

WHITE PAPER

BEYOND BORDERS: ACHIEVING GLOBAL VALUE FROM LOCAL OBSERVATIONAL RESEARCH

5 requirements for designing effective local observational research that can support global product strategies

Effective observational research is paramount to the success of commercialization efforts at global and local scales alike. Study designs must take into consideration the complexity of substantiating the outcomes of drugs and medical devices among diverse patient populations, as well as the distinct commercial and regulatory objectives of the corporate sponsor and its local affiliates. A balanced observational research strategy that integrates local expertise and data requirements with a global research infrastructure can become the critical foundation for the collection of high-quality clinical and patient-reported data necessary to enhance a sponsor's competitive positioning in each of its major markets.

In this paper we will discuss the five requirements for local affiliates to conduct credible observational research, utilizing a centralized research infrastructure, to demonstrate product value in support of both local and global commercial objectives:

1

DEFINE AND DESIGN THE STRATEGY AND COMMUNICATIONS PLAN: BEGIN WITH THE END IN MIND

2

EMBRACE LOCAL STAKEHOLDERS' NEEDS AT THE GLOBAL LEVEL

3

PROVIDE ONGOING SCIENTIFIC OVERSIGHT AND OPERATIONAL LEADERSHIP TO THE OBSERVATIONAL RESEARCH

4

INTEGRATE LOCAL AND GLOBAL RESOURCES TO OPTIMIZE OPERATIONAL EFFICIENCIES

5

IMPLEMENT A MULTIFACETED COMMUNICATIONS PLAN

INTRODUCTION

Deriving global strategic value from observational research

Biopharmaceutical and medical device companies invest a significant amount of their budgets and possibly a decade or more in clinical trials to successfully register a new product. However, obtaining marketing approvals are no longer enough to make a product a commercial success, as demonstrating product value after regulatory approvals has become a key driver of decision making among local healthcare payers, policy makers, healthcare providers (HCPs), and patients around the world. Post-approval observational research can be a powerful clinical and commercial positioning tool for maximizing long-term global product value.

While early registrational clinical trials demonstrate safety and efficacy of a new product in a narrowly-defined patient population and lead to marketing approval by regulatory authorities, observational research is a method to collect compelling, real-world clinical data that boosts a product's value proposition among a broader patient population. In addition to fulfilling the requirements of governments, HTAs (Health Technology Assessment Organizations), and private payers, well-designed observational research can also contribute to new disease management or treatment guidelines, establish therapeutic goals in patient subpopulations, and defend an established

product franchise against new competitors. Observational research can also provide the necessary real-world data that is increasingly required by multiple local stakeholders to enable a sponsor's affiliates to demonstrate product value and to deliver high-level commercial results from their local markets (Figure 1).

Figure 1: Demonstrating product value to the primary observational research stakeholders



Just as the local needs for real-world data are increasing, clinical research budgets are shrinking. As a result, there is a growing need to conduct local observational research more cost-effectively. Sponsors must expend fewer resources, as well as apply those limited resources more strategically and efficiently to accomplish multiple objectives.

Can the results of several local studies be combined to demonstrate product value globally?

How can a centralized research infrastructure support the local affiliates in their markets?

A well-designed observational research can yield comprehensive, high-quality data that are versatile enough to meet the specific goals of each local affiliate, as well as global product positioning objectives. In addition, a centrally managed clinical research infrastructure provides local affiliates access to world-class specialists, standardized procedures, and technologies—high-quality, high-value assets that may not otherwise be available to them. A centrally executed and coordinated observational research strategy, combining a centralized research infrastructure with local market expertise, can help minimize cost and optimize study results to meet both global and local objectives.

Partnering with an experienced CRO to co-develop and/or execute this observational research strategy

provides a comprehensive mechanism to align global and local requirements with the overall research and commercialization objectives, but with customization at the local level that also enables the necessary data collection to support local product messaging. This CRO / Sponsor collaboration can help seamlessly bridge the common, rate-limiting gaps in information that can occur between the global sponsor and the affiliates, and ensure that all critical commercial considerations are identified and adequately addressed.

The following fictional case study for Nouveau Pharma, Inc. illustrates the challenges and opportunities of designing and executing multi-national observational research that meets the objectives of the corporate sponsor and its local affiliates alike.

CASE STUDY: NOUVEAU PHARMA, INC.

Nouveau Pharma, Inc. is preparing for the global marketing approvals and launches of their new treatment for Type 2 Diabetes. This treatment represents a significant advance in mechanism of action (MOA), and could greatly affect diabetes treatment standards.

