COST DRIVERS IN PATIENTS WITH CLASSIC CONGENITAL ADRENAL HYPERPLASIA (CAH)

Mallory Farrar¹, Conor O'Donoghue¹, Ed Paiewonsky¹, Chuck Yonan¹, Ramaa Nathan², Daniel Pfeffer², Vinayak Tiwari², Prasad Shripathi², Pierantonio Russo²

¹Neurocrine Biosciences, Inc., San Diego, CA, USA, ²EVERSANA Life Sciences Inc., Milwaukee, WI, USA

BACKGROUND AND OBJECTIVE

- Classic congenital adrenal hyperplasia (CAH) is a rare genetic disorder characterized by impaired cortisol synthesis and excess androgen production, typically requiring treatment with supraphysiologic doses of glucocorticoids.
- Comprehensive data on the cost of care and trends in rising medical expenses for classic CAH is scarce. This study was conducted to determine the financial burden of illness and to identify the primary cost drivers of care of classic CAH patients.

METHODS

Study Design

• Retrospective analysis of real-world administrative claims data from the IBM MarketScan Research Databases (MarketScan), comprised of deidentified data for over 200 million patients in the USA, between January 2006 and December 2020.

Patient Population

- Classic CAH cohort for analysis included patients with the following criteria:
 - Two or more of CAH related International Classification of Diseases [ICD] codes at least 28 days apart.
 ICD-9 255.2: Adrenogenital disorders ICD-10 E25.0: Congenital adrenogenital disorders associated with enzyme deficiency

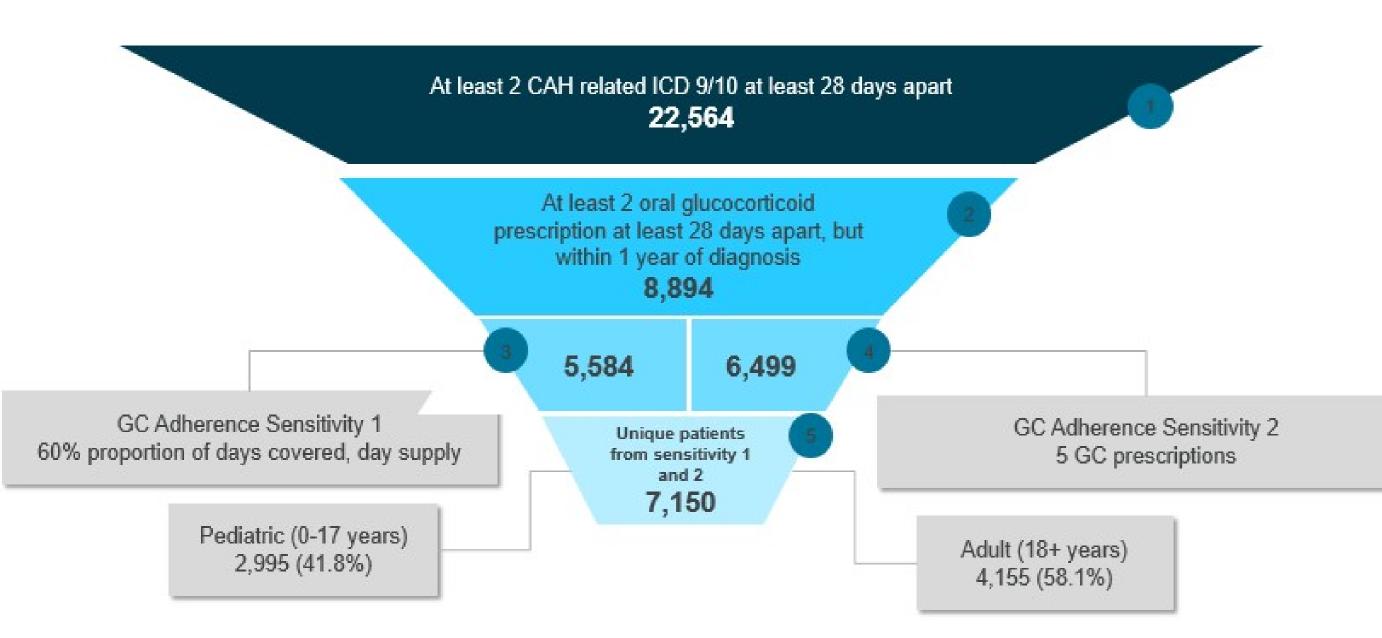
ICD-10 E25.9: Adrenogenital disorders, unspecified

