

PWS on medical claims provided by IQVIA™ Health Plan Claims Data and CMS Medicare fee-for-service claims. Patients were grouped into age bands including: 0-2, 3-8, 9-17, 18-26, 27-49, and ≥50. PWS prevalence and mortality rates were calculated for 2014, then 2018 US census data was used to project rates for the total US population. The presence of select diagnoses and procedures suggestive of a life-threatening event (e.g., mechanical ventilation) with a patient's prompt disenrollment defined as death in the IQVIA data; vital status is indicated in Medicare data.

Results: Overall US diagnosed PWS prevalence was 2.7 per 100k persons (or 1 per 37,037), a prevalence of 8,870 patients in the US in 2018. Diagnosed PWS prevalence 3.9, 5.2, 4.5, 4.2, 2.5, and 1.1 per 100k persons respectively for age bands 0-2, 3-8, 9-17, 18-26, 27-49, and ≥50. The median age of PWS patients was 21 years. The mortality rate was highest among diagnosed PWS patients aged 0-2 years and lowest among those aged 9-17 years and the overall mortality rate was 2.7%. For all respective age bands 0-2, 3-8, 9-17, 18-26, 27-49, and ≥50, the all-cause mortality rate was 5.4%, 3.0%, 1.4%, 2.1%, 2.4%, and 4.5%. The observed median age of death was 23 years (IQR 6-36)

Conclusions: The diagnosed PWS prevalence of 1 per 37,037 persons estimated for the 2018 US population is comparable to the other reported US prevalence estimate. As the current study describes diagnosed patients, it likely represents a lower bound of true PWS prevalence. Annual PWS mortality is ≥3 times higher than the overall US population (2.7% vs 0.8%). This rate appears unchanged from mortality estimates reported for PWS populations in the last several decades despite significant advances in genetic testing and the availability of growth hormone therapies in the US. Aggressive management of serious comorbid conditions, especially in younger PWS patients, should be a clinical priority.

Neuroendocrinology and Pituitary

PITUITARY TUMORS II

Impact of Tumor Characteristics and Preoperative IGF-1 Levels on Postoperative Hormonal Remission Following Endoscopic Transsphenoidal Surgery in Patients with Acromegaly: A Single-Surgeon Series

Tyler Cardinal, BS, Martin Rutkowski, MD, Tanu Patel, BA, Mark Shiroishi, MD, John David Carmichael, MD, Gabriel Zada, MD.

KECK SCHOOL OF MEDICINE OF USC, Los Angeles, CA, USA.

MON-319

Introduction:

Acromegaly is a potentially fatal neuro-endocrinopathy caused by a growth hormone (GH)-secreting pituitary adenoma (PA). A lack of consensus on factors that reliably predict patient outcomes in acromegalic patients following endoscopic endonasal approaches (EEA) warrants additional investigation.

Methods:

Pre- and postoperative tumor and endocrinological characteristics from 55 acromegalic patients who underwent EEA for resection of a GH-secreting PA were evaluated as potential predictors of postoperative hormonal remission (defined as age- and sex- normalized IGF-1 levels).

Results:

The 55 patients included had a mean age of 50.1 ± 13.5 years and a mean follow-up time of 18 ± 17.4 months. Fifty-three patients (96%) presented with dysmorphic craniofacial features, with 22 (40%) presenting with prognathism, 22 (40%) exhibiting frontal bossing, and 18 (33%) presenting with macroglossia. Ten (18%) had microadenomas and 45 (82%) had macroadenomas. Five (9.4%) had giant adenomas. Forty-five (92%) tumors were invasive, with 44 (83%) exhibiting infrasellar invasion, 17 (32%) extending above the sella, and 9 (18%) with cavernous sinus invasion. Thirty-three patients (66%) underwent gross total resection (GTR; mean maximal tumor diameter = 1.52 cm), and 17 (34%) underwent subtotal resection (STR; mean maximal tumor diameter = 2.77 cm). Invasive tumors were significantly larger and Knosp scores were negatively correlated with GTR. Thirty-three patients (65%) achieved hormonal remission after EEA resection alone, which increased to 80% with adjunctive medical therapy. Additionally, 90% of patients who underwent GTR and 63% of patients who underwent STR demonstrated postoperative remission. Six patients (11%) exhibited biochemical remission after postoperative medical therapy with an average time to remission of 5.2 months. These patients all had significantly higher preoperative IGF-1 levels and larger tumors than patients who remitted immediately postoperatively. In all patients preoperative IGF-1 levels were inversely correlated with hormonal remission.

Conclusions:

This study indicates that endoscopic transsphenoidal resection of growth hormone secreting pituitary adenomas is a safe and highly effective management strategy for achieving hormonal remission and tumor control for patients with acromegaly. When combined with postoperative medical therapy, we observed endocrinological remission rates of 80% based on normalized IGF-1 levels. Our results support the conclusions of current literature that smaller and less invasive tumors are more likely to be fully resected. We additionally suggest that patients with lower preoperative IGF-1 are more likely to undergo postoperative biochemical remission, irrespective of tumor size and invasion.

Diabetes Mellitus and Glucose Metabolism

IMPACTS OF METABOLISM ON CLINICAL CHALLENGES

Fecal Microbiota Transplantation Trial for the Improvement of Metabolism (FMT-TRIM): A Randomized Double-Blind Placebo-Controlled Pilot Trial

Elaine Wei-Yin Yu, MD, MMSc¹, Gao Liu, MD, PhD¹, Petr Stastka, BS¹, Michael Chadwick Cheney, NP¹, Jasmin Mahabamunuge, BA¹, Mariam Torres Soto, BA, MA¹, Christopher B. Ford, PhD², Jessica A. Bryant, PhD², Matthew R. Henn, PhD², Elizabeth L. Hohmann, MD¹.

¹Massachusetts General Hospital, Boston, MA, USA, ²Seres Therapeutics, Inc., Cambridge, MA, USA.

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Background: There is intense interest about the therapeutic potential of altering gut microbiota to improve