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Publishing for Payers: A Discussion with a Managed Care Communications Expert & a Journal Editor

March 24, 2021

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How to ask questions

Feel free to ask a question at any time, however all questions will be held until the end the of the presentation.

To ask a question, open the Q&A window, type your question into the Q&A box. Click Send

Note: Check Send Anonymously if you do not want your name attached to your question in the Q&A

We will make every effort to respond to all questions live (out loud)

Faculty

Laura Happe, PharmD, MPH
Editor-in-Chief, Journal of Managed Care and Specialty Pharmacy;
Associate Professor and Director of Online Masters, University of Florida

Evelyn Sarnes, PharmD, MPH
Vice President, Value & Access Communications, Xcenda

Brian Scheckner, PharmD, ISMPP CMPP™
Senior Director, Medical Communications, Neuroscience Franchise, Global Scientific Affairs, Global Medical Affairs, Jazz Pharmaceuticals

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Disclaimer

- Information presented reflects the personal knowledge and opinion of the presenters and does not necessarily represent the position of their current or past employers

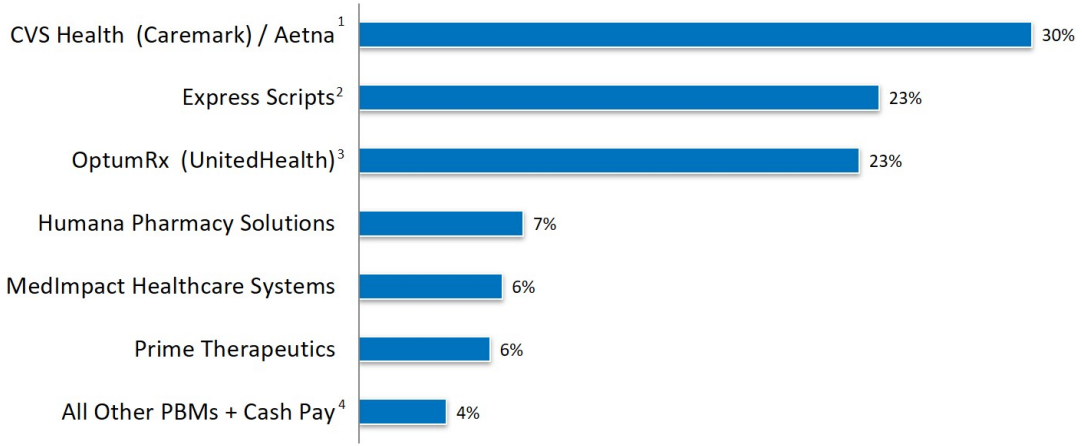
Objectives

- Explain skills needed to produce effective communications for payers
- Identify common managed care pharmacy communication tools (Hint: PIE is not just something you eat at Thanksgiving)
- Describe publication strategies to make your manuscript resonate with payers

Managed care pharmacy

Top pharmacy benefit managers

PBM Market Share, by Total Equivalent Prescription Claims Managed, 2018



1. Includes pro forma combination of claims processed by Aetna. Excludes double counting of network claims for mail choice claims filled at CVS retail pharmacies.
2. Includes Anthem. During 2019, Anthem claims will be transitioning to IngenioRx.
3. Includes Cigna. By the end of 2020, Cigna claims will transition to Express Scripts.
4. Figure includes some cash pay prescriptions that use a discount card processed by one of the 6 PBMs shown on the chart.

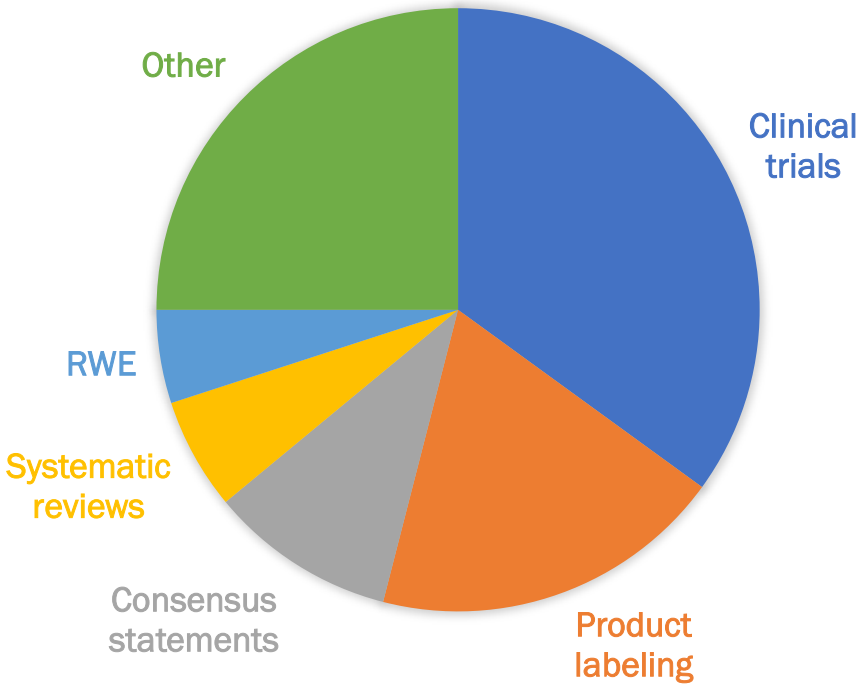
Source: Drug Channels Institute research and estimates. Total equivalent prescription claims includes claims at a PBM's network pharmacies plus prescriptions filled by a PBM's mail and specialty pharmacies. Includes discount card claims. Note that figures may not be comparable with those of previous reports due to changes in publicly reported figures of equivalent prescription claims. Total may not sum due to rounding.

This chart appears as Exhibit 76 in *The 2019 Economic Report on U.S. Pharmacies and Pharmacy Benefit Managers, Drug Channels Institute*. Available at http://drugch.nl/pharmacy

DRUG CHANNELS INSTITUTE

The top 6 PBMs process 95% of all prescription claims.

Data in formulary monographs



Hurwitz JT, Brown M, Graff JS, Peters L, Malone DC. Is real-world evidence used in P&T monographs and therapeutic class reviews? *J Manag Care Spec Pharm*. 2017;23(6):613-620.

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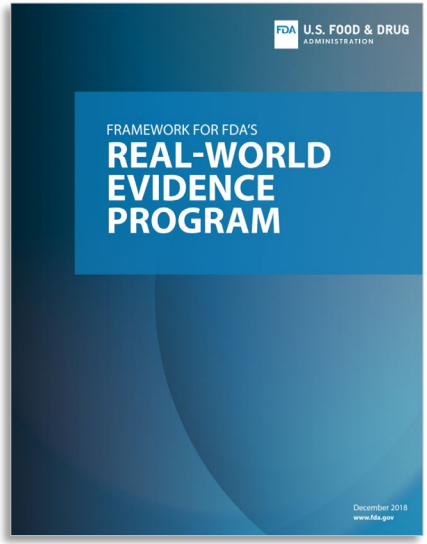
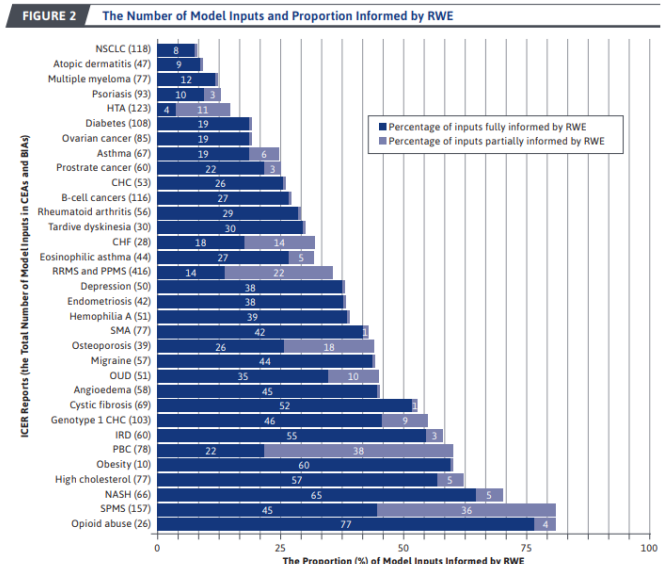
Real-world evidence ▶ RWE