- AND two or more oral glucocorticoids prescribed at least 28 days apart within one year of diagnosis and with either of the two glucocorticoid sensitivities (60% proportion of days covered or five glucocorticoid prescriptions) (Figure 1).
- Patients were stratified into low-cost, medium-cost, and high-cost groups based on medical expenses over a 4-year period (±2 years from index date).

Data Analysis

- The total cost of care was calculated, and the corresponding cost drivers were determined within different categories of demographics, facility type, diagnoses, prescribed medications, inpatient and outpatient procedures.
- Comparisons were carried out with a demographically matched control cohort.

Figure 1: Identification of classic CAH patients from closed claims database



RESULTS

N: Number of patients

Table 1: Classic CAH patient demographics 7

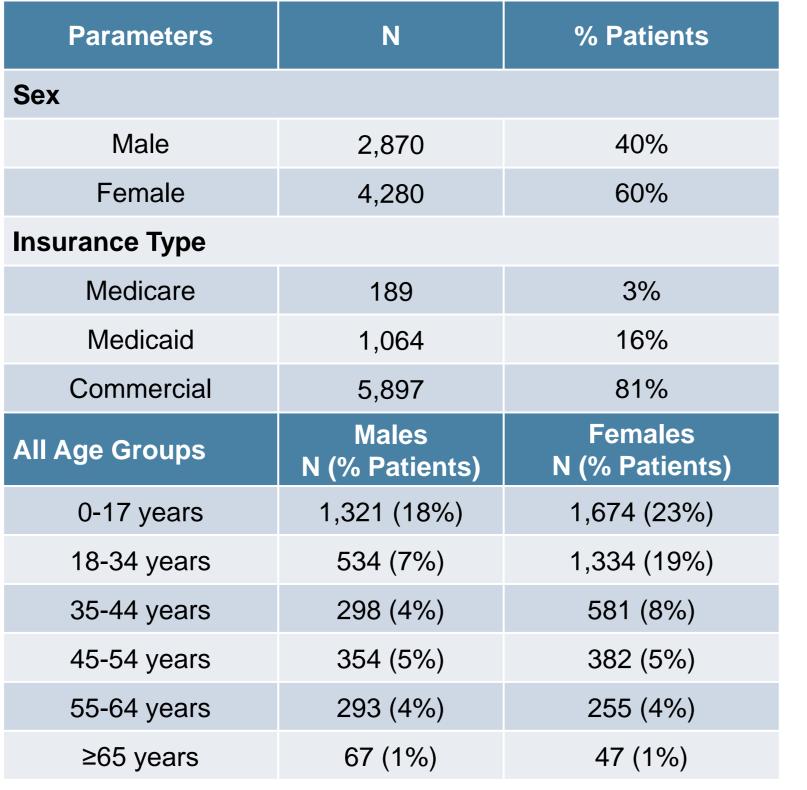


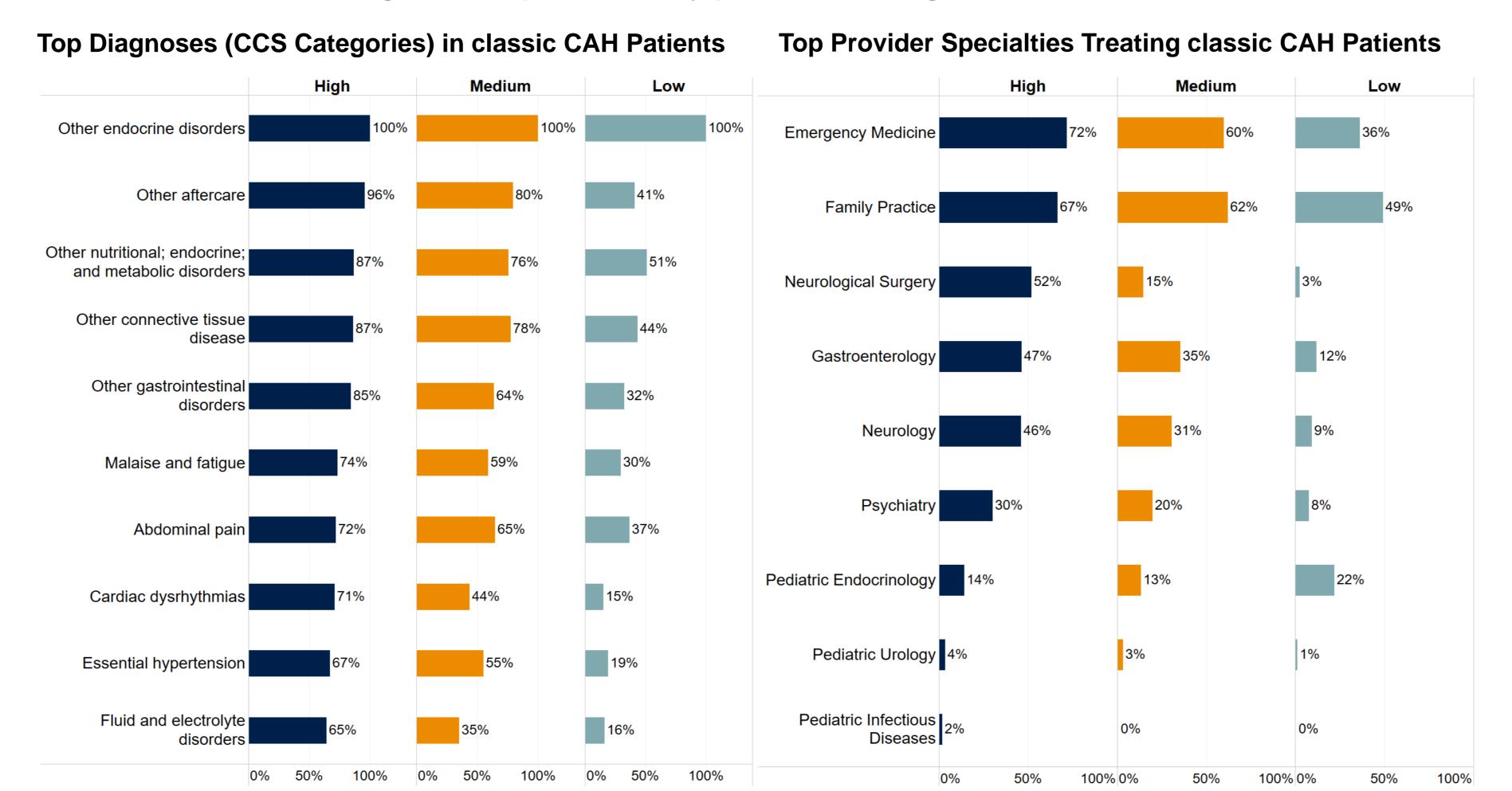
Table 2: Distribution of classic CAH patients by PMPY costs

