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Strategic Partnerships for Unlocking Product Potential

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Integrated Commercialization Services

How can a single partner integrate multiple solutions to help pharmaceutical companies bring products to market?

A recent survey of biopharma CEOs and their marketing leads showed that three out of four biopharma marketing executives are "not very" or just "somewhat" confident they have the right tools, talent, and capabilities to deliver disruptive growth.[1] Another study revealed that 50% of commercial leads in life sciences companies say they don't have a good understanding of what their customers want and need.[2]

These numbers tell us pharma is no stranger to outsourcing commercialization services. To supplement their knowledge and secure the skills to back it up, pharmaceutical manufacturers often turn to commercialization partners—and at many different junctures of the product journey.

From clinical trials through pre-launch and market maturity, so many essential outsourced services converge that it may be difficult for manufacturers to make everything work harmoniously. This speaks to the need for a partner who understands the product journey, shares the manufacturer's goals, and can integrate multiple

solutions to achieve them—with patients top of mind, always.

The Speed of Need

In today's environment, some biotech and pharma companies race through the timeline from discovery to launch at breakneck speed, particularly those who are working to meet the challenges of COVID-19. Other processes, such as the development of cell and gene therapies, may move much more slowly. But in almost every case, a manufacturer will look to the outside for help somewhere along the way.

Considering the complexity of launching a new pharmaceutical product, establishing reasonable timelines early on is critical, as is knowing when things need to happen and in what order. "Complicating this challenge is the fact that in the early stages, some companies may have only one person or very few people responsible for all commercialization decisions," explains Glen Martin, Vice President of Manufacturer Commercialization Strategies at AmerisourceBergen, a provider of integrated commercialization solutions for manufacturers.

Solutions at Every Step

During clinical trials, manufacturers have to think about everything from patient recruitment to transportation and logistics. Having a dependable partner manage all these components allows the manufacturer to leverage provider networks for access to eligible patients, along with global distribution excellence to manage time- and temperature-sensitive storage and transport. Trials can overlap with pre-launch activities, so it's advantageous to work with one partner. "We are often engaged early in the process, helping with global clinical trial logistics," explains Martin. "Looking ahead 14–18 months to regulatory approval and launch, there are many services and solutions we can bring to the table, including assessing the reimbursement landscape and building a plan. There is also growing pressure on manufacturers to produce information in support of health economics and outcomes research or real-world evidence, and that is something we lead in when compared with our competitors."

Once a product is approved, developing its unique value proposition is the next critical step in the commercialization journey, as is defining the distribution channel strategy and designing patient support services. Working with a single partner for these and other pre-launch services can be invaluable for data exchange and overall optimization of the manufacturer's access strategy. "Deploying a manufacturer-centric approach allows our teams to align with the values and vision of each manufacturer," Martin says.

And the work isn't done once the product is in market. Manufacturers will also want to ensure they're working with a partner who offers ongoing support and education

"The pandemic also brought with it a wide range of new demands, not the least of which was the need to create contact-free clinical trials."

that ensures the product gets to patients—whether that means continual refinement of patient support programs, targeted marketing and education for providers, or channel strategy expertise and continued commitment to global distribution excellence.

New Demands for a New Normal

During the recent pandemic, manufacturers have relied more than ever on commercialization partners. This reliance is the result of a wide range of new demands caused by the pandemic, including the need to create contact-free clinical trials, re-evaluate patient support, and pivot field reimbursement and access services.

For instance, in regard to clinical trials, AmerisourceBergen has been working with pharma manufacturers to offer patients in-home treatment options, thus overcoming many of the objections normally associated with participating in a clinical trial. AmerisourceBergen's global specialty logistics business has offered direct-to-patient (DtP) solutions for some time now, but the solution has seen a significant uptick in the wake of the COVID-19 pandemic. DtP services can address the factors that make recruitment for any trial challenging, including proximity, accessibility, and schedule availability.

From expanding patient assistance, setting up web portals for education, and giving virtual injection training to support for finding alternate coverage, leading patient support program providers adapted to the pandemic by helping manufacturers make strategic adjustments for the benefit of patients financially impacted by COVID-19.

Another growing offering are field services, whereby outsourced teams of field reimbursement and access specialists deliver education to provider offices on patient support programs and navigating reimbursement for manufacturers' products. These uniquely qualified teams help reduce patient and provider challenges related to accessing a manufacturer's therapy, but are sometimes overlooked or fraught with misconceptions. In 2020, many manufacturers added outsourced, virtual field support to their access strategies.

