WHITE PAPER

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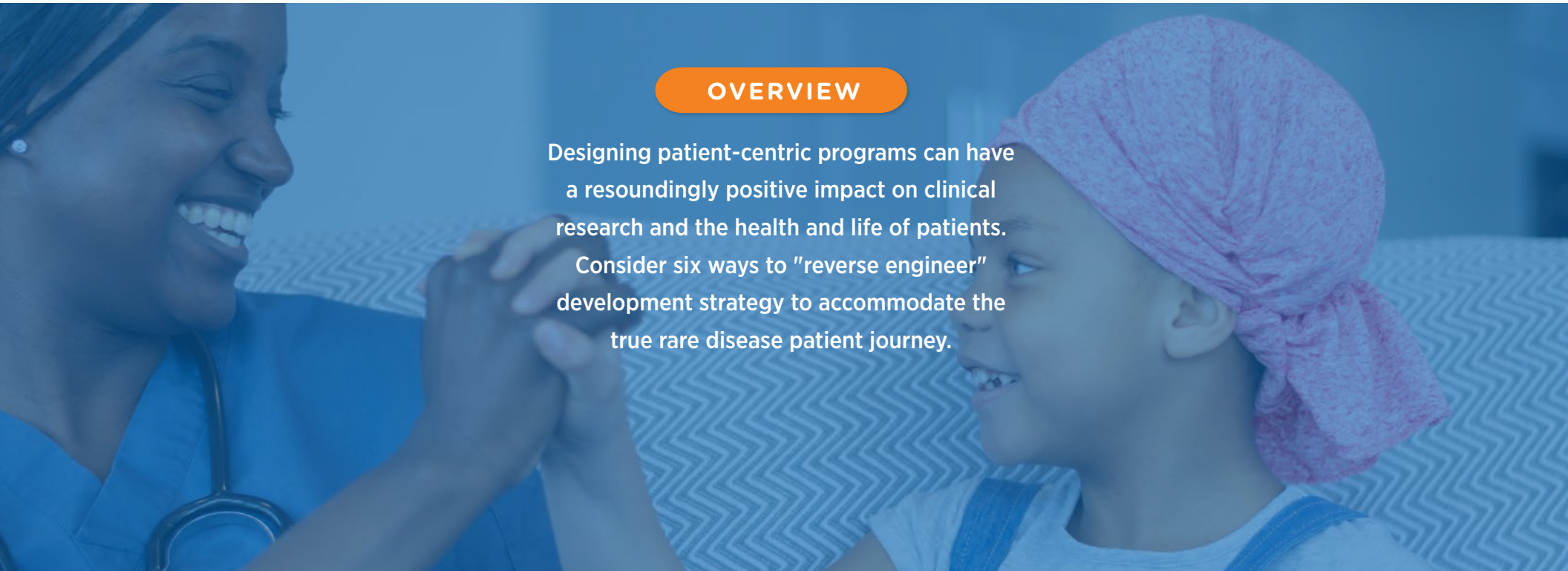
RARE DISEASE

Beginning With the Patient in Mind



OVERVIEW

Designing patient-centric programs can have a resoundingly positive impact on clinical research and the health and life of patients. Consider six ways to "reverse engineer" development strategy to accommodate the true rare disease patient journey.

In order to maximize the chance of development success in small populations like rare diseases, it's critical to understand the patient perspective and what's important to them.

Introduction

Patient centricity is top of mind in drug development today – and rightly so. The rapid evolution of the industry over the past year alone has brightened that spotlight more than ever, making patient centricity imperative. The question becomes: How do we expand perspectives from a myopic scientific lens to apply holistic real-world considerations to drug development strategy and trial design that truly have the patient in mind? Specifically, how do we accommodate the true real-world patient journey in a population that is unlike any other – the rare disease patient?

Involving patients in the drug development process provides context for benefit-risk assessment as well as aids in the development of new assessment tools, study endpoints, and risk communications.[1] Creating genuinely patient-centric drug development programs takes careful consideration and intention; in some cases, it requires an additional financial investment. But this effort can result in generating better outcomes for all stakeholders and reflect the ultimate goal of research and development – an improvement in the lives of more patients.

1. Begin with the patient in mind

In order to maximize the chance of development success in small populations like rare diseases, it's critical to understand the patient perspective and what's important to them. There is a very real difference between a product designed for patients but not with them.

Designing a patient-centric product should begin before a single patient is recruited or enrolled in a trial. It should begin at the first concept of the product through the creation of an initial target product profile (TPP). The TPP helps convey your product concept to important stakeholders – regulators, investors, clinicians, patients, and caregivers – to put a critical eye toward the intended use and labeling for your product. In addition to communicating clinical outcomes, a TPP can also help ensure that actual use in real-world

situations will be achievable and realistic in a patient's life. For instance, a product may have a promising safety and efficacy profile to support clear clinical benefit, but what if it must be administered multiple times a day with special equipment like a nebulizer, requiring the patient to have it accessible at all times? It's important to understand how this may impact patients in their daily lives and understand the trade-offs they may or may not be willing to make to gain relief.[2]

2. Approach patient conversations with compassion

For rare disease patients and caregivers, drug development represents more than a scientific venture – it represents hope. For all patients, but especially those with rare diseases, there is a very real emotional component that cannot be overlooked or discredited. To begin developing patient-centric programs, researchers must first begin from a place of human compassion.

Data suggests that patients' need for information and connection is not currently being met in their patient journey. Meeting that need benefits not only the patients, but also the sponsors, by way of improved patient retention.[3] Digitally savvy patients are increasingly better informed about their health than ever before. When researchers directly engage patients prior to drug development program design, they often learn what matters to them in ways they may not have previously considered.