Cost to Provider – Annual Cost Per Patient



PMPY: Per Member Per Year Cost; N: Number of patients

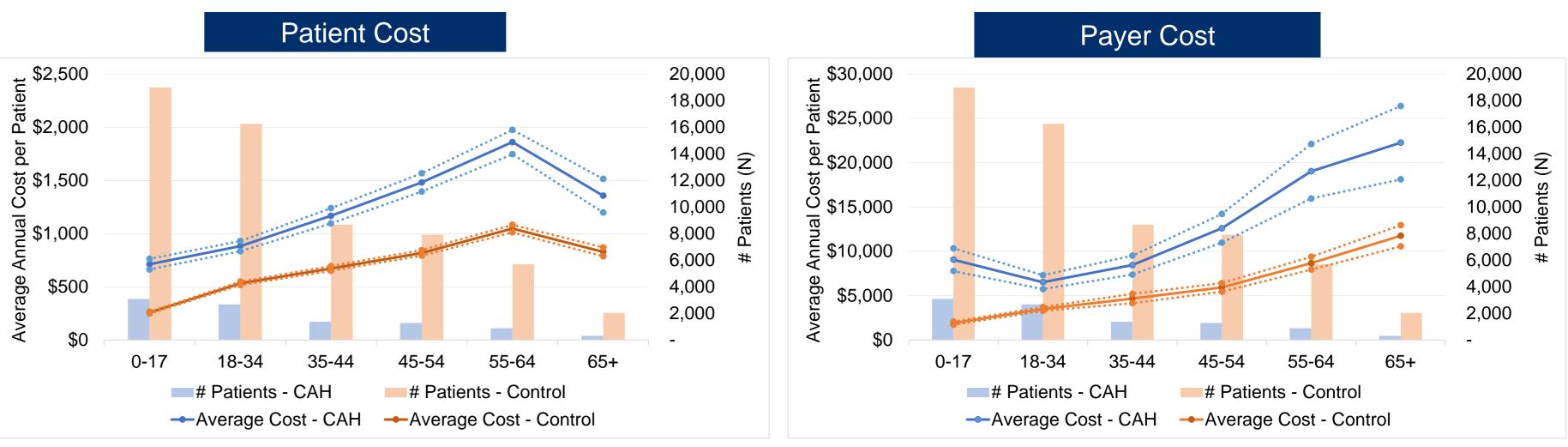
Figure 2: Top features by patient cost segments



