

The New Evidence Landscape: The Impact of Changing Regulatory and Economic Standards on Clinical Innovation and Product Commercialization

Government and commercial payor requirements for drugs and devices are increasingly focused on evidence – evidence of quality, safety, efficacy and value. These new requirements are driving pharmaceutical and life sciences companies to adapt to this “new evidence landscape” by pursuing new strategies that enable them to not only meet these requirements, but get their products to market in a safer, more efficient manner.

On April 20, 2007, United BioSource Corporation and Goldman Sachs, in association with the Center for the Evaluation of Value and Risk in Health at Tufts-New England Medical Center, hosted a symposium in New York City to explore this new evidence landscape. Participants included prominent thought leaders from the government, academia, payors, industry and the investment community.

The key theme that emerged from the day of discussion is that the landscape is shifting – regulators and payors are moving forward with new, evidence-focused requirements. As a result, the pharmaceutical industry is at a “leadership moment”: companies can either react to new evidence requirements, or they can lead the way in adopting new, innovative techniques that truly drive value and elevate the quality of care.

For more information about the symposium, please contact Tess Drahzal at 240 644 0429.

Defining the New Evidence Landscape

Drug and device manufacturers are grappling with intense levels of scrutiny from FDA, payors, consumers and the press, all of whom are concerned about the safety and value of medications in the marketplace. Recent market events are driving the heightened sensitivity to drug safety issues, but there is also increasing concern about the approval of drugs that seem to offer few additional benefits at a much greater cost. As a result, nearly every panelist expects significant health policy reform in the coming years, including increased FDA oversight capabilities and a restructured Medicare reimbursement methodology. The private sector is also expected to increase its efforts to recover health care value, particularly as costs continue to rise.

While broad policy reforms are expected, the specifics of this reform are subject to debate. Dr. Mark McClellan, Former CMS Administrator, made the case for a thoughtful approach, as the blunt instruments of government regulation, price controls and restricted access are no longer sufficient for long term savings. As he noted, these actions “don’t do a good job of replacing low-quality care. They put off the needed work in our healthcare system, of progress towards a fundamentally better, more personalized approach to care, a health system that’s in line with where healthcare and medical technology should be headed today.” Dr. McClellan called for increased use of comparative effectiveness studies and for leading stakeholders to set guidance and standards for developing the next generation of evidence.

Rising Standards for Safety and Effectiveness: The Regulators’ Perspective

In building this next generation of evidence, the regulatory agencies will play a crucial role in shaping the landscape, particularly as their focus expands to include the entire life cycle of a product. As Dan Schultz, Director, FDA Center for Devices and Radiological Health, stated, “Our mission is getting safe and effective [medicines and] devices to market as quickly as possible and making sure that once they get to market, they remain safe and effective.”

Additionally, panelists advocated for building product safety into the product development process at the very beginning in order to screen out compounds that might cause problems or be ineffective in certain populations. Conference panelists addressed drug development requirements that are more complex than the current standards. However, as Rear Admiral Steven Galson, Director, FDA Center for Drug Evaluation and Research, pointed out, these rules will be “in one way more complex than what we do now, but allow adaptation and flexibility.” The panelists agreed that any regulations must support the innovation and development of new methods to produce better clinical data more efficiently.

Rising Standards for Economic Value: The Payors’ Perspective

Regulators are not alone in their evidence focus, however. Public and commercial payors are relying more heavily on clinical evidence to support reimbursement decisions, with the goal of developing new reimbursement methodologies that better reflect the actual value delivered. This is reflected in the rising demand for comparative effectiveness data, with some payors going so far as to develop their own evidence in the absence of other available data.

