



Prader-Willi Syndrome Association (USA) 29th Annual MEDICAL & SCIENTIFIC CONFERENCE

**Thursday, November 16th, 2017
Caribe Royale - Orlando FL.**

PWSA (USA) Abstract Submission Instructions

PWSA (USA) is requesting abstract presentations of recent research studies on the subject of Prader-Willi syndrome for our 29th Annual Medical & Scientific Conference.

To submit, complete a 1-page abstract to be submitted to the Scientific Conference Committee for review **by September 1, 2017**. Abstracts should be sent by e-mail to Ann Manzardo, Conference Coordinator amanzardo@kumc.edu with a copy to Dr. Merlin Butler, Conference Chair at mbutler4@kumc.edu no later than September 1, 2017. You will be notified of acceptance by Friday, September 22nd.

Please limit your abstract to one page in length, single spaced, with title and author information in the following sample format:

Sample:

Growth Hormone Effects in Adults with Prader-Willi Syndrome

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Introduction/Background: Prader-Willi syndrome (PWS) is characterized by short stature, reduced lean body mass and increased fat mass while several trials of growth hormone treatment in children with this disorder have demonstrated beneficial effects on body composition, stature and physical strength and endurance. There is a paucity of data on the use of growth hormone treatment in adults with PWS. Therefore, the main research aim of our study was to examine the effects of growth hormone therapy on body composition, physical activity and energy balance in adults with PWS and the impact of cessation of treatment at 12 months.

Methods: We screened 12 genetically confirmed adults with PWS and enrolled 11 adults (6F:5M; average age \pm SD = 32.3y \pm 11.1y; age range = 23y to 50y). The average weight, height and BMI for the 5 males were 98.9 kg, 154.2 cm and 41.6, respectively and 72.5 kg, 144.7 cm and 34.5 for the 6 females. Nine adults had the 15q11-q13 deletion, one showed maternal disomy 15 and one had an imprinting defect. All subjects had low IGF-1 levels and received daily injections of growth hormone (Genentech Nutropin AQ; 0.0125 mg/kg/day). Growth hormone was maintained within therapeutic range for at least 9 of the 12 months of treatment.

Fasting blood samples were obtained for general chemistry and for IGF-1, glucose, thyroid, insulin and lipid levels. Dual energy x-ray absorptiometry (DEXA) for body composition, voluntary physical activity measured by CSA accelerometers and strength using the one repetition of maximum method (1RM) with two exercises (bench and leg press) and energy expenditure measures by the use of a whole room respiration chamber for an 8 hour duration at each assessment over the two year period (at baseline, at 12 months after onset of growth hormone therapy and at 24 months after one year of cessation of growth hormone treatment).

The data were analyzed using linear mixed modeling to handle non-independence of the data where observations on the same participant were repeatedly collected. In the mixed models, total lean gram, total tissue percent fat, average counts of moderate-vigorous bouts per day, average length of moderate-vigorous bouts per day, IGF-1 and HDL were contrasted between the three measurement periods (baseline, 12 months, 24 months) adjusting for the participant's gender. All analyses were conducted using SAS 9.2 version.

Results/Discussion: In general, total lean gram, moderate-vigorous bouts measures, IGF-1 and HDL increased during the growth hormone treatment (baseline to 12 months) but after treatment offset (12 to 24 months), they decreased back to the baseline levels. In contrast, the participants reduced total tissue percent fat during the first 12 months during growth hormone treatment, but regained more than they lost during the following 12 months. All these changes were significant at 0.05 alpha levels, except for moderate-vigorous bouts changes from 12 to 24 months and HDL change from baseline to 12 months. There were no significant differences between time points for resting metabolic rate, 8 hour energy expenditure, or respiratory quotient (RQ).

Conclusions: The beneficial effects of growth hormone treatment noted in treating children with PWS were identified in our adults during the 12 months growth hormone treatment interval but body composition and physical activity measures and both HDL and IGF-1 levels regressed to baseline after treatment cessation for 12 months. The growth hormone treatment had no apparent effect on energy expenditure.

Prader-Willi Syndrome Association (USA)

PWSA (USA) has provided support for families, created educational materials for distribution and has funded research and encouraged collaboration among researchers, clinicians and families. PWSA (USA) has established Scientific and Clinical Advisory Boards consisting of key professionals, physicians and scientific investigators, many as pioneers in the field to address research, investigations and treatment including for growth hormone in this syndrome.

Registration for the Scientific Day Conference held on Thursday, November 5 is \$125 which includes a continental breakfast and lunch.

Following the Scientific Day Conference

Please consider registering for the **General Conference** on November 17th & 18th directly following the Medical & Scientific Conference. The theme of this year's General Conference is **"Together In Paradise"**.

At the General Conference, experts will present on a large variety of important topics related to PWS with interaction encouraged not only by professionals, but also by the hundreds of parents and people attending the meeting including those with the syndrome from newborn to adulthood.