Nouveau Pharma and their international affiliates must satisfy three different regional needs:

1. The European affiliate's target audience is the European Medicines Agency, which requires a post-marketing commitment for long-term safety and effectiveness of this new MOA, as well as the central reimbursement agencies, which are asking for more real-world data to demonstrate the new product's value compared to treatments currently available to physicians.
2. In Asia-Pacific, the affiliate must map the current standards of diabetes care in a rapidly growing patient population and among their target physicians in order to optimize a product launch strategy.

To satisfy these diverse needs, Nouveau Pharma originally planned:

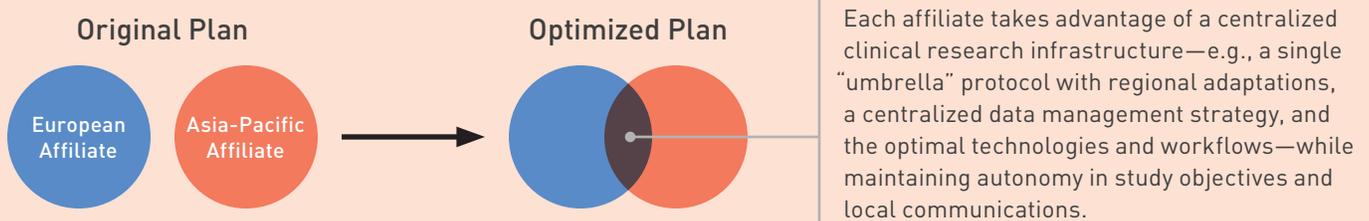
- 2 separate study protocols
- 2 separate project plans
- 2 separate databases and data collection strategies

- 2 separate project teams, each holding weekly meetings and timelines
- 2 separate independent study reports

While this approach might achieve each affiliate's objectives, the data collected would be too localized to potentially extend the conclusions to Nouveau Pharma's other markets. Moreover, the duplicative efforts of the two project teams are an inefficient use of limited resources.

As one example of optimized efficiency (Figure 2), a single "umbrella" protocol with regional adaptations, along with a centralized data management strategy, can standardize data field definitions and database structures across the planned observational research programs. Once the data elements and sources are defined, the optimal technologies and workflows to process the data can be selected to best suit local implementation. In addition, site training, management, and data monitoring approaches can be tailored to each region or country, and managed with a focus on providing each site with comprehensive and tailored study support. A global CRO with international, region-specific experience can be a particularly valuable resource for delivering this level of customized support.

Figure 2. Optimizing observational research programs across regions



1 DEFINE AND DESIGN THE STRATEGY AND COMMUNICATIONS PLAN: BEGIN WITH THE END IN MIND.

At the beginning of any new observational research program, it is critical to “begin with the end in mind” and focus on the final messaging objectives and needs of the target stakeholders.

QUESTIONS THAT SUPPORT THE STUDY DESIGN PROCESS INCLUDE:

- What are the communication objectives—the scientific and clinical messages or product positioning—that each local affiliate needs to relay to their key stakeholders?
- Are there broader healthcare policy or reimbursement issues to address?
- Are there specific prescribing challenges in the region that are linked to physicians’ perceptions about the new product, potentially limiting its uptake and adoption?

Corporate and local marketing managers must define the target stakeholder audiences, the intended use of the data, and the timing and format of the desired communication pieces before study design decisions can be finalized.

In the case study of *Nouveau Pharma, Inc.*, the global launch of their new Type 2 Diabetes medication presents two diverse regional challenges for their post-launch observational

research strategy. Although the purposes of the observational research and the communications that result define how to tailor the product’s value proposition to the needs of each region, there are many common denominators in possible study designs and operational plans that can be uncovered. If each observational research was conducted with only local objectives in mind and without a global perspective, the results would be just that: confined to local applications. On the other hand, a global approach would ensure the collection of data required to support the unique regional purposes as well, creating a global perspective that can be applied across markets.

Local expertise is important. The CRO can foster relationships with local patient advocacy groups, physician organizations, and health care policy makers that possess knowledge of the ultimate needs of the marketplace.

2 EMBRACE LOCAL STAKEHOLDERS' NEEDS AT THE GLOBAL LEVEL.

Achieving corporate-wide objectives for the clinical and commercial success of any new biopharmaceutical product requires an in-depth understanding of regional and country-level differences in medical practices and routine care including:

- Local culture, customs, and effective channels of patient and physician communications
- Local regulatory and ethics committees' guidelines and procedures
- Local market access and reimbursement considerations that may influence product launch, adoption, and long-term acceptance

market. These highly qualified staff understands the local nuances of conducting observational research and can provide the knowledge base and sensitivity to develop productive and lasting relationships in any global or local study environment.

