Specialty Pharmacy Patient Satisfaction Among

Gaucher Community Alliance Patient Community

Abstract

Gaucher disease is a rare genetic disorder caused by a deficiency of the glucocerebrosidase enzyme, leading to the accumulation of glycosphingolipids. Treatment options include enzyme replacement therapy and substrate reduction therapy, which are ongoing specialized treatments aimed at reducing symptoms, improving lifespan, and enhancing quality of life. Specialty pharmacies play a crucial role in providing these specialized medications, and they have seen significant growth and an evolving role in patient care in the United States. The Gaucher Community Alliance conducted a survey among its community to evaluate patient satisfaction with specialty pharmacy services. The survey results, based on responses from 60 participants, indicated that patients are generally satisfied with their specialty pharmacies. While ratings varied across different specialty pharmacies, the findings suggest that patient satisfaction is primarily influenced by specific services, and areas for improvement were identified. The valuable insights garnered from the survey offer opportunities for enhancing patient satisfaction and provide valuable input for decision-making and strategic planning related to specialty pharmacy services.

Introduction

Gaucher Disease

Gaucher disease is a rare, autosomal recessive genetic disorder of metabolism, affecting 1.33-1.75 in 100,000 of the general population and 1 in 850 of people of Jewish (Ashkenazi) descent.[1] [Nalysnyk 2017, p69, col2, para3] Deficient activity of the lysosomal enzyme

glucocerebrosidase results in the accumulation of glucocerebroside lipids in the spleen, liver, bone marrow, and rarely the lungs or central nervous system.[2,3] [NORD 2020, col1, p1, para1; NORD 2020, col, p4, para1] Symptoms range from mild to severe and may include anemia, fatigue, bleeding tendency, enlarged spleen and liver with protruding stomach, and bone pain, as well as loss of bone strength and density with increased risk of fractures.[2] [NORD 2020, p4, col1, para2] Gaucher disease is classified as 3 types, with type 1 being the most prevalent and representing 94% of Gaucher disease cases in the western world.[4] [Charrow 2000, p2837, col1, Table1; Charrow 2000, p2837, col2, para3] Type 1 Gaucher disease varies substantially in patient symptoms and disease course but generally does not affect the nervous system.[2] [NORD 2020, p4, col1, para1; NORD 2020, p4, col1, para2] Patient symptoms can appear at any time but often present later in life.[5] [Stone 2023, p2, col1, para4] In contrast, less common types 2 and 3 are characterized by neurological and visceral involvement that appear early in life.[2,4] [NORD 2020, p4, col1, para3; NORD 2020, p5, col1, para1; NORD 2020, p5, col1, para2; Charrow 2000, p2837, col2, para3] Neurological symptoms include slow development, strabismus, hypertonia, back arching, abnormal head posturing, spasms, and seizures.[2] [NORD 2020, p5, col1, para1; NORD 2020, p5, col1, para2] Type 2 disease has a poor prognosis, with many children dying in infancy and few surviving beyond 2 years, whereas, types 1 and 3 are less severe with varying degrees of chronic ill-health.[2] [NORD 2020, p4, col1, para1; NORD 2020, p5, col1, para1; NORD 2020, p5, col1, para1]

Currently there is no cure for Gaucher disease, and treatments rely on distinct strategies to reduce symptoms through enzyme replacement or substrate reduction.[5] [Stone, p5, col1, para3] Enzyme replacement therapy (ERT) is administered by intravenous infusion of the deficient enzyme.[5] [Stone, p5, col1, para1] Three FDA-approved ERTs are available to treat the visceral manifestations of types 1 and 3 disease by providing recombinant forms of the human glucocerebrosidase enzyme.[5,6] [Stone, p5, col1, para1; Gaucher Disease Treatment, p1, col1, para3] However, these enzymes cannot access the brain due to the blood-brain barrier and therefore are not effective for treating the neurological symptoms associated with types 2 and 3 disease.[5] [Stone, p5, col1, para1] Substrate reduction therapy (SRT) is an orally administered

drug that can inhibit the first step in glycosphingolipid biosynthesis.[5] Currently, 2 FDA-approved SRTs are available to treat type 1 disease by inhibiting glucosylceramide synthase.[5] [Stone, p5, col1, para3]

