

Advance access

Use of named patient programs to enable patient access and enhance global market development

It's a small world, and for pharmaceutical and biotechnology companies, it's getting smaller by the day. Drug pipelines are more visible to the public than ever before. The upsurge in media coverage and Internet use has made it possible for patients and physicians worldwide to keep a close watch on drug candidates in late-stage development – particularly those that address currently unmet medical needs. Once a medicine is approved in one market (and sometimes before it is approved anywhere), a company is likely to receive numerous requests for the drug from individuals around the world who have run out of therapeutic options.

“The reality is that awareness among physicians and patients is only going to continue to grow,” says John Lagus, Vice President of Business and Corporate Development for IDIS, a company focused on the development, implementation and management of named patient programs. “As that happens, companies will need to think about how they’re going to respond, both in the U.S. and internationally.” For many companies, the ethical choice is to meet this demand in a safe, controlled manner by creating a named patient program, or NPP, and fortunately this ethical choice can also yield practical benefits for the sponsoring company.

Named patient programs are not commercial activities; their sole purpose is to provide access to medications that address unmet medical needs – often potentially life-saving treatments. However, once companies have made the decision to implement such a program, it makes sense to evaluate the potential benefits an NPP can provide in the area of global market access and development – especially given the shrinking window of opportunity for gaining a return on investment during new product launches.

On May 7, 2008, *Pharmaceutical Executive* and IDIS presented a live, interactive webcast discussing how experience, insights and relationships gathered through named patient programs can assist companies as they embark on new product launches. This summary will start with the basics of NPP planning and implementation and move on to examine applications in market access and development.

A NAMED PATIENT PRIMER

Named patient programs provide controlled access to innovative drugs in response to requests by physicians in markets outside the United States on behalf



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of specific (or “named”) patients. These requests can occur when:

- A drug is approved in one country but not in the patient’s home country
- A drug is in late-stage clinical trials but is not yet approved
- A launch is not planned in the patient’s home country

There are a number of ways companies can meet pre-approval demand from physicians and patients within the United States, including treatment- and single patient-IND programs. But when U.S.-based companies receive requests from overseas, they can respond by creating a named patient program to manage this international demand in a way that’s ethical, legal and controlled. Named patient programs are conducted in consultation with local health authorities.

Advantages for patients and sponsors

First and foremost, these programs are for the benefit of patients – who may be in desperate need of treatment for life-threatening or debilitating illness with no suitable alternatives available locally. For many patients, license approval in their local market may come too late. In some cases (those involving the European Union registration and approval process, for example), a drug may not clear reimbursement hurdles in an individual country until long after regulatory approval is granted. Or, since a formal launch may not even be planned in some countries, a patient may not have any other opportunity to receive the drug through company-sanctioned channels. In such situations, patients may attempt to obtain treatment by any method possible – often outside the manufacturer’s oversight, which can lead to safety and supply problems.

Named patient programs allow companies to provide access to innovative drugs while maintaining strict control over where the drugs go and how they are used. Sponsoring companies can:

- Determine the treatment criteria used to evaluate physician requests, ensuring that only appropriate patients receive the drug
- Educate physicians and pharmacists on proper use of the drug to minimize errors, particularly in cases where dosage and administration are complex
- Capture additional, real-world safety data

Considering an NPP

Many companies wait until they begin receiving international demand to consider a named patient program, but the best time to initiate an NPP is six to 18 months before the drug is expected to gain marketing authorization, typically when the drug is in or has completed phase III clinical trials. How can a company foresee the need for a named patient program far enough ahead to allow for optimal timing?

Every company must decide whether to create a named patient program based on its individual circumstances, but there are a few indicators that suggest an NPP might be in order. First, does the drug address a currently unmet medical need? Second, is the company seeing signs of future demand? “Companies know their drugs best, and it varies from product to product,” says Lagus. “But if they’re developing a new drug and they’re getting lots of interest from investigators in the U.S., and potentially other countries if they’re doing global studies, that is an indicator.”

