IMPROVED PATIENT OUTCOMES AND FINANCIAL PERFORMANCE

THE VALUE OF INTEGRATED OUTSOURCED SERVICES SUPPORTING RARE DISEASE PHARMACEUTICAL COMPANIES.

DOHMEN®
LIFE SCIENCE SERVICES

By 2020, the global cost of orphan drugs is expected to reach 20 percent of total non-generic drug sales, which are projected at $879 billion.

INTRODUCTION

The drugs developed to treat rare diseases face an improbable journey from development to delivery. If they make it through the decade-long process to receiving FDA approval, they must still survive an arduous trip into the hands of the patient, sometimes passing through six levels of middlemen.

Unfortunately, the convoluted system of orphan drug delivery directly contributes to the skyrocketing costs in healthcare, and as rare drugs proliferate, the impact will multiply. Rare disease drugs already make up a significant portion of the total prescription drug market, according to Evaluate Pharma's 2015 Orphan Drug Report. Orphan drugs made up $114 billion, or 16 percent, of worldwide brand prescription drug sales in 2016. By 2020, the global cost of orphan drugs is expected to reach 20 percent of total non-generic drug sales, which are projected at $879 billion.

The U.S. healthcare system is speeding toward an outcomes-based financial model, and the shift will have repercussions on the specialty drug industry. No matter how Obamacare gets replaced, reimbursement models will start to shift for rare disease drugs and therapies. In 2015, several of the nation's largest private insurance companies teamed up with healthcare systems to create a task force to shift at least 75 percent of their business model to connect payments with patient outcomes by 2020. This shift will impact drug manufacturers, placing an even greater burden on them to provide comprehensive, wrap-around patient service that goes far beyond therapy delivery.

ONLY
58-65%
RARE DISEASE
THERAPY ADHERENCE

Why? Therapy adherence remains a critical concern in small patient populations. Drug manufacturers and healthcare providers should both be concerned by a report from Global Genes showing therapy adherence in the rare space to vary between 58 to 65 percent, meaning that there is a considerable amount of work to be done to help people properly manage their therapy and thus their disease.

Typically, manufacturers outsource patient services to disparate providers – undermining a holistic approach to healthcare. Rising costs and a breakdown in patient service from too many uncoordinated middlemen have created a public perception problem for drug manufacturers: 72 percent of Americans feel that drug costs are unreasonable and 74 percent feel that drug companies put profits before people, according to the August Kaiser Health Tracking poll. This sentiment is heightened in the rare disease community.

Neither orphan drug manufacturers nor their patients with rare diseases are served well by the traditional multi-channel model of drug delivery and patient service. A new integrated model increases adherence and improves patient outcomes, which in turn leads to better financial performance for manufacturers.

$114 BILLION

2016 ORPHAN DRUG MARKET

17%

ADDED MARKUP

$19.4 BILLION

SAVINGS POTENTIAL

CHALLENGES FOR MANUFACTURERS

From purely a financial standpoint, a drug manufacturer has one overarching priority: to get reimbursed for a patient's treatment. Thus, manufacturers have a financial incentive to make sure as many patients as possible get on commercial treatment and stay adherent.

"The most difficult challenge is trying to identify and understand why patients slip through the cracks," said Paul Merrigan, a pharmaceutical executive with more than three decades of commercial operations and marketing expertise.

With the small, complex patient populations typical of the rare disease field, the traditional outsourced services model falls behind on onboarding, patient support, and data collection and analysis—all failings which can harm a drug's brand, or worse, prevent a patient from completing or continuing long-term treatment and limit the manufacturer from getting reimbursed.

For this reason, let's look closer at the traditional model. Rare diseases drugs and the data associated with them typically flow through up to a half-dozen disjointed, uncoordinated middlemen. The process could include a wholesaler/3PL, a distributor, a patient services and reimbursement hub, a Risk Evaluation and Mitigation Strategies (REMS) coordinating center, a healthcare provider, a Pharmacy Benefit Manager (PBM) , and finally, a specialty pharmacy, before the drug is shipped to the patient and makes it into their body.