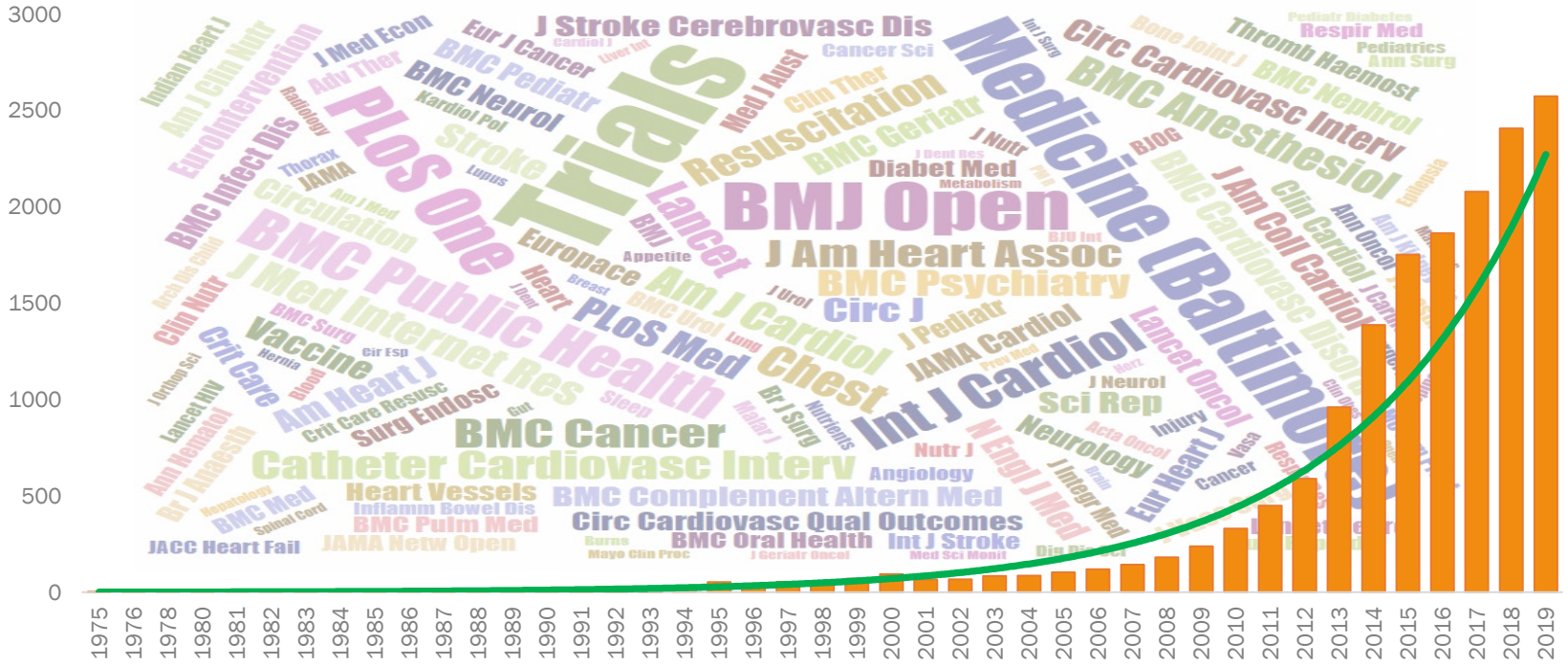
RWE is the clinical evidence regarding the usage and potential benefits or risks of a medical product derived from analysis of data collected in the routine delivery of healthcare.





Lee W, Dayer V, Jiao B, Carlson JJ, Devine B, Veenstra DL. Use of real-world evidence in economic assessments of pharmaceuticals in the United States. *J Manag Care Spec Pharm*. 2021;27(1):5-4.

Growth in RWE publications



Search query: (((((("Health claims"[Title/Abstract]) or ("real-world evidence"[Title/Abstract])) OR ("registry"[Title/Abstract])) OR ("electronic medical records"[Title/Abstract])) OR ("cost-effectiveness analysis"[Title/Abstract])) AND ("clinical study"[Publication Type] OR "observational study"[Publication Type])

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Examples of RWE used by payers

Original Study

Prolonged Duration of Therapy Is Associated With Improved Survival in Patients Treated for Relapsed/Refractory Multiple Myeloma in Routine Clinical Care in the United States

Parameswaran Hari,[1] Dorothy Romanus,[2] Antonio Palumbo,[2] Katarina Luptakova,[2] Robert M. Rifkin,[3] Linh Mai Tran,[4] Aditya Raju,[5] Eileen Farrelly,[5] Stephen J. Noga,[2] Marlo Blazer,[5] Ajai Chari[6]

Abstract

Despite the emerging paradigm favoring continuous therapy, we found that in routine clinical care, myeloma patients at first relapse frequently discontinue treatment before progression, resulting in a therapy duration that is significantly shorter than the interval to the next therapy. We further describe the association between the length of second-line therapy and improved overall survival for patients with relapsed/refractory multiple myeloma.

Background: In clinical trials, an extended therapy duration has been associated with better outcomes in patients with newly diagnosed multiple myeloma (NDMM). However, data on how the therapy duration affects the outcomes for patients with relapsed/refractory multiple myeloma (RRMM) are limited. We conducted a large, retrospective study in the United States to evaluate the effect of the duration of second-line therapy on overall survival. **Patients and Methods:** Adults with NDMM from January 2008 to June 2015 were followed up to identify their second-line therapy. The duration of therapy (DOT) and time to next therapy (TTNT), as a proxy for progression-free survival, were estimated using the Kaplan-Meier method. The relationship between the duration of second-line therapy and overall survival was evaluated with a logistic marginal structural model to mitigate the risk of treatment selection and survival bias. **Results:** A total of 628 NDMM patients developed a relapse after initial therapy. The median DOT for second-line therapy was 6.9 months (95% confidence interval [CI], 5.9-7.7 months), which was shorter than the corresponding TTNT (median, 15.1 months; 95% CI, 13.4-17.3 months). Each additional month of second-line therapy was associated with a reduced adjusted risk of death at 1 year (odds ratio, 0.78; 95% CI, 0.77-0.83; $P < .001$). **Conclusion:** In a large database capturing a heterogeneous patient population and varied treatment patterns reflecting routine clinical care, we found a clinical benefit for continued longer DOT at first relapse. Despite the emerging paradigm favoring continuous therapy, second-line progression-free survival (utilizing TTNT as the proxy) was more than twofold longer than the DOT. Understanding the barriers to extended DOT could help to improve the outcomes for RRMM patients.

Clinical Lymphoma, Myeloma & Leukemia, Vol. 18, No. 2, 152-60 © 2018 The Authors. Published by Elsevier Inc. This is an open access article under the CC BY-NC-ND license (http://creativecommons.org/licenses/by-nc-nd/4.0/).