Initiating an NPP

Once a company has decided to establish a named patient program, the first step is to get the timing right. Demand for a medicine often appears about 12 months prior to approval. If the program begins too early (say, 24 months prior to approval), supplies of the drug may not be adequate to meet the needs of both the NPP and any ongoing clinical trials. If the program is started too late, too few patients may be served to justify the effort. And to avoid compromising recruitment, companies may want to wait until patient accrual ends for any local registration trials.

Ideally, planning for an NPP should begin six months before the program starts, allowing time to:

- Prepare documents and contracts
- Assemble educational materials for physicians and pharmacists, including the package insert, drug background, and dosage and administration information
- Establish treatment criteria

This also allows sufficient time to bring key stakeholders into the process (see Figure 1), including:

- Medical affairs
- Clinical development

- Regulatory affairs
- Supply chain management
- Pharmacovigilance
- Global commercial group
- Affiliates

A multifunctional team is necessary to handle the logistical and tactical elements involved: Are supplies of the drug adequate to support the program? What clinical restrictions should be considered when formulating treatment criteria? What educational information will be necessary to ensure that physicians and pharmacists understand how to use the drug properly? What data should the sponsor collect?

Getting stakeholder groups together early in the process will maximize the benefit to both the patient and the company sponsoring the NPP. It's particularly important to come to a consensus on certain crucial points, such as treatment criteria, when the program will start and end, and pricing. "That same cross-functional team can also consider the benefits that may arise from a market development standpoint – the earlier, the better," says Susan Nemetz, President of The Nemetz Group and former Vice President of International Commercial Operations at Millennium Pharmaceuticals.

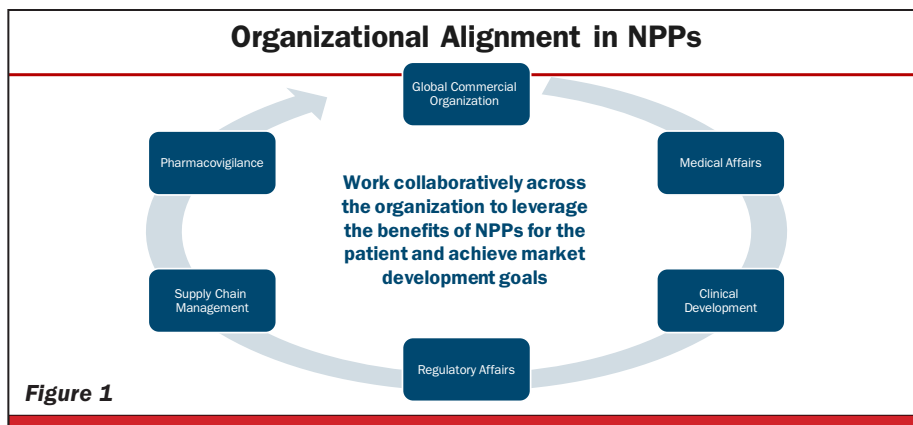


Figure 1

A named patient program specialist (typically an outside partner) can be a critical member of the team. Most countries have their own regulations governing pre-approval access through named patient programs. An NPP specialist can serve as a liaison with international sites, coordinating with physicians and pharmacists, helping to meet all the necessary regulatory requirements, and ensuring stringent control of the drug. Most importantly, partnering with an NPP specialist allows the sponsor company to accomplish these goals without diverting valuable time or internal resources.