In many cases, the price of the therapy is marked up, or discounted, in various ways at each level, which often lacks transparency and inflates the price. As a result, manufacturers, knowing that they will have to provide discounts along the supply chain, may be compelled to price the product higher as their net price will be significantly lower – similar to being subjected to the mandatory rebates and discounts provided to government programs like Medicaid, 340B hospitals and the Military's Federal Supply Schedule. According to Milliman research, payer cost is 17 percent higher than manufacturer cost. It's estimated that payers are covering $19.4 billion in markups (17 percent of $114 billion) that could include:

- Wholesaler margin,
- Pharmacy margin,
- PBM spread (i.e., the difference between what the PBMs charge their customers and what they pay the pharmacies), and
- PBM retention of a portion of rebates.

That's $19.4 billion in the rare disease market alone, going to maybe six different players that sit between an innovative life science company producing critically important therapies and a rare disease patient that relies upon them. Some of the markup can happen without a drug manufacturer's approval or knowledge.

Marie E. Lamont, president of Patient Services for Dohmen Life Science Services (DLSS), cited the example of a company that suffered damage to its reputation after a doctor discovered a disparity of more than $50,000 in price between two identical therapies sold to two different patients. The therapy was provided through two different distribution channels, and under one it saw outrageous markup, and under the other it didn't.

"The physician told the manufacturer, I don't want to do business with you because you're gouging prices," Lamont said. "But under the traditional industry model, the manufacturer had no control of the price after selling to the distributor. And in this case, that meant little control of their reputation either."

Onboarding challenges

Simply getting a patient onto a therapy can be a challenge. "Once they go through a long, frustrating journey to diagnosis, then it's a matter of getting prescribed and beginning to take therapy," Merrigan said. "There's a whole second journey of benefit verification, hub patient and reimbursement services, financial assistance and finding out which specialty pharmacy will serve the patient based on their insurance coverage."

869 MILLION

HOURS OF LOST PRODUCTIVITY IN PRIOR AUTHORIZATION PROCESS

The average rare disease takes 7 years to get correctly diagnosed after the onset of symptoms. But following that diagnosis, it can still take more than a year for a patient to begin treatment because of the multi-step process required by their insurer to approve reimbursement of the drug. That process, called prior authorization, is typically handled by the physician, and is responsible for 868.4 million hours of lost productivity per year among American healthcare providers. If the insurer denies coverage, the responsibility falls to the patient to appeal and almost always requires the assistance of the provider's office staff. Meanwhile, the patient, who may have remained unaware of the reason for the delay in authorization, continues to suffer from the disease, which in some cases could mean suffering irreparable damage from a degenerative disease.

Patient support challenges

The day a patient starts therapy, the drug manufacturer then turns its attention to optimizing adherence, mitigating side effects and monitoring outcomes.

"If the patient doesn't do well, for instance if the drug doesn't deliver on the value of the clinical performance, for whatever reason, then your revenue stream is at risk," Merrigan said. "If the manufacturer doesn't get paid for the product they are delivering, then no one is going to innovate and bring new products to market."

Rare disease drugs come with a potential for major side effects and requirements ranging from a highly specialized diet to extremely unpleasant physical effects – all impacting the probability of short and long-term adherence.

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But with small patient populations, large specialty pharmacies that handle hundreds of drugs may not be experts in the disease or the patient experience of one particular drug.

"You don't want to trust a large, big box retail pharmacy to be highly educated on a rare disease," Merrigan said. "They have no core team dedicated to the disease and high turnover.That's just one thing of many that they do. That might be a detriment to ultimately the patient's experience with your product."

Unfortunately, many patients learn to dread contacting their insurance company. "I had to deal with my insurance company this week, and I knew as soon as I picked up the phone I would be in a voicemail nightmare," said Mary Pierce, Alpha-1 patient and DLSS patient advocate. "I got shuttled around two or three times at least."

Patients also question their physicians' expertise regarding their clinical needs. Given the difficulty of obtaining an accurate diagnosis and the fact that 80 percent of rare diseases have a genetic component, family members often know more about available treatments than healthcare professionals, who may have limited experience with the disease and scant information on how best to treat it.

Without support from a knowledgeable physician or a pharmacist, some patients will stop therapy after experiencing side effects, or their outcomes will suffer for other reasons. In either case, if the patient could be helped, but stops taking the therapy, it's a "lose-lose" situation for the patient and the manufacturer, and for that matter, the whole system.