Keywords: DOT, Overall survival, Progression-free survival, RRMM, Time to next therapy

Introduction

Multiple myeloma (MM) is the second-most common hematologic malignancy and primarily affects the elderly population.[1] Novel therapies have been associated with a significant improvement in overall survival (OS) for patients with MM[2,3]; however, relapse will be inevitable for most patients. Consensus has been increasing that prolonged therapy for patients with newly diagnosed MM (NDMM) correlates with improved patient

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Clinical and epidemiological research

OPEN ACCESS

EXTENDED REPORT

The comparative effectiveness of abatacept versus anti-tumour necrosis factor switching for rheumatoid arthritis patients previously treated with an anti-tumour necrosis factor

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Handling editor Tore K Kvien

► Additional material is published online only. To view please visit the journal online (http://dx.doi.org/10.1136/annrheumdis-2013-203936).

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ABSTRACT

Objective We compared the effectiveness of abatacept (ABA) versus a subsequent anti-tumour necrosis factor inhibitor (anti-TNF) in rheumatoid arthritis (RA) patients with prior anti-TNF use.

Methods We identified RA patients from a large observational US cohort (2/1/2000–8/7/2011) who had discontinued at least one anti-TNF and initiated either ABA or a subsequent anti-TNF. Using propensity score (PS) matching (n:1 match), effectiveness was measured at 6 and 12 months after initiation based on mean change in Clinical Disease Activity Index (CDAI), modified American College of Rheumatology (mACR) 20, 50 and 70 responses, modified Health Assessment Questionnaire (mHAQ) and CDAI remission in adjusted regression models.

Results The PS-matched groups included 431 ABA and 746 anti-TNF users at 6 months and 311 ABA and 493 anti-TNF users at 12 months. In adjusted analyses comparing ABA to anti-TNF following treatment with ABA and anti-TNF, the difference in weighted mean change in CDAI (range 6–8) at 6 months (0.46, 95% CI −0.82 to 1.73) and 12 months was similar (−1.64, 95% CI −3.47 to 0.19). The mACR20 responses were similar at 6 (28–32%, p=0.73) and 12 months (35–37%, p=0.48) as were the mACR50 and mACR70 (12 months: 20–22%, p=0.25 and 10–12%, p=0.49, respectively). Meaningful change in mHAQ was similar at 6 and 12 months (30–33%, p=0.41 and 29–30%, p=0.39, respectively) as was CDAI remission rates (9–10%, p=0.42 and 12–13%, p=0.91, respectively).

Conclusions RA patients with prior anti-TNF exposures had similar outcomes if they switched to a new anti-TNF as compared with initiation of ABA.

INTRODUCTION

Rheumatoid arthritis (RA) affects an estimated 1.3 million Americans and is associated with substantial morbidity and mortality.[1] To prevent the development of permanent joint damage and deformity as well as functional impairment, the current treatment paradigm is to treat patients with active disease with disease modifying antirheumatic drugs (DMARDs).[2] The typical approach is to introduce a conventional non-biologic DMARD first, followed by combination non-biologic DMARDs or biologics in non-responders.[2] The initial biologic used in the vast majority of patients is the anti-tumour necrosis factor (anti-TNF). These agents were the first class of biologics approved by the Food and Drug Administration (FDA) for the treatment of RA.

However, little is known regarding what to do when patients have an inadequate response to the first anti-TNF agent. Changing mechanism of action has been shown to be beneficial in comparisons of rituximab versus a subsequent anti-TNF[4–7] as demonstrated by improvements in disease activity as well as persistence of therapy. But it is unclear whether any change in mechanism of action will result in improvement or if the results seen were related specifically to B cell depletion. Further exploration is needed to assess whether switching to abatacept (ABA), as a different example of changing mechanism of action, also provides a benefit among those with inadequate response to an anti-TNF. To date, there have been no randomised controlled trials (RCTs) comparing the effectiveness of switching to ABA as compared with a subsequent anti-TNF, and a recent meta-analysis using indirect methods suggested that use of an anti-TNF was associated with a higher probably of achieving an American College of Rheumatology (ACR) 50 response than ABA, although not all indirect comparisons have shown a difference between ABA and anti-TNF agents.[8–10] In biologic-naïve patients, a few studies have compared responses to ABA versus an anti-TNF.[11–14] A similar efficacy was found when comparing responses to ABA and infliximab, although there have been concerns that the results may not be generalisable to the US population of RA patients due to the lower doses of infliximab used (3 mg/kg every 8 weeks) and lack of the usual infliximab loading dose. Additionally, ABA (in combination with methotrexate (MTX)) has been compared with adalimumab with MTX among biologic-naïve RA patients with similar ACR response rates.[14]

Given the absence of head-to-head RCTs addressing best treatment approaches in patients with inadequate response to an anti-TNF agent, comparative effectiveness studies using observational data from registries can be employed.[15] Therefore, the aim of the present study was to compare the clinical effectiveness of ABA versus a subsequent anti-TNF agent among RA patients with previous anti-TNF exposure in a large US cohort of RA patients using the

CrossMark

To cite: Harrold LR, Reed GW, Kremer JM, *et al*. *Ann Rheum Dis* 2015;**74**:430–436.

ORIGINAL INVESTIGATION

Glucagonlike Peptide 1–Based Therapies and Risk of Hospitalization for Acute Pancreatitis in Type 2 Diabetes Mellitus

A Population-Based Matched Case-Control Study

Sonal Singh, MD, MPH; Hsien-Yen Chang, PhD; Thomas M. Richards, MS; Jonathan P. Weiner, DrPH; Jeanne M. Clark, MD, MPH; Jodi B. Segal, MD, MPH

Importance: Acute pancreatitis has significant morbidity and mortality. Previous studies have raised the possibility that glucagonlike peptide 1 (GLP-1)–based therapies, including a GLP-1 mimetic (exenatide) and a dipeptidyl peptidase 4 inhibitor (sitagliptin phosphate), may increase the risk of acute pancreatitis.

Objective: To test whether GLP-1–based therapies such as exenatide and sitagliptin are associated with an increased risk of acute pancreatitis. We used conditional logistic regression to analyze the data.

Design: Population-based case-control study.

Setting: A large administrative database in the United States from February 1, 2005, through December 31, 2008.

Participants: Adults with type 2 diabetes mellitus aged 18 to 64 years. We identified 1269 hospitalized cases with acute pancreatitis using a validated algorithm and 1269 control subjects matched for age category, sex, enrollment pattern, and diabetes complications.

Main Outcome Measure: Hospitalization for acute pancreatitis.

Results: The mean age of included individuals was 52 years, and 57.45% were male. Cases were significantly more likely than controls to have hypertriglyceridemia (12.92% vs 8.35%), alcohol use (3.23% vs 0.24%), gallstones (9.06% vs 1.34), tobacco abuse (16.39% vs 5.52%), obesity (19.62% vs 9.77%), biliary and pancreatic cancer (2.84% vs 0%), cystic fibrosis (0.79% vs 0%), and any neoplasm (29.94% vs 18.05%). After adjusting for available confounders and metformin hydrochloride use, current use of GLP-1–based therapies within 30 days (adjusted odds ratio, 2.24 [95% CI, 1.36-3.68]) and recent use past 30 days and less than 2 years (2.01 [1.37-3.18]) were associated with significantly increased odds of acute pancreatitis relative to the odds in nonusers.