Building awareness

Because a named patient program is not a commercial activity, the sponsor may not promote it or target specific physicians. So how does word get out? The key is drawing a distinct line between information and promotion:

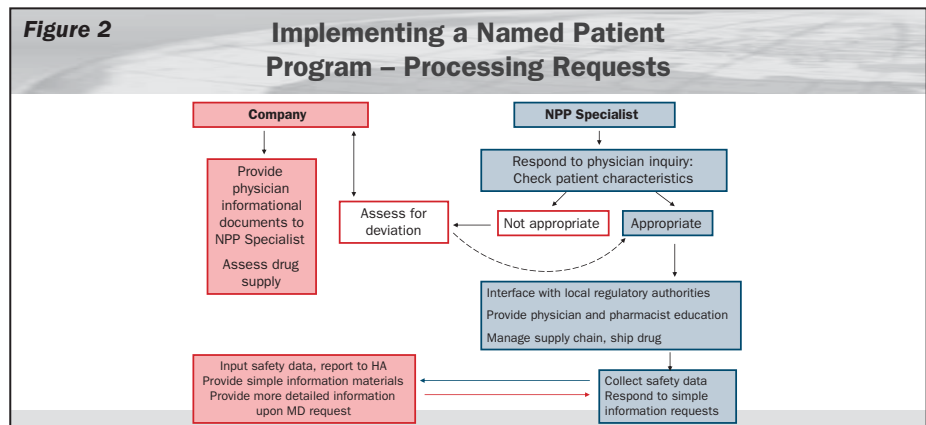
- Sponsoring companies can issue a press release announcing the existence of a strategic partnership to offer medicines through named patient programs.
- If a physician calls the company to request access to a drug, that physician can be redirected to the named patient program specialist.

- Companies can respond to unsolicited inquiries and provide requested information about the drug.

The important thing is to be ready for these requests, particularly at medical meetings. “At U.S. meetings such as ASCO and ASH, many of the attendees are international physicians, and they’re going to presentations where physicians are talking about how a new medication is being used,” says Lagus. Some companies may be surprised by how much international awareness already exists, he adds. “We’re seeing cases where U.S.-trained physicians return to their home country after using a drug in the United States or even serving as a co-investigator in a registration trial. These physicians want access to these new medicines not available in their countries on behalf of their patients.”

Processing requests

Upon receipt of a physician request for pre-approval access, the sponsoring company or its partner NPP specialist first determines whether the patient meets the treatment criteria. If not, a waiver may be requested. If so, the company or its specialist partner collects information on the individual case for safety and pharmacovigilance purposes



– such as the patient’s location and year of birth, as well as the prescriber’s name, country and hospital – and provides the physician with educational materials. Paperwork is then prepared as required by local regulations and the drug is shipped to the physician or pharmacist. Adverse event data are reported to the company (either directly or via the NPP specialist) to be entered into the pharmacovigilance database and reported to the appropriate health authorities. Figure 2 illustrates the roles of the company and the NPP specialist in processing physician requests.

Concluding the program

The decision to end a named patient program is specific to the individual company and product. A program might last until marketing authorization is obtained, or even longer if the drug distribution network is not yet in place or reimbursement has not been established (in some countries, negotiations can last more than a year).

In many situations, marketing authorization may be granted in one region long before others. “Unlike the United States, where a company can launch from day one in all fifty states, in Europe companies often need to conduct staged launches – even if they’ve received a centralized EMEA approval,” says Lagus. “A named patient program allows a company that has already launched in some European countries to continue to provide that medication to patients in other European countries where the drug is not yet commercially available. It gives the company flexibility in how they handle Europe in particular, but also as they think about launching around the rest of the world.”

PREPARED FOR LAUNCH

Once a company has decided to imple-

ment an NPP, it makes sense to look at ways the program can address some of the current barriers to global market access and development, including external factors such as:

- Multiple approvals required for registration, pricing and reimbursement
- Staggered launch opportunities and associated timing challenges
- Increased competition for customers’ time and attention

A named patient program can also address internal barriers, such as:

- Functional and geographic silos and a resulting lack of alignment on product strategy
- Incomplete market data
- An incomplete understanding of customers and their needs
- Tension between traditional methods and the need for innovative approaches to respond to changing market dynamics

“In the past, named patient programs haven’t really been considered for the benefits they provide in the market development arena,” says Nemetz. “That may be partly because these programs cannot be promotional, so the people who think from a purely commercial perspective don’t consider this option.”

