p=.005). CGM users also performed more frequent daily BG monitoring (5.2±1.9 vs 4.2±1.9, p=.0002) and were less likely to have HbA1c ≥9% after 18 months (27% vs 42%, p=.03). In summary, we found distinct socio-demographic and diabetes-specific factors associated with device use in adolescents with T1D. These findings provide an opportunity to address barriers associated with device non-use in order to expand device implementation, especially in underserved adolescents with T1D.

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Comparison of Quality of Life Responses From Caregiver and Children Aged ≥7 Years Using the Quality of Life in Short Stature Youth (QoLISSY) Questionnaire, Following 12 Months of Growth Hormone Treatment With Either a Weekly Somatrogon or a Daily Genotropin Injection Schedule

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Objective: Paediatric growth hormone deficiency (pGHD) affects 1/4,000 children. Treatment with daily sub-cutaneous injections of recombinant human growth hormone (r-hGH) increase height velocity and quality of life (QoL). A recent randomised controlled clinical trial (NCT02968004) evaluated the efficacy/safety of weekly Somatrogon (hGH-CTP) and daily Genotropin in pGHD. QoL (an exploratory endpoint) was evaluated using the validated Quality of Life in Short Stature Youth (QoLISSY) questionnaire, which includes three subscales (physical, social, emotional) and total score.
Methods: The QoLISSY core module was administered to patients (aged 3-11 years [girls], 3-12 years [boys]) and parents in US, UK, Australia, New Zealand, Belarus, Russia, Ukraine and Spain, at Baseline (BL) and 12 months after treatment start. The QoLISSY-CHILD was completed by children aged ≥7 years; QoLISSY-PARENT was completed by the Caregiver for children <7 years, and for some children aged ≥7 years. We report here only the QoLISSY results for children aged ≥7 years (reported from either child or parent).
Results: For Total QoLISSY-PARENT, for children aged ≥7 years in the Somatrogon group (N=26), mean scores are 53.65 (BL) and 65.52 (month 12) with mean change of 13.01 (95% Confidence Interval [CI]: 3.99, 22.02). In the Genotropin group (N=28), mean scores are 55.89 (BL) and 63.66 (month 12) with mean change of 6.60 (CI:-0.21, 13.40). For Total QoLISSY-CHILD in the Somatrogon group (N=35), mean scores are 61.48 (BL) and 74.69 (month 12) with mean change of 13.00 (CI: 5.81, 20.19). In the Genotropin group (N=35), these scores are 60.96 (BL) and 69.03 (Month 12) with mean change of 7.84 (CI: 2.71, 12.97). Scores of >70 indicate a good QoL.
Conclusions: QoL in children aged ≥7 years improved, following 12 months of either treatment, whether this was reported by caregiver or child. However, these data show that the baseline scores and 12 month scores from the QoLISSY-PARENT in both treatment groups were numerically lower than those reported by the child. This is consistent with the literature[1], in which the caregivers generally report lower QoL scores on behalf of the child.
[1]Explaining parent-child (dis)agreement in generic and short stature-specific health-related quality of life reports: do family and social relationships matter? Quitmann et al *Health and Quality of Life Outcomes* 2016 vol 14, Article 150

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Diagnosis of Childhood and Adolescent Growth Hormone Deficiency Using Transcriptomic Data

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Background: We have shown that gene expression (GE) data have promise as a novel tool to aid in the diagnosis of childhood growth hormone deficiency (GHD)[1]. Our previous study compared GE data in children with GHD to healthy control children of normal stature. The aim of this study was to assess the utility of GE data in the diagnosis of GHD in childhood and adolescence using non-GHD short stature children as a control group.
Methods: GE data were obtained from patients undergoing growth hormone stimulation testing via a sample of blood taken at the start of the test. Arginine and glucagon stimulation tests with a cut-off for peak GH of <7mcg/L (IDS iSYS assay) were used for the diagnosis of GHD. GE was assessed in peripheral blood mononuclear cells via RNA-seq using the Illumina HiSeq 4000 platform. Data were taken for the 271 genes whose expression was utilised in our previous study. The synthetic minority oversampling technique was used to balance the dataset and a random forest algorithm applied to predict GHD status. Boruta was used to assess which of the genes were contributing to the predictive capacity.
Results: Twenty-four patients were recruited to the study, with eight subsequently diagnosed with GHD. Of the eight patients diagnosed with GHD, three had two stimulation tests and five had one stimulation test with anterior pituitary hypoplasia (in addition one patient had an arachnoid cyst and another a thin stalk). Median (range) peak GH was 2.5 (0.1 - 5) mcg/L in the GHD group and 11.0 (7.4 - 31) mcg/L in the non-GHD group.
There were no significant differences in gender, age, auxology (height SDS, weight SDS, BMI SDS) or biochemistry (IGF-I or IGFBP-3 SDS) between the GHD and non-GHD subjects. 82 of the 271 genes used in our previous study were above the threshold of detection for RNA-seq in this study. A random forest algorithm using these 82 genes gave an AUC of 0.97 (95% CI 0.93 - 1.0) for the diagnosis of GHD. Boruta was able to identify 65/82 genes with predictive capacity greater than permuted data within the dataset. Using a gene ontology approach the top fifty

biological processes generated 16 clusters by affinity propagation which included regulation of TORC1 signalling and inositol phosphate metabolism.
Conclusion: This study demonstrates highly accurate diagnosis of childhood GHD using a combination of GE data and random forest analysis and validates the findings of our original study.
[1]Murray *etal* (2018) JCI Insight 3(7): e93247

