The Growing Role of Disease Registries in EVIDENCE GENERATION

Contributed by

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The current policy environment has broadened the scope of evidence that life-sciences companies must provide to healthcare stakeholders. In the United States, the focus is on comparative effectiveness research (CER), and in many other parts of the world, relative effectiveness/efficacy.

Public and private payer organizations require evidence on real-world effectiveness and value to support coverage and reimbursement decisions. Regulatory authorities require information on real-world safety. And physicians need information on real-world effectiveness and safety — ideally for specific patient subgroups to help guide treatment at the point of care.

In addition to focusing upon real-world evidence, the current policy environment has dramatically broadened the scope of comparators. It's no longer sufficient for a life-sciences company to present evidence about the relative safety, effectiveness, or value of its product relative to another pharmaceutical product. For a patient with a given condition such as diabetes, all possible treatments are relevant comparators including, but not limited to, other drugs, surgical procedures, diet, and disease management.

It's simply not possible to generate this breadth of evidence through a series of randomized clinical trials. The cost and time required to generate all of the needed evidence would be prohibitive.

Consequently, mechanisms for capturing the relevant real-world data are needed. Disease registries will be an important means to this end. In some geographies, existing observational datasets such as claims data and electronic medical records can answer many of the questions. However, the data often will be incomplete.

For example, claims data lack clinical depth; electronic medical record data lacks breadth of healthcare utilization, comorbidities, concomitant drugs, etc. Both lack information on patient reported outcomes, which are relevant for some disease areas. Moreover, in many countries it's difficult for life-sciences companies to gain access to observational data, even when it already exists. In these cases, life-sciences companies must generally conduct late-phase studies to collect the primary data themselves.

However, once in place, disease registries can provide an effective mechanism for generating real-world evidence in a wide variety of areas:

- Informing clinical development — helping to identify priorities of compounds, as well as trial design
- Understanding the burden of illness
- Understanding disease progression
- Observing real-world treatment patterns, switching patterns, uptake on new products, etc.
- Observing patient outcomes — both effectiveness and safety measures
- Understanding heterogeneity of treatment effects
- Demonstrating the value of treatments

Research Design, Data Quality, and Statistical Methods

A major challenge to the effective use of evidence generated from observational data is having an understanding of when such evidence is likely to be reliable. The analysis of observational data can be fraught with problems, including the inability to control for confounders and poor data measurement. These issues result in a common statistical problem that leads to bias in treatment estimates. From a research design standpoint, randomization solves these problems and as a result, randomized trials are at the top of the evidence hierarchy.

However, it's important to understand that analyses of observational data are not always doomed to be biased. If data are collected on the relevant variables and the data are of high quality, most of the concerns about the analysis of observational data are resolved. In other words, it's not so much a question of a randomized control trial versus an observational study. The more appropriate questions are: Have the right data been collected? And, are good procedures in place to ensure the quality of the data being collected?

To improve the reliability of evidence from observational data, it's very important to collect data on all of the necessary variables, as well as to ensure high quality (limited measurement error). High-quality data, in combination with appropriate statistical methodologies from epidemiology, health services research, and econometrics will address many of the limitations that threaten the validity of conclusions reached from observational data. PV

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