

The value of an **expert specialty pharmacy** in treating rare diseases

We've divided our paper into three sections based on audience:

BIOPHARMA →

PROVIDER →

PATIENT →

What is a rare disease?

A rare disease is defined as a condition affecting 200,000 Americans or fewer. About 7,000 rare diseases have been identified, 90% of which remain unmet with approved treatment options.¹

In 2023, 51% of novel drug approvals granted by the Center for Drug Evaluation and Research (CDER) received orphan drug designation as they target rare diseases, rare cancers, or tumors.²



Patients with rare diseases face undue and unpredictable challenges in accessing effective, affordable treatments.



Pharmaceutical companies struggle to define a channel distribution strategy that balances the need for specialized capabilities with an affordable distribution structure.

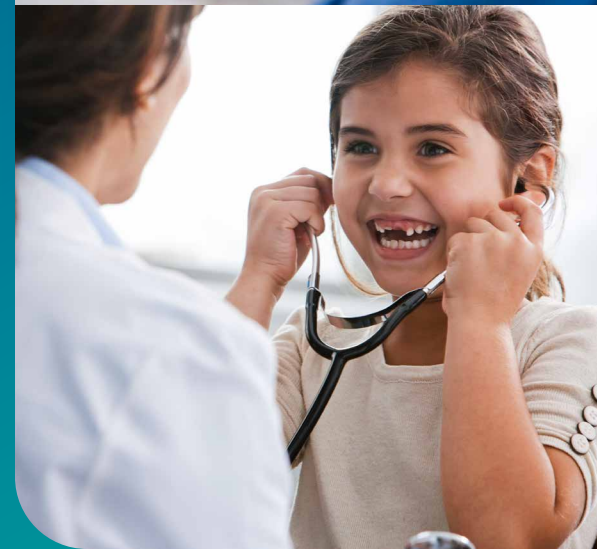
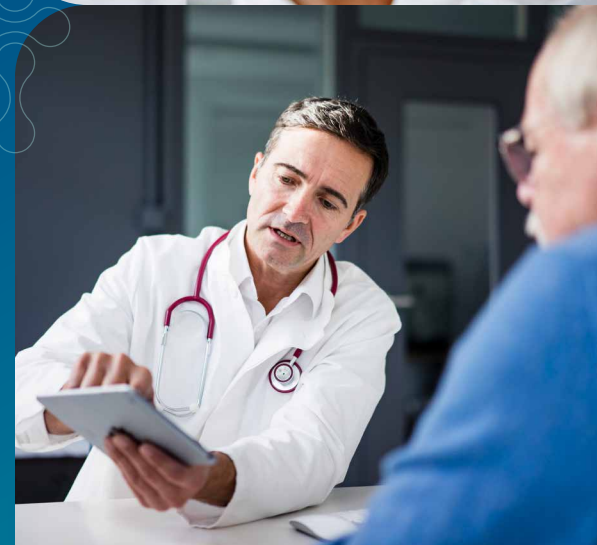


Providers want to keep their patients adherent to their therapy but face a heavy lift with administrative duties related to patient care.

Each of these considerations underscore the collective challenges shaping the increasingly complex rare disease landscape. In this paper, we outline how an experienced specialty pharmacy partner can curb the effects of such challenges with industry experience and specialized capabilities for serious, challenging, and under-addressed conditions.

1. <https://rarediseases.org/>

2. <https://www.fda.gov/media/175253/download?attachment>



BIOPHARMA



Supporting the orphan commercialization process

The journey from clinical development to commercial access presents distinctive challenges. As biopharma manufacturers shift their focus from drug discovery and regulatory functions to commercial planning, they must leverage the commercial expertise residing within their selected specialty pharmacy partners.

Rare drug manufacturers — many of which are new organizations, almost exclusively focusing on small, orphan populations — are seeking smooth commercialization paths that run parallel with simplified treatment journeys for their patients.

