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Working Toward Common Goals AGENCY-CLIENT RELATIONSHIPS

A JOURNEY OF DISCOVERY with Frank Baldino

Deriving Value from PATIENT REGISTRIES

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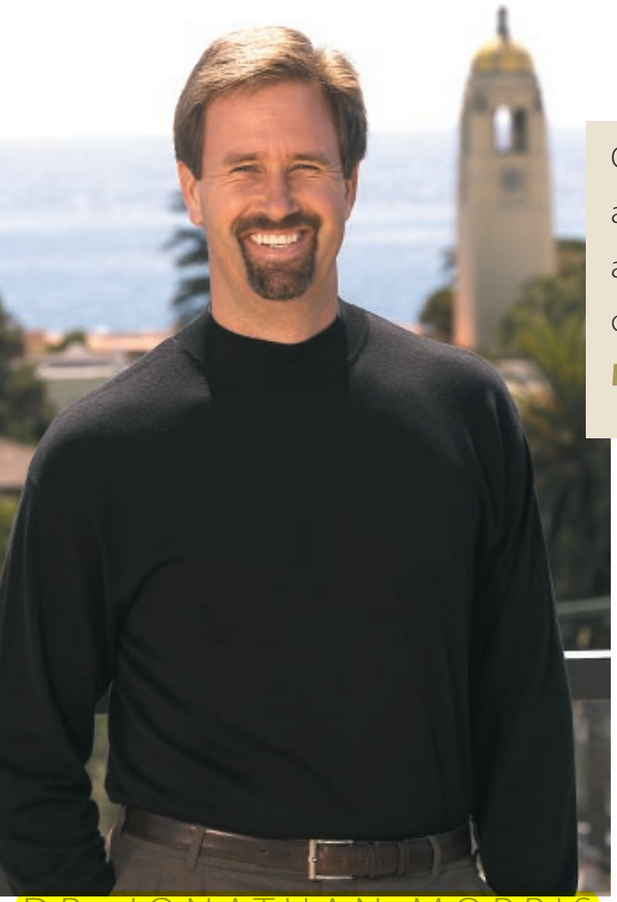
BY DENISE MYSHKO

PATIENT REGISTRIES
OBSERVATIONS OF THE REAL WORLD

DR. HERVE CASPARD

There is no such thing as a standard registry. **WE HAVE TO STRIKE A BALANCE: DO WE ASK A LOT OF QUESTIONS OR JUST A FEW?** We also need to consider the work load for the investigators.

Through patient registries, **PHARMACEUTICAL COMPANIES CAN OBTAIN REAL-WORLD EFFECTIVENESS DATA ABOUT PRODUCTS,** including adverse events and outcomes in various patient populations, that go beyond the limited confines of controlled trials.



DR. JONATHAN MORRIS

On the regulatory front, experts predict that agencies will request more registries as a postmarketing commitment.

"As regulatory agencies strive to approve products earlier, they are looking to ensure that biopharmaceutical companies have in place risk-management strategies," Dr. Verst-Brasch says. "While the FDA may not require a safety-registry study, many companies are being proactive and conducting such a registry as a risk-mitigation approach. Risk management is the new buzzword in this heightened regulatory environment."

According to Christopher-Paul Milne, DVM, MPH, JD, assistant director of Tufts Center for the Study of Drug Development, risk management efforts are just beginning.

"There weren't many risk-management specific PMCs in 2003, but I think we will see more as they are incorporated into the risk-management plan concept," he says.

Clinical-drug development and safety assessments have become much more sophisticated in recent years, with more of an emphasis on defining risk in a more quantitative way, says Sandy Kweder, M.D., deputy director of the Office of New Drugs, at the U.S. Food and Drug Administration.

> Companies that view registries as an effective growth tool and not just a burden or an obligation **WILL BE THE MOST SUCCESSFUL.**

"To define the risk, companies often need to go beyond gathering the data that might be available in a typical new drug application," she says.

In light of FDA requests for more post-marketing data on newly approved drugs, Sean L. Hart, B.S., MBA, CEO of BBCI, says the agency is likely to request restricted access or product-specific registries that identify specific safety signals over time.

"FDA officials are discussing risk-management strategies, and that is why we're hearing more about registries and large simple safety studies," he says. "The FDA is talking more about registries and large, simple safety studies as part of its new overall risk management/risk assessment pharmacovigilance efforts."

Additionally, registries can help define the safety parameters for drugs with a narrow therapeutic window.