In practice, the global CRO should act as an extension of the local affiliate's core team, understanding that physicians are important decision makers and using the observational research as an opportunity to strengthen and expand relationships between the affiliate's target prescribers and the sponsor. Local knowledge and experience are essential to effective county-level interactions among the study physicians, the sponsor, and the various stakeholders in the local area. To this end, the CRO must employ highly qualified local staff experienced in conducting relevant observational research in their home

3 PROVIDE ONGOING SCIENTIFIC OVERSIGHT AND OPERATIONAL LEADERSHIP TO THE OBSERVATIONAL RESEARCH.

In our dynamic, healthcare delivery world, constant change is a given. A global CRO can provide the perspective, research, and training to keep complex engagements adaptive and ahead of trends and challenges.

A centrally managed infrastructure provides methodological expertise and operational efficiencies to assist local affiliates in support of product launches, market access plans, and post-approval regulatory requirements.

A centralized team of world-class, observational research specialists should be comprised of a strong bench of globally available subject matter experts (SMEs) who represent a wide area of expertise, including:

- Pharmacoepidemiologists and biostatisticians with extensive backgrounds in study design and analytical methodologies best suited for observational research
- Observational research experts trained in all aspects of observational research implementation and delivery
- Health economists and health-related outcomes research specialists
- Patient safety experts up to date on the latest post-market pharmacovigilance requirements

- Reimbursement and market access strategists who understand the competitive landscape and the factors that influence market adoption and successful product commercialization

These observational research specialists should have appropriate SOPs, observational research training materials, and promote continuing education of the project teams as advancements in observational research methodologies, new legislation, and/or changes in health-care policies emerge over time, all of which can be a stimulus for future observational research programs to ensure sponsors' long-term research and commercial needs are met.

4 INTEGRATE LOCAL AND GLOBAL RESOURCES TO OPTIMIZE OPERATIONAL EFFICIENCIES.

When this research infrastructure of local and central experts is fully formed and functioning, the cohesive group's performance exceeds the sum of its parts. Local and global teams complement each other to create a continuum of expertise—from the country-appropriate ways to engage physicians and patients in observational research to globe-spanning strategies for data collection and communication of new information.

While the local teams have the experience to run the study on the ground, the resources available in a centralized global infrastructure support the local efforts by providing:

- A strong bench of SMEs
- SOPs, work instructions, and related process documents
- Coordinated technology development and evolution, such as electronic data capture systems, study web portals, and mobile patient-reported outcomes applications
- A common library of document templates and forms
- Effective, cross-study project tracking tools and communications

These resources are central to a global network of research “hubs” that work to a standardized set of research principles and processes,

promoting uniformity and adherence to the same set of technical and quality standards. They facilitate consistent, high-quality project deliverables. This centralized approach also efficiently utilizes limited global resources to maximize project team productivity, expedite timelines, and potentially reduce post-launch research budget needs.

5 *IMPLEMENT A MULTIFACETED COMMUNICATIONS PLAN.*

As a result of “beginning with the end in mind” and identifying the critical scientific and clinical messages and product positioning to communicate product value, the local observational research designs and operational plans can be harmonized.

The desired communication pieces—meeting abstracts, manuscripts, publications, and exhibitions—should align with the needs of the local target audiences, as well as support corporate commercialization goals. Clear, standardized data can advance important clinical evidence outside of their regional context to support consistent product branding and clinical messaging globally.

With the support of a global CRO that understands today’s highly-regulated clinical development and medical communications environment, a well-executed communications plan allows local affiliates to deliver a wide range of clinical information to multiple stakeholders. Communication of healthcare discoveries and scientific knowledge may foster improved product adoption, accelerating the benefits of new treatments to patients in need. By integrating

the vast data generated from local physician practices with the strategic vision and resources afforded by a central infrastructure, sponsors can transform their observational research programs from limited, localized post-marketing efforts to sources of powerful, clinical information of global, strategic value.

SUMMARY

Leverage the value of a globally-integrated development team.

For the local affiliate, approaching observational research programs on a study-by-study basis without taking advantage of available global resources means having to navigate increasingly complicated observational research requirements and to satisfy an increasing number of diverse stakeholders on their own. Beginning with the end in mind, a CRO approaches an operational strategy and communications plan from a global perspective that can optimize the sponsor's global competitive commercial position, while simultaneously customizing to

local affiliates' needs to strategically conserve resources and to strengthen their regional marketing position.

When local affiliates take advantage of a strong, centralized research infrastructure, study results from individual regions or countries may be better compared to results from other areas. The resulting synchronization facilitates compatibility of study designs, uniformity in standards and work processes, and more opportunities to link data to uncover important patient trends and promote more consistent and compelling

global clinical messaging. Local affiliates benefit from expert support and state of the art technology to assist in observational research design and implementation, while the global team develops the mission-critical communications to support the sponsor's global commercialization efforts as a whole.

Our regional leads are always available for a conversation.

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