The industry continues to see worldwide changes in the supply chain and the commercialization journey as all stakeholders adapt to the impact of the COVID-19 pandemic. Even those manufacturers who aren't involved in therapies for coronavirus are impacted. And at times like these, a nimble, innovative partner is both a lifeline and a market mover. "Our partners really relied on us during the pandemic and the support we were able to deliver has significantly improved our stature in the industry," says Martin.

While each of these services on its own is an example of how a commercialization partner can be innovative, nimble, and patient-focused, collectively they demonstrate how

a partner who provides integrated services is always looking for solutions where gaps exist.

How Does it Work?

AmerisourceBergen starts every partnership with in-depth backgrounding. "We learn as much as we can about the product, its attributes, the targeted disease state, and the patient population," explains Martin. "We need to understand [the manufacturer's] commercialization objectives along with any other characteristics of their situation that may influence our work. One example is that the manufacturer may use a group purchasing organization (GPO) and we can work that into the plan."

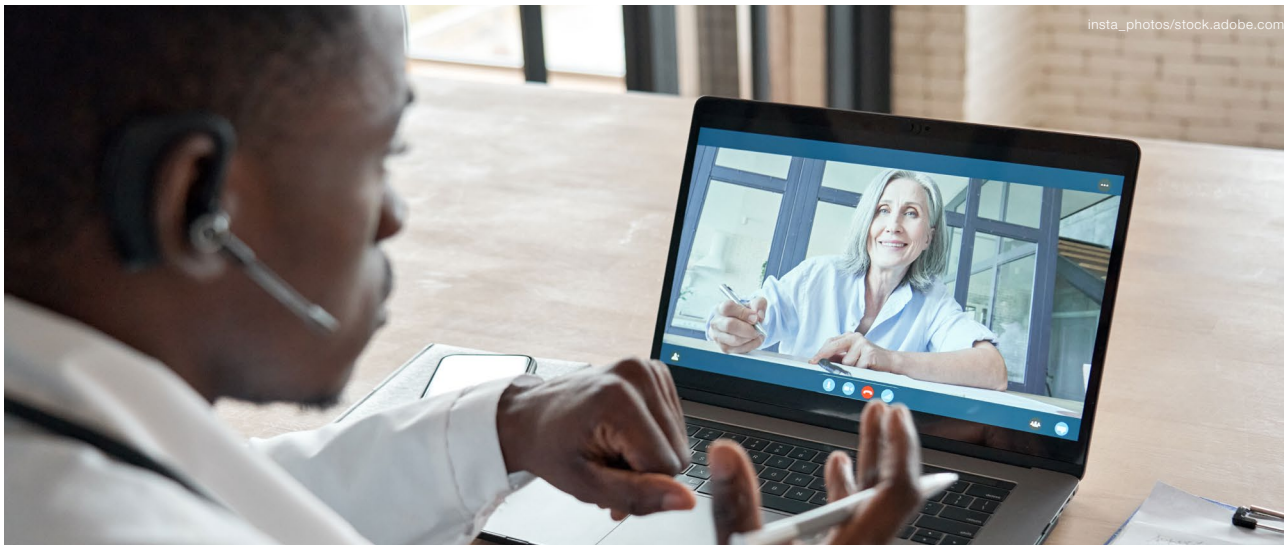
After AmerisourceBergen completes its initial assessment phase, the company employs its proprietary product signature tool, which breaks down important product attributes and identifies AmerisourceBergen service offerings from among its 70+ commercialization solutions. The goal is always to get the right product to the right patient at the right time, while optimizing the commercial success of the manufacturer's product and aligning with the manufacturer's specific goals and resources. A recommended set of solutions will take into account the product's mode of administration, billing method, patient population, site of care, product cost recommendations, and more.

"At AmerisourceBergen, we've assembled, united, and integrated many different solutions and we're delivering them in such a way that makes it easy for manufacturers to work with us," comments Martin. "We've created a centralized manufacturer commercialization strategy function that enables us to pull it all together, creating a blueprint for success."

References

1. Accenture, "Biopharma Marketing - Harnessing Change for Growth," Published Dec. 9, 2019. Available at https://www.accenture.com/us-en/insights/life-sciences/biopharma-marketing-harnessing-growth.

2. Accenture, "Growth Through Commercial: Be life years ahead," Published 2019. Available at https://www.accenture.com/us-en/services/life-sciences/commercial-services.



Is a Contact-Free Clinical Trial Possible?

Mike Sweeney and Kim McLeod

Innovative ways to reach patients and gather evidence for drug approval.

Since the COVID-19 pandemic began, the healthcare industry has seen its share of surprises—including the sudden emergence of *virtual everything*. But there's one part of the pharma supply chain where we've heard a resounding "we're ready" when it comes to moving medicine forward with remote solutions.