The right specialty pharmacy partner should fortify these efforts, accelerate the learning curve, and work to minimize potential duplicative services that could impact program efficiency and the overall bottom line.

When identifying and evaluating the right channel distribution strategy, there are several critical factors to be considered. Chief among them is striking a balance between both access and cost

containment. To answer this need, biopharma can leverage established pharmacy partners, capable of collaboratively coordinating care for patients across various stakeholders and entities. This experience powers expediency and ensures that any redundancies in services are avoided.

The ideal partnership should offer a long-term vision balanced with real-time tactility. There should also be a good line of sight between the launch and long-term corporate priorities, e.g., planned product expansions.

Biologics understands the importance of providing biopharma partners with a unified, multidisciplinary team to support their unique needs.



“In the dynamic world of orphan product development, Biologics is confident in our experience working with our biopharma partners to develop detailed commercialization plans, providing unique support along the way. With **unwavering dedication to our patients and partners**, we navigate the ever-changing landscape, confident that our commitment to innovation and patient impact will prevail.”

— **Ela Lourido**, Vice President and General Manager, Biologics by McKesson



Biologics has more than 30 years of industry experience. We assist our customers as they navigate the complex and fragmented healthcare industry for highly complex orphan-designated therapies. We have been thoughtfully uniting with biopharma partners offering supportive expertise to solve the complex, new challenges surrounding rare disease therapies.



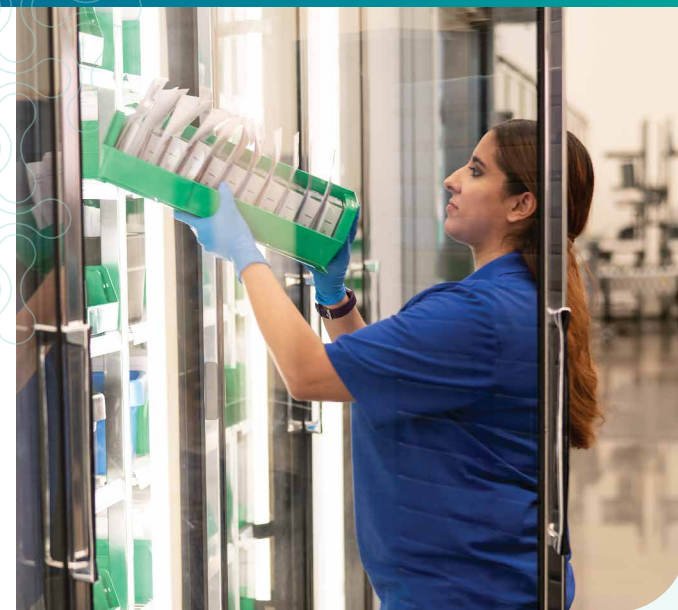
Biologics helps our biopharma partners collect meaningful data. As an extension of our strategic and data-driven thinking, we empower post-marketing analysis and capture of real-world observations at the population level. These include Quality of Life (QoL) assessments, Patient-Reported Outcomes (PROs), and other long-term valuations to strengthen evidence generation.



Biologics meets premier accreditation standards. Underscoring our remarkable commitment to providing enhanced patient outcomes, Biologics has received dual accreditation as a Utilization Review Accreditation Commission (URAC) 4.0 Specialty Pharmacy with Rare Disease Pharmacy Center of Excellence Designation and Accreditation Commission for Health Care (ACHC) Specialty Pharmacy Accreditation with Distinction in Oncology and Distinction in Rare Diseases and Orphan Drugs. Recognition from these premier third-party organizations validates the quality and efficiency of our pharmacy operations, primarily concerning our patient management programs.