CCS: Clinical Classifications Software

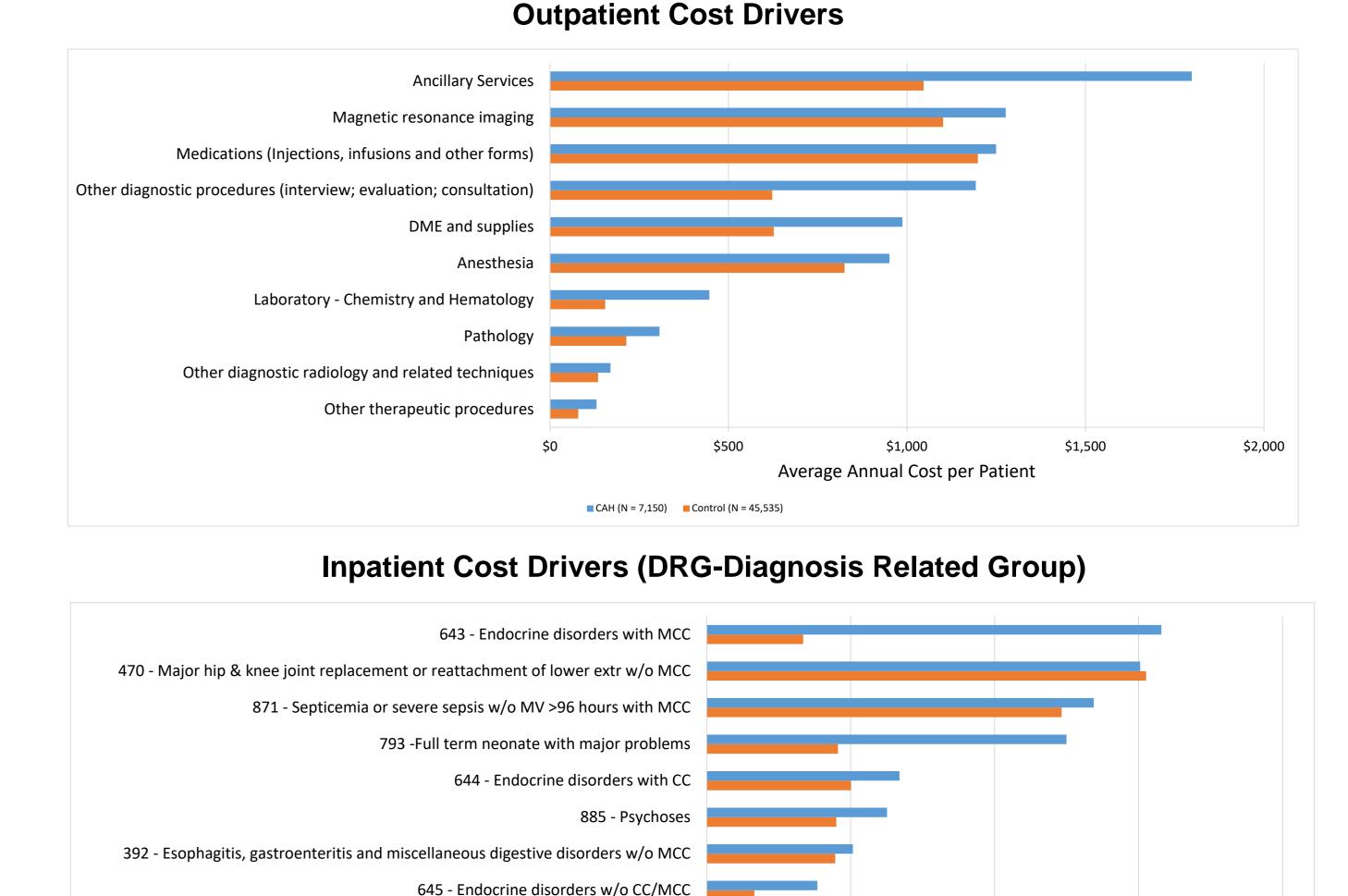
- The study included 7,150 patients with classic CAH (41.8% pediatric, 58.2% adults, 40% male) ages <1-90 years (Table 1).
- Visits to physicians of various specialties including emergency medicine, family practice, neurological surgery and gastroenterology were higher for high-cost patients compared to the medium-cost and low-cost patient segments (**Figure 2**).
- High-cost patients of all ages were more likely to visit the emergency department (94%, vs. 82% medium-cost and 55% low-cost patients). 89% of high-cost patients had inpatient stays compared to 67% medium-cost and 25% of low-cost patients.
- High-cost patients (Per Member Per Year [PMPY]=\$81,020; n=209) accounted for 2.9% of CAH patients, 12% were medium-cost patients (PMPY=\$22,012; n=855), and 85% were low-cost patients (PMPY=\$5,822; n=6,073) (**Table 2**).

Figure 3: Average annual cost per patient paid to provider by age group



Patient Cost: Average annual out of pocket costs paid by patient to provider; Payer Cost: Average annual costs paid by payer to provider; N: Number of patients

Figure 4: Average annual cost per patient paid to provider by outpatient and inpatient costs



MCC: Major complication or comorbidity; CC: Complication or Comorbidity; MV: Mechanical ventilation; DME: Durable medical equipment; N: Number of patients; w/o: without; Misc.: Miscellaneous

794 - Neonate with other significant problems

641 - Misc disorders of nutrition, metabolism, fluids and electrolytes w/o MCC

- The average annual cost per patient calculated across inpatient services, outpatient services and prescriptions was higher for classic CAH patients for all age groups, compared to the demographically matched control cohort (**Figure 3**).
- Ancillary services, magnetic resonance imaging (MRI), medications and other diagnostic
 procedures including interviews, evaluations and consultations were the high outpatient
 cost drivers in classic CAH patients (Figure 4).
- Endocrine disorders with MCC (major complication or comorbidity), hip and joint replacements, septicemia or severe sepsis were the high inpatient cost drivers (**Figure 4**).

CONCLUSIONS

- Economic burden of treating classic CAH patients was significantly higher than treating a demographically matched control population.
- ✓ Key cost drivers in the care of classic CAH patients include inpatient hospitalizations, ancillary services, and diagnostic procedures.
- Current and emerging therapies for classic CAH should be evaluated for their potential to impact these cost drivers.