World Courier's Mike Sweeney and Xcenda's Kim McLeod share their insights on how the two businesses were already prepared to meet manufacturers' needs when COVID-19 made it necessary to adopt new methods of reaching patients for drug studies—albeit with solutions that address different ends of the research spectrum. Here, they share perspective on the future of data collection and how to adapt clinical trial logistics in light of COVID-19.

Q: Patient recruitment is a challenge even when we aren't in the midst of a global pandemic. What innovative methods can trial sponsors consider to find patients and continue research now that we're seeing even higher drop-off and potential research site closures due to COVID-19?

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MIKE SWEENEY: Recruiting the targeted number of eligible patients for a clinical trial can require a substantial investment of time and money. Offering patients an in-home treatment option helps overcome many of the objections normally associated with participating in a clinical trial. Direct-to-patient (DtP) services can address the factors that make recruitment for any trial challenging: proximity, accessibility and schedule availability. And the reasons for adopting it as a model are amplified in the current context. Overall, the patient mindset is shifting, and sponsors want to support that. They want to make life easier for the patients who are unable or unwilling to enter healthcare settings.

KIM MCLEOD: At the post-approval stage, innovative manufacturers may consider virtual patient recruitment for observational, real-world studies. For this type of study design, electronic health record (EHR) data can be queried to identify potentially eligible patients. Then, a health system and CRO can work together to perform digital recruitment outreach to those patients. All patient-reported data is collected via online portal during the follow-up period. No in-person physician visits are required and all patient-reported data, EHR and claims data can be linked together into one study database. This approach can be used to define real-world drug use, including clinical outcomes, treatment patterns, burden of disease and even the impact on healthcare resource utilization.

Q: Are there specific products or disease states where "going virtual" makes more sense?

MS: DtP is most suitable for rare diseases, but beyond that it can work for any therapeutic area. Everything from mental health to oncology or neurology. What we've seen during COVID-19 is DtP being utilized for more drugs that don't require temperature control. Generally, though, we recommend manufacturers/trial sponsors ask themselves a few questions to vet whether or not their product is a fit for DtP:

- Are any known or inherent risks likely in the patient's response to the drug?
- Is there sufficient product stability data to ensure it can safely be transported and stored (at home) at the correct temperature?
- Can the product be self-administered, or administered by a home healthcare professional?
- Can the visit schedule/administration be continued at home without interruption?

KM: When you're looking at collecting data for rare disease, a qualified partner like Xcenda can leverage relationships with IDNs, GPOs and clinical site networks to access physicians that other study partners may not be able to reach in order to identify viable patients. They can also provide guidance on what data to pull, as not everything on record is necessarily relevant strategically for

IS A CONTACT-FREE CLINICAL TRIAL POSSIBLE?

"The coronavirus pandemic has forced healthcare and pharma to be more innovative."

a product launch. When products are already in market, virtual solutions are useful when the patient population is small and spread out across the country or even the globe. It's traditionally hard to find these patients with these serious conditions, especially in a concentrated geographic location.

Q: How realistic are these solutions? Are you seeing pharma companies ask for them?

MS: World Courier has been actively offering DtP for years, but by the end of April we had seen the volume of our daily DtP shipments increase by five times that of the pre-COVID average. We're also seeing more flexibility from regulators on "alternative secure delivery methods" as they prioritize patient safety. We should expect there to be lasting ramifications for the supply chain as a result of this pandemic, and the impact of DtP is one of those. Though there is debate across pharma about the upfront cost, it's important to look at the opportunity to create value through this solution in the medium and long-term—for patients, sponsors and the healthcare system.

KM: Not only realistic, but incredibly relevant right now. The coronavirus pandemic has forced healthcare and pharma to be more innovative. If we don't pioneer some of these methods, we won't be able to recruit patients—especially for some of the investigational drugs that we can't halt research on.

We're being approached to help manufacturers complete that RWE piece of the study already. Telemedicine is bridging the gap too. There are so many solutions that no one would have been brave enough to try before COVID-19 forced us into new ways of thinking. A lot of our manufacturer partners can't afford to wait and see how it works for someone else.

Mike Sweeney

Mike Sweeney is the Senior Director of Patient Centric Logistics at World Courier.

Kim McLeod

Kim McLeod, MPH, is the Senior Director of Prospective Clinical Research with Xcenda.

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The Future of Payer Engagement: How Pharma Can Break Through

Matt Sarnes and Allen Lising

Payers and IDNs representing over 275 million covered lives access a single platform for pertinent product information. Learn how to meet them where they are.

Xcenda's Matt Sarnes and FormularyDecisions' Allen Lising share insight on the product information payers and other healthcare decision makers (HCDMs) find most valuable.

Q: How much progress has the healthcare industry made in driving meaningful dialogue on product value between pharmaceutical companies and formulary decision makers?

