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better overall survival. Conclusions: This study provided external comparators for efficacy evidence from single-arm tumor-agnostic trials. Pembrolizumab was associated with significant prolonged PFS but not OS versus chemotherapy in patients with metastatic CRC or EC.

RWD20

ANALYSIS OF US REAL WORLD EVIDENCE DATA TO ASSESS AND COMPARE THE EFFECTIVENESS OF TWO CHEMOTHERAPY REGIMENS: FOLFIRINOX VS GEMCITABINE/NAB-PACLITAXEL FOR FIRST LINE METASTATIC PANCREATIC CANCER

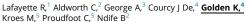


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Objectives: To demonstrate the validity of our analysis techniques utilizing realworld data to assess and compare the effectiveness of two chemotherapy regimens that have not been directly compared in randomized clinical trials: FOLFIRINOX (combined leucovorin calcium, fluorouracil, irinotecan and oxaliplatin) and gemcitabine plus nab-paclitaxel for first line metastatic pancreatic cancer. Methods: Our proprietary detection algorithm identifies attributes of chemotherapy regimens in a large population of individual patients in US medical claims and pharmacy prescription fill data. Key attributes examined: chemotherapeutic agents, duration, timing, and associations to preceding and following treatments. With a cohort of 17k pancreatic cancer patients treated either with FOLFIRINOX or gemcitabine/nabpaclitaxel as first line therapy in the last 5 years, we identified a subset of 2439 metastatic patients aged 50+ at the time of the first line starting date, who completed first line treatment prior to December 31st, 2021. Using medical claims made through June 30th, 2022, a death surrogate event was estimated as last medical event of any type prior to the observation end date. Overall Survival (OS) analysis was made using the Kaplan-Meier method. The cohort was split by chemotherapy regimen. Results: The analysis demonstrates survival benefit of FOLFIRNOX over gemcitabine/ nab-paclitaxel: FOLFIRINOX 12.5 months vs gemcitabine plus nab-paclitaxel 10.25 months. Comparable outcomes can be found in publications based on the results of randomized clinical trials, separately for each chemotherapy regimen, and retrospective analyzes. Conclusions: Survival benefit was shown in patients with metastatic pancreatic cancer treated with FOLFIRNOX over gemcitabine/nab-paclitaxel. Our proprietary detection algorithm identifies attributes of chemotherapy regimens in claims data and can be used to provide insights into oncology treatment regimens in large populations of oncology patients, which is especially important when comparative analyzes of clinical effectiveness are limited.

RWD21

IMMUNOGLOBULIN A NEPHROPATHY PATIENT REPORTED HEALTH UTILITY AND QUALITY OF LIFE: **EVIDENCE FROM REAL-WORLD DATA**



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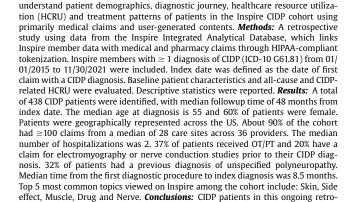
Objectives: Immunoglobulin A nephropathy (IgAN) is a rare kidney disease with an annual incidence of $\sim 25/1000000$ worldwide. Approximately 50% of IgAN patients with proteinuria ≥1 g/day progress to kidney failure within 15 years. There are limited published health related quality of life (HRQoL) data from IgAN patients in clinical practice to date, here we report results from a multi-country study and describe patient-reported health utility and QoL split by proteinuria and estimated glomerular filtration rate (eGFR). Methods: The Adelphi IgAN Disease Specific Programme was a point-in-time survey of IgAN-treating nephrologists and their patients in France, Germany, Italy, Spain, the UK (EU5), the US, China, and Japan, between June-October 2021. Physicians reported patient demographics and clinical characteristics. Patients completed: EQ-5D-5L (1 = perfect health - 0 = death, US tariff), EQ-VAS (0 worst to 100 best imaginable health) and Kidney Disease QoL (KDQOL; higher scores = better QoL). Patients who answered all of the above were split by proteinuria (P1<1g/day≤P2) and eGFR (G1≥45mL/min/1.73m²>G2), analyses were descriptive. Results: Overall, 883 patients had matched physician reported proteinuria (536 (P1), 307 (P2)) and 894 eGFR (714 (G1), 136 (G2)) at survey. Mean patient age was 42 years, 56% of patients with matched proteinuria and 57% with matched eGFR were male respectively. Overall mean EQ-5D-5L scores at survey were 0.87 (P1) vs 0.79 (P2), and 0.86 (G1) vs 0.71 (G2). Overall mean EQ-VAS scores were 75.5 (P1) vs 67.5 (P2) and 73.8 (G1) vs 60.0 (G2). Overall mean KDQOL burden of kidney disease scores were 60.1 (P1) vs 46.7 (P2) and 56.9 (G1) vs 39.4 (G2). Conclusions: EQ-5D-5L, EQ-VAS and KDQoL results suggest worsening HRQoL and health utility with increasing IgAN severity (higher proteinuria/ lower eGFR) highlighting an unmet burden of disease.