"If the population can be defined through an electronic registry — and this really only can be done electronically — then companies will be able to collect data and authorize the dispensing of the drug based on the parameters defined in the registry," Dr. Gliklich says. "Therefore, more drugs with a narrow safety window could become available for more appropriate populations. The FDA is going to be evaluating this carefully. As a result, this will be a huge opportunity for sponsors because drugs that weren't necessarily thought safe enough for large populations might be safe for 10% of the population."

REGISTRIES VS. CLINICAL TRIALS

Patient registries can be product-oriented, disease-oriented, or focused on a particular patient population. Unlike Phase III or Phase IV trials, registries are not blinded. They may have a comparator group, but there are no inclusion and exclusion criteria for patient recruitment. In other words, all patients taking a particular drug or using a particular device or who have a certain disease are eligible to participate.

Registries generally focus on effectiveness while Phase III trials look at efficacy. Registries typically are observational where trials are randomized. And registries tend to be larger than clinical trials.

Mr. Hart says to be a true registry, there has to be prospective enrollment of patients receiving a particular drug or a series of drugs within a specific disease. The patients need to be tracked to evaluate a specific effectiveness or safety outcome.

"I say effectiveness because we're really not proving efficacy," he says. "There has to be a given endpoint, and there has to be a well-constructed data collection method with rigorous follow up. In the past, some registries didn't have these criteria."

A registry does not try to show that a product is having a particular effect. A registry

> Once a drug is on the market, **WE WANT TO UNDERSTAND HOW IT PERFORMS** in a wider group of patients beyond the patients meeting the inclusion/exclusion criteria of the clinical trials.

JOHN WALT

ways for a small company with a new technology is marketing."

Mr. Patrick says the company originally expected enrollment of about 500 patients.

"We didn't fully anticipate the interest that the medical community would have in a registry to better understand how the technology can be used," he says. "The next time, we will set our sights a little higher and use an electronic system to more effectively manage the enormous amount of data that is generated."

Today, less than 10% of registries use electronic systems for data capture, says Jonathan Morris, M.D., chairman, CEO, and president of ProSanos Corp.

"The regulatory environment for prospective randomized clinical trials has helped define the specifications for EDC implementation," he says. "In the registry or postlaunch environment, there hasn't been the same regulatory or business push, so many companies are still trying to figure out exactly what they should do and how they should do it."

Dr. Gliklich says pharma companies need to go beyond traditional EDC systems to collect information for registries.

"Because registries serve as a reporting system and as an active knowledge base, there has to be the ability to generate patient-management and practice-management tools for sites," he says. "The idea is for a registry to become a population-management tool that providers can access."

REGISTRY CHALLENGES

As with any study, conducting a registry poses some challenges.

"Patients may or may not see their physician," says Leanne R. Larson, senior VP of patient registries, at Ovation Research Group. "They may or may not take their medication. They may or may not complete questionnaires. Companies have to overcome similar issues with physicians."

She says companies likely will be working with sites that may or may not have a research infrastructure or may even be research naive.

"Companies have to design and manage a registry program in a way that doesn't burden the sites," Ms. Larson says.

Registries are different from trials, and every aspect needs to be addressed from the perspective of the strategic goals of the sponsoring organization, Mr. Trotter says.

"One of the challenges that we often face is working with internal groups that may or may not have a consistent understanding of the registry," he says.

"Each registry is different and should be tailored to address specific needs," says Herve Caspard, M.D., Sc.D., director of epidemiology, U.S. health outcomes/medical, at Sanofi-Synthelabo Inc. "For example, we should not hesitate to simplify the case report form when we just want a little information."

Mr. Webb says because the return on investment (ROI) for registries is a little softer, measuring their value can be a challenge as well.

"There are objective ways to measure ROI, such as market-share data and the effects a registry can have in terms of penetrating and expanding the marketplace, as well as patterns of product use," he says. "From a scientific standpoint, registry data can be disseminated through abstracts and manuscripts, scientific presentations, and investigator meetings to help them improve outcomes for their patients."

Dr. Morris points out that there is another value that often doesn't get added into the equation: the prospective capture of data that can be used for a scientific marketing plan.

"The use of registry data by providers themselves to potentially change their own prescribing behavior or increase visibility and awareness, can be a much more effective selling tool than a pure sales detail or other traditional marketing tactics," he says. "Having a registry that can be used by specific providers or institutions is good medicine and good marketing."✦

We view registries as an **IMPORTANT OPPORTUNITY FOR OUR CLIENTS TO BETTER UNDERSTAND** how diseases are managed in the real world. Registries fill an important niche between formal clinical trials and unstructured views of the marketplace.

JEFFREY TROTTER

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

Experts on this topic

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