What Lies Ahead? 2014

Trends to Watch: Medical Product Development in North America

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"What Lies Ahead?" for 2014

DIA has released its second annual "What Lies Ahead?" report, providing experts' insights into the year ahead for pharmaceuticals, biotechnology, and medical devices.

Thought leaders from industry, academia, government, and patient organizations were asked to choose the trends they foresee shaping the world of medical product development and access during the coming year. These diverse respondents were closely aligned in their selections of the year's most influential trends, despite sometimes unique perspectives on medical product issues. "The concurrence of multiple stakeholders on the trends that will be most important in 2014 lends credibility to their feedback," said Susan Cantrell, Director, DIA Americas.

For 2014, thought leaders once again placed a focus on collaboration in the Top 10 trends but dropped it from the 2013 top spot. Three different trends tied for the 2014 top position: the evolution of patient/consumer engagement, regulatory agency support of innovation, and learning how to use big data.

The importance of both patient/consumer engagement and big data were among the Top 10 trends of 2013, and on 2014's list, they share the top spot. The shift in focus for both issues attests to progress in engaging all stakeholders in the discussion about the development of effective, safe, and patient-focused therapies. Regulatory agency support of innovation was a surprise top trend, acknowledging the increasingly critical role regulatory agencies play in balancing safety with innovation to meet the needs of patients.

Based on our experts' responses, personalized medicine and companion diagnostics will continue to be among the top priorities in 2014, as will the push for clinical trial data transparency. The implementation of risk-based monitoring in clinical trials appears for the first time in the Top 10 list and underscores a commitment to patient protection and data integrity despite pressure to conserve resources when conducting trials.

"Our thought leaders' insights were quite accurate last year, and we believe these projections of the future landscape can be valuable to companies as they strategize to successfully meet the ever-changing needs of the marketplace," said Cantrell. "We're pleased to present the 2014 report."

What Lies Ahead for Therapeutic Innovation & Regulatory Science in 2014:

1. A Three-Way Tie for the Top Trend of 2014:

Evolution of Patient/Consumer Engagement. The goal of therapies is to improve patient health and health care outcomes, and the patient/consumer has been recognized as an important stakeholder in their development. Industry at large, beyond the early adopters of patient engagement, is evolving in its understanding of the full potential of patient input. Moving beyond the informed patient's treatment choice at the point of care, industry is acknowledging that patient input can accelerate the development of therapies for the disease community as a whole. Patient input can inform the design of clinical trials, the endpoints/outcomes sought, the weighing of benefit vs risk, the products that will be developed, and those that will stay on the market. Industry continues to look for the best ways to engage patients in meaningful dialogue and to optimize their input.

Learning How to Utilize Big Data. In an incredibly short period of time, industry has recognized the power of big data for purposes ranging from innovation and discovery to the assessment of real-world outcomes of treatments. Industry and its partners have learned what constitutes big data, how to access it, and what to use it for. But, even veterans of real-world data analysis have been humbled by the task of leveraging these large data sets to their full potential. Traditional analytical approaches are not adequate to go deep into the data to understand patients in the complex dimensions made possible by the integration of multiple data sets. A new field of analytics is emerging that will take full advantage of these big data sets, even to answer evidence questions. Also important is that there are many types of companies in the biopharmaceutical and device industry, with different questions and different abilities to work with the data. New skill development for industry professionals of all disciplines is needed, from those who will work with the data to those who will conceptualize what they want from it.

Regulatory Agency Support of Innovation. As science drives innovation, it is important that regulatory agencies balance their efforts to ensure safety of new types of products, such as live biotherapies and oligonucleotide therapies, with efforts to foster innovation. FDA has risen to the challenge, backed by FDASIA provisions and user fee commitments, by expediting regulatory pathways and working collaboratively with sponsors throughout the drug development process. The Agency's commitment to regulatory science and participation as a stakeholder in problem-solving consortia further its understanding of how innovation can be fostered. There are new areas that require guidance from the Agency, such as nanotechnology, biosimilars, regenerative medicine, and cellular therapies.

4. Importance of Collaboration.

Collaboration as an avenue for innovation was identified as the top trend for 2013 by DIA thought leaders and appears again on this year's list. The perception of collaboration is moving from that of a "novel approach" to a necessary tool for progress in today's environment. Collaboration among the stakeholders in the "health care ecosystem" is considered key in fulfilling the "triple aim" of health care reform: providing high quality care, with better health outcomes for patients, while reducing cost. A recent survey of biopharmaceutical, payer, and provider executives indicated that there is strong agreement on the need to collaborate with other stakeholders; fewer than 20%, however, said they have made progress in this area. Biopharma has an important role to play in collaborations for innovation and improved care and outcomes but must form relationships with diverse stakeholders to be fully effective. Positioning itself as an effective collaborator will be a focus for the biopharma industry this year.

