THE BENEFITS OF Prelaunch Access

Contributed by Natalie Douglas, CEO, IDIS

PRELAUNCH ACCESS TO DRUGS CAN PROVIDE MONTHS AND PERHAPS YEARS OF TREATMENT FOR PATIENTS WITH UNMET MEDICAL NEEDS WHEN ALL OTHER THERAPEUTIC OPTIONS HAVE FAILED OR NO OTHER OPTIONS ARE AVAILABLE.

The benefits of named-patient programs are best realized when a company incorporates planning for such programs early in a drug's clinical development stages and includes all relevant stakeholders in the process.

Connie Loughman of Indiana recently gained worldwide attention with a YouTube video she created, which advocated access to an investigational pancreatic cancer treatment for herself and fellow patient, actor Patrick Swayze. Pamela Northcott from Wales, diagnosed with kidney cancer, was told her treatment options were limited. Two drugs considered to be standard treatments were not available to Pamela as they had yet to be approved by The National Institute for Health and Clinical Excellence. Pamela challenged the Welsh Assembly government for access.

Transparency of drug-development pipelines, intense media coverage of healthcare issues, and global access to information about drugs in the prelaunch phase via Websites and blogs have created a more educated and empowered population of patients. Heightened awareness, in combination with patients taking a more determined approach to their care, has brought with it an increase in demand for access to these medications despite their prelaunch status, particularly among those patients who have exhausted all other therapeutic options.

Pharmaceutical and biotechnology companies have various options for responding to requests for access to drugs prior to their launch. Before responding to these requests, however, companies should understand their options and the regulations governing them, and involve key stakeholders when planning for early access.

ENABLING PRELAUNCH ACCESS

Governments worldwide have created provisions for granting access to prelaunch drugs for patients who have exhausted all alternative treatment options and who do not match clinical-trial entry criteria. Often grouped under the labels of compassionate use, expanded access, or named-patient supply, these programs are governed by rules that vary by country.

In 1987, the U.S. Food and Drug Administration (FDA) formally put in place regulations to facilitate patient access to prelaunch drugs. FDA-recognized expanded access programs (EAPs) include treatment investigational new drugs (treatment INDs), treatment protocols, and single-patient INDs. Treatment INDs and treatment protocols allow a cohort of patients who would not otherwise qualify for clinical trials to gain access to a prelaunch drug, generally when the drug is in Phase III trials. A single-patient IND is a request from a physician to the FDA that an individual patient be allowed access to a prelaunch drug on an emergency or compassionate basis — when all other options have failed.

WHEN DEMAND IS GLOBAL

When a drug is approaching launch or has been approved in the United States but not yet in other countries, U.S.-based pharmaceutical and biotechnology companies can expect to receive requests for these drugs from around the world. As such, companies should be familiar with the various regulations defining prelaunch access on a global basis.

Canada's well-defined Special Access Program (SAP) provides access to nonmarketed drugs to practitioners treating patients with serious or life-threatening illnesses when conventional therapies have failed, are unsuitable, or are unavailable. In this procedure the practitioner is responsible for initiating a request on behalf of a patient and ensuring that the decision to prescribe the drug is supported either by credible evidence available in the medical literature or provided by the manufacturer. This process is time-intensive as each case requires a review, followed by an authorization by Canada Health to enable an unregistered drug to be administered to a patient with an otherwise unmet medical need.

In Australia, patients can access experimental drugs via the Special Access Scheme, while in Japan Named-Patient Access programs allow access to drugs with an expectation that the drug first be approved in the exporting country.

Almost 20 years ago, a European Union (EU) Council Directive laid out the framework for the supply of unregulated medicines in response to unsolicited requests for use by individual patients "on his or her personal responsibility." The current legal basis for access to prelaunched medicines in the EU is Article 5 of Directive 2001/83/EC. This legislation offered the possibility of prelaunch use as an exception to the rule that medicines must be authorized

before use or used within the context of an approved clinical trial. In 2004, Regulation 726/2004 further evolved compassionate use programs as the EU expanded.

While prelaunch use is permitted by EU legislation, these programs are governed by individual member states; each of the 30 member states of the European Economic Area (EEA) has its own nationalized regulations regarding the import of prelaunch medicines. It should be noted that even after a drug receives approval, authorities in each country can take several months to make reimbursement decisions, which can delay the ability of patients to access these drugs.

In a majority of EU member states, an authorization from a competent authority is required to initiate named-patient supply; in other countries it may be retrospective. For example, the French regulatory agency (AFSSAPS) is primarily concerned with the science supporting a patient's request for access to a prelaunch drug and has in place a proactive approval system. In contrast to the French system, in Ireland the prime concern is transparency of supply and patient safety, so retrospective notification is required.

Interestingly, the liability for use of unauthorized drugs falls to different parties, depending on the member state. In most countries, the responsibility resides with the patient's physician; in others (Germany, Greece, and Sweden, for example), liability resides with the company. France and the United Kingdom split the responsibility between the company (responsible for faulty products) and the physician (responsible for any clinical negligence).