Specialty Pharmacy

Complex, chronic, and/or rare conditions, such as Gaucher disease, require specialized therapies, high-cost medications, and specialized care management, all of which can be provided by specialty pharmacies.[7] [NASP, p1, col1, para1] Specialty medications provided by such pharmacies often have greater complexity compared with conventional prescription drugs due to drug storage, handling, and delivery requirements.[8] [Zuckerman 2019, p1, col1, para1] Other factors increasing their complexity may include administration processes, side effect management, manufacturer restrictions, payer authorization or compliance with specific benefit requirements, high costs, patient financial hardship, or some combination of these factors.[7,8] [Zuckerman 2019, p1, col1, para1; NASP, p1, col1, para3] By providing specialized medications, comprehensive care management, and patient support, specialty pharmacies contribute to improved patient outcomes, enhanced medication adherence, and better overall management of chronic and complex diseases.[8] [Zuckerman 2019, p2, col1, para3; Zuckerman 2019, p13, col1, para6]

Specialty medicines accounted for 55% of the prescription medicine market share for net spending in 2021, up from 28% in 2011, which has been driven by growth in autoimmune and oncology treatments.[9] [Fein-Long 2022, slide16] The increasing prevalence and complexity of these treatments demands specialized expertise to deliver personalized care, facilitating an emerging role for specialty pharmacies, as reflected in their 315% growth from 2015 to 2021.[9] [Fein-Long 2022, slide4] The fastest-growing types of specialty pharmacy are health-system specialty pharmacies (HSSP), accounting for a third of the sector in 2021.[9] [Fein-Long 2022, slide4] However, pharmacy benefit managers (PBMs), such as CVS Health, Express Scripts, OptumRx, and Alliance Rx Walgreens still dominate specialty dispensing, representing over three quarters of the specialty drug dispensing share in 2021.[9] [Fein-Long 2022, slide5]

Given the evolving position of specialty pharmacies in supporting optimal patient outcomes and their pivotal role in facilitating access to treatment for Gaucher patients, the Gaucher Community Alliance conducted a survey among US based patients to assess patient satisfaction with their services.

Methods

Survey Design

A survey was sent out to a Gaucher patient listserv of approximately 800 individual emails from March to April of 2023 in 3 separate mailings. The survey was also posted on Facebook within various Gaucher disease groups. The survey prompts are listed in Table 1 and include a 5-point Likert scale to assess patient satisfaction with specialty pharmacy as well as an open-ended question for the patient to share anything regarding their specialty pharmacy.

Table 1: Survey Prompts

Survey Prompts
The specialty pharmacy I currently use for treatment is:
I am happy with the service provided by my specialty pharmacy. (1-5 with 5 being the best service)
How many months of treatment does your pharmacy send you at each delivery?
Please tell us anything you would like us to know about your specialty pharmacy.

Results

Survey Participation

Seventy patients responded to the survey. Among the responses, 10 participants listed no specialty pharmacy or inappropriate answers, indicating a lack of understanding of the question. Therefore, the analysis omitted those 10 participants and was based on responses from 60 members.

Satisfaction Rating Analysis

Mean satisfaction ratings for the remaining 60 patients were stratified by specialty pharmacy and shown in Figure 1. Individual scores were also plotted to emphasize the difference in sampling among specialty pharmacies, which likely impact the mean rating. Mean satisfaction ratings were 4 or higher for all specialty pharmacies listed except for Alliance Rx Walgreens, Carelon Rx (IngenioRx), and Coram. However, the mean satisfaction ratings of these 3 specialty pharmacies should be evaluated with caution given their small sample size of 2-4 respondents each. Low ratings (≤2) and high ratings (≥4) were observed across most specialty pharmacies and generally independent of specialty pharmacy when considering that some had extremely small sample sizes (Figure 1).

