Optimizing care pathways for patients with rare diseases:

Insights and recommendations from the 2024 Becker's-Alnylam survey

NP-USA-00896.

Rare diseases can be difficult to recognize, which often limits patient access to necessary care and ultimately impacts patient outcomes.

Ensuring patients are connected to the most appropriate, effective care pathways starts with the health system's commitment to better identifying and diagnosing individuals with these conditions.

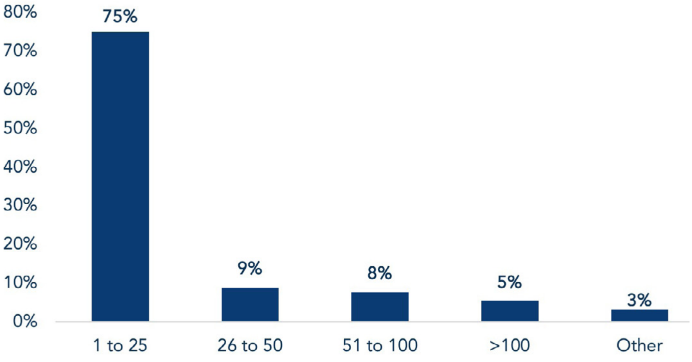
Becker's Healthcare recently conducted a survey in partnership with Alnylam Pharmaceuticals to learn more about the care of rare diseases and the role of health system pharmacy operations.

Alnylam has led the translation of RNA interference (RNAi) into a whole new class of medicines for conditions including hereditary ATTR (hATTR) amyloidosis with polyneuropathy, acute hepatic porphyria (AHP) and primary hyperoxaluria type 1 (PH1). The company collaborates with leading health systems to co-identify quality improvement data-driven projects that advance patient outcomes and optimize operational care pathways to expand patient access.

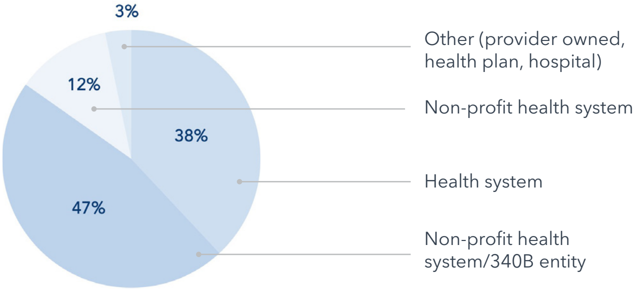
Ninety-two executives including senior pharmacy, service line leaders and administrators responded to the Becker's-Alnylam survey. Nearly all the participants (97%) represent health systems and over half (54%) are from a nonprofit or 340B entity health system. Three-quarters of survey participants work for institutions with between one and 25 hospitals, and over half (52%) work for institutions that have more than 500 beds at their flagship facility.

Demographics of Survey Respondents

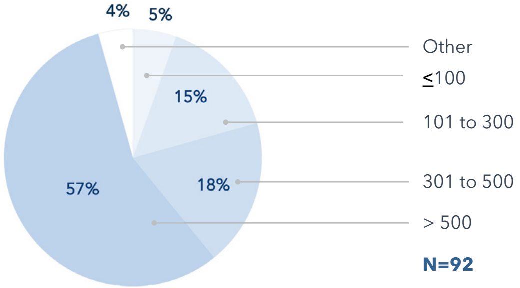
How many hospitals are in your health system?



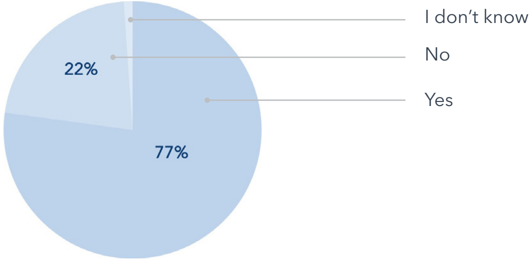
Please select the most accurate description of your organization. Please select all that apply



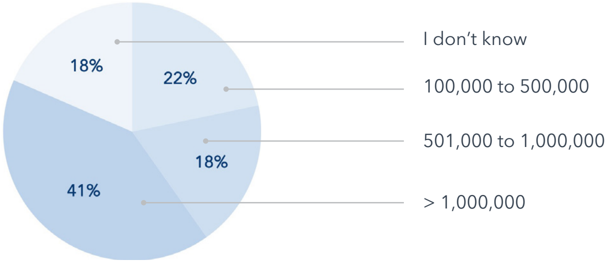
What is the size of your hospital(s) or flagship facilities?



