When Clinical Trials Are Not Enough: How Expanded Access Programs Benefit Clinical to Commercial Transitions

durbin
part of the uniphar group

Pharmaceutical leaders face growing pressure to control costs, improve clinical trial operations, and successfully commercialize treatments geared toward highly specialized patient populations. Expanded Access Programs (EAPs) are currently an underutilized resource that assist in these areas while also fulfilling altruistic missions.

While pharma, biopharma, and medical device companies implement EAPs primarily to serve seriously ill patients who lack other treatment options, EAPs also help ease the transition from clinical development to commercial launch. By giving patients early access to investigational treatments, drug and device developers obtain valuable real-world data (RWD), enhance communication with key opinion leaders (KOLs) and patient advocacy groups, and create an avenue for ethical, sustainable access. Increased market share is a valuable byproduct.

Here, we explore how pharma and biotech companies can use EAPs to not only serve their patient communities, but also as a component of clinical and commercial development.

THE PRICE OF REINVENTION

To improve recruitment rates, patient centricity, and diversity, among other issues, regulatory agencies are encouraging drug developers to adopt decentralized clinical trial models wherever possible. By implementing remote participation, patient monitoring, and source data review, clinical trial sponsors can shorten timelines and expand participant pools. However, the additional expense that comes from implementing new technology, hiring and training staff, and overall change management makes the shift from in-person to decentralized trials a complicated endeavor.[1]

Meanwhile, health systems, payers, and patients are protesting rising pharmaceutical prices with increasing frequency. Although pharmaceuticals provide clinically meaningful benefits, the price of those benefits has risen exponentially over the past 15 years.

In the U.S. for example, treatments for multiple sclerosis were priced between $8,000 to $11,000 a year in the mid-1990s. In 2015, new treatments cost about $60,000 a year. Rare disease patients also face unsustainable drug prices. In 2016, the median annual U.S. price for each patient treated with top-selling orphan drugs was $83,883. That's 5.5 times the median annual cost for top-selling non-orphan drugs.[2]

The cost issues associated with derailed clinical trials, implementing decentralized trial models, and adjusting drug prices puts drug and biologic developers in a difficult business position. They must juggle all these issues while maintaining an edge in a highly competitive marketplace, as well as uphold a positive reputation among stockholders, payers, and patient populations.

EAPs won't fully alleviate these pressures, but they do present a way to augment drug development strategies and ease the transition to commercialization.

At the same time, the organizations that implement EAPs share a common mission: bring vital medicines to patients who need them, especially those who have no other options.

TAP INTO A RICH SOURCE OF REAL-WORLD DATA

Regulatory agencies, payers, and drug developers use RWD collected outside of clinical trials to inform approval and reimbursement, expand indications, and confirm or deny important safety information.

EAPs present an opportunity to collect RWD from patients with high unmet medical needs who may benefit from investigational treatment, but cannot participate in clinical trials. The data collected often provide valuable information on diseases and patient populations sponsors often know little about, as investigational drugs are only tested in well-controlled patient groups.

For example, if a clinical trial only includes patients aged 50 and under per its eligibility criteria, the sponsor will not have data on patients over age 50. The sponsor could, however, implement an EAP with different eligibility criteria, which benefits another group of patients as well as the drug program overall.

The more insights into how a drug responds in patients with rare diseases, rare forms of cancer with low survival rates, and other critical conditions, the stronger its safety and efficacy profile. A clearer safety and efficacy profile helps streamline the path to commercialization.

Awareness of the benefits of EAP data is increasing among sponsors. A study published in British Journal of Clinical Pharmacology found that the number of approvals in the U.S. and EU that used EAP data increased nearly four-fold from 1993-2013 to 2014-2018. In 13 cases, EAP data formed the main evidence for approval.[3]

Research also shows that any adverse events experienced during an EAP rarely influence the drug development program. A study conducted by the FDA's Center for Drug Evaluation and Research found the incidence of clinical holds related to adverse events observed in EAP patients was .2%.[4]

BUILD AWARENESS WITH KEY OPINION LEADERS

When developing therapies for patients with serious diseases and no alternative treatment options, it's likely that a limited number of physicians will know about investigative drugs. What if sponsors could launch a program that not only helped more patients but also raised awareness among physicians, investigators, and key opinion leaders (KOLs)?