ALLEN LISING: As the number and complexity of products coming to market continues to increase, payers are asking for product information to guide their formulary planning and budget forecasting earlier in the development process.

Supporting the bi-directional exchange of information between payers and life science organizations is one of the keys to unlocking real value in pharmaceutical-led care by driving value-based reimbursement decisions. When we joined Xcenda in 2019, the most exciting aspect of combining the FormularyDecisions technology and payer community with Xcenda's leading global market access and health

economics expertise was the opportunity to deliver more information on pipeline and approved products directly to payers, and deliver more actionable insights on payer needs to manufacturers.

Q: Where are you seeing the most activity in terms of information exchange between pharma and payers?

MATT SARNES: Data from FormularyDecisions shows the therapeutic categories most reviewed by HCDMs in 2019 were oncology, neurology, infectious disease, rare diseases and autoimmune disorders. The high activity in these disease areas aligns with product launch activity in these categories, with oncology having 10+ products come to market in 2019.

Interestingly, HCDMs not only reviewed pre-approval product information, but also information on existing products in those same therapeutic categories. Of the almost 23,000 hits on oncology products in 2019, existing oncology product pages accounted for more than two-thirds of the page views. This likely reflects payers' need to understand both the new product and competitive products in that space.

The top product in terms of reviews by payers in 2019 was actually not an oncology product, but a rare disease product, Zolgensma, with almost 2,000 page views over the year.

Q: When are HCDMs most likely to review product information?

MS: For the first quarter of 2020, HCDM activity on the platform continues to align around product launches. In fact, five of the top 10 most reviewed products during the quarter launched in either Q4 2019 or Q1 2020. Our data also shows that HCDM activity around a new product or indication starts to increase significantly 4-6 months pre-launch and stays elevated through a five-month post-launch period.

Q: Where/how are payers accessing the information they need on these products?

MS: The use of FormularyDecisions among HCDMs continues to increase. That's on top of the existing base of 2,100 registered users representing 275 million covered lives. In 2019, we saw more than 1,200 logins per month. Session time—the amount of time a payer spends evaluating information, so to speak—also increased by 35% (or almost 30 minutes per session). This indicates that HCDMs are finding the relevant and consolidated information useful.

AL: Our community of payers has expressed the desire for manufacturers to put more information on the platform, especially during the pipeline phase when there is a dearth of information. When manufacturers add information to the platform, whether clinical trial information in the public domain or an eDossier for payers to request, we see double-digit growth in overall product activity by HCDMs on those products.

FormularyDecisions continues to be a valuable centralized resource where HCDMs can conveniently access information when they need it and directly request more information from the more than 500 manufacturers connected to the platform as needed. More than 1,700 new dossier requests came through the platform in 2019. We also see a significant uptick in engagement with PIE webinars and other features that facilitate collaboration.

Q: What does the future look like for this kind of information exchange?

MS: Data from first quarter 2020 showed a greater than 100% spike in user activity in both March and April, with over 2,000 page views in the platform per working day. Even though the world was already on the path to various forms of digital engagement and immediate access to data, the COVID-19 pandemic certainly accelerated both innovation and consumption of technology in the healthcare space. Now that many manufacturers are finding themselves launching products in a virtual world, we'll no doubt continue to see use of the platform grow.

Matt Sarnes

Matt Sarnes, PharmD, is the Senior Vice President, Business Development & Strategy at Xcenda.

Allen Lising

Allen Lising is the Managing Director of FormularyDecisions at Xcenda.

AmerisourceBergen

You see a product. We see a path.

What's your vision for commercialization success?

Whether you're part of a global powerhouse or a one-man show, you're an innovator. You want to make the most of scientific advances and move health forward to change patient lives for the better.

We do, too. And AmerisourceBergen is the only commercialization partner with the combination of proven solutions, diverse perspectives, and unmatched scale to make that happen. We build partnerships that unlock product potential and get medications to patients who need them.

Let's work together to envision new paths to healthier futures around the world.

See more

Rethink Field Services: Three Myths Keeping Access Barriers in Front of Your Product

Melissa Mulchahey

Is it time to reconsider how important field services are to your commercialization strategy?

As more pharma manufacturers are forced to launch products in a virtual environment, evaluating the services that will be a part of that launch will be essential. One critical extension of the manufacturer's team is the Field Reimbursement and Access Specialist (FRAS). These provider-facing associates play a critical role in reducing patient and prescriber challenges related to accessing your therapy. As such, they're an essential part of the matrixed team needed to facilitate product adoption and expand access.