Does your health system have/own in-house specialty pharmacy services?



Approximately how many patients does your health system/organization serve each year?



Education and multidisciplinary collaboration are key to improving rare disease diagnoses

The survey results demonstrated that healthcare organizations tend to use a range of typically disparate processes to uncover people with rare diseases.

48% of healthcare organizations rely on patient clinical questionnaires for each specialty department

36% rely on the genetics department or team

39% search the EHR system (conducted by individual clinic or the pharmacy team)

13% utilize family cascade screening

While multiple pathways can help to identify patients with rare diseases, over 25% of survey respondents indicated they don't know their organization's current approach.

"I think this is the case because rare disease is such a siloed channel," Tim Affeldt, PharmD, Vice President, Specialty Pharmacy and Infusion Pharmacy Operations at Fairview Pharmacy Services/M Health Fairview in Minneapolis, said. "There often isn't a system approach to identifying rare disease cases. You might develop a relationship with a particular rare disease provider or clinic from a pharmacy services perspective and understand how they identify patients. But you probably don't have a relationship with every rare disease provider."

These findings suggest:

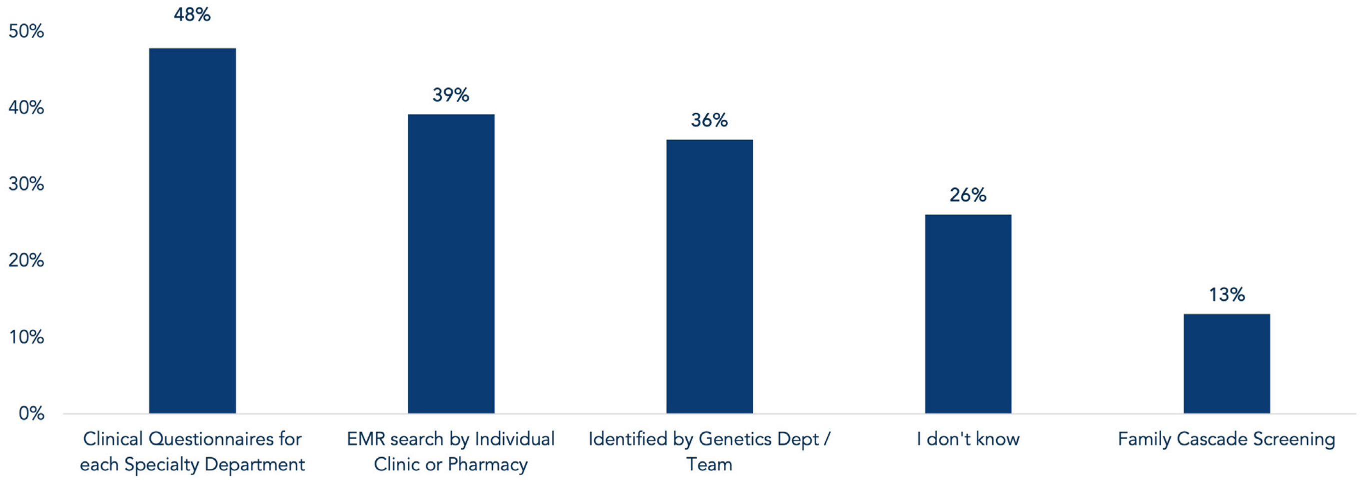
Education is needed so that key stakeholders can expand their awareness of how individuals with rare diseases are identified.

Greater internal **collaboration**, including care coordination, could help institutions identify patients with rare diseases by expanding appropriate usage of the EHR and family cascade screening.