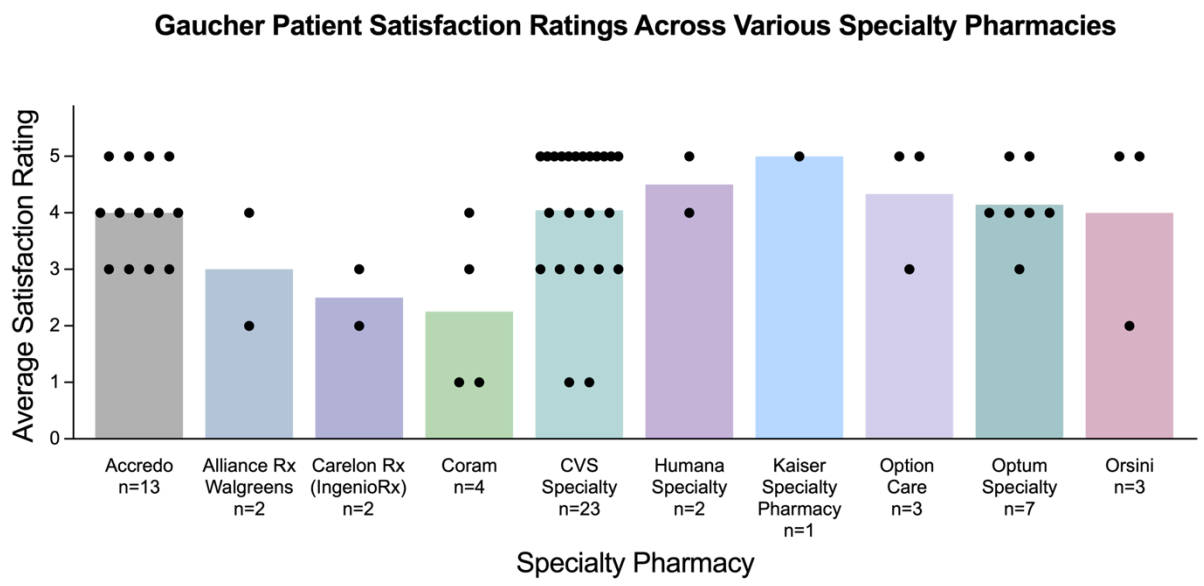

Figure 1: Gaucher Patient Satisfaction Ratings Across Various Specialty Pharmacies. Bars represent mean satisfaction ratings stratified by specialty pharmacy. Individual scores for each specialty pharmacy are plotted as black circles. Note that Coram is a subsidiary of CVS Specialty.

Treatment Supply per Order

Almost all respondents (57/60) indicated receiving 1 month of treatment supply per prescription order. Three respondents reported receiving a 2-month supply, which was independent of specialty pharmacy as orders were from 3 different specialty pharmacies.

Patient Reflection

Among the 60 participants included in the analysis, 42 responded when asked to share anything about their specialty pharmacy. The average satisfaction rating for participants who responded to this prompt was 3.8, whereas it was 4.4 for those not responding, suggesting that satisfied patients were less likely to share information. Among the participants, 19 had positive comments, 17 had negative comments, and 6 had neutral comments about their current specialty pharmacies. The average satisfaction rating was 4.8 among those with positive comments, 2.4 among those with negative comments, and 3.8 among those with neutral comments.

Analysis of the responses revealed that a longer treatment supply per order is desirable, with several respondents preferring a 2-month, 3-month, or quarterly supply rather than the 1-month supply they are currently receiving. One respondent noted that the 1-month supply is particularly challenging with frequent traveling and often leads to situations without medication. Another respondent expressed dissatisfaction with having to go through the authorization process each month, despite the prescription being good for a year.

> *"[Filling my prescription] is a nightmare every month. My prescription is good for a year, but they require an authorization every month. Most months it isn't authorized until the last moment, and I stress if I will receive my meds in time. I have no choice in the matter, so I have to deal with it."*

Several respondents communicated the inconvenience of having to speak with someone live each time an order needs to be filled because it consistently takes time out of their busy schedules, especially during business hours. Participants noted refilling prescriptions electronically or through an app is more convenient than speaking to an individual on the phone. However, some respondents had positive comments regarding their monthly live phone

conversations with their assigned representative. Overall, respondents emphasized that a consistent point person at the specialty pharmacy is desirable and helpful.

> *"I have been able to establish a relationship with one person who reaches out to make the drug shipment as smooth as possible. I'm grateful for her!"*

Two respondents reported using specialty pharmacies for decades and recently observing a decline in the quality of service, specifically noting late or lost shipments, missing supplies, unrefrigerated medication, and poor customer service. Also noted were major disruptions when CVS Specialty acquired other specialty pharmacies, leading to uncertainty in supply and dependence on the pharmaceutical company to cover treatment.

> *"In the last 10 years, I have experienced horrible customer service, lost shipments, shipments sent to the wrong address, missing supplies, medication that isn't refrigerated and more… The hardest part about living with Gaucher disease is having to deal with the team at my specialty pharmacy."*

Respondents noted frustration when specialty pharmacies do not handle insurance issues in a timely manner or forget to bill the copay program, resulting in incorrect billing. It is also frustrating to participants when specialty pharmacies attempt to change their infusion nurses, especially since their choice of specialty pharmacy is often dictated by insurance, making them feel powerless.