Conclusions and Relevance: In this administrative database study of US adults with type 2 diabetes mellitus, treatment with the GLP-1–based therapies sitagliptin and exenatide was associated with increased odds of hospitalization for acute pancreatitis.

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Published online February 25, 2013.
doi:10.1001/jamainternmed.2013.2720

Author Affiliations: Department of Medicine, The Johns Hopkins University School of Medicine (Drs Singh, Clark, and Segal); and Center for Public Health and Human Rights (Dr Singh) and Department of Health Policy and Management (Drs Chang and Weiner and Mr Richards), The Johns Hopkins Bloomberg School of Public Health, Baltimore, Maryland.

THE GLUCAGONLIKE PEPTIDE 1 (GLP-1)–based therapies include a GLP-1 mimetic, exenatide, and a dipeptidyl peptidase 4 inhibitor, sitagliptin phosphate. These therapies are used in patients with type 2 diabetes mellitus to lower blood glucose levels. Exenatide is a subcutaneous injectable GLP-1 agonist that enhances glucose-mediated insulin secretion. Sitagliptin is an oral dipeptidyl peptidase 4 inhibitor that prevents the proteolysis of endogenous GLP-1.[1]

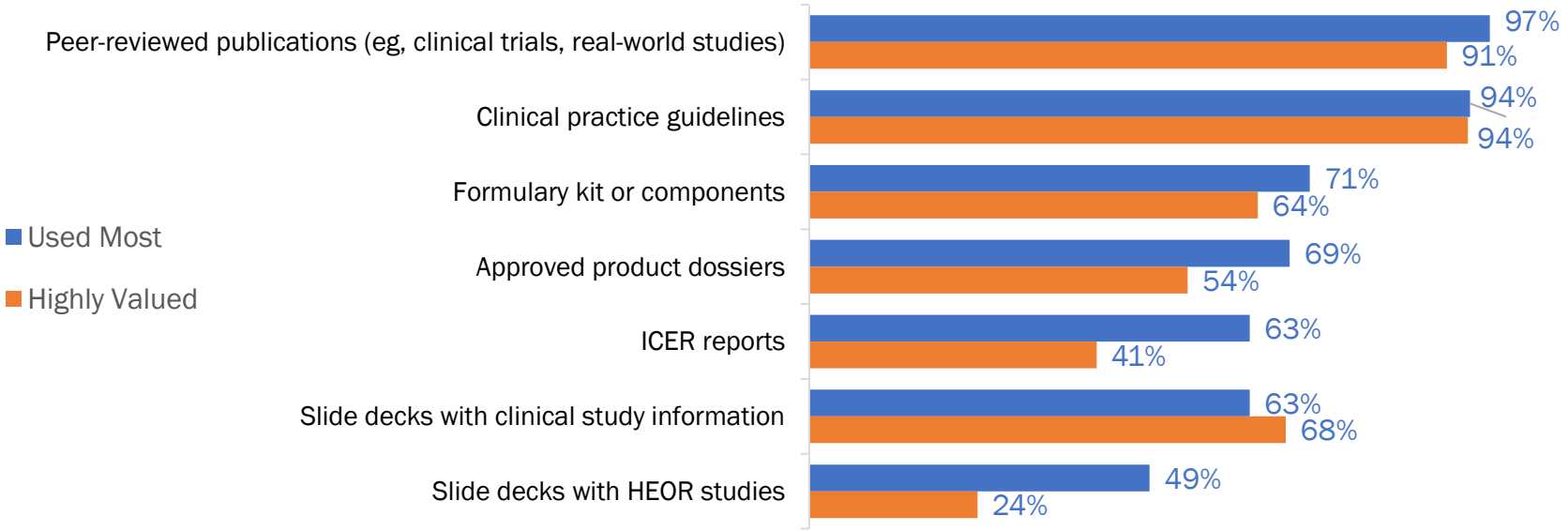
Sitagliptin and exenatide have been shown to cause acute pancreatitis in rodent models via amplification of ductal replication and induction of acinar to ductal metaplasia.[2,3] Mouse models have also shown that GLP-1–based therapy may induce focal proliferation in the exocrine pancreas and accelerate formation of dysplastic lesions and pancreatitis.[4] Reports of spontaneous acute pancreatitis in humans have been published[5,6]; however, the strength of this association and causality cannot be inferred from these reports.

See Invited Commentary at end of article

Previous observational studies of this association have yielded inconsistent re-

Value of publications for payers

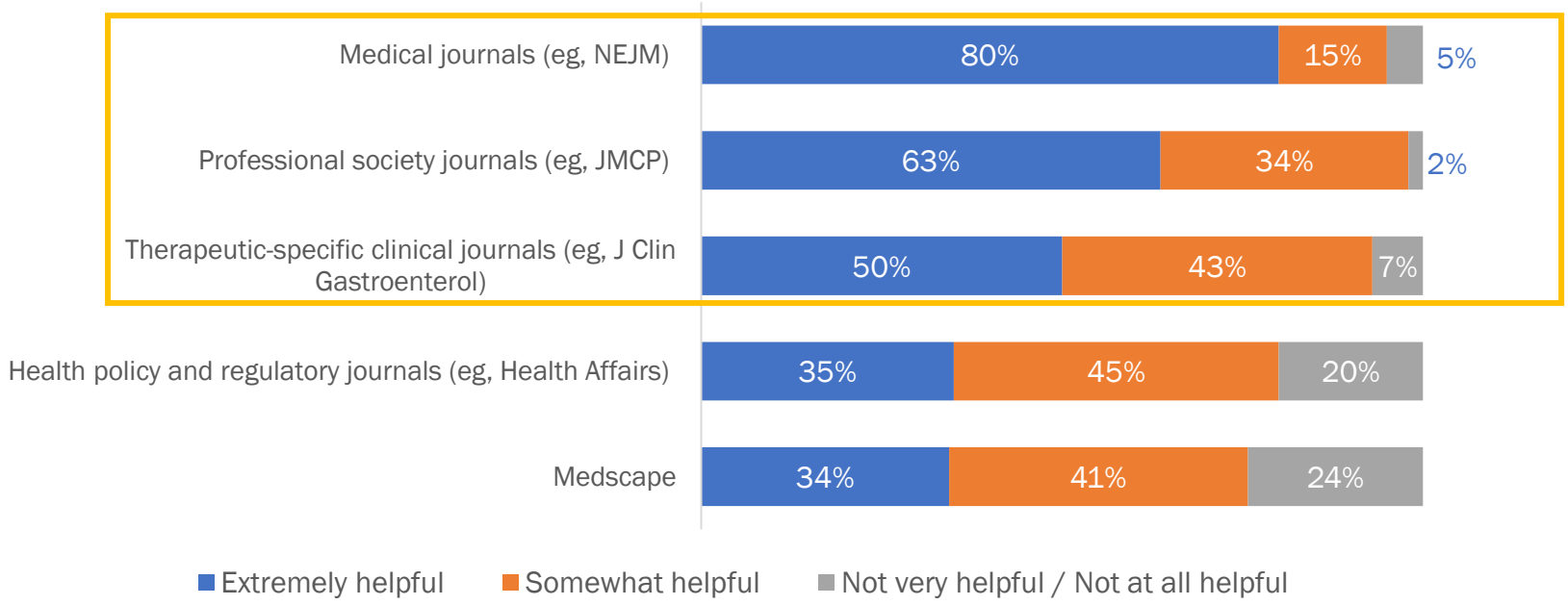
Most Used Sources of Information and Corresponding Value for Medical Policy/Formulary Decision-Making