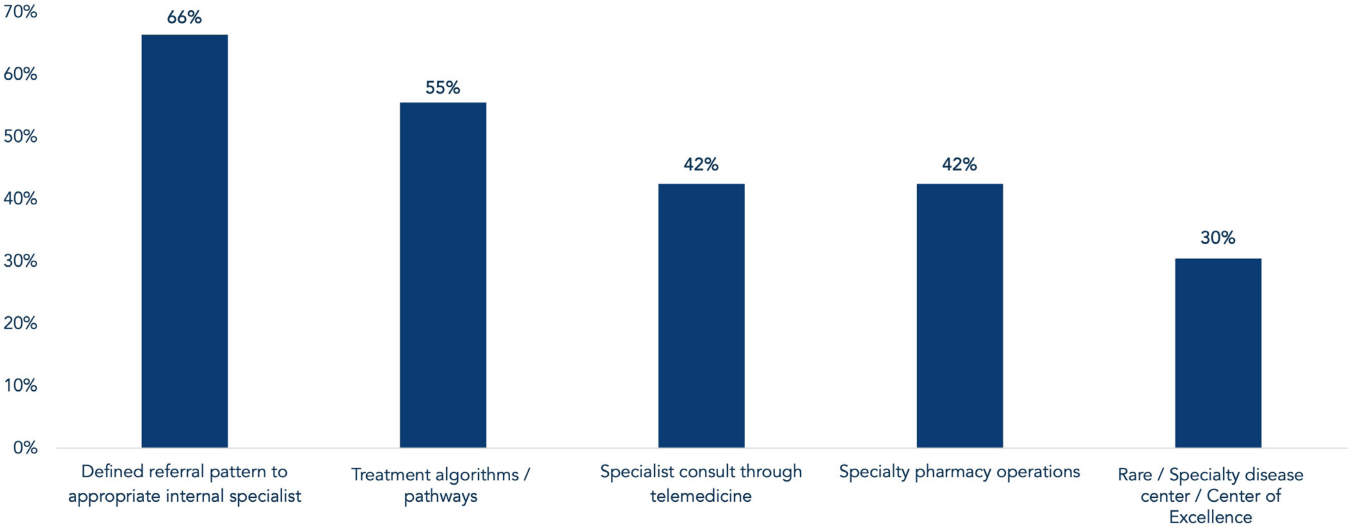
Fairview Pharmacy Services, for example, has created a rare disease committee which includes a variety of stakeholders, including nurses, pharmacy, finance and more.

"I think the rare disease committee will go a long way toward developing a more standardized approach for identifying patients with rare disease across the health system," Mr. Affeldt said.

How does your health system identify patients who need appropriate treatment for rare / specialty diseases? Select all that apply.



What infrastructure and processes do you have in place to diagnose and manage rare / specialty disease patients? Select all that apply.



Similarly, when it comes to diagnosing and treating patients, the Becker's-Alnylam survey found a majority of organizations use at least one of these processes:

66%

of survey respondents indicated they rely on defined referral patterns between specialists, with 9% noting that was the only process they use for diagnosing and treating patients with rare diseases.

55%

said their organization leverages treatment algorithms and pathways, with 8% noting this is the sole process they utilize.

Additional education in diagnosis and treatment processes, pathways or best practices could also help health systems find the best-performing combinations of methods tailored to their myriad priorities and the needs of their patients with rare diseases.

BEST PRACTICE RECOMMENDATION

Accelerate time to care and reduce provider burden by:

1. Ensuring robust disease education is available to all care delivery providers
2. Utilizing appropriate diagnostic/treatment algorithms in the EHR
3. Orchestrating tight care coordination processes across specialties and
4. Enabling a leadership role for pharmacy operations

A flexible approach to administering rare disease therapies – Quality and financial opportunities

The majority of survey participants said their organization uses different sites of care to administer rare disease therapies. Nearly all (85%) utilize the local hospital's outpatient infusion clinic, and one-third (36%) have home infusion or hospital-at-home services available to patients, both of which can provide important access to patients closer to their homes. Interestingly, 16% of respondents only listed one location for infusion site (typically an outpatient/ambulatory clinic), and 11% of respondents indicated that the main/flagship hospital campus was the sole location for administration of drugs. Relying solely on ambulatory outpatient clinics limits patient choice and access, and especially impacts people who live far away from the clinics.

Considering the rare disease patient journey, which can be long, arduous and frequently includes symptom progression, offering patients a variety of treatment administration venues (such as health system infusion centers, physician offices or at-home nurse visits) provides critical flexibility advantages. Integrating site-of-care shared decision-making between the patient and care team supports elevated patient satisfaction and quality of care measures. This strategy also enables a more predictable economic model that is better aligned with the health system's priorities and patient needs.

Over half of the organizations surveyed use independent infusion centers that are not part of the health system. Unfortunately, sending patients with rare diseases to an independent infusion center can negatively impact patient retention, quality of care and the financial well-being of health systems. Additionally, data related to treatments administered at third-party, independent infusion centers are not typically integrated with the health system EHR. As a result, when patients with rare diseases interact with their health system physicians and pharmacists, those providers may not have access to critical information.

When health systems refer patients to independent infusion centers, they are also unable to capture the economic benefits of the buy-and-bill reimbursement model. In this model, healthcare organizations purchase drugs directly from the wholesaler and then bill payers for the drug and administration costs.

