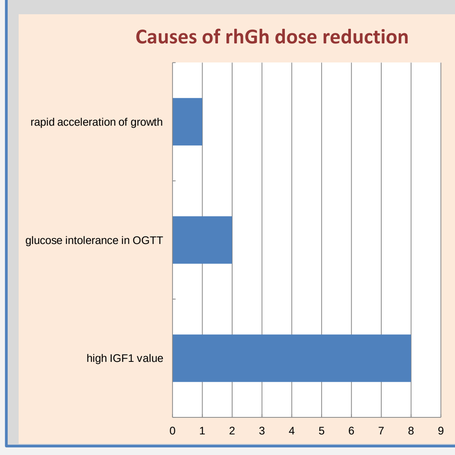


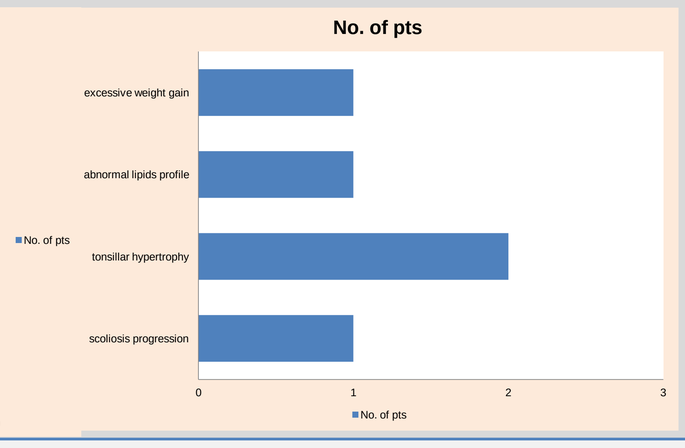
Comparison of growth rate, weight, BMI dynamism



Changes in dosage of rhGH

- 10/12 (83,3%) required rhGH dose reduction.
- 2/12 (16,7%) patients had their treatment interrupted, one boy because of excessive weight gain (36 kg over the time of 3 years) and one girl due to episodes of fainting.





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