A NOVEL PATIENT-CENTERED REAL-WORLD EVIDENCE STUDY DESIGNED TO BETTER UNDERSTAND CHRONIC INFLAMMATORY DEMYELINATING POLYNEUROPATHY USING LONGITUDINAL DATA IN THE UNITED STATES



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spective analysis had a median of 4+ years of healthcare visit data since diagnosis.

Majority of patients had 100+ claims, which indicate substantial healthcare

resource utilization. CIDP patients endure a long diagnostic journey to undergo

Objectives: Chronic inflammatory demyelinating polyneuropathy (CIDP) is a rare

autoimmune inflammatory disorder of the peripheral nervous system. Evidence is

limited with the real-world patient population. The aim of the study is to better

RWD23

appropriate treatment.

BARRIERS TO PERSISTING ON PROPHYLACTIC TREATMENT FOR C1-ESTERASE INHIBITOR DEFICIENT HEREDITARY ANGIOEDEMA; OBSERVATIONS FROM **COMMUNITY IMMUNOLOGY**



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Objectives: Prophylactic therapies are an important tool in HAE management though persistency on therapy may be suboptimal. To determine reasons for discontinuation, we reviewed office visit notes and EMR data generated from community care. Methods: Data: PIONEER-HAE, a database containing EMR, continuity of care documents, and extracted visit note data, specific to patients managed by the Consortium of Independent Immunology Clinics (CIIC). Unstructured data were extracted into electronic forms by clinically-trained scribes and included attack details, on demand treatment, symptoms onset and diagnosis, family history, comorbidities, prophylactic treatment initiation, discontinuation, and reasons for discontinuation. Form data were subjected to logical checks, random audit (1-2%), and tertiary data review after combining with CCD and EMR data. Patient selection: Type I or II HAE treated with FDA-approved prophylactic drugs between 2018-2022. Drug episodes, defined as prophylactic drug-specific treatment without drug-free periods of >90 days, occurring between 2018-2022 were examined. Results: Study population characteristics (n=183): 63% (116) female, mean (median) age 43 (43) years with 15% (27) \geq 65 and 5% (9) <18 years old, predominantly white (87%, 116/ 134), commercial insurance coverage (73%, 134), and type I HAE (90%, 164). Drug episodes (n=292): 14% (40) androgens, 2% (5) anti-fibrinolytics, 39% (114) C1-esterase inhibitors, 46% (133) kallikrein inhibitors. As of Dec 2022, 174 drug episodes were active. For the remaining 118 discontinued episodes, 91 had documented discontinuation reasons: 26% (24/91) lack/loss of efficacy, 23% (21/91) comorbids or observed/anticipated adverse events (e.g. weight gain, concern over liver damage), 23% (21/91) patient preferences (e.g. anticipated pregnancy, prefer different drug), and 16% (15/91) payer/payment issues (e.g. denied, can't afford). Conclusions: In this study of prophylactic HAE treatments, 74% of evaluable episodes were discontinued for reasons other than lack of efficacy. These data serve as an important first step towards 1) awareness of real-world factors impacting persistency and 2) optimized disease management.

RWD24 **VENO-OCCLUSIVE DISEASE (VOD) INCIDENCE AND** TREATMENT TRENDS INSIGHTS FROM LATEST SYSTEMATIC REVIEW OF CASE REPORTS (2018-2023)



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Objectives: For ultra rare diseases independent case reports provide useful realworld evidence on disease burden and treatment trends. The objective of this review was to understand incidence and treatment trends for Veno-Occlusive Disease (VOD) using latest published case reports. Methods: PubMed, Medline and NOVEL-VOD databases were searched for English language publications dated January 1, 2018 to December 31, 2022. Case reports were selected for inclusion if publications reported incidence or treatment for VOD (also called SOS-Sinusoidal Obstruction Syndrome). Selected publications were tagged as epidemiology, treatment trends, defibortide treated, pulmonary or hepatic VOD. Each category of case reports were reviewed to