"We want the patient experience to be so smooth that they think about us not just for our drug, but as a company that has made the whole experience they go through easy," Merrigan said.

Data challenges

One additional challenge the traditional model presents concerns data collection, analysis and reporting. With many rare diseases, the clinical trials aren't long enough to demonstrate outcomes, they just provide hope of an outcome. To maintain its FDA approval, a manufacturer may need to continue to gather data on patient outcomes, or it may want to gather data for its own continuous improvement purposes.

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To be effective, this data must be gathered in a consistent, standardized way with minimal variation allowing for accurate analyses. Fragmented hub and delivery operations make it nearly impossible to gather insightful data to analyze and act upon patient feedback and program enhancements.

To solve the problem of fragmented data across a myriad of providers, especially when multiple specialty pharmacies are involved, yet another middleman has

"If you had just one specialty pharmacy, you could be in a better position to have them provide accurate and complete data in a format that's easy to analyze and track important metrics."

joined the fray. "The data aggregator brings all the data together across the information and supply chain," Merrigan said. That incurs yet another cost, but may be necessary to ensure quality patient services and effective business decision-making.

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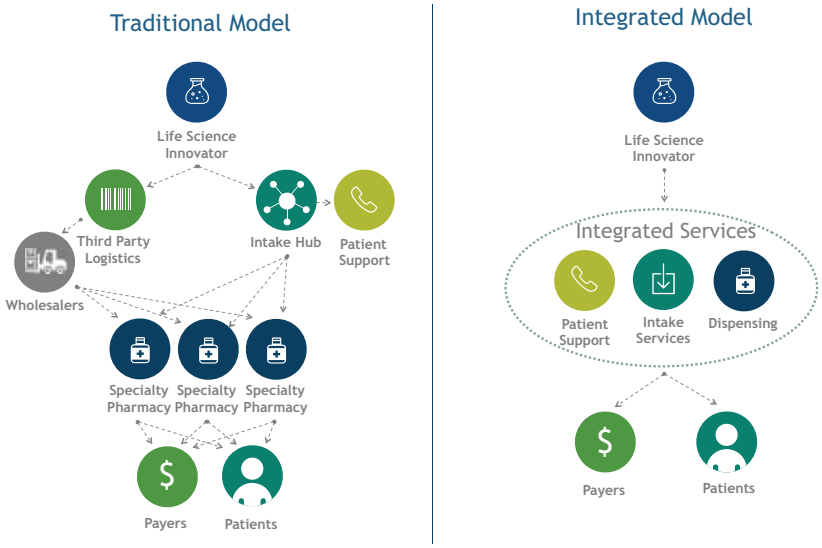
AN INTEGRATED MODEL PROVIDES AN ALTERNATIVE

The challenges of onboarding, inconsistent patient support and fragmented patient data could all be mitigated if one company followed each patient from diagnosis through therapy adherence to create a better quality of life.

"In an ideal situation, everyone in the industry would like to do everything themselves," Merrigan said. "But it costs a lot of money and the infrastructure investment is huge. Often it's not even possible for a manufacturer to cover."

A new model is emerging in the rare disease field: One company with a broad range of services develops a tailored program for a manufacturer's needs, providing significant advantages to the manufacturer and their patients.

DLSS is a pioneer in this integrated outsourced services model. DLSS signs on to serve as an extension of the drug manufacturer to deliver high-touch care as the exclusive distributor of therapy and the patient services provider, taking the place of the wholesaler, specialty pharmacy and the hub.



"It's a partnership with the manufacturer, their patients and us," said Lamont. "We create a stronger, more simplified connection."

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Better for the manufacturer

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Operating on a fee-for-service basis, DLSS never adds markup to the price of a drug. As the exclusive provider of a therapy, DLSS assigns dedicated teams to serve an entire patient population as experts in overcoming prior authorization hurdles, managing side effects, and holding a patient's hand through a difficult time.

Merrigan cites one example where the new model delivered better patient outcomes for a therapy that induced major tolerability issues if not managed properly. The therapy required patients to go on an ultra-low-fat diet mandating that no more than 15 percent of calories come from fat. If they didn't follow their eating plan, the interaction of the drug with excess fat intake could cause diarrhea, bloating and vomiting, which could cause terrible adherence and ongoing compliance issues.