Robert McDonough, Medical Director of Aetna, pointed out that the payor focus is one of “efficacy vs. effectiveness...the improvement must be attainable outside the investigational setting, so that it is effective in actual clinical practice.” Other panelists suggested that the focus on comparative effectiveness is gaining traction in the consumer marketplace, and many trusted consumer brands are vying to serve as the source for the best care at the lowest price. Patients are taking advantage of resources such as the free information database provided by Consumers Union, which is based on drug class reviews of Drug Effectiveness Review Project (DERP). While there is skepticism about the methodology employed by the Consumers Union and other groups, panelists concurred that better evidence on the most effective treatments will drive value for both payors and patients.

Currently, the evidence that can most effectively drive value in the marketplace is not widely gathered. Panelists including Dr. Sean Sullivan of the University of Washington, and Peter Bach, a former CMS official currently of Sloan Kettering, concurred that industry needs to produce evidence beyond the data required for regulatory approval in order to support product value in the marketplace- efficacy endpoints. They also commented that traditional phase III trial designs are not often suitable for coverage decision making.

Industry Responds: Product Commercialization and Reimbursement Strategies

Only the collection of the right evidence, at the right time, can provide doctors and patients with the information they need to make the best informed decisions. In using this evidence to make better decisions, however, there must be a balance between safety and effectiveness, risk and benefit. As Geno Germano, President, U.S. and General Manager of Wyeth Pharmaceuticals expressed, “the principles of evidence-based medicine must be applied broadly, across all medical interventions, to achieve both value and quality healthcare.” Panelists also recommended the pharmaceutical industry play a leadership role in developing consistent standards for evidence that accommodate the perspectives of all stakeholders.

Setting clear and consistent standards is important, and Josh Offman, Vice President, Global Coverage and Reimbursement at Amgen pointed out that comparative effectiveness measures only work if the correct evidence is gathered at the beginning; “too often in the payor community today, the lack of evidence is equated with equivalence. And unfortunately for innovators...that becomes a real challenge.”

Building on the remarks of other panelists, Burt Adelman, Executive Vice President, Portfolio Strategy, Biogen Idec highlighted the industry’s commitment to making some of these changes, “we need to improve the process of information gathering and transparency as it relates to the collection of safety information.”

All panelists highlighted the need for patient evidence to be infused into the commercialization process.

Industry Responds: Clinical Design Solutions

As a response to changing evidence standards, pharmaceutical companies are embracing nontraditional, tactical methods of clinical development, including adaptive clinical trials. Some of these approaches hold great promise for increasing the performance and efficiency of the product development process. Wyeth Pharmaceuticals is one company pioneering these novel methods, and Michael Krams, Assistant Vice President, Adaptive Trials, Clinical Development at Wyeth explained, “the idea [for] this different approach with regulators is to effectively argue for an ongoing interaction with health authorities where we [have] dialog from the very early design stage onwards.” Krams went on to elaborate that the development process at Wyeth is designed to allow them to “fail faster and more efficiently,” while also ensuring that company resources are allocated to developing the most promising compounds based on the clinical data.

Beyond adaptive clinical trials, companies are exploring other methods to develop better data more efficiently. Gretchen Dieck, Senior Vice President, Safety and Risk Management at Pfizer discussed another option – large, real-life studies, which, “are like large simple trial design because you get patients that may have been on other drugs...[however], these studies are very costly because, in some instances, they’re carried out in multiple countries.” Despite their cost, the benefits of large streamlined studies are substantial and Pfizer is making significant investment in this area.

Also on the panel was Robert Butz, Vice President and General Manager Global Clinical Development, MDS, Inc. Mr. Butz, along with the other panelists, emphasized that the new evidence landscape is both real and permanent. Future success will rely on the integrating the current development infrastructure with analytical approaches specifically designed to either kill development or arrive at optimal dosing parameters much earlier in the product development cycle.

About UBC

United BioSource Corporation, is a global pharmaceutical services organization that combines deep scientific knowledge with broad execution expertise across the entire lifecycle continuum. Our focus is on generating real-world data to support the development and commercialization of medical products for emerging and established life science companies.