That’s unfortunate, because the nature and structure of named patient programs can yield significant benefits in three key market development categories (see Figure 3).

Assessment and planning

To plan an effective international launch, companies need to understand the market they’re entering, the current treatment pathway, and what unmet need the drug addresses within that market. By its very nature, a named patient pro-



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POTENTIAL BENEFITS FOR MARKET DEVELOPMENT:

ASSESSMENT AND PLANNING

- Deeper insight into therapeutic area
- Knowledge of treatment patterns, standards of care, decision-making process
- Identification of appropriate patients and protocols
- Enhanced accuracy of launch forecast
- Refinement of demand estimates
- Prioritization of target markets and identification of new markets
- Foundation for partnering strategy (out-licensing)

CULTIVATION AND RELATIONSHIP DEVELOPMENT

- Access to and relationship-building with KOLs and physicians
- Information on payer interaction and pricing
- Potential to begin setting payer expectations through price used in program
- Response to ex-U.S. demand
- Support for launch of field-based personnel (MSLs) to support physicians
- Input to customer segmentation

DEPLOYMENT AND OPERATIONS

- Insight into optimal supply chain and operations infrastructure
- Testing and resolution of potential supply chain issues
- Testing and modification of core commercial systems (sales tracking, CRM)
- Refinement of key forecast assumptions
- Insight into core processes and standard operating procedures
- Support for launch of field sales force

Figure 3

gram provides two important insights: If a company is receiving pre-approval demand for a drug, knowing where that demand is coming from can help define the unmet need. Also, knowing about patients' demographics, diagnosis and previous treatment at the time their physicians make the decision to request the medication helps the company understand where the drug fits into the current treatment pathway.

The sponsoring company can also receive informal feedback through physician interactions. This can give the team a more complete picture of:

- Patterns of demand and how the drug will ultimately be used in that market
- Whether the drug meets local physicians' expectations
- Whether the educational support provided to physicians and

pharmacists hits the mark or needs to be adjusted prior to launch

Cultivation and relationship building

Named patient programs naturally involve interaction – and thus, foster relationship development – with local key opinion leaders and other physicians who would be likely prescribers post-launch. This can give the sponsoring company a better idea of the needs, beliefs and priorities of the physician segments most likely to adopt the drug.

Named patient programs can also help reduce the risk of preventable adverse events and increase the likelihood that patients' experiences will be positive. "The reality today is that once a drug is approved in one market, you'll see international pharmacies and wholesalers seeking to obtain the product in another

er country on behalf of a specific physician,” says Lagus. Providing access through a named patient program gives the manufacturer the control necessary to ensure that only suitable patients receive the treatment, healthcare providers have all the educational support they need to optimize dosage and administration, and all safety precautions are taken. This is crucial to the welfare of the patient, and it’s also critical to the health of a commercial launch. Says Lagus, “These physicians are potentially long-term customers, and companies need to make sure that these potential customers have the best possible experience with their new medicine.”

Participating physicians may also serve as resources for regulators and other local healthcare providers who are considering the benefits the drug can provide to patients in that country. “Post-approval, physicians who have experience with the medication may ultimately be the go-to physicians locally or regionally for others who are trying to gain that experience,” says Nemetz. “We know that peer-to-peer interaction has a huge impact on the success of commercialization.”

In most countries, pricing decisions related to an NPP (whether to charge and how much to charge) are left up to the sponsoring company. If a company chooses to charge for named patient access to the drug and the fee is close to the anticipated market price, the program can begin to set payer expectations about pricing prior to launch.