But that initial connection between researchers and patients must be reframed from an austere approach – as in regarding patients as merely "human subjects," for example – to a more human-centric approach. In doing so, researchers can authentically and holistically understand the true patient journey and its ultimate implications to research and development. Sponsors can begin by accessing patient platforms and networks, carrying out patient focus groups, and collaborating with patient advocacy organizations.

Establishing trust is a strong focus area of the drug development industry today, given the past misconceptions around big pharma and clinical research being focused exclusively on profits. Patients deserve to see that researchers are sincerely focused on breakthrough treatments, and that they want to improve the quality of life for patients. Authentically understanding the entire journey from a very human perspective can go a long way.[4]

While establishing communication is paramount, more must be done. The frequency and nomenclature of that communication must also be carefully considered. While researchers may worry that "plain" language may affect accuracy– a belief that can lead them to use inaccessible scientific language – technical language may be difficult for many patients to understand, especially if there is a language barrier. It can even be off-putting to the parent caregiver of a pediatric rare disease patient who is understandably emotionally invested.

However, simple language shifts can bridge this gap while ensuring scientific rigor. "Clinical office" can take the place of "site," for example. Substituting "patient" for "human subject," or "adherence" for "compliance" demonstrates compassion for patients while removing subtle barriers between them and researchers.

3. Consider a patient guide

For a rare disease patient or caregiver, receiving a diagnosis may be the start of the journey. But the path is not linear, and it can be a frightening and confusing road that evolves as the patient progresses over time. A patient navigator, someone who is an assigned partner to that patient, can make a tremendous difference in the life of the patient – and ultimately, the outcome of the drug development program.

By bringing in care and not relying on the site to provide it, this patient "concierge" can act as a guide for the rare disease patient and caregivers, interpreting scientific language in a manner that is accessible, and even making an impact on the daily life of patients by thinking ahead about their daily needs and lifestyle.

For instance, a rare disease pediatric patient's parent may have to shuttle them to a site several hours away, returning home late in the evening. If the child is young, or if there are other children the parent is caring for, small lifestyle considerations can make a tremendous impact on that parent's life. For example, the patient concierge can think ahead and have dinner delivered when the tired family returns home from the site visit or set up transportation if needed. Beyond enabling those patients to stay in studies, this approach creates a patient-centric program that makes a difference at a very emotional level.

From the sponsor perspective, rare disease patient representatives can advise design in such a way that the potential benefit and burden of participation are addressed and the chosen outcome measures and inclusion and exclusion criteria are optimized.[5]

4. Anticipate return on investment

Patients, caregivers, and patient advocacy organizations are increasingly doing more to fund, discover, and develop treatments for rare and ultra-rare diseases that afflict their children, themselves, their friends, and their communities, and should be considered key partners in the drug development process.[6] Involving patients and their advocates early in development and trial design not only lends itself toward patient centricity, but also potentially impacts the financial considerations.

Truly patient-centric programs require sponsors to increase

Minimize nonadherence and patient dropout by using patient-centric logistics to enable program participation with minimal disruption to daily life. Especially in rare disease programs, direct-to-patient (DTP) makes participation more convenient for a patient population with limited or no ability to travel by providing delivery of clinical materials to the patient's home, home pickup and delivery of collected biological samples, and global home healthcare services.[11]

budgets, engage in institutional review board and ethics committee reviews, create patient-led roles, organize engagement activities, and more.[7] But payment models are increasingly focused on the real-world impact of treatment and devices, and regulatory bodies examine real-world evidence for information on the performance of a product in a clinical setting against a broader patient population to ensure the product supports a large percentage of patients. Understanding what evidence will best support a product's value story is paramount for sponsors.

Further, investing in patient centricity is proven to increase engagement and retention – a financial impact that offsets other costs. Options such as in-home care, virtual trials, mobile health, or place-based care[8] are shown to reduce dropout rates and no-shows to site visits,[9] allowing for both cost savings and patient-centered care.

5. Streamline administrative timelines

While one enduring goal of clinical research is to expedite timelines to get products to market faster, several contributing factors come into play, many of which are out of the control of researchers. However, when designing a patient-centric program, there are simple shifts in timelines that can certainly be mitigated.

Specialization equals speed. Especially in the pediatric rare disease space, partnering with teams who specialize in the therapeutic focus area will inherently impact performance – and its impact on the patient. Further, there are small gap times that cause unnecessary delays – such as administrative lags in communications – that can be assessed and addressed, adding up to reduced timelines. Having a strategic partner who is responsive and adaptive ensures your drug development program is moving as fast as it possibly can.

Tackling any "red tape" concerns that impede progress should also be addressed straight away. For example, although the benefits of patient-facing digital technologies are well documented, including improved patient experience, compliance, and engagement, the barriers to adoption are primarily those that do not directly involve the patient, such as organizational and corporate cultural challenges, business-related challenges, and regulatory challenges.[10] Eliminating those barriers prior to the start of the program improves the patient's experience.

6. Work to establish pricing accessible to patients

When patients, payers, and policymakers hold divergent views on the value and uncertainty of therapies, high prices come into question.[12] Gene-based therapies, while potentially life altering for rare disease patients, may be challenged by insurance companies and paired with high prices.

On the patient side, high prices must be justified, making considerations for impact on quality of life balanced with financial security, as well as the impact on patient caregivers' lost productivity and potential income, as they share the patient's daily journey. Now more than ever, the input of patients as consumers must be considered by developers to find the true value of a product as identified by those who will ultimately use it.[13]

Conclusion

For patients, researchers, and patient caregivers and advocates, designing patient-centric programs isn't a burden, a marketing tactic, or a bandwagon. It should be the North Star that disrupts the drug development process and has a resoundingly positive impact on clinical research and, ultimately, the health and life of patients.

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