Poster Tour Guide Packet

ISPOR 2022

Poster Session:	**In-Person and Virtual Poster Session 4**
Tour Name:	**Rare and Orphan Diseases**
Tour Time:	Tuesday, May 17, 2022, 5:30 - 6:30 PM
Tour Area:	Area A, Prince George Exhibit Hall

Acceptance Code:	EE15
Abstract Title:	Economic Burden of Transfusion-Dependent Beta-Thalassemia in the United States
Presenting Author:	Chukwukadibia Udeze

Abstract Body:

OBJECTIVES: Transfusion-dependent beta-thalassemia (TDT) is a genetic blood disorder whose treatment is characterized by lifelong red blood cell transfusions (RBCTs) and iron chelation therapy (ICT). This study aims to describe the economic burden of patients with TDT in the United States (US).

METHODS: This retrospective cohort study used administrative claims from US MarketScan Commercial, Medicare, and Medicaid Multi-state databases to identify patients with >1 inpatient or >2 outpatient claims for beta-thalassemia or Hemoglobin E/beta-thalassemia between March 1, 2010 – March 1, 2019. Eligible patients with TDT were required to have ≥8 RBCTs in any 12-month period on or after the date of the earliest qualifying beta-thalassemia claim (first RBCT date = index date) and at least 12 months of post-index continuous enrollment with medical and pharmacy benefits. At least 3 days between service dates of RBCT claims were required to be considered discrete RBCTs. Patients were followed from index date to insurance disenrollment, death, or end of study period (March 1, 2020), whichever came first. Patients with hematopoietic stem cell transplant or sickle cell disease claims were excluded from this analysis. Baseline demographics, RBCT frequency, healthcare resource utilization, and costs were summarized per patient per year (PPPY).

RESULTS: A total of 4,504 patients with a diagnosis of beta-thalassemia were identified, of which 198 met criteria for TDT and other inclusion/exclusion criteria. Mean patient age at index date was 22.6 years and 56.6% were female; 81.8% of patients were in the Commercial database, 16.7% in Medicaid, and 1.5% in Medicare. Patients received a mean 12.52 RBCTs annually. Mean total annual healthcare costs were $137,987 PPPY. Mean annual costs of RBCTs were $27,887 and ICT were $70,402 PPPY.

CONCLUSION: There is a significant economic burden with care of TDT patients in the US driven by chronic RBCTs and ICTs.

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Acceptance Code:	EE48
Abstract Title:	Cost-Effectiveness of Alternative Diagnostic Testing Pathways with Whole Exome Sequencing (WES) in a Rare Disease Patient Population: The Canadian Care-for-Rare SOLVE (SOLVE) Multi-Centre Observational Cohort
Presenting Author:	Koen Degeling

Abstract Body:

Background: Patients suspected of having a rare genetic disease often experience lengthy and costly diagnostic testing pathways. Our objective was to determine the time-to-diagnosis, associated testing costs, and cost-effectiveness for patients with rare disease of suspected genetic etiology who received WES testing at alternative places in the diagnostic pathway.

Methods: We designed a discrete event simulation model of the diagnostic pathway, with the report of the first test result as starting point. We defined and populated the simulation based on data from the electronic medical records for 169 patients from our C4R-SOLVE cohort. Five alternative pathways were modelled: noWES, and WES as the 1st, 2nd, 3rd, or 4th test (Tiers 1-4, respectively), where WES was the last test performed. Outcomes included: 1) achieving a diagnostic result as the primary effectiveness measure modelled using logistic regression; 2) time-to-diagnosis modelled using a standard and mixture parametric time-to-event distributions; and 3) patient-level total test costs modelled using empirical distributions. We applied discounting at 1.5% and quantified uncertainty using probabilistic analyses and expert-defined scenario analyses.

Results: Compared to molecular and specialized diagnostic tests (noWES), WES increased diagnostic yield from 20% to 42%. WES decreased time-to-diagnosis by 1.9 and -0.1 years, (Tier1 and Tier2, respectively, and increased the time by 0.7 and 1.5 years (Tier3 and Tier4, respectively). Test costs per pathway were CDN$3,289, CDN$2,683, CDN$3,404, CDN$4,512, and CDN$5,298 for No WES, and Tiers1-4, respectively. The incremental cost per additional diagnosis (95% Confidence Interval) was CDN$-2,762 ($-6,305;305), CDN$491 ($-2,395;$3,489), CDN$5,620 ($2,666;$10,052) and CDN$9,321 ($6,579;$14,634) for Tiers 1-4, respectively. The scenario analyses yielded similar results.