Survey of Xcenda's Managed Care Network, Sept 2020 (N=35 US pharmacy/medical directors)
Q2a. Which of the following sources of information does your organization use to support medical policy and/or formulary decision-making?
Q2b. How useful are each of the following sources of information for medical policy and/or formulary decision-making within your organization? (Showing % Extremely/Very Useful)

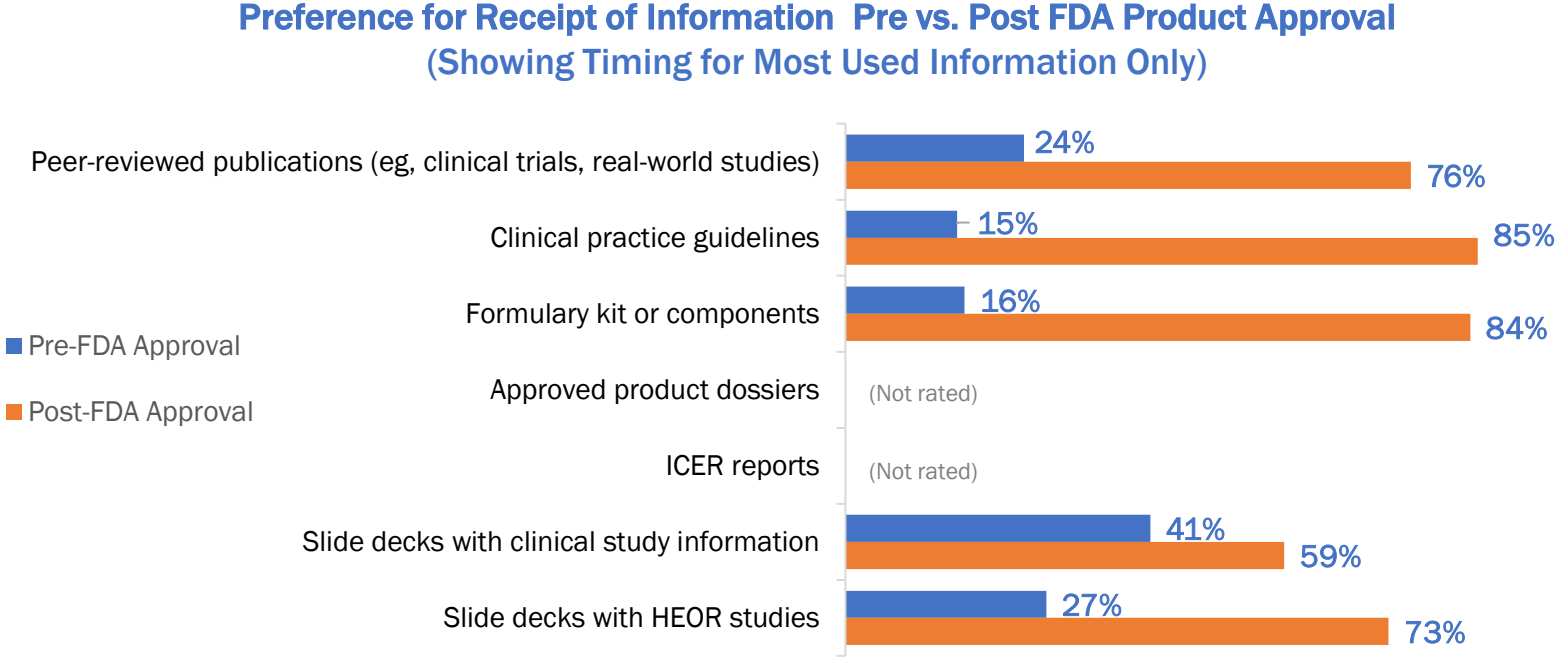
What type of journals do payers read?

Assessment of "Helpfulness" of Journal/Media Types



Survey of Xcenda's Managed Care Network, June 2019 (N=41 US pharmacy/medical directors)
Q408. How helpful to your role are each of the following sources?

Timing of information relative to product approval



Survey of Xcenda's Managed Care Network, Sept 2020 (N=35 US pharmacy/medical directors)
Q2a. Which of the following sources of information does your organization use to support medical policy and/or formulary decision-making?
Q3. How useful are each of the following sources of information for medical policy and/or formulary decision-making within your organization? Before vs. After Product Approval (Choose One)

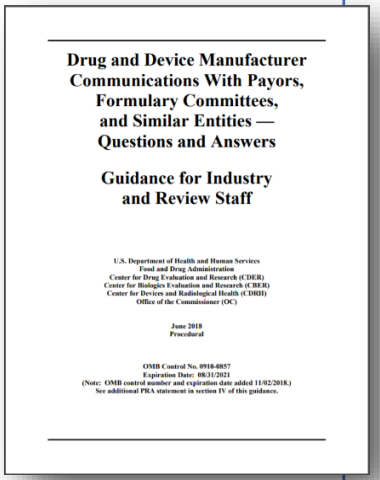
Pre-approval Information Exchange

FDA Guidance on HCEI and PIE June 2018

"Drug and Device Manufacturer Communications With Payers, Formulary Committees, and Similar Entities — Questions and Answers Guidance for Industry and Review Staff"

Provides a pathway to start communicating with payers earlier in the product's lifecycle, as well as post-approval HCEI

- Clarifies how biopharmaceutical manufacturers can communicate **truthful and non-misleading information to payers across a product's lifecycle**
- Allows manufacturers to share certain HCEI on **approved products or devices** with payers
- Allows manufacturers to share information with payers on **unapproved products or unapproved uses of cleared products,** as long as certain conditions are met ("PIE")
- Note that recommendations from the FDA guidance are **non-binding**



Drug and Device Manufacturer Communications With Payors, Formulary Committees, and Similar Entities — Questions and Answers

Guidance for Industry and Review Staff

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)
Center for Devices and Radiological Health (CDRH)
Office of the Commissioner (OC)

June 2018
Procedural

OMB Control No. 0910-0857
Expiration Date: 08/31/2021
(Note: OMB control number and expiration date added 11/02/2018.)
See additional PRA statement in section IV of this guidance.

Key: FDA – Food and Drug Administration; FDAMA – Food and Drug Administration Modernization Act; HCEI – healthcare economic information; PIE – pre-approval information exchange.
Source: Food and Drug Administration. Drug and device manufacturer communications with payors, formulary committees, and similar entities – questions and answers. Guidance for industry and review staff. https://www.fda.gov/media/102683/download. June 2018. Accessed December 12, 2019.

