Launch Excellence and Patient Centricity

When it comes to the pharmaceutical industry, there are often two schools of thought that guide perceptions from the general public. The first is that the industry's primary goal is to maximize profits, and it is willing to meet that goal by creating and marketing as many treatments as possible to as many people as possible at the highest price possible. This school of thought isn't entirely without merit as we've seen the pitfalls of unethical marketing fueling the opioid crisis[1] and each day there are television ads promoting pharmaceutical products to the masses. The second school of thought is that the industry's primary goals are to identify treatments to heal patients safely and to deliver those treatments to patients as efficiently as possible. This school of thought is also merited with many organizations focused specifically on rare diseases and most organizations focused on areas of unmet need for patients. While the first school of thought has a mostly negative connotation and the second has a mostly positive one, in reality, both are correct. Like any industry, revenue is necessary to sustain a business and this fact is no different for the pharmaceutical industry; but, in pharma, patients are the customer and the products often have the ability to change their lives for the better, so there is an ethical component to development and access. It is this relationship between the industry, the customer, and the product that is unique and creates a nuance that is worth exploring.

During a traditional drug development process, patients themselves enter the process around the time the drug enters the clinic for Phase 1 studies, but it is imperative to obtain a deep understanding of the patient population as early as possible to allow for the identification of opportunities to optimize patient considerations. Each therapeutic area and indication has varying demographic profiles and can also present different challenges during the patient journey from pre-diagnosis to diagnosis and from treatment to access and quality of life. Centering the patient from the initiation of clinical development through launch and beyond benefits both the patients who will have positive health impacts and the manufacturers who will have positive impact on their bottom lines.

Understanding the Patient Population

The drug development process is long and can be a complicated endeavor for organizations, and the majority of the earlier stages are rightfully focused on the science

[1] Purdue Pharma, Sacklers reach $7.4 billion national opioid settlement | CNN Business

behind the drug. However, there is a point where the science starts to gel and the indication becomes clear, and this is exactly the time when grasping a deep understanding of the patient population is ideal. This is the first step for organizations to become more patient centric and the most important step to get right.

A deep understanding of the patient population consists of three components:

- demography,
- epidemiology,
- and the patient journey.

Epidemiology is not a new topic for drug developers, but demography is a less common topic that should also be considered when thinking about patient populations. Epidemiology and demography are similar in that both focus on examining populations with an eye on trends, but while demography focuses on the macro-level (aggregate level), epidemiology focuses on the micro-level (individual level).[2] Studying the demography of an indication's patient population could add an additional layer of understanding at a macro-level that could help organizations engage with patients better at a micro-level.

Let's use sickle-cell anemia as an example to dig into the details of epidemiology and demography. For sickle cell, epidemiology tells us that SCD affects about 100,000 people in the US and more than 90% of those people are African American, with 3-9% being Hispanic or Latino. Epidemiology also provides us with an ability to look-ahead, saying that SCD occurs in about 1 of every 365 African American births and 1 of every 16,300 Hispanic-American births. For most organizations, this information would be enough to feel good about understanding the patient population, so they would stop here and use this information for demand forecasting and other launch activities. But, what else can we learn about African Americans and Hispanic Americans that could help us be more patient centric in drug development?

This is where demography comes into our analysis. Demography provides more details about these patient populations, answering questions about topics[3] such as:

- Age and Gender
- Income brackets
- Geographical location
- Language preferences, and
- Household composition

[2] Demography and epidemiology: past and future directions | demotrends
[3] Demographic Survey Questions: A Complete Guide

The addition of demographic insights to epidemiological insights enhances our understanding of the patient population and allows for more strategic decision making during the drug development process. Early in the process, organizations can already start to think through detailed patient personas to understand customer needs and preferences. If we know that African Americans are concentrated in the Southeast United States, for instance, then we could focus our clinical sites there. They can also identify market trends that could create opportunities to reach the patient more probable, thereby ensuring a better chance at fair representation in research and development. If we know that the patient population is of a lower income bracket, then we may provide transportation support or another type of patient service. Regardless of the therapy area, a demographic study could shed light on the patient population and uncover opportunities to be more patient centric throughout the drug development process.