WHAT IS AN EAP?

A potential pathway for a patient with a life-threatening or serious disease or condition to gain access to an investigational medical product (drug, biologic, or medical device) for treatment outside of clinical trials when no comparable or satisfactory alternative therapy options are available.[5]

Early adopting physicians may become brand advocates, while KOLs would be better prepared to move the product forward. Early KOL support may positively influence regulatory, reimbursement, and prescribing decisions.[6]

Because physicians administer EAP products as a treatment before commercial launch, EAPs help raise awareness about the product among the physician community. When the drug becomes approved for use, physicians are more inclined to prescribe the drug because they know more about it and see it working in their EAP patients.

The same result holds true with patient advocacy groups. When their members learn about a drug's EAP and the clinical data it produces, they may be more likely to request information about the product from their doctor. Awareness of the EAP generally helps build loyalty and positive relationships among the disease community, which raises the odds of a successful launch.

When designing an EAP, sponsors could benefit from involvement of patient advocacy groups and KOLs in the design process, depending on the disease indication. By improving collaboration, sponsors learn what these stakeholders need and produce an EAP that will ultimately provide better outcomes and increased engagement.

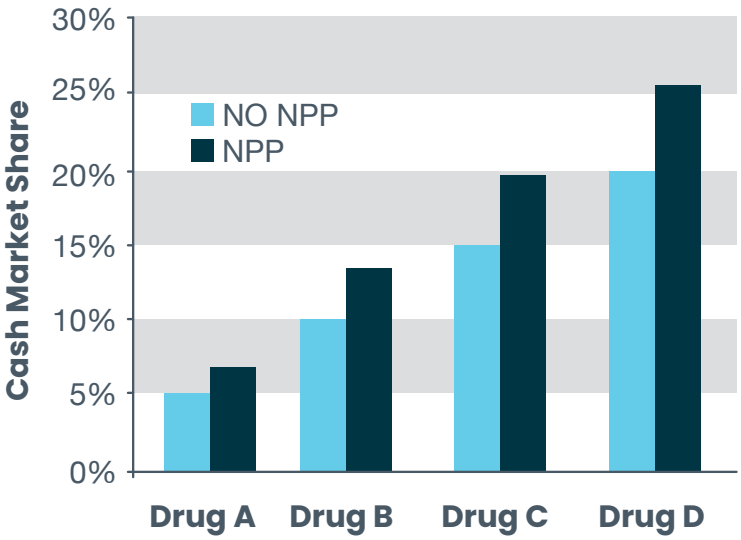
INCREASE MARKET SHARE

The true purpose of an EAP is to help patients who have exhausted all other available treatment options, including clinical trials. However, these programs also have a place in global market access plans and strategies.

Because the FDA and regulatory agencies in other countries grant EAP requests to sponsors, EAPs ensure "controlled and compliant supply of these preapproved drugs, thereby reducing counterfeit opportunities."[6]

By increasing access to — and awareness of — an investigational drug, a sponsor may increase its market share. A study conducted by Eularis to assess the financial impact of an EAP on product launch found the odds of spending $1 on the drug was 1.36 times higher if the drug was made available through an EAP compared to a drug without an EAP. And the larger the drug's market share, the larger the impact of the EAP.[7]

Offering expanded access comes at a cost — specifically, costs associated with manufacturing high-value drugs and implementing the EAP. However, opportunities exist in many countries for compensation or formal reimbursement. Early planning is key to take advantage of these opportunities while maintaining alignment with market access strategies.



Examples of EAP advantage to cash market share.[7]

AN ETHICAL, CONTROLLED WAY TO IMPROVE ACCESS

Operational benefits aside, the most valuable reason to implement an EAP remains the same for all treatments: to provide an ethical, sustainable means to provide access to potentially lifesaving therapies to patients in need. When designed and implemented well, EAPs can have a powerful impact on patient outcomes.

