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Examination of Treatment Patterns in Patients with Classic Congenital Adrenal Hyperplasia (CAH) Compared to Treatment Guidelines

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Introduction: Classic congenital adrenal hyperplasia (CAH) is a rare autosomal recessive disorder, usually due to a deficiency in the 21-hydroxylase enzyme, that results in impaired cortisol synthesis and excess androgen production. Recommended guidelines for management of CAH include assessment of androgen control by routine clinical investigations and patient examination for treating both disease-related and drug-related symptoms. The objective of this study was to identify treatment patterns in patients with classic CAH with reference to guideline-directed care in the United States (US).

Methods: A retrospective analysis of real-world data on patients with classic CAH was carried out using claims data between Oct 2012 and July 2020 (8 years) from the EVERSANA open claims database of 290 million US citizens. The initial cohort included patients with two or more CAH-related ICD 9/ICD 10 codes at least 28 days apart, and two or more oral glucocorticoids (GCs) prescribed at least 28 days apart within one year of CAH diagnosis. The final CAH cohort combined unique patients identified from two sub-cohorts based on GC adherence (sensitivity 1: patients with 60% proportion of days covered and sensitivity 2: patients with five or more GC prescriptions from date of diagnosis to end of study period or available data for that patient). Information on demographic and clinical characteristics, comorbidities, prescribed medications (including corticosteroids), procedures, and referrals were analysed.

Results: The final cohort included 11,765 patients (overall mean age 28.7 years) of which 36.9% (n=4,338) were pediatric (0-17 years of age; mean age 8.4 years) and 63.1% were adults (n=7,427; mean age 40.5 years). The majority of CAH patients (62%; n=7,260) reported hydrocortisone use, with a higher proportion of pediatric patients on hydrocortisone (96%; n=4,158) compared to adults (42%; n=3,102). Other frequently prescribed corticosteroids were prednisone (30% pediatric; 68% adults), fludrocortisone (65% pediatric; 34% adults), dexamethasone (25% pediatric; 50% adults), and methylprednisolone (6% pediatric; 43% adults). Among diagnostic procedures and labs, total testosterone (47% pediatric; 46% adults), thyroid stimulating hormone (29% pediatric; 55% adults), 17-hydroxyprogesterone (62% pediatric; 33% adults), androstenedione (55% pediatric; 20% adults), glycated hemoglobin (18% pediatric; 41% adults) and free thyroxine (23% pediatric; 36% adults) were most common. Furthermore, bone age studies were reported in 19% of pediatric patients (51% of pediatric CAH patient population). Only 44% of adult CAH patients and 34% of pediatric CAH patients visited an endocrinologist or pediatric endocrinologist between Oct 2012 and July 2020.

Conclusions: In line with guidelines, most pediatric patients received short-acting hydrocortisone. However, a large proportion of patients with classic CAH were not treated by an endocrinologist and did not receive guideline-recommended therapy or laboratory testing or bone age assessments, putting them at risk for poor disease control and GC-related adverse events.

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