Biologics offers deep, dynamic capabilities to navigate market uncertainties. To position a product for success, a biopharma manufacturer should select strategic partnerships that understand brand and product challenges as well as remain highly engaged and responsive to the current and future direction of the market. Biologics anticipates orphan product development to prove resilient in the face of recent policy reforms. Experienced pharmacy partners are accustomed to managing lifecycle needs, including developments impacting the economic model of an orphan product. Having weathered various, similar headwinds, your pharmacy partnership should be leveraged in managing future priorities and investments.



Biologics biopharma insights

- Supports **upwards of 100 therapies** that have received FDA Orphan Drug Designation
- Contracted to support **over 115 active limited drug distribution programs**
- Participates in highly limited distribution networks of 1 of 3 or fewer specialty pharmacy providers for **over 40 therapies**
- Offers **18 exclusive therapies**, with 8 in rare

PROVIDERS



Working together to deliver exceptional care to every patient

Not every specialty pharmacy is equipped to care for patients with rare diseases or provide a dedicated therapeutic focus. Experienced specialty pharmacies, such as Biologics by McKesson, understand the challenges for patients who are particularly prone to therapeutic disruption and may benefit from individualized support.

Because rare diseases impact a smaller percentage of the population, navigating the journey from diagnosis to treatment of these complex, chronic conditions can be challenging. These disease states are often so rare that common information and scientific knowledge are often lacking — making it unrealistic to expect doctors to be familiar with these thousands of rare conditions, or their treatment options.

Therapies for rare diseases are often high cost and require more clinical support and, often, administration. Specialty pharmacies receive

additional training and education in dispensing rare disease drugs. As such, Biologics is able to provide hands-on support for both providers and patients during the entire treatment journey.

Understanding rare and orphan diseases

With nearly three decades of experience in supporting patients with rare conditions, Biologics understands the challenges of this community and strategically targets highly complex therapeutic areas requiring the high-touch support of a specialty pharmacy provider.

Let the specialty pharmacy lighten the administrative load



Biologics is adept at navigating patient benefits and the complexities of patient coverage, including helping to connect patients with organizations that provide financial assistance.



We ensure providers are equipped with the most current scientific literature for payer determinations, formulary exceptions, prior authorizations or appeals.



Closed loop communication keeps providers aware of patient challenges or progress while they are not in their provider's direct care.

Caring for patients with rare diseases, especially in pediatrics, is an arduous journey and **caregivers play a pivotal role** in ensuring their wellbeing.



Specialty pharmacies have the capability to have dedicated rare disease clinicians to support your patient's journey

Outstanding patient care starts with an established support system designed to help people living with rare diseases. With newer therapies, the specialty pharmacy is often asked to play a key role in educating patients, managing care, and recognizing adverse events with an emphasis on medication adherence.

Enhancing caregiver support for patients on rare disease drugs

Caring for patients with rare diseases, especially in pediatrics, is an arduous journey and caregivers play a pivotal role in ensuring their wellbeing. However, they often face numerous obstacles that can hinder their ability to provide optimal care.

Caregivers of patients on rare disease drugs face a myriad of challenges that demand our empathy and attention. It is crucial to acknowledge and comprehend these hurdles to develop effective support systems. Some key challenges include:



Limited awareness and resources

Rare diseases often receive minimal attention, resulting in a lack of awareness and limited resources for both patients and caregivers.



Emotional and psychological burden

Caring for a patient, specifically a child, with a rare disease can be emotionally and psychologically draining for caregivers, leading to stress, anxiety, and burnout.



Financial constraints

Rare disease treatments can be expensive, placing a significant financial burden on caregivers who may struggle to afford necessary medications, therapies, and support services.



Isolation and lack of support networks

Caregivers may feel isolated due to the rarity of their loved one's condition, making it challenging to find others who can relate to their experiences and provide much-needed support.

Addressing the challenges faced by caregivers of patients on rare disease drugs requires a collaborative effort from various stakeholders. Biologics supports caregivers (once registered) by helping them update pivotal health information, connecting them with financial assistance, communicating with them on behalf of the patient, and ordering refills — all to give caregivers more time to focus on caring for their loved ones.