Strategies to make your communication tools resonate with payers

Know your audience

Data-driven

Value clinical + economic data

Make population-level decisions

Detail-oriented

Not swayed by traditional marketing

Not absent of emotion

Busy

Speak their language

OCREVUS adherence and persistence data

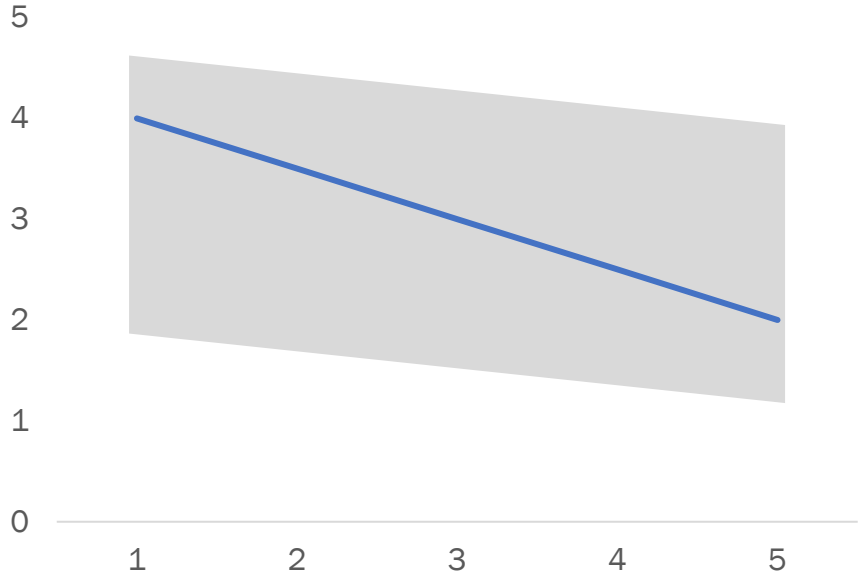
Adherence and persistence are important considerations when it comes to your patient's dosing regimen

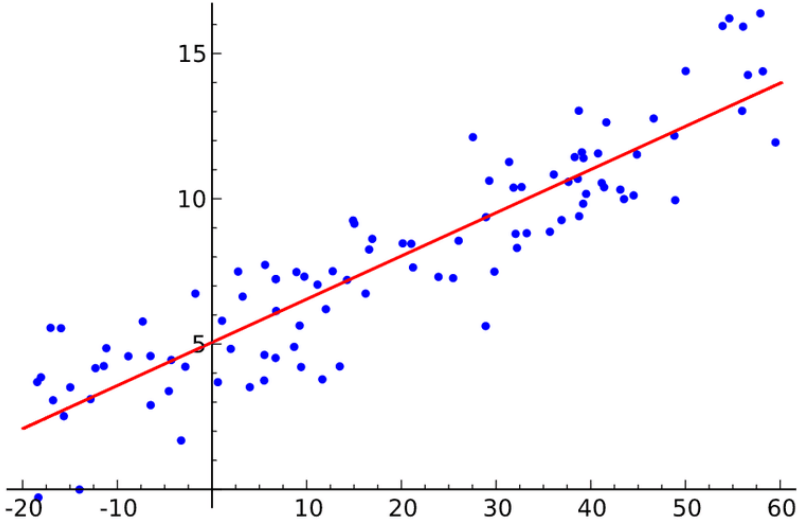
There are many reasons patients and providers choose to start or discontinue treatment, from adverse events to patient preference.

In the OCREVUS RMS clinical trials, the percentage of patients who completed the clinical trials at year 2 were[1]:

OPERA I			OPERA II		
89%	vs	83%	86%	vs	77%
OCREVUS		Rebif	OCREVUS		Rebif

Speak their language





Speak their language

RESEARCH

Burden of Alcohol Abuse or Dependence Among Long-Term Opioid Users with Chronic Noncancer Pain

Pamela B. Landsman-Blumberg, MPH, DrPH; Nathaniel Katz, MD, MS; Kavita Gajria, MS; Anna D. Coutinho, BPharm, PhD; Paul P. Yeung, MD, MPH; and Richard White, PhD, MPH, MBE

ABSTRACT

BACKGROUND: Substance abuse disorders among chronic noncancer pain (CNCP) patients add to the clinical challenges and economic burden of caring for such patients. Despite potential risks, some CNCP patients with a history of alcohol abuse or dependence (AAD) and pain that is refractory to nonopioid treatment options may still need opioids for pain management. However, there is a lack of data on adverse outcomes in long-term opioid users with CNCP and a history of substance abuse or AAD disorders.

OBJECTIVE: To compare adverse outcomes and all-cause health care costs among CNCP patients on long-term opioid treatment with and without a previous diagnosis of AAD.

METHODS: Using MarketScan claims databases (2006-2012), CNCP patients with ≥90 days of opioid supply after CNCP diagnosis and continuous enrollment 12 months before CNCP diagnosis (baseline period) and 12 months after opioid start (post-index period) were identified. AAD was defined by diagnosis codes at any time before opioid initiation. Outcomes included opioid overdose, accident, and injury episodes identified by ICD-9-CM diagnoses codes. T-tests and Mann-Whitney tests compared continuous measures, and chi-square and Fisher's exact tests compared categorical measures between those with and without AAD. Multivariable analyses for outcomes were conducted, adjusting for baseline differences between cohorts.

RESULTS: Of 21,203 CNCP patients with long-term opioid treatment, 750 (3.5%) had an AAD diagnosis before opioid initiation. AAD patients were significantly younger (48.4 [SD ± 11.4] years vs. 52.8 [SD ± 14.8] years), less likely to be enrolled in Medicare (17% commercial vs. 4% Medicare), and more likely to be male (67% vs. 48%; all $P < 0.001$). There were no differences in type or number of CNCP diagnoses or Charlson Comorbidity

What is already known about this subject

- A history of alcohol abuse or dependence (AAD) is considered a strong predictor of potential opioid abuse or misuse and may be considered a relative contraindication to the use of opioids in chronic noncancer pain (CNCP) patients.
- Opioids may be a necessary pain relief treatment option among patients with a history of AAD and who have CNCP that is refractory to nonopioid treatments.
- There are limited published data available to describe the adverse outcomes of long-term opioid use in patients with CNCP and a history of AAD.

What this study adds

- In this cohort of patients with CNCP and long-term opioid use, the risks of overdose, accident, or injury were higher among patients with a history of AAD compared with those without AAD.
- During the 1 year following the initiation of long-term opioid therapy, CNCP patients with a history of AAD had higher health care costs than those without a history of AAD.
- The risks and benefits of treating patients with a history of AAD with long-term opioid therapy must be carefully considered.

Substance abuse disorders are common among chronic noncancer pain (CNCP) patients, adding to the clini-

interrupted by nocturia [13, 14]. OAB affects more than 37 million adults in the USA [7, 15] and poses a substantial burden to the healthcare system, with a projected cost of $82.6 billion in 2020 [15]. The syndrome is an often overlooked comorbid condition among patients with depression, and patients with both depression and OAB may experience a compounding of the high cost of care associated with each separate condition [11].

An association between urinary incontinence and depression, especially among women, has been denoted in several studies, and outcomes related to OAB and depression have been explored independently within the literature [16]. Little is known, however, about the specific impact of co-occurring OAB among patients with depression. Given the prevalence of both conditions and the potential for poor outcomes for patients resulting from their comorbidity, a better understanding of how these conditions interact is needed. How costs and workplace productivity are affected by the conditions co-occurring can address part of that knowledge gap. This study characterizes the healthcare costs, healthcare resource utilization (HCRU), and workplace productivity among two cohorts of patients with depression, those with and those without OAB.

METHODS

Data Source

This study was conducted using data from the IBM MarketScan Commercial Claims and Encounters Database, the IBM MarketScan Medicare and Supplemental and Coordination of Benefits Database, and the MarketScan Health and Productivity Management Database. The Commercial database contains longitudinal medical and drug information, including paid amounts, for several million individuals (in-

3 million individuals annually. The MarketScan Health and Productivity database includes data on workplace absence, short-term disability, and worker's compensation for a subset of enrollees in the Commercial database. MarketScan databases are Health Insurance Portability and Accountability Act (HIPAA) compliant. This study was conducted using anonymized patient data from MarketScan and as such review board approval and patient consent were not necessary.