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Economic Burden of Growth Hormone Deficiency in a US Pediatric Population

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Pediatric GHD is a rare disorder of short stature that is currently treated with daily injections of GH. In addition to short stature, GHD is associated with other comorbidities such as impaired musculoskeletal development, cardiovascular disease, and decreased quality of life. The objective of this study was to analyze GH utilization, adherence, and healthcare costs among children with GHD who had either Medicaid or commercial health insurance. Children (age <18 years) with a diagnosis of GHD between January 1, 2007, and December 31, 2017 were identified in the IBM Marketscan Commercial and Medicaid Databases. Children with GHD were direct matched (1:3) to controls without GHD (or other short stature-related disorder) on age, gender, plan type, region, and race (Medicaid only). The index date was set as the date of the first GHD diagnosis during the selection window for GHD patients and using random assignment for controls. Patients were followed for the 12 months prior to the index date until the end of continuous database enrollment or December 31, 2018. Baseline comorbidities and medications were measured during the 12 months pre-index. Treatment patterns and all-cause and GHD-related healthcare costs were measured during the variable follow-up period. Multivariable modeling was used to compare costs between GHD patients and controls and between GH treated and untreated GHD patients while adjusting for baseline characteristics. There were 6,820 Medicaid and 14,070 commercial patients with GHD who met the study inclusion criteria. Mean (SD) age at index was 9.5 (4.5) years for Medicaid patients and 11.1 (3.7) years for commercial patients. A majority of patients were male (>65%) and followed for at least 3.7 years. Overall, 63.2% of Medicaid and 68.4% of commercial patients were treated with GH during follow-up. Among Medicaid patients, the treatment rate was highest among white males and lowest among black females. Adherence, as measured by proportion of days covered, was low, with 18.4% of Medicaid patients and 32.3% of commercial patients considered "adherent" (PDC ≥ 0.8). Nearly half (49.1%) of treated Medicaid patients and 24.3% of commercial patients discontinued GH therapy before age 13. After adjusting for baseline characteristics, all-cause non-GH costs were 5.7times higher (Δ$19,309) for Medicaid patients and 5.5 times higher (Δ$12,305) for commercial patients than matched non-GHD controls. Adjusted all-cause non-GH costs were 0.6 times lower (Δ$14,416) for treated Medicaid patients and 0.7 times lower (Δ$7,650) for treated commercial patients than for untreated patients. Pediatric GHD presents a significant healthcare burden, and many patients remain untreated or undertreated. Untreated GHD was associated with higher non-GH healthcare costs than treated GHD. Strategies to improve adherence may reduce the healthcare burden faced by these patients.

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Effectiveness and Safety of Early Growth Hormone Treatment in Children Born Small for Gestational Age: Long-Term Data From NordiNet® International Outcome Study (IOS) and ANSWER Program

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Background: Growth hormone (GH) is indicated for the treatment of short stature in children born small for gestational age (SGA) who fail to show catch-up growth. In pilot studies, early initiation of GH has been associated with favorable growth responses in short SGA children. However, few studies have examined the short- and long-term effects of initiating GH in children born SGA who are <4 years (yr) of age, compared with those starting later. This analysis therefore investigated the effect of age at GH initiation on long-term effectiveness and safety in children born SGA.
Methods: The NordiNet IOS (NCT00960128) and ANSWER (NCT01009905) programs are complementary, non-interventional, multicenter studies that evaluated the long-term effectiveness and safety of Norditropin (somatropin; Novo Nordisk A/S, Denmark) as prescribed in real-life clinical practice. In this analysis, children born SGA who were prepubertal at GH initiation were grouped according to age at GH start: <4 yr, 4-6 yr, and ≥6 yr. Patient characteristics at birth and at GH start, auxological measurements, and adverse events were evaluated in each group.
Results: Overall, 3351 SGA patients were included in the effectiveness set (age at GH start: <4 yr [n=389, 54.8% male]; 4-6 yr [n=1048, 57.6% male]; and ≥6 yr [n=1914, 56.6% male]). The proportion of patients born pre-term (<37-week gestation) was 38.6% in the <4 yr group, 36.1% in the 4-6 yr group, and 28.2% in the ≥6 yr group. Mean (SD) birth length standard deviation score (SDS) and birth weight SDS were: −2.9 (1.6) and −2.3 (1.2) in the <4 yr group, −2.8 (1.3) and −2.2 (1.1) in the 4-6 yr group, and −2.5 (1.4) and −2.0 (1.2) in the ≥6 yr group. Mean (SD) age at GH start was 3.1 (0.7), 4.9 (0.6), and 9.3 (2.2) years in the <4 yr, 4-6 yr, and ≥6 yr groups, respectively. Mean (SD) GH duration and daily GH dose in the study were: 3.5 (3.1) yr and 0.044 (0.016) mg/kg in the <4 yr group, 4.1 (3.1) yr and