But as the pharma landscape evolves, field services can be overlooked, often with the misconception that it's too cookie-cutter a solution for today's dynamic specialty products. Add to that the social distancing restrictions of the current COVID-19 pandemic, and field services is an area of opportunity rife with misperceptions. These three are some of the current biggest myths—and the ones that may have the biggest impact on your product should you continue to believe them.

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A new age of pharma field services. Learn what to look for.

Myth #1: I only need field services for buy-and-bill products.

Truth: Though one size doesn't fit all, field services can break down access barriers for oral products, injectables, and other self-administered drugs.

Many manufacturers believe that field reimbursement and access support isn't necessary for oral products or self-administered specialty drugs. But given the high cost of these products and the measures payers take to control those costs (e.g., prior authorization and step therapy), the access barriers are the same as those that exist for provider-administered drugs. A FRAS who can educate providers on patient assistance programs (PAPs) and help patients navigate affordability is still monumental in removing those obstacles. As well, the FRAS plays a critical role in helping answer provider questions about product access channels, prior authorization requirements, and more.

Myth #2: Field services are only impactful in person.

Truth: The COVID-19 pandemic has made virtual support a valuable part of the FRAS toolkit.

Many providers have successfully adopted telehealth to continue delivering care during the coronavirus pandemic, and field services is no different. In fact, flexibility is a critical success factor for field solutions. Today's FRAS incorporates live, phone, and virtual activities depending on provider needs and abilities, and adapts technology to support virtual engagement. Examples include video support, virtual toolkits and digital provider resources rather than print materials, as well as virtual manager "ridealongs."

Myth #3: Field services aren't designed for small pharma.

Truth: Size doesn't matter.

The notion that only large manufacturers with big budgets or products for massive patient populations need field services is ill-conceived, as is the idea that only niche partners can provide that support. Most outsourced field services companies have a diverse portfolio that spans from "big five" pharma to "first product to market" pharma.

Manufacturers of novel or orphan products often have an even more critical need for field services in their efforts to leave no patient or script behind in a very challenging payer landscape. The FRAS can also help drive awareness of that drug's patient support program and reach providers and patients who might not otherwise know of the availability of those services for the product. At the same time, the outsourced partner delivering field services can offer better support to manufacturers as part of a network of companies that offers patient support services and specialty physician services when that partner is able to access expertise and insights from across the patient journey.

RETHINK FIELD SERVICES: THREE MYTHS KEEPING ACCESS BARRIERS IN FRONT OF YOUR PRODUCT

Small and mid-sized pharma has an even more critical need to leverage the experience and synergies inherent to partnering with a trusted outsourced field services organization. The seasoned partner will work with these clients to design a customized program to bring the greatest impact possible.

Separating fact from fiction

Just as no two products are alike, no single model of support works for every product. The right field services partner recognizes that and has the expertise to address your product's specific barriers to access. Just don't let misconceptions about what to expect from field services become the barriers.

Melissa Mulchahey

Melissa Mulchahey is the Vice President of Field Services Client Engagement and Business Development at Xcenda.

Commercializing an Orphan Drug: Why "Just" Serving a High Unmet Medical Need Won't Do the Job

Wolfram Lux and Simone Seiter

What it takes to win and be successful when commercializing an orphan drug.

Orphan and ultra-orphan diseases are the new Promised Land for the biopharmaceutical industry. Although tiny in patient population compared to well-known diseases, the 7,000 rare diseases known today affect up to 30 million patients in the US, almost 10% of its population. An alarming 95% of these diseases have not yet a medication or therapy, and diagnosis takes five to seven years on average, as they are not top-of-mind for physicians.

However, today they have a voice. Countless patient advocacy groups have driven the attention of the industry, payers, and policy makers toward diseases so rare that sometimes not even 50 patients in a country are affected.

While there were only a few players addressing the medical needs of these diseases ten years ago, the field of small research-driven biotech companies has expanded to an entire new industry that has begun harvesting the fruits of their labor and paving the way for others. On that journey, they have resolved tremendous biologic, scientific, and technical challenges, while also learning

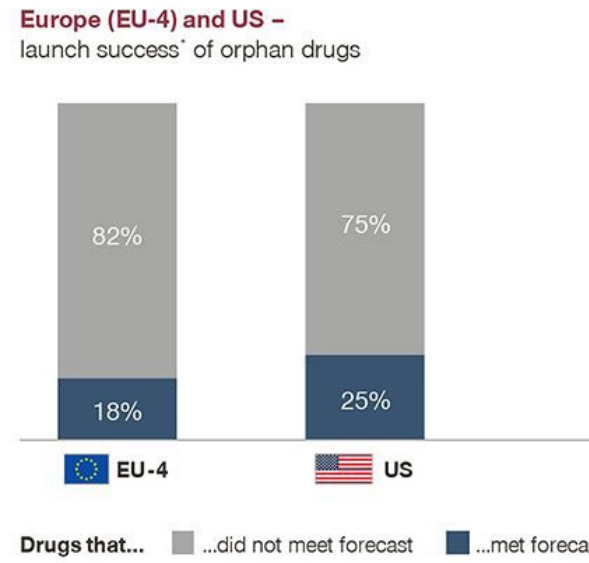
that commercializing these drugs does not follow the usual principles of the established pharmaceutical industry.

