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NAMED PATIENT PROGRAMS YIELD SIGNIFICANT RESULTS

Access to information about drug development and clinical pipelines has become readily available on a worldwide basis. Physicians and patients are now more informed than ever before about innovative drugs in late-stage development, and they are keenly aware of drugs that have been approved in foreign countries but not yet approved in their own. As a result, global demand for innovative medicines in late-stage development or medicines approved in a limited number of countries has grown substantially over the past few years, motivating pharmaceutical and biotechnology companies as well as regulatory bodies to find ways to provide access to these drugs in a controlled, legal, and ethical way. In particular, the robust pipelines of the American companies have attracted a marked increase in requests for these medicines from overseas.

For U.S. patients, there are three avenues by which companies can offer access to pre-approved medicines: clinical trials, investigational new drug (INDs), and single patient INDs. The latter two are often referred to as "compassionate use" or "expanded access."

For requests originating outside of the United States, however, named patient programs are used to address this demand. These programs allow companies to systematically respond to requests for drugs either in late-stage development or drugs approved in the United States but not yet in the requestor's country, directly from physicians or via national health services — depending on local regulations — on behalf of specific, or "named" patients. Thus, the pharmaceutical or biotechnology company can ensure that only patients meeting set selection criteria have access to the drug. This reduces the risk that a physician in an unlicensed market seeking a particular drug will attempt to procure the product from a licensed market on his or her own and without any proper training in the drug's use.

MULTIPLE STAKEHOLDER BENEFITS

Patients are, of course, the biggest beneficiaries of these programs. Named patient programs allow physicians to obtain drugs on behalf of their patients, most often for those suffering from chronic life-threatening diseases, for example, cancer, infectious diseases such as AIDS, or rare diseases that require an orphan drug.

Pharmaceutical and biotechnology companies also realize significant benefits from use of named patient programs, including the generation of physician and patient goodwill, opportunities for physician and pharmacist engagement and education, real-life data capture, product visibility, additional market intelligence, as well as revenue.

In addition, these programs can provide data on physicians and prescribing rationale to assist early-stage brand advocacy. A named patient program also can speed uptake of a product after its official launch within a market. Physicians and pharmacists who participate in these programs will be familiar with the product and will likely become early adopters once marketing approval has been granted. These individuals can often act as references for other healthcare providers in the market due to their premarket experience. Information on the volume of pre-approval requests can also help manufacturers refine postapproval market forecasts.

MANAGING THE PROGRAM

The collection of additional pharmacovigilence data is a significant factor in many companies' decision to launch a named patient program. Adverse event data are collected and reported via a fully compliant pharmacovigilance plan. The adverse event data gathered through such a program could be used as supporting evidence for a company's market access strategy.

Companies are allowed to charge for the drugs distributed through named patient programs. In most countries, compensation comes through the national health system or directly from the patient, though the manufacturer can still make the medication free of charge if it chooses. For smaller companies, the ability to charge for this access can lead to an important boost in revenue.

Some companies choose to manage these programs in-house; most, however, do not have the necessary administrative and logistical resources nor the regulatory expertise to effectively and efficiently handle these drug requests while remaining in compliance with local regulations. Each of the 30 member states in the European Economic Area, for example, has its own set of nationalized regulations governing named patient programs.

IN CONCLUSION

In summary, named patient programs provide pharmaceutical and biotech companies of all sizes with a legal and ethical means to:

- Make innovative medicines available to meet preapproval demand from physicians and patients.
- Create a prelaunch pool of physicians and pharmacists instructed in the proper use of a new drug.
- Gather adverse event data that can be incorporated into a drug's safety profile.
- Improve forecasting based on the volume of prelaunch requests received from various markets.

Regardless of whether a company manages its program internally or partners with a named patient program specialist, companies that do not consider setting up such programs, particularly for drugs that will likely generate large prelaunch demand, are putting themselves at a disadvantage.

Natalie Douglas is CEO of IDIS, Weybridge, United Kingdom; IDIS provides strategic solutions for managing prelaunch access to FDA-approved drugs, and in the last three years has managed more than 40 named patient programs in partnership with pharmaceutical and biotech companies worldwide. For more information, visit idispharma.com.

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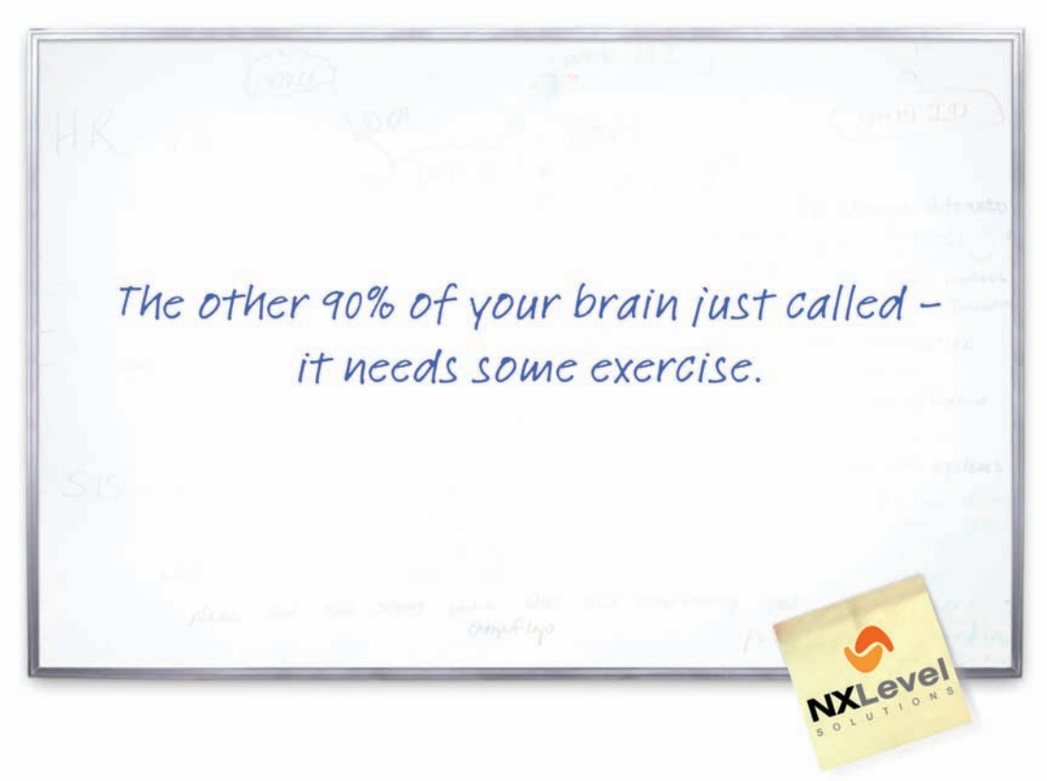

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