Study Design/Search Strategy

This was a retrospective cohort analysis of HCRU, costs, and workplace productivity comparing a cohort of individuals with depression and OAB (the case cohort) to a propensity score matched cohort with depression but no OAB (the control cohort). Cohorts were constructed from a prevalent population of patients with depression (identified on the basis of medical and prescription claims for depression, Appendix 1 in the electronic supplementary material, ESM). The first diagnosis of OAB (or date of first prescription for an OAB medication) served as index for the case cohort ("OAB index date"). For patients with depression and without OAB, a proxy index date was constructed using the Harvey et al. [17] method selected randomly from within an April 1, 2012 to December 31, 2015 identification period. The 6-month period prior to the index date was the pre-index (baseline) period and the 12-month period following the index date was the post-index period. The full study period was October 1, 2011 to December 31, 2016.

Individuals in both cohorts were required to be at least 18 years of age with continuous insurance enrollment (in the pre-index through the post-index periods) and have a diagnosis of depression during an October 1, 2011 to December 31, 2015 identification period.

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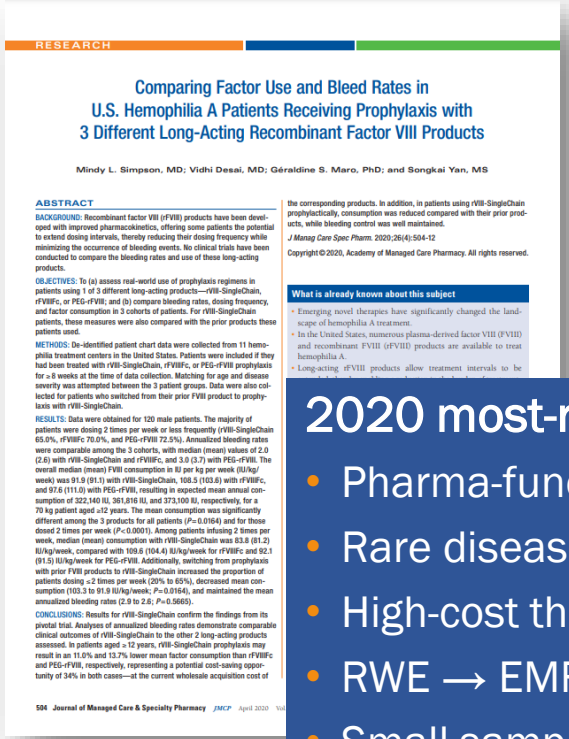

RESEARCH

Comparing Factor Use and Bleed Rates in U.S. Hemophilia A Patients Receiving Prophylaxis with 3 Different Long-Acting Recombinant Factor VIII Products

Mindy L. Simpson, MD; Vidhi Desai, MD; Géraldine S. Maro, PhD; and Songkai Yan, MS

ABSTRACT

BACKGROUND: Recombinant factor VIII (rFVIII) products have been developed with improved pharmacokinetics, offering some patients the potential to extend dosing intervals, thereby reducing their dosing frequency while minimizing the occurrence of bleeding events. No clinical trials have been conducted to compare the bleeding rates and use of these long-acting products.

OBJECTIVES: To (a) assess real-world use of prophylaxis regimens in patients using 1 of 3 different long-acting products—rFVIII-SingleChain, rFVIIIFc, or PEG-rFVIII; and (b) compare bleeding rates, dosing frequency, and factor consumption in 3 cohorts of patients. For rFVIII-SingleChain patients, these measures were also compared with the prior products these patients used.

METHODS: De-identified patient chart data were collected from 11 hemophilia treatment centers in the United States. Patients were included if they had been treated with rFVIII-SingleChain, rFVIIIFc, or PEG-rFVIII prophylaxis for ≥ 8 weeks at the time of data collection. Matching for age and disease severity was attempted between the 3 patient groups. Data were also collected for patients who switched from their prior FVIII product to prophylaxis with rFVIII-SingleChain.

RESULTS: Data were obtained for 120 male patients. The majority of patients were dosing 2 times per week or less frequently (rFVIII-SingleChain 65.0%, rFVIIIFc 70.0%, and PEG-rFVIII 72.5%). Annualized bleeding rates were comparable among the 3 cohorts, with median (mean) values of 2.0 (2.6) with rFVIII-SingleChain and rFVIIIFc, and 3.0 (3.7) with PEG-rFVIII. The overall median (mean) FVIII consumption in IU per kg per week (IU/kg/week) was 91.9 (91.1) with rFVIII-SingleChain, 108.5 (103.6) with rFVIIIFc, and 97.6 (111.0) with PEG-rFVIII, resulting in expected mean annual consumption of 322,140 IU, 361,816 IU, and 373,100 IU, respectively, for a 70 kg patient aged ≥12 years. The mean consumption was significantly different among the 3 products for all patients ($P = 0.0164$) and for those dosed 2 times per week ($P < 0.0001$). Among patients infusing 2 times per week, median (mean) consumption with rFVIII-SingleChain was 83.8 (81.2) IU/kg/week, compared with 109.6 (104.4) IU/kg/week for rFVIIIFc and 92.1 (91.5) IU/kg/week for PEG-rFVIII. Additionally, switching from prophylaxis with prior FVIII products to rFVIII-SingleChain increased the proportion of patients dosing ≤ 2 times per week (20% to 65%), decreased mean consumption (103.3 to 91.9 IU/kg/week; $P = 0.0164$), and maintained the mean annualized bleeding rates (2.9 to 2.6; $P = 0.5665$).

CONCLUSIONS: Results for rFVIII-SingleChain confirm the findings from its pivotal trial. Analyses of annualized bleeding rates demonstrate comparable clinical outcomes of rFVIII-SingleChain to the other 2 long-acting products assessed. In patients aged ≥ 12 years, rFVIII-SingleChain prophylaxis may result in an 11.0% and 13.7% lower mean factor consumption than rFVIIIFc and PEG-rFVIII, respectively, representing a potential cost-saving opportunity of 34% in both cases—at the current wholesale acquisition cost of the corresponding products. In addition, in patients using rFVIII-SingleChain prophylactically, consumption was reduced compared with their prior products, while bleeding control was well maintained.

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What is already known about this subject

- Emerging novel therapies have significantly changed the landscape of hemophilia A treatment.
- In the United States, numerous plasma-derived factor VIII (FVIII) and recombinant FVIII (rFVIII) products are available to treat hemophilia A.
- Long-acting rFVIII products allow treatment intervals to be

2020 most-read paper

- Pharma-funded
- Rare disease
- High-cost therapeutic area
- RWE → EMR data
- Small sample size, N=120

In addition to efficacy, another important consideration is the factor consumption when evaluating the overall benefit associated with any given product. While it is not a direct outcome of treatment, consumption of units in conjunction with bleed rates may provide a more comprehensive evaluation of treatment effectiveness, showing that patients can achieve similar bleed rates with a reduction in FVIII consumption.[21] Indeed, an indirect comparison of clinical trials showed patients using a long-acting rFVIII product were able to reduce their FVIII consumption compared with using SHL FVIII therapy.[9] Therefore, if reduced consumption is realized in clinical practice, then long-acting products have the potential to improve the management of hemophilia by reducing treatment burden and improving patient quality of life. Also important, if the price is the same or lower, reducing consumption will lead to the reduction of the associated costs of treatment. Here, we investigated

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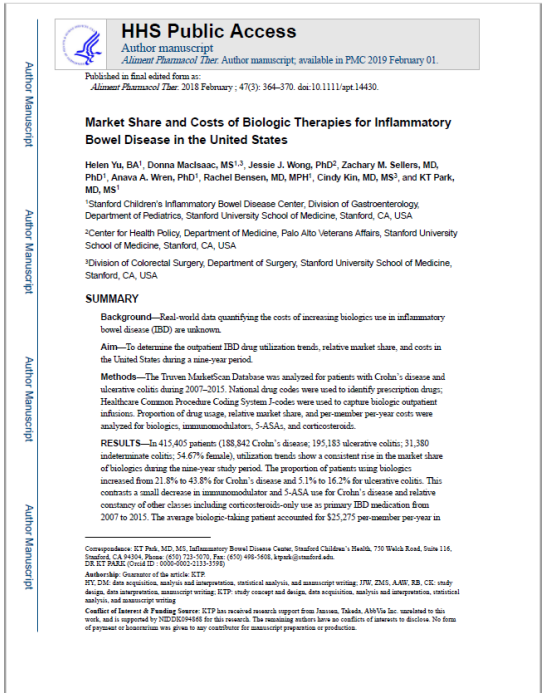

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Market Share and Costs of Biologic Therapies for Inflammatory Bowel Disease in the United States

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SUMMARY

Background—Real-world data quantifying the costs of increasing biologics use in inflammatory bowel disease (IBD) are unknown.