Conclusions: WES in the diagnostic pathway for patients suspected of having a rare disease can increase the diagnostic yield and reduce the time-to-diagnosis and test costs with the benefits being greater the earlier in the pathway that WES is implemented.

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Acceptance Code:	HSD85
Abstract Title:	Real-World Usage Patterns and Costs of IVIG Treatment in Adults with Generalized Myasthenia Gravis in the United States
Presenting Author:	Cynthia Qi

Abstract Body:

OBJECTIVES: Intravenous immunoglobulin (IVIg) is used in the treatment of patients with generalized myasthenia gravis (gMG), a rare autoimmune disorder. The objective of this study was to assess real-world IVIg usage patterns and its implications on costs over 1 year post-initiation in adults with gMG in the United States.

METHODS: Adults (≥18 years) with gMG who initiated IVIg were identified from Symphony Health's Integrated Dataverse (IDV)®, January 1, 2014 – December 31, 2019, unprojected de-identified patient prescription and medical claims, January 2020 dataset. IVIg courses were defined as ≥1 IVIg claims filed consecutively with ≤5 days between claims. Patients who received ≥6 IVIg courses over 1 year post-initiation were defined as chronic users (CU), while those who received ≤5 were defined as intermittent users (IU). Mean all-cause medical costs included medical service and pharmacy costs, and were assessed over 1 year post-IVIg initiation.

RESULTS: Among 1627 patients with gMG who initiated IVIg during the study period, 928 (57.0%) were IU and 699 (43.0%) were CU during the first year. 41.9% (389/928) of IU and 46.6% (326/699) of CU had at least one exacerbation or crisis event (E/C) during the year preceding IVIg initiation. Mean annual medical costs per patient for CU were 2.2- to 2.9-fold greater compared with IU (Had prior E/C: IU $73,970, CU $164,223; No prior E/C: IU $53,766, CU $156,356). Although 43.9% of patients initiated IVIg after at least 1 E/C during the preceding year, 53.6% (IU, 491; CU, 381) still experienced at least 1 E/C during the year post-IVIg initiation.

CONCLUSION: CU comprised 43.0% of IVIg initiators, and costs incurred for CU were significantly greater than that for IU. Regardless of the frequency of IVIg treatment, myasthenic exacerbations were still common.

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Acceptance Code:	EE500
Abstract Title:	Comparison of Healthcare Resource Utilization and Costs in Patients with and without Bullous Pemphigoid: A Retrospective Analysis of US Health Insurance Claims Data
Presenting Author:	Xiao Xu

Abstract Body:

OBJECTIVES: To evaluate the impact of bullous pemphigoid (BP) on healthcare resource utilization (HCRU) and costs in a retrospective analysis of 2015–2019 US administrative health insurance claims data (MarketScan).

METHODS: Patients were included in the BP cohort if they had ≥6 months of continuous health plan enrollment before the first observed BP diagnosis (index date) and ≥1 pharmacy claim for BP therapy within 30 days of index date. The BP cohort was matched to non-BP patients (non-BP cohort) based on demographic and clinical characteristics. Follow-up ran from the index date to either health plan disenrollment or end of study database. All-cause per-patient per month (PPPM) and annual HCRU and associated costs were assessed overall and by care setting.

RESULTS: The BP and non-BP cohorts comprised 1,108 and 4,621 patients, respectively. At baseline, patients (~50% female; mean age: BP 73.6, non-BP 71.1) had similar Charlson Comorbidity Index (CCI) scores (BP 3.25 vs. non-BP 2.80). During follow-up, the BP cohort had a higher CCI score (mean: 4.97 vs. 3.25), and higher PPPM HCRU, resulting in higher mean total PPPM medical encounter costs ($2,801 vs. $2,384) versus baseline. The non-BP cohort had similar PPPM HCRU and associated costs at follow-up versus baseline. Including medication costs, the mean total PPPM cost decreased by $213 during follow-up for the non-BP cohort (baseline $1,566, follow-up $1,353) but increased by $358 for the BP cohort (baseline $2,856, follow-up $3,214). The BP cohort had higher annualized PPPM costs both at baseline (Δ$15,480) and follow-up (Δ$22,332) than the non-BP cohort.

CONCLUSIONS: BP patients had higher disease burden in terms of comorbidities, HCRU, and associated costs than non-BP patients of a similar age. Particularly after diagnosis, BP patients were hospitalized more frequently, possibly due to treatment-associated comorbidities and outcomes. More effective and targeted treatment is needed for BP.

