Introduction

Passage of the 21st Century Cures Act heralds a new era in drug and device development. No longer are sponsors bound to traditional clinical trials to prove safety and efficacy. More flexible, faster and cheaper research methods, including observational studies, are now acceptable in the FDA's approval process, either as supplements to or substitutes for clinical trials.

Observational studies allow drug- and devicemakers to collect real-world usage information from patients and physicians as well as harvest data from existing studies – from university research to patient data registries – to use as evidence of a product's efficacy and safety.

Data from these studies can save manufacturers time and money compared to lengthy clinical research and help them get to market faster. The drawback is that successful observational research requires an entirely different set of procedures and careful planning to ensure the real-world evidence collected is valid and reliable. And it requires an understanding of the kind of evidence the FDA and, perhaps more importantly, other marketplace stakeholders will accept.

This management report addresses the opportunities and challenges that observational studies can present. It covers the most effective uses of observational research in both the pre-approval and post-approval phases; how to identify stakeholders and determine what kind of data they need; and how the FDA's view of observational research is evolving.

This report is largely drawn from comments made during an FDAnews webinar by Jeff Trotter, president of Continuum Clinical, which in 2017 published the 10th edition of its Survey on Observational Research. A 30-year veteran of the healthcare industry, Trotter is a thought leader in the design and implementation of research studies generating real-world evidence.