Benefits of Demography (graphic)

- Detailed Buyer Personas
- Early market trending
- Fair representation
- Targeted marketing
- Tailored services

Another important aspect of understanding the patient population after epidemiology and demography is the patient journey. The patient journey is an analysis of the steps that a patient would take through a disease state from when symptoms first appear, through when they are treated and managed. The steps of the patient journey analysis include pre-diagnosis or origination, diagnosis, treatment, access, and adherence. For each of these steps, the patient experience is examined. For example, in the diagnosis step, organizations would learn how would a patient be diagnosed, what is that process like, and how long would it take. The key to using the patient journey in an effort to becoming more patient centric in drug development is focusing in on the lens through which the journey is viewed. Typically, the “patient” journey is really a view of the journey of the patient from the physician’s point of view. While this view is also important to understand, taking a look at the patient journey from the patient’s point of view is where organizations can be more patient centric, and this view has come to be known as the “emotional patient journey”. The emotional patient journey adds the human factor back into what can be a methodical stepwise process of diagnosis and treatment, and it brings to light how the patient may be feeling and what barriers may present. For example, having to visit the clinic four times to complete diagnosis and treatment may be easy to check the box on paper but to the patient it may be overwhelming. In this example, a potential reduction in patient visits

could be an area where an organization could focus on when centering the patient more effectively.

Patient Journey Steps (graphic)

- pre-diagnosis or origination,
- diagnosis,
- treatment,
- fulfillment/access, and
- adherence

Patient Centricity in Drug Development

When we think about launch excellence, we're usually focusing on the final 18 months before the drug or device comes to market and all of the activities that should be performed and initiatives that should be implemented as the best practice to increase the chances for success in the marketplace. Those final 18 months before launch, known as the launch phase, do provide an environment for organizations to be patient centric, but there are opportunities throughout the drug development process, including in the early development phase, clinical trial phase.

Early Development Phase

The early development phase is when you have a thorough understanding of the patient population, but you have not entered the clinic yet. This phase is the perfect time to use the knowledge you've gathered to connect with the patients and caregivers who will eventually make up your customer base to determine what is important to them. There will be some general principles that are important to most patient populations and then there will be some that are more specific to certain patient populations or patients with certain diseases.

In general patients and caregivers are looking for transparent communication and unbiased information about their disease state delivered in a convenient and easy-to-understand way.[4] They also want to be empowered to make informed choices about their care and to have access to resources and support programs that improve their quality of life. When it comes to more specifics, there are two research modalities that work well to dive deeper into patient insights.

[4] Defining patient centricity with patients for patients and caregivers: a collaborative endeavour | BMJ Innovations

- Patient Advisory Boards: Organizations can establish advisory boards consisting of patients and caregivers to provide insights into disease burden and treatment needs. This can help shape research priorities and clinical study designs.
    - Sanofi Genzyme has implemented Patient Advisory Panels to obtain input on aspects of planned clinical trials from the perspectives of potential participants. This practice has been applied to various therapeutic areas, making the integration of patient perspectives a consistent practice throughout the clinical trial lifecycle.[5]
    - Roche has created the "Patient Partnership" program, which includes initiatives like the "Patient Advisory Boards" where patients provide direct feedback on their experiences with Roche's products and services. This feedback is used to make improvements and ensure that patient needs are met.[6]
- Surveys and Focus Groups: Conduct surveys and focus groups to gather patient perspectives on unmet needs and potential treatment outcomes. Surveys are appropriate for a larger audience and focus groups allow for patients to interact with each other as well. Both of these modalities can also guide early-stage research and development.
    - Pfizer has developed the "Patient and Health Impact" team, which focuses on understanding patient experiences and outcomes. They use this information to guide drug development and improve patient support programs.[7]

Interacting with the patient population directly before the clinical trial phase provides a chance to make adjustments that could benefit not only the patient but also the product's success at launch.

