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PRO97
CLINICAL AND ECONOMIC BURDEN OF FOCAL SEGMENTAL GLOMERULOSCLEROSIS (FSGS) IN THE US:RESULTS FROM OPTUM CLAIMS

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Objectives: FSGS is a histopathologic syndrome with substantial unmet need and significant societal burden of illness. Given the limited data on clinical and economic characteristics of FSGS patients relative to individuals without FSGS, this retrospective observational study considers healthcare resource utilization (HRU) and costs in patients with focal segmental glomerulosclerosis (FSGS) compared to a matched non-FSGS cohort. ***Methods:*** Claims data from Optum's de-identified Clinformatics® Data Mart Database from October 2015 through June 2019 were used to identify a FSGS cohort and a cohort of patients without FSGS. Patients were matched 2:1 by age, sex and race. All FSGS patients had >= 6 months of continuous enrollment prior to and after their first FSGS medical claim (index date). Controls had the same data requirements around the index date as their matched FSGS patient. Individuals with any cancer diag-nosis were excluded. ***Results:*** Study included 768 FSGS patients and 1,536 matched non-FSGS controls. The FSGS cohort was comprised of more Medicare patients compared to the non-FSGS comparator (39.1% vs 33.1%, p=0.0045). FSGS patients had higher mean Charlson Comorbidity Index (CCI) (2.6 vs 0.5, p<.0001) and greater pro-portion with CCI >=3 (43.6% vs 6.0%, p<.0001). More FSGS patients had all-cause hospital admissions (21.2% vs 3.1%, p<.0001), surgeries (51.4% vs 17.1, p<.0001) and ER visits (24.6% vs 9.8%, p<.0001) in the six months after FSGS identification. FSGS patients also had higher mean outpatient visits (34.1 vs 11.2, p<.0001) and prescriptions (21.3 vs 7.7, p<.0001) than the non-FSGS patients. Median total costs for inpatient, outpatient and prescriptions during the 6 months post-index were $7,936 and $565 (p<0.0001) for FSGS patients and non-FSGS controls, respectively. ***Conclusions:*** This retrospective analysis of FSGS demonstrated increased healthcare resource utilization and costs among FSGS patients compared with those without FSGS. This remarkable societal burden warrants further study of this rare disease.

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PRO98
USING AI/ML TO IDENTIFY AND DIAGNOSIS PATIENTS WITH RARE DISEASES: A LANDSCAPE ASSESSMENT

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Objectives: Every year, HEOR researchers expand the depth and breadth of research available for the use of artificial intelligence (AI) and machine learning (ML). Our ability to examine terabytes of data is greatly expanding the way we can tackle previous intractable problems. One area of recent interest for the application of AI/ML is the ability to predict patients that may eventually be diagnosed with rare diseases as well as our ability to aid physicians with more accurate and/or earlier confirmation of a rare disease diagnosis. Both of these research tracts ultimately afford the medical community better tools to help hasten the delivery of treatments to these patients. ***Methods:*** Our landscape assessment evaluated two questions: what research is being conducted (A) to enhance identification of variables that may predict rare disease diagnosis in the future and (B) to evaluate uses of AI/ML based algorithms in medical practice to enable easier diagnosis of rare disease patients (hereinafter called identify and diagnosis tracts, respectively). Both research tracts used PubMed to identify research conducted in these areas over the past five years that was published in English. ***Results:*** Identify tract found 61 articles. Research was conducted primary in areas associated with CNS, oncological, and genetic disorders. In this identification research, genomic data are used most when predicting rare disease; computational imaging data is second-most common. Although many computation algorithms are represented in the research, the most common are Deep Learning, Neural Networks, and Random Forest. For diagnosis tract, there are far fewer studies. Beyond six studies reviewed by Brasil et al. (2019), two additional papers were found. The diseases, AI/ML methods, and research exhibited no fixed pattern. ***Conclusions:*** The breadth of research in these fields is far greater than the depth in any one facet. Eventually, we will need both to establish strong methods.

PRO99
EVALUATING THE HTA IMPACT OF REAL-WORLD EVIDENCE FROM FORMAL ORPHAN DRUG REGISTRIES

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Objectives: To perform a comparative assessment of the formal registry requirements in Germany and Brazil for orphan therapies with limited clinical evidence in order to pinpoint the future role of real-world evidence (RWE) in health technology assessments (HTAs). ***Methods:*** This study conducted a targeted policy and literature review of the orphan drug registry requirements of the G-BA (Federal Joint Committee) in Germany and CONITEC (National Committee for Health Technology Incorporation) in Brazil, two HTA bodies with differential priorities for market access decision-making. To understand the impact of the formally generated real-world evidence, CBPartners also prepared an analogue assessment of recent orphan therapies that went through these registry pathways. ***Results:*** In early 2019, the G-BA obtained the authority to implement registries for any orphan drug with a conditional approval; the collected data would then be used for a re-assessment of that drug's added benefit rating. To-date, no changes to the initial benefit assessment outcomes have occurred. That said, given the G-BA's direct role in designing these registries, all data produced are likely to be considered in the re-assessment. In Brazil, for recent orphan drug approvals, CONITEC has recommended the implementation of patient registries as part of conditional public funding. However, while the data collected will trigger a re-assessment after three years, no orphan drugs have been negatively impacted thus far. Because CONITEC has less rigorous requirements, there may be more heterogeneity of impact for the real-world evidence that is ultimately produced. ***Conclusions:*** RWE from formal registries is unlikely to have a significant positive impact on future HTA decisions but may contribute to worsened outcomes if efficacy or safety perform worse than expected. Manufacturers will need to be more cautious about producing RWE outside of formal registries, as HTA bodies with more experience may be highly selective on what data they accept.