How specialty pharmacy drives interdisciplinary collaboration

Clinical support can mitigate the wide array of barriers to adherence, interconnecting determinants such as emotional wellness, ability to maintain activity levels, quality of life, family support and more. A specialty pharmacy must develop targeted interventions personalized to the individual patient's needs and circumstances. Biologics often explores various opportunities to tailor patient engagement, providing a dedicated resource for guidance and support. Through this level of commitment, our clinicians can provide guidance in line with the patient population, and in collaboration with their physicians.

A specialty pharmacy experienced in treating patients with rare diseases has several methods and frequencies for patient engagement and calls, texts or emails them based on their communication preferences. Patients are more likely to be receptive to clinical guidance when received via their preferred communication method.



It can identify when patients over- or under-utilize a therapy, as well as the factors leading to these adherence issues.



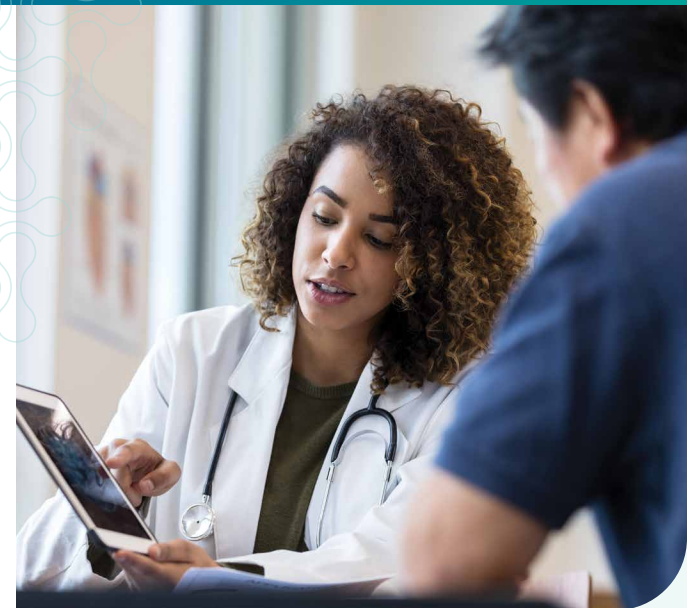
It is accustomed to collaborating to achieve a common goal. Distribution of orphan drugs to patients is a multi-stakeholder process. Your specialty pharmacy should communicate with the multidisciplinary teams in place supporting the patient to ensure an uninterrupted chain of communication.



It can pinpoint the most vulnerable patients. Employing risk-based segmentation to identify patients who may benefit from additional touchpoints can help to improve medication adherence. Patients with chronic, and oftentimes life-threatening rare diseases are more likely to be readmitted to the hospital, stay in the hospital longer and incur significantly greater healthcare costs. By establishing consistent outreach from the beginning of therapy, a specialty pharmacy can gain insights into a patient's situation, tailor their level of support, and adjust their plan of care as needed in lockstep with the provider.