Inconsistency in scheduling refills and customer service is upsetting to respondents, who reported it has led to delayed treatments and missed infusions. Respondents were also dissatisfied when specialty pharmacies do not refer to special instructions from the customer, such as requests for smaller shipments with less ice, different delivery addresses, or delivery to the doctor's office.

> *"Every time they changed case managers, they messed up the shipment or didn't call at all. I felt like I was babysitting them."*

In addition to those already mentioned, favorable features of specialty pharmacy service included reminder emails and calls to refill prescriptions, packaging each infusion dose, ability to request a specific delivery date, on-time shipments, ability to honor special requests from patients, and ensuring all supplies are sent or at least immediately sent if there are missing components.

> *"They have been very good to us! They work hard to always have drug and supplies ready for us. They rarely have issues, but if they do, they have a courier bring anything we are missing ASAP. We highly recommend them."*

Discussion

Current FDA-approved treatments for Gaucher disease include 3 ERTs, namely Cerezyme® (imiglucerase), VPRIV® (velaglucerase alfa), Elelyso® (taliglucerase alfa), and 2 SRTs, including Cerdelga® (eliglustat) and Zavesca® (miglustat).[6] [Gaucher Disease Treatment, p1, col1, para3] Patients receive ERT via intravenous infusion every 2 weeks, either at an infusion center or at home, whereas SRTs are oral treatments. [Gaucher Disease Treatment, p2, col1, para2] The overall goals of these specialized treatments are to lessen signs and symptoms of disease, restore the highest level of patient wellness, prevent progression and new complications, and achieve a longer lifespan and higher quality of life.[10] [Roh 2022, p507, col1, para2] Specialized medicines for treating Gaucher disease have historically been prescribed through specialty pharmacy, and specialty pharmacy selection is often dictated by the insurance company.[8,11] [Kober 2008, p51, col1, para2; Zuckerman 2019, p12, col1, para1] As a result, the patient is in a position where they lack control over their own care regarding access to life-changing specialty medication, as these decisions are ultimately determined by external parties.[8] [Zuckerman 2019, p12, col1, para1; Zuckerman 2019, p12, col1, para2]

Specialty pharmacies were designed to provide an integrated approach to healthcare for individuals suffering from chronic disease and complex illness to attain enhanced clinical and economic results while facilitating prompt patient access to comprehensive care.[7] [NASP, p1, col1, para5] Their role is to connect patients who are severely ill with the life-changing but often costly medications indicated for their conditions, provide treatment-related care services, and support patients facing reimbursement challenges.[7] [NASP, p1, col1, para6; NASP, p1, col2, para1] Specialty pharmacies should ideally serve as the intermediary between the "5 Ps", being the patient, pharmaceutical company, prescriber, payer, and pipeline of new medications.[7] [NASP, p1, col2, para1] Hence, a high-performing specialty pharmacy can be a valuable partner for patients in achieving the best possible results. Conversely, inadequate specialty pharmacy services can be detrimental to the patient, given its pivotal role in ensuring optimal care and facilitating access to transformative treatments. In a chronic disease like Gaucher that requires monthly access to therapy, sub-optimal specialty pharmacy care can be especially impactful considering the frequent periodicity of interaction.

Given the intended role of specialty pharmacy and patients' limited control over their care, it is imperative to frequently assess the performance of specialty pharmacies using various metrics. The objective of this survey was to evaluate the level of satisfaction among patients with Gaucher disease regarding their current specialty pharmacy. Survey results revealed that patients are generally satisfied with their specialty pharmacy, with mean satisfaction ratings of 4 or higher for most specialty pharmacies. Low ratings (≤2) and high ratings (≥4) were observed across most reported specialty pharmacies and were largely independent of specialty pharmacy, indicating that patient satisfaction is primarily influenced by individual experiences with specialty pharmacy services rather than the shortcomings of a single specialty pharmacy. Therefore, all specialty pharmacies can glean valuable insights regarding the aspects of care substantially impacting patients' quality of life as indicated by the findings of this survey (Figure 2).