What makes rare diseases different from specialty diseases?

The fact that there are only few patients in rare diseases means they are distinguished from specialty diseases in several ways:

- **Partnerships driven by strong commitment to patient communities:** Patient advocacy groups are a driving force behind drug development and provide crucial input for biotechs
- **Fewer resources:** Many biotechs are small, research-driven organizations with limited resources
- **CoE model:** Technical and medical requirements for application are typically very high, requiring well-trained and experienced facilities
- **Especially challenging market access:** Challenging manufacturing and small lot production drive production costs, calling for six-digit reimbursement prices that challenge established payer agendas and payment models
- **Relentless patient focus:** Every single patient counts
- **Need for proactive patient identification:** A logistical challenge of unique relevance in rare diseases

These characteristics establish an entire new business model, set apart from the ethical pharma, generics, and OTC business. However, the rules of the game have not yet been fully laid out, and many things remain in flux while new ideas and approaches are tested along the product life-cycle. As a consequence, most biotechs in the orphan space have failed to meet their own expectations when looking at orphan launches between 2015 and 2019:



Three in four orphans launches missed launch goals so far, as stated by analysts. Established pharma only missed out on 55% of their launches. In the EU, almost 90% of orphan drugs continue to fail in year two after launch. In the US, still over 60% of orphan drugs continue to fail in year two after launch.

What are the major building blocks of success?

Whilst an entire new industry is still trying to find its ways around, Simon-Kucher has been looking at the findings so far and identified four prerequisites for commercial success.

1. **Master the patient journey before anything else**
   successful companies have recognized that the patient journey starts with effective patient engagement. As there are only few patients spread across significant geographies, finding them and facilitating traffic to a center of excellence is where the business starts and is how patients get access to desperately needed therapy. The logistical challenges to counteract the existing dilution effect, however, are enormous, and exceed everything we are used to from established pharma.
2. **Optimize trail design and endpoints to maximize asset value**
   The tiny patient population means that classical multi-arm and potentially even biomarker-backed RCT designs slow down recruiting and study speed. New ideas currently being considered include synthetic-control arms with external data or multi-domain endpoint concepts, currently under FDA review. Put simply, why should we keep using single primary endpoints to evaluate complex and sometime multi-organ diseases?
3. **Overcome current payer paradigms for commercializing innovative orphan drugs**
   Payers' agendas and incentives are still mainly based on an annual (or short-term) perspective and common payment models are suboptimal for the often highly front-loaded therapy costs of rare diseases. Players and payers around the world are discussing new payment models and risk-share agreement forms in order to offset the risk of potentially vanishing therapeutic effects over time, e.g. in gene-therapies. Rare disease players should consider trade-offs between high-quality evidence, risk, and speed to market when thinking about their P&MA strategy. Successful players have aimed at evolving the level of cooperation between treatment centers and payers to advance fair and predictable payment schemes for their therapies on the basis of e.g. outcome-based agreements, including payment installment plans.
4. **Ensure supply chain is embedded deeply in commercial planning**
   Often, one production lot means only one or few patients. Therefore, supply chain management, which is typically relatively rigid, needs to be agile and allow for flexibility to accommodate commercial needs. Close alignment with daily or weekly commercial operations is key to providing patients with access. The supply chain needs to be fully GMP-compliant and production processes scalable on various levels where necessary, e.g. in terms of digitalization, product release procedures, supply of ingredients, stockholding, cold-chain transport, etc. Successful players also consider the fact that many commercial production slots are taken by larger scale diseases, and therefore build capabilities and capacity early or build in-house.