In a majority of EU member states, an authorization from a competent authority is required to initiate named-patient supply while in other countries, all that is required is an import certificate. In Austria, Greece, and Spain the drug company is expected to provide named-patient products free of charge to individual patients; in other member states, either the patient, the hospital, or the national insurance system pays.

Despite these differences, the reporting of adverse events is expected in all EU member states (set forth in Article 83). The marketing authorization holder or manufacturer must report serious adverse reactions (SARs) occurring within the EU to the competent authority of the member state in which the incident occurred.

PREPARING FOR PRELAUNCH ACCESS

Below are key considerations when implementing a named-patient program, the process by which companies can enable physicians, on behalf of their patients, to access medicines approved or nearing approval in other countries before marketing approval has been granted in their home country, in a legal and ethical way.

Named-patient programs are typically put

THROUGHOUT THE PRODUCT LIFE CYCLE

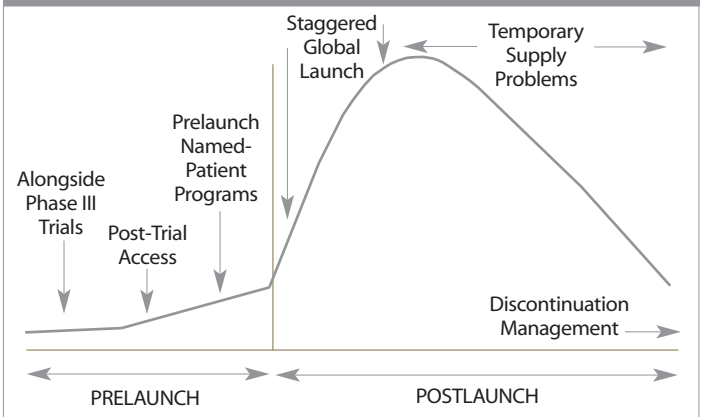
NAMED-PATIENT PROGRAMS CAN BE USED TO PROVIDE PATIENT ACCESS AT A NUMBER OF STAGES THROUGHOUT A PRODUCT'S LIFE CYCLE

- During Phase III, to patients with an unmet medical need who do not meet the clinical trial inclusion criteria
- Bridging the gap between the end of Phase III and receipt of marketing authorization
- In conjunction with a global launch while approval and reimbursement are being sought across countries worldwide

NAMED-PATIENT PROGRAMS ALSO PROVIDE AN ALTERNATIVE ROUTE TO PATIENT ACCESS AS PART OF A GLOBAL COMMERCIALIZATION STRATEGY WHERE

- A formal launch is not planned
- A sales infrastructure does not exist
- It is not commercially viable to seek a marketing authorization

WHEN CAN A NAMED-PATIENT MEDICINE BE USED



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forth at any stage post-Phase II and can run in parallel with Phase III trials until commercial launch. Timing for a prelaunch program should be carefully thought through, ensuring care is taken not to interfere with established clinical trials. If the program is started too late, there may not be sufficient use of the program. In cases where demand for the drug is extremely high, however, initiating the program within just a few months of expected authorization is warranted.

Sufficient planning time must be allowed to bring key stakeholders into the process; these stakeholders include medical affairs, clinical development, regulatory, supply chain, pharmacovigilance, and the global commercial group and affiliates. In addition, adequate time must be allocated to develop information for physicians and pharmacists regarding dosing, administration, and restrictions, and to establish treatment criteria to ensure proper selection of patients.

COMMUNICATING AVAILABILITY

Companies providing access to named-patient drugs must exercise caution when communicating such programs. Sponsoring companies can provide information regarding prelaunch access following an unsolicited request to physicians at medical conferences, for example, but cannot solicit interest from target physicians, advocacy groups, or patients, nor can the company use its salesforce to promote such programs.

BENEFITS TO THE SPONSORING COMPANY

In addition to providing significant benefit to patients with unmet medical needs, these programs can offer important benefits to the sponsor in the areas of assessment and planning, relationship building, and operations.

Assessment and Planning

By their very nature, these programs provide two important insights: If a company is receiving prelaunch 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 new treatment fits into the current treatment pathway.

Relationship building

Named-patient programs foster relationship development with local key opinion leaders and other physicians who would be likely prescribers postlaunch. 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. Postlaunch, 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.

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. In the context of a named-patient program, the sponsoring company can work out any problems in the supply chain; fine-tune its educational support for physicians, pharmacists, and patients; and coordinate internal functions ahead of time, thereby increasing the chances that the launch will run smoothly.

PATIENT BENEFITS

Prelaunch access to drugs can provide months and perhaps years of treatment for patients with unmet medical needs when all other therapeutic options have failed or no other options are available. The benefits of named-patient programs are best realized when a company incorporates planning for such programs early in a drug's clinical development stages and includes all relevant stakeholders in the process. Some companies prefer to manage their programs in-house, while others prefer to partner with a named-patient program specialist, but regardless of the approach, these programs are vital for meeting global demand for prelaunch medicines.

IDIS assists sponsors in the field of named-patient medicine sourcing and supply. For more information, visit idispharma.com. Natalie Douglas can be reached at: ndouglas@idispharma.com.✦

PharmaVOICE welcomes comments about this article. E-mail us at feedback@pharmavoice.com.

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