Aim—To determine the outpatient IBD drug utilization trends, relative market share, and costs in the United States during a nine-year period.

Methods—The Truven MarketScan Database was analyzed for patients with Crohn's disease and ulcerative colitis during 2007–2015. National drug codes were used to identify prescription drugs; Healthcare Common Procedure Coding System J-codes were used to capture biologic outpatient infusions. Proportion of drug usage, relative market share, and per-member per-year costs were analyzed for biologics, immunomodulators, 5-ASAs, and corticosteroids.

RESULTS—In 415,405 patients (188,842 Crohn's disease; 195,183 ulcerative colitis; 31,380 indeterminate colitis; 54.67% female), utilization trends show a consistent rise in the market share of biologics during the nine-year study period. The proportion of patients using biologics increased from 21.8% to 43.8% for Crohn's disease and 5.1% to 16.2% for ulcerative colitis. This contrasts a small decrease in immunomodulator and 5-ASA use for Crohn's disease and relative constancy of other classes including corticosteroids-only use as primary IBD medication from 2007 to 2015. The average biologic-taking patient accounted for $25,275 per-member per-year in

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Authorship: Guarantor of the article: KTP.
HY, DM: data acquisition, analysis and interpretation, statistical analysis, and manuscript writing; JJW, ZMS, AAW, RB, CK: study design, data interpretation, manuscript writing; KTP: study concept and design, data acquisition, analysis and interpretation, statistical analysis, and manuscript writing

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INTRODUCTION

Crohn's disease (CD) and ulcerative colitis (UC) are inflammatory bowel diseases (IBD) with typical chronic relapsing and re[illegible] [1] affecting an increasing proportion of the population in the U[illegible] improperly treated,[4] recent developn[illegible] health outcomes in IBD. Expensive, therapy options for disease maintena[illegible]

As a result of increasing biologics us[illegible] care services to outpatient pharmacy[illegible] costs account for nearly one-half of [illegible]

Per-Member Per-Year IBD Medication Costs

Figure 3 shows the PMPY costs allocated t[illegible] biologics, immunomodulators, 5-ASAs, or [illegible] medication. Among patients on biologic th[illegible] IBD medications increased from 2007 to 2[illegible] 2015. For outpatient IBD medications, the [illegible] $25,275 PMPY in 2007 and $36,051 PMP[illegible] patient accounted for $23,616 PMPY in 20[illegible] cost trends for biologics greatly exceed cos[illegible] corticosteroids. Although 5-ASA-taking pa[illegible] than $5,000 PMPY in 2015 sharply contras[illegible] biologic-taking patients. Compared to biolo[illegible] immunomodulator-taking or corticosteroid-[illegible] of the total costs attributed to outpatient IBD medications. An all-medication comparison [illegible] shows that the share of PMPY costs for biologics increased from 72.9% to 85.7% in 2015. In pediatric patients, PMPY costs attributed to biologics increased from 81.7% to 94.9% in 2015.

DISCUSSION

We present the most comprehensive analysis to date of real-world outpatient pharmaceutical utilization and costs in IBD in the US. Our research specifically aimed to address an undescribed health services impact of prescription IBD medication use with a focus on biologics. In our study, we determined the PMPY costs of outpatient IBD medications, quantifying the cost impact of increasing biologics use in the US. We found that outpatient IBD medication use is driven by increasing biologics use as mainstay therapy for disease control. A key finding in our study highlights that the PMPY costs of an average patient in 2015 receiving biologic therapy ($36,051/year) is nearly eight times costlier (<$5,000/year) and approximately thirty-six times costlier (~$1,000) than an average patient receiving 5-ASAs and immunomodulators, respectively.

The aggregate data we outline here also indicate that increasing market share trends for both biologics and 5-ASAs will likely continue if the current utilization trajectory occurs. From a clinician perspective, corticosteroid-sparing therapy plans are evidence-based and necessary to optimize long-term health outcomes, maximize quality of life, and minimize opportunity loss.[12,13,14,15] Considering biologics' effectiveness in achieving mucosal healing,[16,17,18,19] and drug industry's active development pipelines and research investment in newer therapies, extrapolation of market share and cost trends for biologics is reasonable and possibly underestimated for the foreseeable future.

Another interesting finding in our analysis is that biologics' market share in pediatric patients outpaces the market share in all patients. Pediatric patients also had less corticosteroid-only maintenance therapy. This may reflect the known phenotypic difference between adult- versus pediatric-onset IBD.[20] Children with IBD often present with worse

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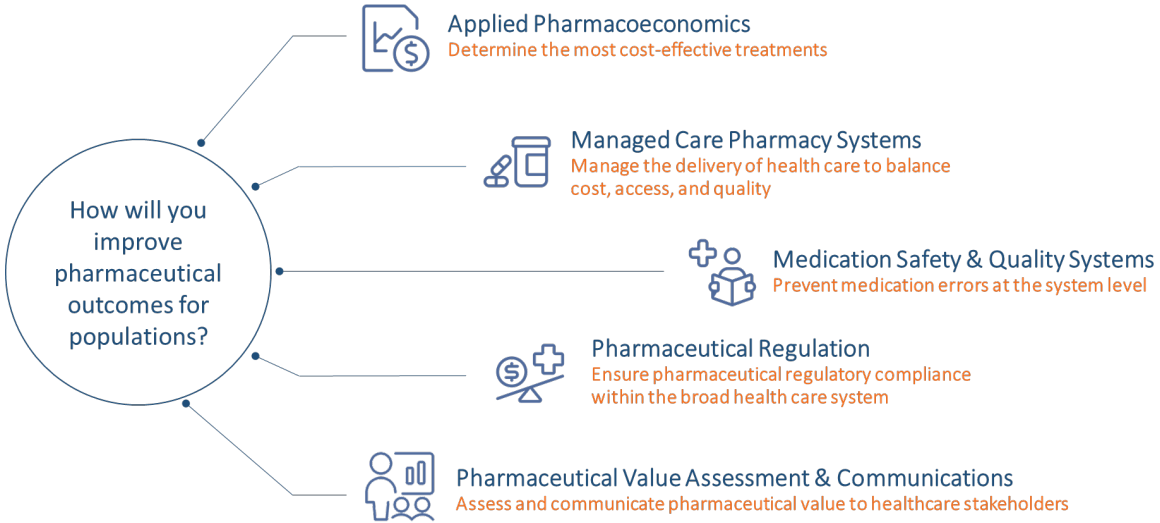

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