The primary endpoint will evaluate annualized height velocity at 52 weeks of TransCon CNP or placebo. Key secondary endpoints will examine change in upper to lower body segment ratio at week 52, safety, pharmacokinetics (PK), and quality of life associated with weekly TransCon CNP.

Conclusion: CNP is targeted specifically to the underlying pathology of ACH through inhibition FGFR3. No effective medicinal product is currently available for the treatment of ACH. TransCon CNP is designed to provide sustained exposure of CNP. This ongoing Phase 2 ACcomplisH trial is designed to assess the efficacy, safety, and PK of TransCon CNP by weekly administration compared to placebo.

Pediatric Endocrinology
PEDIATRIC ENDOCRINOLOGY: ADRENAL, THYROID, AND GENETIC DISORDERS

Efficacy of Levothyroxine on the Frequency and Severity of Migraine in Children with Subclinical Hypothyroidism

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Introduction: Migraine is the most frequent primary intermittent headache in children. Hypothyroidism can exacerbate primary headaches and be a risk factor for persistent headache. Subclinical hypothyroidism is diagnosed when the level of TSH is high and the plasma concentration of free T4 is normal. **Objective:** To evaluate the efficacy of levothyroxine in the frequency and severity of migraine in children with subclinical hypothyroidism. Materials and **methods:**Prospective, observational and analytical study. Pediatric patients with a diagnosis of migraine and subclinical hypothyroidism (TSH >4.5 mU/L and normal concentration of free T4). Exclusion criteria: Hemiplegic migraine, basilar, retinal migraine, periodic childhood syndromes, epilepsy, asthma, diabetes mellitus, nephropathy, liver disease, previously diagnosed thyroid disease. Evaluation (for six months) of the frequency (monthly) and severity (severity score) of migraine in children with subclinical hypothyroidism treated with levothyroxine, during three months. **Results:** Patients attended: 31; excluded: 14; studied: 17 (9 girls). Age: 11.2 + - 1.89 years. The monthly frequency of migraine before treatment with levothyroxine was 14.75 + - 4.1 and after therapy, 5.39 + - 2.1 (p = 0.001); migraine severity score before treatment was 7.2 + - 1.2 and after therapy, 4.05 + - 1.31 (p = 0.001). Conclusions:Levothyroxine therapy in children with migraine and subclinical hypothyroidism was effective in decreasing the frequency and intensity of migraine in patients.

Pediatric Endocrinology
PEDIATRIC ENDOCRINOLOGY: ADRENAL, THYROID, AND GENETIC DISORDERS

Evaluation of Methods That Estimate Glomerular Filtration Rate in Patients With Prader-Willi Syndrome

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Background: Prader-Willi syndrome (PWS) is a rare, complex, multisystem disorder caused by the loss of multiple paternally expressed genes on chromosome 15q11-13 and is present in 1/15,000-30,000 individuals. Characteristics of PWS include low muscle mass and hypotonia, accumulation of excess body fat, short stature, hyperphagia, behavioral problems, cognitive disabilities, developmental delays, and hypogonadism. In these patients, serum creatinine (SCr)-based methods to calculate estimated glomerular filtration rate (eGFR) can lead to inaccurate results. eGFR is reported to be negatively correlated to muscle mass and PWS-associated low lean body mass may contribute to low SCr levels. Therefore, eGFR calculations may not accurately reflect PWS patient's renal function. A more accurate, non-invasive, inexpensive means to monitor renal function for this patient population is desirable. **Objective:** To assess methods of estimating renal function in pediatric PWS patients and summarize the relationship between eGFR and patient-specific factors. **Methods:** The pre-treatment data of patients ≥4 years old with genetically confirmed PWS participating in an investigational study of DCCR (diazoxide choline) were evaluated. Lean body mass was measured using dual X-ray absorptiometry. Lean body mass and age were correlated to eGFR/creatinine clearance (CrCl) values calculated using four different equations: Bedside Schwartz (BS), Chronic Kidney Disease Epidemiology Collaboration (CKD-EPI), Modification of Diet in Renal Disease (MDRD), and Cockcroft-Gault (C-G). **Results:** Of the 124 patients enrolled in the study, 99 were <18 years old and 103 were taking growth hormone (GH). Mean SCr was below normal range at 0.52 mg/dL, with only two subjects (1.6%) having SCr in the normal range (0.84 to 1.21 mg/dL). eGFR calculated using BS was 120±22 mL/min/1.73m^2; CKD-EPI 154±23 mL/min/1.73m^2; MDRD 211±77 mL/min/1.73m^2; and CrCl by C-G 191±80 mL/min. Among the three eGFR equations, CKD-EPI presented with the most reasonable eGFR values <200 mL/min/1.73m^2. When stratified by different age groups, SCr increased with age while eGFR decreased (and surprisingly CrCl by C-G increased). When correlating the eGFR values to various parameters, lean mass and age showed significant negative correlations with eGFR for BS, CKD-EPI, and MDRD. In contrast, for C-G there were significant positive correlations between CrCl and both lean mass and age. **Conclusion:** In PWS, the combination of low SCr and excess accumulation of body fat results in eGFR values that likely overestimate actual renal function in PWS patients. The inconsistent trends in correlation values between eGFR or CrCl by C-G and both lean mass and age indicate current SCr-based methods to estimate renal function in PWS may be inadequate. The use of CKD-EPI or other non-SCr-based methods to monitor renal function should be considered in PWS.