Deployment and operations

When launching a new drug, a company must align its deployment and operations objectives around its overall strategy, while tailoring tactics to the local situation. If a cross-functional team has already been assembled to oversee a

named patient program, the company has a head start. “This kind of program gives you the opportunity to get everyone on the same page very early on,” says Nemetz. “You’re able to identify the gaps in previous planning, whether they’re specific, tangible things – like how the drug gets from the manufacturing site to the institution – or more alignment-oriented gaps. For instance, are patient education programs consistent with what the marketing, medical and clinical teams all think is appropriate?”

In the context of a relatively small, manageable program, the sponsoring company’s team can work out any kinks in the supply chain, fine-tune its educational support for physicians, pharmacists and patients, and coordinate internal functions ahead of time – increasing the chances that the launch will run smoothly right off the bat. Says Nemetz, “Having that real-time experience in a given therapeutic area with your own drug and your own team is well worth the time and effort.”

DOING THE RIGHT THING – AND BENEFITING

Once a drug that relieves an unmet need is approved in one market, desperately ill patients in other regions are likely to seek access to it. The question is whether the manufacturer will assume control of this process or leave patients’ and physicians’ experiences with the drug up to chance. And if companies do choose to implement a named patient program, they owe it to themselves to find out how the program can enhance the success of their international commercial launches. This is one case in which the best interests of patients, physicians and pharmaceutical companies intersect – with named patient programs, the ethical choice is also the practical choice.

*To replay the free
webcast, visit
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MarketAccess](http://PharmExec.com/MarketAccess).*



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CASE SCENARIO 1

Controlling safety, supply and customer experiences

A mid-sized European company had received approval in one European country for a novel orphan drug but was still awaiting pan-European approval and launch. Given the high degree of media awareness of the drug and the volume of demand from European physicians, the company initiated a named patient program.

“The drug’s dosing and administration required some additional education,” says Lagus. “The company was concerned that if physicians acquired the product from other sources without proper education being given, they might see adverse experiences that could give the brand a poor image. And second, they were concerned that if wholesalers or traders were trying to acquire the drug, there wouldn’t be sufficient supplies left in the market where the drug was already approved.”

The company also hoped to clarify its launch forecast and set proper pricing expectations among healthcare providers and payers.

Results

Through the program, the company was able to:

- Provide the drug to 100 patients at more than 20 centers across Europe over the six months prior to pan-European launch
- Supply pharmacists and physicians with the necessary educational materials to guarantee proper administration of the drug; as a result, no medical errors due to improper administration were reported
- Control the drug supply and guarantee availability in the market where the drug was already approved
- Improve its understanding of the adoption cycle, enhancing the accuracy of market forecasts
- Begin to set payer expectations by setting a price that was close to the anticipated market price

CASE SCENARIO 2

Gauging international demand

A large U.S. company was awaiting approval for a novel oncology agent in late-stage development, and already its supply chain and medical affairs groups were being overwhelmed by compassionate use requests – both at home and overseas – spurring the creation of a named patient program.

The company hoped to develop a better understanding of international oncology markets, foster relationships with physicians in those markets and gain insights that would help determine where to set up commercial operations and where to partner.

Results

Through the program, the company was able to:

- Treat more than 700 patients over 24 months
- Engage nearly 350 physicians in 30 countries
- Gain greater insight into the therapeutic area
- Effectively address demand during the product’s phased European launch

Given its initial focus on Europe, the company was surprised by the number of requests coming from other markets such as the Middle East and Southeast Asia. “Companies can’t always anticipate where demand will come,” says Lagus. “A company might initially want to implement a named patient program in just three countries in Europe, but soon they start to see demand coming from other countries.”

The ability to more accurately gauge international demand – as well as the complexity of establishing access in various markets – served as a foundation for the company’s future partnering strategy. “For a U.S.-based company, setting up commercial operations outside the United States is not easy to do,” says Nemetz. “You would definitely want to do it in a place where you had the greatest likelihood of success.”