Insights and recommendations for those preparing for their go-to-market

Based on these four building blocks of success, we investigated what it specifically takes to win and be successful commercializing an orphan drug. We analyzed previous orphan launches and spoke with selected players to understand what made them succeed, identifying four major areas that made the difference in commercialization success:

1. Launch process planning
2. Build-up of the commercial model
3. Customer engagement along the patient journey
4. Partnership with and utilization of patient advocacy groups

Launch process planning for an orphan drug is different in four ways:

1. **Supply chain planning:** Ensure availability of the product on time by deeply embedding it in daily commercial planning and management. Successful players said allowing and enabling flexible management is key to avoiding shortcomings in the logistical chain and building trust among patients, HCPs, and all stakeholders along the patient journey.
2. **Set-up planning for customer centers of excellence:** Point of therapy are specialized centers which involve complex logistics when ordering and applying a rare disease therapy. After initial diagnosis, everything else takes place in designated centers of excellence, making their identification and set-up process key. This requires a true understanding of the daily reality and motivation of all stakeholders involved, on an account level. According to our interview partners, installing a smooth ordering, management, and application process needs to be facilitated on a case-by-case basis, and a one-size-fits-all toolbox or approach typically doesn't work.
3. **Communication planning:** Enforce tight and regular internal communication routines to enable quick reactions to sub-national market dynamics. This requires installing direct patient support, education, internal processes, and trained staff early on. The players who we talked to stressed the need to watch the fine balance between the natural interest of physicians in their upcoming launch and the risk of over-the-top communication, especially now, in times of COVID-19.
4. **General level of planning detail:** Given the tiny patient populations in many diseases, often spread across a geography, successful players learned that things don't run the same way everywhere within a country. "Don't take country processes and dynamics for granted" was their

"Ensure availability of the product on time by deeply embedding it in daily commercial planning and management."

take-home message. This means finding more detailed answers to well-known questions and watching granular dynamics to allow for flexible adjustment of planning and execution.

The build-up of the commercial model for an orphan drug has significant impact on success

1. **Organizational capabilities and organization:** Building a patient-centric organization that enables access to patients was key for the front-runners. This is the design of flexible, focused, and efficient commercial organizations (at efficient scale), working truly cross-functionally to coordinate launch preparations. The players hired market specialists with a broader and therefore less common set of skills to engage customers effectively. In such organizations, a few individuals need to be able to perform on various levels, e.g. general commercial tasks, corporate management, and strong communication. The classical silo-role profile hardly exists in a well-working orphan drug organization.
2. **Stakeholder engagement:** Orphan diseases create a universe of challenges in their own right. Engaging early with stakeholders, such as patient advocacy groups, physicians, payers, and policy makers to build and sustain disease awareness and motivate early diagnosis and treatment is much more pronounced than in e.g. specialty diseases. The reason for this need is the limited awareness, lack of strong data, and often suboptimal facilitation of the patient journey and treatment logistic chain. Clarifying and strengthening the pathways to these disease patients and expert networks provides key input for trial design, develops market understanding, and helps to win supporters who lobby for disease development and awareness.
3. **Value proposition:** In the context of limited awareness and evidence for an orphan disease, payer and physician expectations on the benefits, risks, and challenges of a drug should be shaped in an effective but also realistic way. Articulate the value of the new treatment option and communicate the paradigm of a new optimal treatment strategy. Develop optimal pricing along the parameters laid out above and evaluate new payment models carefully. Consider a contracting strategy supported by payer-relevant clinical and health economic evidence from a payer-rationalized trial design.

Customer engagement along the patient journey is the source of your business

Many players apply what has worked successfully for the pharma industry over the years. Earlier in this article, we identified answering common question in more granularity as a key success factor. This is especially true when looking at the patient journey in orphan diseases. Here successfully customer engagement truly starts at the very beginning.

After having mastered all of the abovementioned challenges in pre-launch

times and during set-up of your organization, now the market holds the fruits for you to harvest. BUT these fruits are not low-hanging. In fact they rather sit toward the top of the tree. Now it is about people … and the following elements have proven to make or break the deal for an orphan once you have made it to market:

1. Find the patients, pool the patients, and facilitate traffic effectively to your center(s) of excellence
2. Understand your HCPs' daily reality and motivation – rare diseases are not top-of-mind and HCPs have loaded agendas that do not circulate around your drug
3. Let physicians, nurses, and other HCPs know about your treatment and the value of your therapy
4. Ensure availability of your product through the supply chain and ensure access for patients in a smooth, hassle-free process for all stakeholders during therapy
5. Establish physician confidence in your therapy and provide help with patient management
6. Support and educate patients directly and early before launch

Partnership with and utilization of patient advocacy groups (PAGs)

PAGs have always been an integral part of customer engagement in the pharmaceutical industry. However, their role was long marginalized to rather being add-on support for general marketing and sales activities. Their role and benefit is a completely different story in orphan diseases, where many research and development initiatives have not only been influenced but also substantially initiated, driven, and funded by them.

"PAGs have always been an integral part of customer engagement in the pharmaceutical industry."