"We needed pharmacies that really understood patient education and support to follow a diet," Merrigan said. "We had our own dieticians, but we needed the pharmacy to be on board since they are on the front line with patients."

When DLSS builds a highly customized patient program, it securely stores and analyzes all patient data, while making sure to protect patient-identifying details. Having a single data source, the manufacturer and DLSS ideally work together on continuous program improvement based on metrics and trending, including payer relations, financial performance and patient outcomes.

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Better for the patient

Under the integrated model, a patient feels supported at every point of the journey following diagnosis. To expedite the onboarding process, DLSS often provides support to physicians' offices to ensure the prior authorization and appeals process is completed as quickly as possible.

Alpha-1 patient advocate Mary Pierce shared an anecdote about an Alpha-1 patient whose physician initially ordered a treatment carried through a home care agency.

DLSS can maintain relationships with patients from their clinical trials all the way through the drug's commercialization and into treatment.

"The physician's staff had a terrible time getting answers when the home care agency was calling for prior authorizations," Pierce said. "The patient never even went on the first therapy. They finally referred the patient to us, and our team helped facilitate the prior authorization with the physician and their office, and the patient is now getting the necessary care."

DLSS can maintain relationships with patients from their clinical trials all the way through the drug's commercialization and into treatment. One of the wrap-around services DLSS offers concerns financial assistance, which is offered by third-party, non-profit 501c3 organizations.

"The financial issues are usually the worst part of it," Pierce said. "So we work to help patients find alternate funding sources and where possible help them with forms or understanding the requirements for financial assistance."

DLSS also provides high-touch patient support during treatment to provide peace of mind and convenience. To increase adherence, DLSS can arrange for treatment during a patient's vacation, rather than always shipping a drug to the patient's home. Having a portable service model that wraps around the therapy regardless of a patient's geography or their insurer empowers caregivers to manage patient outcomes with care protocols and technologies tailored to the unique requirements of a specific disease and patient community.

DLSS also excels at patient education, helping patients and their families understand how they will need to adapt their lives to succeed on therapy. Through these close interactions with patients, DLSS builds deep, specific disease-state expertise that it shares with patient organizations, helping to build patient engagement and a sense of community, which may be lacking for many rare diseases.

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THE POWER OF THE MIDDLEMEN

The DLSS integrated partnership model provides clear advantages in patient support, patient outcomes and data collection, because it was developed specifically for the rare disease drug market.

In contrast, the traditional model was developed to serve large patient populations taking drugs that don't have complex safety profiles or require high levels of support. Because the traditional model incorporates multiple players and allows more profit opportunities for disparate service providers, it maintains wide support from payers, the medical and pharmacy benefit companies.

"The challenge with an exclusive model like DLSS is that all the other channel partners — specialty pharmacies, payers — get offended," Merrigan said. "The model impacts their pocketbooks. And each one of those channel partners can impact the manufacturer's future business."

So while a manufacturer might determine that DLSS offers the best service for its rare disease product, if that same manufacturer makes a future product that doesn't require a high-touch patient services model and opts to use the traditional model, it may not get the best terms from payers.

The payer relationship and its impact on access affect rare disease patients, especially if it results in higher insurance co-pays if the specialty pharmacy is not in-network with the payer. For exclusive models that focus on maximizing outcomes for rare disease patients, part of their expertise is in doing the work necessary to obtain in-network access for each patient through effective contracting, leveraging payer relationships and accessing all possible financial resources for patients.

"Any growing pharma company has to think about all their future business and future products," Merrigan said. "Migrating from one model to the other is really painful in the short run, for the company and for the patient."

CONCLUSION

Providing outstanding patient service and support is paramount for all rare drug manufacturers. Given the shifts in healthcare legislation, rare drug manufacturers will continue to be pressured to link drug therapy to outcomes, meaning those capable of providing the best patient outcomes will fare best financially.

The traditional model of rare drug distribution and patient service — developed with distribution scale as its top priority — fails to provide the level of patient service needed to ensure positive patient outcomes and experience.

An alternative model, such as the DLSS integrated service and exclusive direct-to-patient offering, provides the level of service rare drug manufacturers need to ensure the highest levels of patient adherence to drive better outcomes, successfully grow their business, and fulfill their mission. That's better for patients, manufacturers, payers and the industry overall.