CLINICAL TRIALS ALONE ARE NOT ENOUGH

Companies that design, implement, and manage EAPs can realize a variety of benefits from their programs, but only when these programs are executed well. Creating a successful program requires regulatory, clinical, and supply chain expertise specific to expanded access. An experienced EAP consultancy is a valuable resource to aid sponsors in designing a sustainable program that addresses the needs of all stakeholders, from healthcare professionals and board members to patients and caregivers.

In 2018, Biogen launched an EAP for nusinersen (now Spinraza®) to treat patients with SMA. Sam Lucas, now Durbin's senior vice president of Expanded Access, led the program on the sponsor side. The EAP focused on the most severe form of the disease, infantile-onset SMA, which has a high risk of mortality.

The program enrolled 835 individuals across 30 countries. Participants could access nusinersen treatment up to three years before drug reimbursement and commercial launch. Early access was critical, as the disease has a 30% mortality rate at two years old. In addition to helping these young patients improve motor function, the EAP generated useful data for the sponsor and for disease registries.[8]

These types of positive results can do wonders for a brand's image and reputation. The positive press may influence public perception of the company as well as stock prices.

To manage the data burden, including RWD collection and analysis, a consultancy with an easily integrated platform is crucial. Durbin offers uniphi, a secure, built-for-purpose, proprietary online portal that acts as a central repository for physicians and sponsors. Uniphi serves as an efficient single tool for EAP process flow management, registrations, orders, and reporting.

When developed and executed correctly, with proper due diligence and care from an experienced vendor, expanded access provides benefits that far outweigh any perceived risks. And in a competitive industry, it's a tool too valuable to ignore.

REFERENCES

[1] Agrawal, Guarav, et al. No place like home? Stepping up the decentralization of clinical trials. McKinsey & Company, June 10, 2021

[2] Morgan SG, Bathula HS, Moon S. Pricing of pharmaceuticals is becoming a major challenge for health systems. BMJ. 2020;368:l4627. Published 2020 Jan 13. doi:10.1136/bmj.l4627

[3] Polak TB, van Rosmalen J, Uyl-de Groot CA. Expanded Access as a source of real-world data: An overview of FDA and EMA approvals. Br J Clin Pharmacol. 2020;86(9):1819-1826. doi:10.1111/bcp.14284

[4] Jarow JP, Lemery S, Bugin K, Khozin S, Moscicki R. Expanded Access of Investigational Drugs: The Experience of the Center of Drug Evaluation and Research Over a 10-Year Period. Ther Innov Regul Sci. 2016;50(6):705-709. doi:10.1177/2168479016656030

[5] FDA, Expanded Access https://www.fda.gov/news-events/public-health-focus/expanded-access

[6] Patil S. Early access programs: Benefits, challenges, and key considerations for successful implementation. Perspect Clin Res. 2016;7(1):4-8. doi:10.4103/2229-3485.173779

[7] Bates AK. Implementing a Pre-Launch Named Patient Programme: Evidence of Increased Market Share. Journal of Medical Marketing. 2008;8(4):319-324. doi:10.1057/jmm.2008.25

[8] Yong J, Moffett M, Lucas S. Implementing a Global Expanded Access Program (EAP) for Infantile-Onset Spinal Muscular Atrophy (Type I): Understanding the Imperative, Impact and Challenges. J Neuromuscul Dis. 2019;6(2):227-231. doi:10.3233/JND-190387

ABOUT DURBIN EAP

Durbin is a specialist medical supplier distributing unlicensed pharmaceuticals to 160 different countries. Durbin works in partnership with global pharmaceutical and biotech companies to provide Expanded Access Programs, including Named Patient Supply and Cohort Programs.

The company has 25 years' experience designing and implementing EAPs from concept and specialises in developing robust and compliant voluntary data collection initiatives, which run seamlessly alongside the programs they manage.

Those initiatives help partners capture real-world insights from outside the clinical trial environment that can then have a variety of potential uses, ranging from regulatory and payor negotiations to informing future study design.

durbin
part of the uniphar group

durbin-eap.com