The survey indicated that patients desire the option for a longer treatment supply per order (ie, 2-3 month or quarterly supply), which would lead to less disruption to their lives, alleviate traveling issues, and reduce the frequency of the authorization process if mandated by insurance. Patients emphasized the importance of having a dedicated representative at the specialty pharmacy when issues occur. However, patients prefer the option of refilling prescriptions electronically or through an app at their own convenience rather than being required to speak to a live representative.

Specialty pharmacies can ensure efficient workflow by promptly handling insurance issues and integrating prompts to alert staff if patients are utilizing a copay program so as not to induce patient anxiety resulting from incorrect billing of high-cost specialty medications. Furthermore, specialty pharmacies can set up consistently timed refill reminders to prevent delayed treatments and missed infusions. Any supply and delivery issues should be proactively and immediately addressed by the specialty pharmacy to put the patient at ease. Specialty pharmacies can increase patient satisfaction by integrating any special requests into their file and ensuring adherence. It is also important for specialty pharmacies to understand that patients develop relationships with their infusion nurses and requiring them to switch may be disruptive and cause distress.

The suggested improvements are relatively simple to integrate into operations but may substantially improve patient quality of life. The negative impact of failing to implement such changes is demonstrated by some of the survey responses, for example: “the hardest part about living with Gaucher disease is having to deal with the team at my specialty pharmacy.” Gaucher disease is a highly debilitating chronic disease that substantially limits major life activities and causes appreciable pain. The notion that dealing with the specialty pharmacy is the worst part of the disease reflects the power of specialty pharmacy to impact patient quality of life and underscores its responsibility to ensure fair and equitable treatment of people who are already coping with a debilitating illness. The suggested minor adjustments to enhance patient

convenience for routinely refilled specialty medications are not specific to Gaucher disease and would likely benefit most specialty pharmacy patients.

The specialty pharmacy market is increasingly competitive and projected to grow by 8% per year through 2025 with the expected approval of emerging specialty medications.[9,12] [Fein-Long 2022, slide4; Fein-Long 2022, slide5; West 2022, p1, col1, para2] However, growing payer control continues to challenge patient access to medication, with high-cost specialty medicine being the most regulated.[9] [Fein-Long 2022, slide31] As a result, an increasing number of healthcare systems have established in-house specialty pharmacies (ie, HSSPs) to facilitate access to specialty medications.[8] [Zuckerman 2019, p2, col1, para2] Benefits of the vertical HSSP model have been demonstrated regarding clinical outcomes, financial impact to patients and the health system, higher patient and provider satisfaction, and enhanced efficiency in specialty pharmacy practice.[13] [Zuckerman 2022, p1907, col2, box] Payers and PBMs are now seeking partnerships with these HSSPs to benefit from the improved outcomes and lower costs.[12] [West 2022, p1, col1, para2; West 2022, p5, col1, para1]

As specialty pharmacies strive to differentiate themselves to acquire and retain customers, it behooves stakeholders to learn from insights revealed in assessments such as this survey. For example, survey participants who have been using specialty pharmacies for decades reported a recent decline in the quality of service. Additionally, major disruptions to patients accessing treatments were reported during transition periods following change of specialty pharmacy ownership. The insights garnered from this survey and others like it provide opportunities to inform decision-making and strategic planning regarding specialty pharmacy services.

Insights to Improve Patient Satisfaction with Specialty Pharmacy Services

- Option for longer treatment supply per order (ie, 2-3 month or quarterly supply)
- Dedicated patient representative at the specialty pharmacy
- Option of refilling prescriptions electronically or through an app
- Consistently timed refill reminders to prevent delayed treatments and missed infusions
- Prompt handling of issues leading to incorrect patient billing
- Proactive and immediate management of any supply or delivery issues
- Adherence to special patient requests when possible
- Prevention of treatment disruptions during transition periods/ownership changes

Figure 2: Summarized insights to improve patient satisfaction with specialty pharmacy services based on survey responses from Gaucher patients

Conclusions

Specialty pharmacies have been instrumental in providing specialized treatments and contributing to the management of Gaucher disease, resulting in improved patient outcomes. While the survey results indicate overall member satisfaction with their specialty pharmacies, some services warrant improvement, such as longer treatment supplies, dedicated representatives, and electronic prescription refills. These improvements can enhance patient convenience and minimize disruptions to their lives. In the evolving and competitive specialty pharmacy market, it is crucial for stakeholders to leverage insights gained from such surveys to deliver optimal care and customer satisfaction.

For more information

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