PAGs play various roles along the drug development pathway and contribute significantly to value generation. Winning players have done a good job of living up to their expectations, right from the start of drug development:

1. **Discovery and pre-clinical:** Market shapers who push for development of products with high unmet medical need, providing deep insights into the patient journey and daily needs of patients and their families
2. **Trial readiness/Phase I:** Partners providing trial design insights and funding for early research
3. **Phases II & III:** Influencers, acting as potential partners in market access by informing value dossier development
4. **Regulatory approval/launch:** Market shapers, again influencing payer negotiations, expediting reviews, and creating lanes of access for patient sub-populations
5. **Post-launch:** Influencers, again strongly influencing engagement on access levers, pricing negotiations, and downstream coverage, as well as building on center capabilities.

PAGs in the course of go-to-market preparations are key stakeholders and should

be embedded tightly in the commercial planning and clinical development process.

Conclusion: Launching an orphan is different and timely preparation is key!

After evaluating the orphan disease and manufacturer scene, and analyzing the findings from the frontrunners in ideal and not so ideal conditions, we came to the conclusion that the orphan drug business constitutes a new business model, set apart from the ethical Rx-business in pharma, OTC, and generics.

Preparing a launch and commercializing an orphan drug has to be done differently, with launch excellence in this space built on a few specific areas. And the timelines for engagement and launch start much earlier than we know from ethical pharma, beginning as early as Phases I and II.

The question is, are you also prepared for it and are you prepared in a timely manner?

Dr. Wolfram Lux

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Patient Support in the Time of COVID-19: Five Ways to Adapt

Lash Group

Five ways pharma can ensure access, affordability and adherence during a global pandemic.

As social distancing and stay-at-home orders became commonplace in response to the COVID-19 pandemic, many patients chose to put off non-urgent doctor's visits, eliminating a common point of contact with their healthcare providers. At the same time, millions of Americans have lost their jobs and, as a result, their insurance coverage.

Many pharmaceutical manufacturers already utilize patient assistance programs (PAPs) to help patients with issues related to medication affordability and adherence. But the COVID-19 pandemic is now requiring them to reassess patient needs and determine how to adjust their programs accordingly.

"We're still early in evaluating what's happening with patients right now," says Nicole Dunn, Vice President of Client Delivery at Lash Group. "We've seen patient assistance programs, for example, expand their income and insurance coverage criteria, recognizing that some individuals will need assistance for the short term. We're also seeing manufacturers reposition some of their clinical support

since patients are unable or unwilling to go to the doctor right now."

Here are five adjustments manufacturers can consider to ensure patients experience uninterrupted access to medication—during the coronavirus pandemic and in the months that follow.

1. **Expanding patient assistance.** Patients who would typically not qualify for patient assistance programs may now face new financial pressures. Manufacturers should consider expanding eligibility for such programs, even temporarily, so that cash-strapped patients do not ration their medication or forego it entirely. Manufacturers may also want to expand the types of services patient assistance programs offer. For instance, instead of refilling patients' prescriptions on a monthly basis, manufacturers can help patients get 90-day refills to reduce the frequency of refills or trips to the pharmacy. Manufacturers can also connect patients with resources that would benefit them if they have been impacted by COVID-19.
2. **Thinking outside the doctor's office.** Under normal circumstances, many patients learn about and enroll in patient assistance programs at the doctor's office. But during the pandemic, when most people are not making in-person visits, manufacturers may need to set up a website where patients can learn about and enroll in patient assistance programs. Consider a direct patient portal that makes submitting documentation from home into the program easy.
3. **Exploring alternative coverage.** Many people who were accustomed to getting their health insurance through their employer will now have to navigate enrolling in COBRA, Medicaid or a marketplace plan. A disruption in insurance coverage could also cause a disruption in access to medication. To ensure that doesn't happen, patient assistance programs can work with patients directly to help them understand their coverage options and enroll in a health plan.
4. **Providing personalized virtual assistance.** Patients who aren't seeing their doctors in person may have lingering questions about their medication that could affect adherence, especially for patients with chronic or rare diseases. Patient assistance programs can help by offering virtual injection and administration training.
5. **Streamlining benefits verification.** The process of verifying a patient's benefits can disrupt patient care even under normal circumstances. In order to make things go as smoothly as possible amid shifting patient coverage, manufacturers should determine if electronic benefit verification solutions

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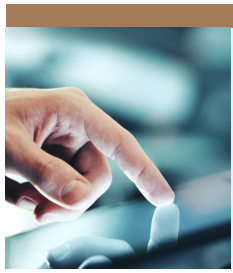

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are right for their programs and for the patients they support. Other technologies, like "digital assistants" that make automated calls to payers, can also help the process go more quickly for patients.

While COVID-19 has thrown patients a lot of curveballs, it doesn't have to stop them from getting the medication they need. Manufacturers can make a few strategic adjustments in order to ensure their therapies remain affordable and accessible to every patient who needs them.