Creating internal treatment venues instead of referring patients with rare diseases to independent infusion centers fills care gaps, optimizes the individual patient care/experience and improves patient retention. This has a positive impact on both patients and the bottom line. In addition, increasing buy-and-bill activity can lead to higher reimbursement rates, which bolster an organization's financial sustainability.

"For infusions, we often use our hospital outpatient department areas, which are infusion sites located in the four walls of our pharmacy," Binita Patel, PharmD, Vice President, Pharmacy Services at Memorial Hermann Health System in Houston, said. "We also look at patient convenience. If it's a subcutaneous injection, a clinic office is fine."

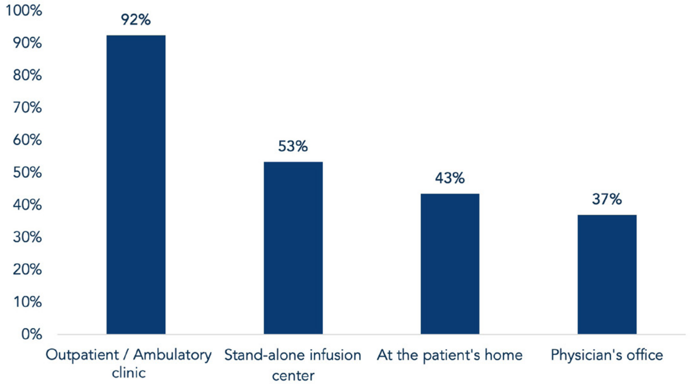
Aside from ambulatory outpatient clinics and independent infusion centers, 43% of survey participants indicated their organizations also use home care delivery to administer rare disease therapies, while 37% of health systems leverage physician offices. For administration of rare disease treatments, healthcare leaders may consider increasing their organizations' utilization of physician offices. This setting improves care quality for patients with rare diseases, while enabling health systems to control the drug lifecycle through the buy-and-bill model.

In some instances, health systems have integrated HCP-administered therapies into the physician clinic by embedding the health system pharmacy in the clinic as a site of care. As Nick Gazda, PharmD, MS, BCPS, CSP, Director of Oncology and Infusion Pharmacy Services at Cone Health explains: "Pharmacy can play an integral role in optimizing operations and clinical value for HCP-administered therapies. Having a coordinated and cohesive pharmacy team can help reduce denials, optimize site of care and patient preference, and support high quality clinical care through ensuring patient education and adherence."

BEST PRACTICE RECOMMENDATION

Enhance the experience for patients with rare diseases through optimizing site-of-care flexibility including infusion center, home infusion and provider clinic options as appropriate. Robust OCP planning in anticipation of new therapy market entrants ensures health system priorities are aligned with patient journey objectives.

At what sites of care does your organization administer infusion services? Select all that apply.



How the buy-and-bill model for rare disease treatments supports the Quintuple Aim

The majority of survey participants (75%) view care continuity and care quality as the primary advantages of buy-and-bill, compared to sending referrals to an external provider, and 39% cited patient satisfaction as the primary advantage. These findings are consistent with the priorities of the Quintuple Aim in health care, which focuses on improving patient experience, patient outcomes, clinician well-being, and health equity, while also lowering the cost of care.

As health systems make decisions about pursuing a buy-and-bill strategy, most take extra steps during benefit determination for high list-price drugs. Most respondents in the Becker's-Alnylam survey also indicated their organizations will consider buy-and-bill as long as payers will provide adequate reimbursement for treatments. Developing a proactive, cross-functional benefits verification strategy with drug manufacturers can help address these issues.

"We start by asking whether we can access the drug," Ms. Patel said. "That's based on the payer restrictions. We don't allow white bagging in our hospitals. We have to be able to purchase the drug, so we know the chain of custody. Once we access it, can we get reimbursed for it? We do all of this as part of the pharmacy process, before the patient hits the chair."

According to survey participants, payer considerations are the greatest potential barrier to buy-and-bill programs for rare disease treatments. Respondents noted the most significant concerns or barriers to these programs are:

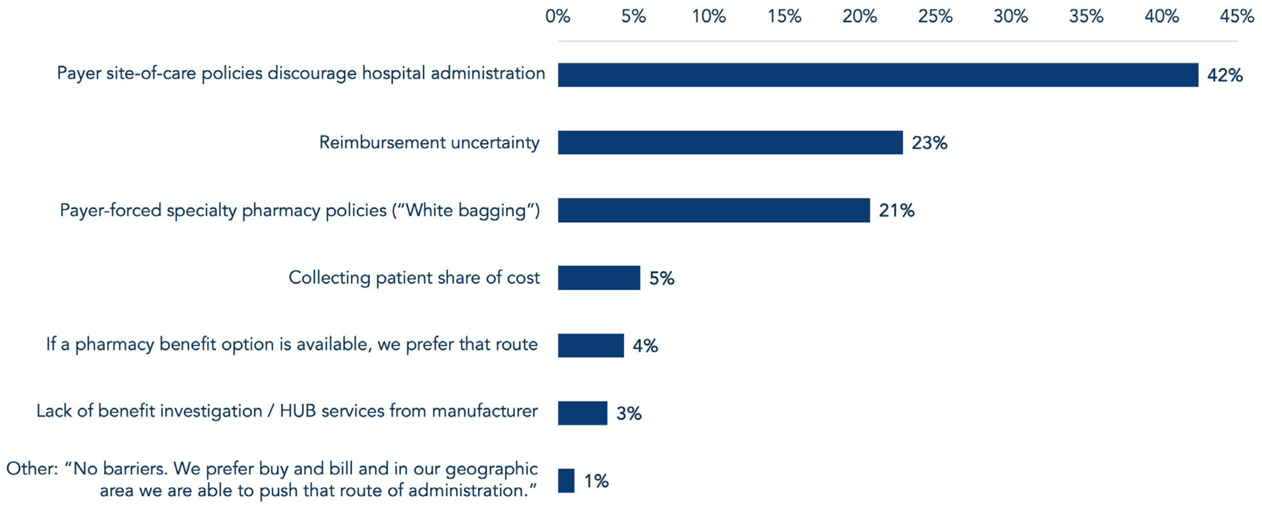
Site-of-care policies	**42%**
Reimbursement uncertainty	**23%**
Payer-enforced pharmacy policies – also known as "white bagging"	**20%**

To verify coverage, most health systems use cross-functional resources. Over 50% of survey respondents rely heavily on pharmacy operations to verify coverage and oversee financial processes for buy-and-bill. The same proportion of survey respondents indicated they actively engage manufacturer resources.

BEST PRACTICE RECOMMENDATION

Effective implementation of a buy-and-bill strategy for rare diseases supports care continuity and care quality goal achievement. Deploying health system cross-functional resources, including pharmacy operations as well as engaging manufacturers, are critical components.

Greatest potential barriers to conducting buy & bill in rare / specialty diseases



Case Study: **Implementing innovative operational care pathways for patients with rare diseases**

To improve treatment for patients with rare diseases, Alnylam Pharmaceuticals recently worked with an academic medical center in Southern California to optimize an operational care pathway (OCP) for patients with hATTR amyloidosis with polyneuropathy. Teams from both organizations worked closely together to co-develop a new workflow and an impact analysis that ensured alignment with the health system's operating goals.

As part of the project, Alnylam Pharmaceuticals educated the formulary committee about a treatment option for patients with hATTR amyloidosis with polyneuropathy, supported healthcare providers with disease and clinical management information, and informed the care team about product billing and coding requirements, as well as prior authorization, claims and appeals processes.

The new operational care pathway for patients with hATTR amyloidosis with polyneuropathy has five key steps:

1. The patient is scheduled for treatment at the prescribing clinic.

2. Nurses order treatment through a Pyxis™ medication-dispensing system and the pharmacy tech loads the medication into Pyxis™.

3. The nurse administers treatment in the clinic setting.

4. The pharmacist tracks the next dose and notifies the nursing team.

5. The nurses submit the order and existing prior authorization form.

With the new OCP, patients with hATTR amyloidosis with polyneuropathy patients can receive treatment in the prescribing clinic, which enhances their care experience. Since inception, the OCP has decreased the time from diagnosis to starting therapy, and increased treatment adherence among patients. From an operational perspective, process complexities have been reduced and clinic nurses can now work at the top of their license.

When it comes to identifying and treating patients with rare diseases, it's important for health systems to recognize there is more than one "right" approach. It's essential to look beyond the lens of the specialty pharmacy alone and consider more broadly what's best for patients, as well as the health system.

Implementing best practices related to diagnostic pathways, treatment administration sites, and reimbursement processes by integrating the buy-and-bill model is an effective way to optimize OCPs for patients with rare diseases.

This strategy enables health systems to significantly improve patient care, while also confronting the economic headwinds facing the healthcare sector. While the number of patients with rare diseases may be relatively small, the value of each patient to the health system is significant when viewed through the priorities of Quintuple Aim.