PRO100
EVALUATION OF TREATMENT PATTERNS OF FABRY DISEASE UTILIZING MEDICAL CLAIMS ANALYSES OF A GERMAN SICKNESS FUND DATABASE

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Objectives: Fabry disease (FD) is a rare disease due to absent/deficient alpha-galactosidase which has a debilitating impact on affected untreated patients. Analyses were conducted to evaluate the treatment patterns of FD in Germany on a representative database covered by the German statutory health insurance system consisting of 5.5 million individuals during the study period from 2010 – 2017. ***Methods:*** FD confirmed cases had ICD-10-GM codes (E75.2) for sphingolipidosis and ATCC (Anatomic-Therapeutic-Chemical Classification) code for approved FD therapies. Analyses were conducted on ICD-10-GM codes, EBM-Codes (*Einheitlicher Bewertungsmaßstab*) for outpatient care, OPS (Operation and Procedure Classification system) codes for inpatient care, and sick leaves documented in the database for four quarters pre-and post-index date (i.e., quarter of first diagnosis). ***Results:*** Forty-six FD patients were treated with an approved therapy (agalsidase alfa, n=37; agalsidase beta, n=12; migalastat, n=8) yielding an estimated treated FD prevalence of 0.85 per 100,000 insured patients from 2010-2017. The cohort included 41% (n=19) females; mean age was 42 years (SD=15); mean follow-up was > 5 years (SD=3). Post-index the top three associated comorbidities were essential hypertension (44%), chronic kidney disease (37%), and back pain (33%). Most patients (52%) had at least one sick leave post-index, seven patients (15%) had at least one sick leave pre-index. Overall mean duration of sick leaves was 17 days (median=2) post- and 21 days (median=0) pre-index. Most patients received their first diagnosis in outpatient care (76%, n=35). Specialists consulted in outpatient care post-index were nephrologists (46%), internal medicine (44%) and ophthalmologists (28%). Thirty-three patients (71%) had ≥1 hospital stay post-index, and nine patients (20%) had ≥1 hospital stay pre-index. Overall mean hospital stays pre- and post-index was 0.6 (SD=1.4) and 1.7 (SD=1.7), respectively. ***Conclusions:*** The study quantified the burden of FD on patient productivity and healthcare utilization from insurance claims analysis in Germany.

PRO101
CHARACTERIZING RARE AND OTHER UNCLASSIFIED DISEASES LACKING ICD DIAGNOSIS CODES: AN EMR APPLICATION IN HEREDITARY ANGIOEDEMA (HAE)

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Objectives: To characterize patients in a US EMR database with provider-specified diagnosis of HAE, a rare genetic condition of the immune system not easily identified in other data sources due to non-specific ICD-9/10-CM claims codes. ***Methods:*** From the IQVIA US Ambulatory EMR database of 75 million patients (2006 forward), cases were identified as having HAE if they had any record between 1/1/2006-10/31/2019 with provider-specified diagnosis of HAE based on SNOMED-CT (Systematize Nomenclature of Medicine, Clinical Terms) codes or a problem description of "%HERED%ANGIO%EDEMA%. Patient demographics and clinical profiles were summarized for those who had at least 2 visits for provider-specified HAE diagnosis. ***Results:*** 1,453 patients had a provider-specified diagnosis of HAE in EMR for a final cohort of 759 HAE patients with ≥2 HAE visits. Mean database observation period 1.9±1.7 years, 72.1% women, mean age 45.2±19.5 years at first diagnosis and 68.0% Caucasian. Documented family history of HAE for 9.9% patients (14.6% when looking for any family history of angioedema). From co-morbid conditions assessed (ICD-9/10-CM definitions): 46.6% allergy/immune, 36.5% hypertension, 31.0% GERD, 25.2% anxiety, 19.8% depression, 19.2% asthma, 15.8% osteoarthritis, 13.2% insomnia and 10.9% migraine. HAE prescriptions were identified in 330 patients (43.5%), of which: 50.6% icatibant acetate, 45.8% danazol and 39.7% C1 esterase inhibitor (human). Mean number of monthly visits (any reason) during the 12 months pre-index were 1.1±1.7 visits/month vs mean of 1.3±1.5 visits/month during the 12 months post-index. ***Conclusions:*** An HAE patient cohort was successfully identified in the EMR data based on provider-specified diagnosis. The patient and clinical profile appeared comparable to earlier reports for this patient population. To our knowledge this represents the largest HAE cohort from US data that involved a provider-specified diagnosis HAE while not requiring a definition based on non-specific ICD-9/10-CM diagnosis codes or other case-finding logic/algorithms.

PRO102
CHARACTERISTICS AND OUTCOMES OF PEDIATRIC HEMOPHILIA-A PATIENTS IN THE INPATIENT SETTING

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