Clinical Trial Phase

An organization's first broad opportunity for patient interaction is when clinical trials initiate and one could argue that this is the beginning of the marketing journey. Building a strong relationship with the patient population you aim to serve is the foundation of patient centricity in clinical trials. In fact, Novartis has implemented a program called "Patient Engagement in Research" which involves patients in the design and execution of clinical trials. This helps ensure that the trials are more patient-friendly and address real patient

[5] Sanofi-Case-Study-full.pdf
[6] Patient centricity: How is the pharma industry addressing patient reach? | Access to Medicine
[7] Top 20 Pharma Company Creates Global Change in Patient Centricity - A Life in a Day

needs.[8] There a number of ways for organizations to bring a patient centric mindset into the clinic to build trust, including:

- Patient Trust and Engagement: A commitment to DEI can build trust with diverse patient communities, encouraging greater participation in clinical trials and patient engagement programs.[9]
- Patient-Centric Trial Design: Involve patients in the design of clinical trials to ensure protocols are patient-friendly. This includes simplifying procedures, reducing the frequency of visits, and considering patient-reported outcomes.[10]
- Real-Time Feedback: Use digital tools and patient portals to collect real-time feedback from trial participants. This can help identify and address issues promptly, improving patient retention and engagement.[11]
- Patient-Centric Trial Results: Provide lay-language clinical trial results summaries for patients that are easy to review and digest.[12]

Although opportunities abound for organizations to become more patient centric during the clinical trial phase of drug development, this phase is also under a lot of scrutiny both internally from senior management and executive leadership as well as potentially externally from investors if the company is publicly traded. Because of this additional pressure and the temptation to go without the patient voice in the clinical trial phase to expedite the process, it is important to plan for and gain alignment for those plans in the early development phase.

Launch Phase

The clinical trial phase and the launch phase overlap at around the end of Phase 2 or the beginning of Phase 3, but patient centricity in the launch phase takes on new challenges: regulatory and marketing.

Recently regulatory bodies have placed more value on incorporating patient voices, with the FDA expressing their commitment.[13] Historically, patients have been bystanders in the regulatory process, and it is a step forward to see those who will interact with these therapies the most have a seat at the table as well. Organizations planning for regulatory

[8] Why patient centricity is key to long-term pharma company success | Arthur D. Little
[9] TCB-The-ROI-of-Inclusion-final.pdf
[10] Bringing the Patient Voice into Clinical Trials | PharmaVoice
[11] How To Integrate The Patient Voice Into Trial Design
[12] Mapping the Landscape of Patient-centric Activities Within Clinical Research - ScienceDirect
[13] FDA's commitment to incorporating the patient voice into their decision-making process - Patient Engagement for Medicines Development

submissions should consider patient centricity as a bonus as regulatory bodies have had positive responses to patient feedback. For example, recently, we've seen:

- *Patient Input in Regulatory Submissions: Organizations including patient perspectives in regulatory submissions to provide a comprehensive view of the treatment's benefits and risks. This can support a more patient-centered evaluation by regulatory bodies.*
- *Public Hearings and Consultations: Participate in public hearings and consultations organized by regulatory agencies to ensure patient voices are heard during the approval process.*
- *Regulatory Compliance: Regulatory bodies are increasingly emphasizing the importance of DEI in clinical research, especially when a disease state affects a particular patient population. Companies that prioritize representation in their trials may find it easier to meet regulatory requirements and gain market approval.*[14]

Regulatory bodies approve products for use in humans but they also regulate marketing materials for product promotion. Developing a marketing plan in the launch phase is one of the strengths of the patient centric ethos of drug development, but there still tends to be a focus on the HCPs as the customer as opposed to the patient as the end user, and therefore, the actual customer. Incorporating the patient into marketing development, along with the physician, is a better way to target the audience.

As a foundation for marketing, patient research is key. ATU (Attitudes, Trials, and Usage) studies are common in the launch phase as marketers try to gain perspective, but they are typically done with physicians. They can and should also be done with patients. Patient ATUs, along with the modalities we discussed earlier, such as focus groups and advisory panels, increase organization's knowledge of patient insights. With this knowledge, organizations can develop culturally competent products and services and can improve patient satisfaction and loyalty.

Patient Centricity and ROI

The bottom line is that the patient is the customer, or at the very least, one of the customers. In this era of inclusion, patients are demanding to have a voice in how products are created, tested, and delivered to them, and the more organizations can meet this demand, the more successful they will be.

Evaluating ROI patient-centric initiatives involves measuring both financial and non-financial outcomes, such as patient satisfaction, health outcomes, and market reach.

[14] gx-lshc-dei-patient-experience.pdf

While the immediate financial ROI might be challenging to quantify, the long-term benefits of improved patient outcomes, enhanced reputation, and regulatory advantages can be substantial. At Scimitar, we support our clients in demonstrating the strategic value of patient centricity for organizations in both the short and long term.