

## Let Real World Evidence and CTI Make Your Case

The CTI team of clinical and scientific experts develop evidence from real-world data around the value of products in terms of patientfocused, economic, clinical, and humanistic outcomes from normal clinical practice, outside of clinical trials. Understanding the results of using particular healthcare practices or products can inform key stakeholders, such as clinicians, patients, payers, hospitals, and other providers, about the benefits, risks, and costs of various treatment choices.

The International Society for Pharmacoeconomics and Outcomes Research (ISPOR) Real-World Data Task Force1 has defined six sources of real-world data, which are used to generate evidence through analytical techniques to answer specific research questions. These data sources are described as:

- 1. Supplements to traditional randomized controlled trials (RCTs)
- 2. Large, simple trials (practical clinical trials)
- 3. Patient/disease registries
- 4. Administrative/claims data
- 5. Health-related surveys
- Electronic health/medical records (EHRs/EMRs) and medical chart reviews, which are still often in paper form

#### Recent HECOR Case Study

#### **Background:**

Adrenal insufficiency (AI) is a rare, life-threatening endocrine disease characterized by insufficient production of corticosteroid hormones. In a study recently published in the Journal of the Endocrine Society.2 CTI researchers and others conducted a study designed to estimate the annual health care burden for patients with AI using real-world data.

#### **Methods:**

Using a US-based payer >10,000 patients database. were identified for the study, including >1,000 patients with primary AI (PAI), >8,800 with pituitary disorder-associated AI (PIT), and >500 with congenital adrenal hyperplasia (CAH). Patients were matched to controls, based on age, gender, insurance, and region. The researchers used multivariable regression models to estimate expenditures.

#### Key Results:

- Total annual health care expenditure estimates were significantly higher in all AI cohorts: PAI \$18,624 vs \$4320; PIT \$32,218 vs \$6956; CAH \$7677 vs \$4203.
- Patients with AI had more frequent inpatient hospital stays, and up to 8-10 times more hospital days annually.
- Patients on multiple steroid therapies had higher expenditures than patients using hydrocortisone alone. In PAI and PIT cohorts taking hydrocortisone

only, greater adherence to therapy was associated with lower expenditures.

- Conclusions:
- Patients with AI have a substantial annual health care burden. Expenditures vary dependingon the underlying cause and treatment and are reduced with higher corticosteroid adherence.

#### **Implications:**

This study provided insights for insurers and its sponsor, which has a product to treat AI, into the significant cost of healthcare for patients with AI, and the importance of treating patients effectively. Patients who were able to take their medication consistently as directed could have reduced costs.

1)Garrison LP Jr, Neumann PJ, Erickson P, Marshall D, Mullins CD. Using real-world data for coverage and payment decisions: the ISPOR Real-World Data Task Force report. Value Health 2007;10(5):326-35.

2)Gunnarsson C, Ryan M, Marelli C, Baker ER, Stewart PM, Johannsson G, Biller BMK. Health care burden in patients with adrenal insufficiency. J Endocr Soc 2017; 1(5): 512-523.

СТІ continuously provides clients with research our the on outcomes and health economics of products. procedures. and devices, to facilitate better understanding the provision of healthcare services to patients in the US and around the world.



# About the CTI HECOR Team

At CTI, the HECOR team regularly consults, designs, implements, and publishes research corresponding to the following key offerings:

- Small, medium, and large healthcare-associated database analyses
- Simple and comprehensive, systematic literature reviews and meta-analyses
- Medical chart and electronic medical record (EMR) review studies
- Registries and prospective observational studies
- Burden-of-Illness studies
- Budget impact studies and economic modeling
- Data visualization analytics
- Scientific publications (conference abstracts and posters, and journal manuscripts)
- Strategic research consulting

### Meet the Team



#### Candace Gunnarsson, Ed.D., M.A. Vice President, Health Economics and Outcomes Research Specialties: Real World Data Analytics and Product Value Strategy

William (Bill) Irish, Ph.D., M.Sc. Vice President-Biostatistics & Health Outcomes Research Specialties: Clinical Trial Design, Statistical and Economic Modeling





#### Frank Ernst, Pharm.D., M.S.

**Executive Director, Health Economics & Outcomes Research** Specialties: medical chart review studies, retrospective hospital and claims databases, registry data analyses

Tina Hunter, Ph.D., M.S. Senior Director, Biostatistics & Health Outcomes Research Specialties: Big Data and Real World Analytical & Statistical Methodology



Michael Minshall, M.P.H. Senior Director, Health Economics & Outcomes Research Specialties: Client/Payer Economic Strategy and Implementation

For more information about how our HECOR team can help you, visit www.ctifacts.com or contact us at info@ctifacts.com.