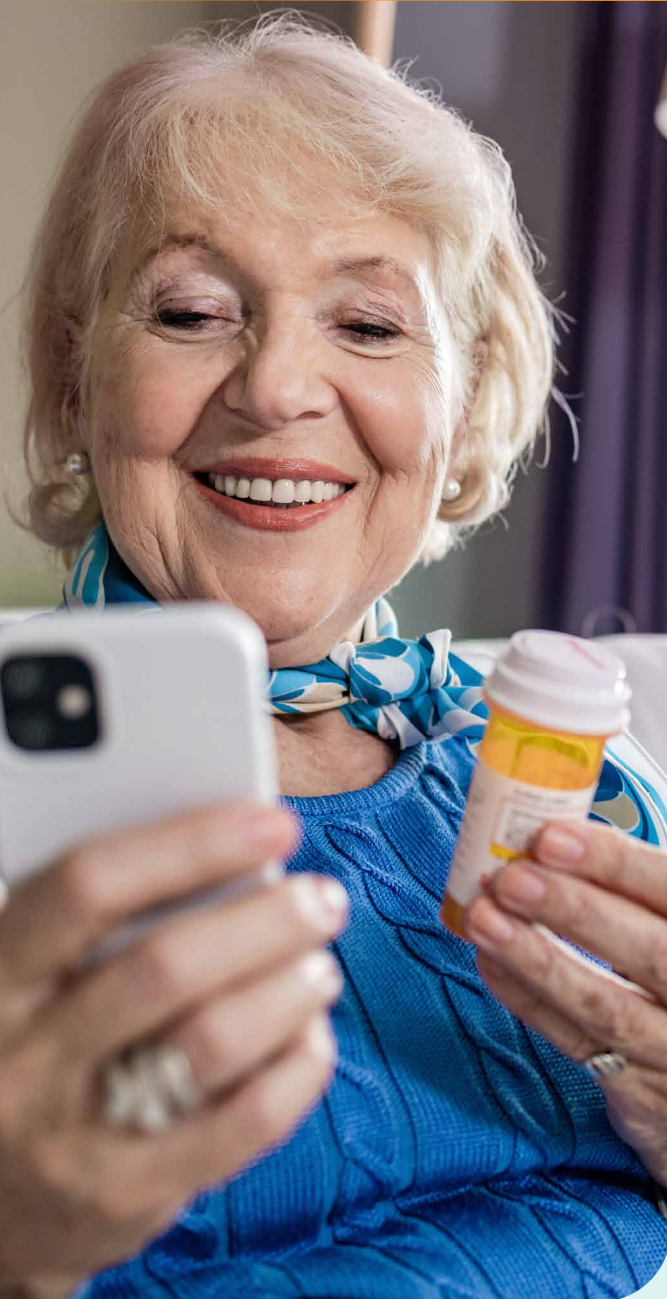
It pursues continuous clinical personalization. Frequently working with smaller subsets of patients and disease states allows the specialty pharmacy to concentrate on providing care that is consistent in its clinical focus and quality — while also personalized and specific in its content and delivery.



Biologics provider insights

- Industry-leading access to **over 200 rare and oncology therapies**
- **Extensive payer networks** and exclusive limited drug distribution agreements
- **Patient adherence programs** to promote favorable outcomes

PATIENTS



Providing personalized care to every patient is at the heart of what Biologics does

All too commonly, rare diseases may take years to accurately diagnose, and then patients are often faced with limited treatment options, or none. Many patients have spent years independently managing their condition, so the care and support of a dedicated team is welcomed. Allowing a specialty pharmacy to participate in a patient's care is an enormous privilege and an opportunity to demonstrate Biologics' concentrated skill set.

A specialty pharmacy can help patients navigate the following complexities:



Provide thoughtful and compassionate education on how to access a therapy that could change the trajectory of their life.



Educate patients regarding additional financial resources that may help cover medication costs, or other ancillary needs such as treatment-related travel.



Ensure known/avoidable adverse events are identified before they threaten to disrupt therapy.

Biologics is **devoted to providing our patients with the highest level of care** during every phase of their treatment.



Biologics patient insights

- **Over \$123M** in financial assistance was secured for patients in 2023
- **70+ clinicians** to focus on individualized treatment journeys
- **Dedicated teams** to place heightened focus on programs with a degree of holistic complexity

Biologics by McKesson's service model helps oncology and rare and orphan patients navigate the complex healthcare landscape.

With three decades of experience supporting patients with rare cancers, Biologics has emerged through evolving market dynamics as a pioneer in the industry and strategically branched out to support additional, highly complex therapeutic areas that require the high-touch support of a specialty pharmacy provider.

For more information, visit our website at biologics.mckesson.com/rare.