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Acceptance Code:	PCR102
Abstract Title:	Leveraging Social Media for Patient Experience Insights in Rare Disease
Presenting Author:	Rachel Black

Abstract Body:

OBJECTIVES: Understanding patient-centered concerns can aid clinicians and manufacturers in the development of tools that assess patient experience and improve care. The objective was to determine the feasibility of utilizing social media to generate insights on patient experience around treatment, quality of life, and burden of illness for a sample rare disease.

METHODS: We developed a prototype tool that uses data from the cystic fibrosis subreddit /r/CysticFibrosis on Reddit.com. Discussion threads ("posts," in Reddit terminology) from July to December 2021 were reviewed and tagged as relevant if they contained discussion points of potential relevance to clinicians or manufacturers. Individual statements within threads were then manually labelled to one of four main categories (Patient Journey, Treatment Effect, Treatment Switching, and Life Milestones). Within Patient Journey, threads were subcategorized into Psychosocial Impact of Disease, Disease Burden, and Symptoms. Treatment Effects were labeled as Positive, Negative, or Neutral. Reasons for Treatment Switching were captured. Milestones were further categorized by type of event.

RESULTS: 57 threads from the CysticFibrosis subreddit were tagged over the 6 month period, of which 31 were deemed relevant. 103 comments had at least one label, with 117 labels being applied (51 Patient Journey, 39 Treatment Effect, 24 Milestones, 1 Treatment Switching, 2 Other). Within the Patient Journey category, the psychosocial impact of disease (N=22), disease burden (N=14), symptoms (N=11), and other (N=2) were mentioned. Within Treatment Effects, 15 were Positive, 21 were Negative, and 2 were Neutral. Trikafta (N=23), Kaftrio (N=1), Kalydeco (N=1), and Orkambi (N=2) were mentioned. Milestones included going to college (N=4), having kids (N=3), marriage (N=2), career (N=11), advanced degree (N=1) and other (N=3).

CONCLUSION: The results of the prototype demonstrated that social media hold considerable potential for obtaining real-time, treatment-level insight into patient experiences that may not be readily identifiable through traditional research methods.

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Acceptance Code:	HSD67
Abstract Title:	The Burden of Systemic Glucocorticoid (GC) Use in Anti-Neutrophilic Cytoplasmic Autoantibody (ANCA)-Associated Vasculitis Patients
Presenting Author:	Kathryn Fitch

Abstract Body:

OBJECTIVES: Quantify the amount of GC use in newly diagnosed granulomatosis with polyangiitis (GPA) and microscopic polyangiitis (MPA) commercially insured and Medicare fee for service (FFS) populations.

METHODS: Using the 2016-2019 Milliman proprietary commercial claims data and Medicare 100% FFS Innovator Research data, GPA and MPA were identified in index years 2017 and 2018 as those with at least two qualifying claims coded with a disease ICD-10-CM code. Newly diagnosed patients were identified as those with no claim coded with GPA or MPA 12 months prior to their first claim in 2017 or 2018. Oral and injectable GC use was identified in non-inpatient claims using NDC and HCPCS codes. Use of GCs during inpatient stays is not identifiable, as such this analysis underestimates the total GC load. The prednisolone-equivalent dose in milligrams (mg) was calculated by converting GC scripts/claims to a prednisolone-equivalent basis.

RESULTS: We identified 390 (GPA) and 70 (MPA) commercially insured and 3,719 (GPA) and 1,168 (MPA) Medicare FFS newly diagnosed patients with GC use who also had eligibility for 12 months prior to their first GC use date through 18 months after their first GC use date. The cumulative prednisolone-equivalent doses at 18 months were: 32% and 33% used 1-999 mg; 32% and 39% used 1,000-4,999 mg; 25% and 16% used 5,000-9,999 mg; and 11% and 13% had 10,000+ mg, respectively for the commercial population. The cumulative prednisolone-equivalent doses through 18 months were: 46% and 26% used 1-999 mg; 29% and 37% used 1,000-4,999 mg; 19% and 28% used 5,000-9,999 mg; and 6% and 8% used 10,000+ mg, respectively for the Medicare FFS population.

CONCLUSIONS: Most patients newly diagnosed with GPA and MPA were treated with high cumulative amounts of GCs. Given side-effects reported with high GC doses, this report highlights the need for GC sparing therapies.