5. Personalized Medicine/Tailored Therapies and Companion Diagnostics.

The focus on personalized medicine moved up slightly from the 6th most important trend in 2013 to the 5th in 2014. Scientific advances are improving our understanding of disease states, mechanisms of action of new and existing treatments, and reasons for variable responses to therapies among individuals. Industry has recognized that there is potential for success of therapies in smaller, more appropriately identified patient groups. Companies, with the cooperation of regulatory agencies, are optimizing processes for developing companion diagnostics for new and existing therapeutic products. Within the past year, the Supreme Court ruled that naturally occurring DNA is not patentable, opening the field for new companies to compete in the development of genetic testing options, and a number of gene patents are soon to expire, broadening the impact of the Court's decision. Lower cost testing may pave the way to payer coverage of genetic testing, opening access to precision diagnostics for many more patients. At the same time, questions of affordability of precision medicine are being pondered. Personalized medicine is becoming a reality and is moving along a continuum where both benefits and limitations are being identified.

6. Clinical Trial Data Transparency.

Though clinical trial data transparency will continue in 2014 to be an area of debate, many changes have occurred in 2013, paving the way for a system in which data sharing is the norm. The EMA put forth for comment its plan for the release of clinical trial data and received over 1000 comments, delaying the release of final documents until the agency is able to review and assimilate the feedback. GSK released its plan to share data upon request to qualified researchers, and FDA has discussed in various forums the benefits of the prudent release of clinical trial data. Supporters and opponents both have rational points to make, and 2014 will be a year of debate and working through challenges as we move toward consensus or compromise on the question of sharing of detailed data from clinical research.

7. Implementation of Risk-Based Monitoring in Clinical Trials.

FDA, through its risk-based monitoring guidance, has acknowledged that verification of all source documents is not always an effective monitoring approach for clinical trials. It recommends instead the design of a tailored plan to address patient protection and data integrity risks specific to the study. Translating this concept into clinical practice presents a number of challenges to sponsors, and though TransCelerate Pharma has initiated a project to establish a standard framework to help in this regard, sponsors will be tasked with strategizing how to best allocate their study resources based on risk.

8. Focus on Unmet Medical Needs.

In 2014, this issue has moved up in the top ten trends. In both mature and developing markets, the search for innovation is focusing on unmet medical needs. Products that duplicate available treatments are no longer valued unless they are meaningfully superior or lower in cost. In developing markets, products must fill country-specific needs at competitive prices in order to be successful. In mature markets, there is unmet medical need in diseases such as Alzheimer's and Parkinson's as well as certain cancers. Rare diseases also represent a large collective disease burden and will continue to be a focus of therapeutic development. Expect public discussion of endpoint development and selection, use of surrogate endpoints and accelerated approval, reasonable safety exposures, and other complex issues as we work to address the needs of rare disease populations, say our thought leaders.

9. Data Standardization and Interoperability.

This was an "honorable mention" trend in 2013 and is gaining importance for a number of reasons. Standardized, fully electronic data and analysis sets will be required for all submissions to FDA by the year 2018. This has driven the adoption of data standards such as CDISC. Beyond individual clinical trial data sets, the ability to easily combine multiple data sets for big data analysis is greatly enhanced by the application of data standards, and the interoperability of data systems relies on the application of data standards to allow seamless exchange of accurate data. As an example, the goal of extracting clinical research data from electronic health records relies on the adoption of data and operational standards by both enterprises. Industry will be focusing on the adoption of data standards and on interoperability of data systems in the coming year.

10. Tied for Trend Number 10:

Continued Importance of Global Markets. It is important for companies to work in global markets, especially in developing markets like China, pan-Asia, Russia and Eastern Europe, India, and Brazil, Argentina, Venezuela, and other Latin American countries, as this is where the majority of future growth will be. But the approach to developing markets is maturing; from previous learnings, the challenges of these regions are better understood. The economics, culture, regulatory and health care infrastructures are unique to each country and must be strategically assessed. Successful companies conduct early stage analysis and planning to account for varying global factors when selecting countries for expansion. They also create partnerships to address these varying needs and to build infrastructure, including training of the workforce.

A Top 10 trend in 2013, the topic of global markets just managed to remain above the cut for 2014. It is not really less important this year; it is, as one thought leader expressed, "almost an established fact of life and no longer a trend."

An Explosion of Mobile Health Applications. In 2013, this was trend number 7 according to DIA thought leaders, who agreed that regulatory agencies will need to establish rules and guidelines for the appropriate utilization of this new technology. As 2014 begins, FDA has issued its guidance providing clarity on the types of mobile applications it will regulate, and many believe that this will facilitate innovation among developers. As mobile technologies become more reliable, sophisticated, and interoperable, the potential for development is limitless. Companies are utilizing the technology for patient-reported data, monitoring, and simple communication. Context-based applications can collect data about the patient's environment and prompt reminders or medical team alerts. The promise of enhanced patient-provider relationships resulting in higher quality care is discussed, but some warn that this ability to exchange information is only a tool and that trust and attention to the relationship must be a priority.

One important trend from the 2013 Top 10 that almost made it to the 2014 list deserves an honorable mention:

11. Meaningful Benefit-Risk Assessments Still in Development.

From numerous frameworks for assessing the balance of therapeutic product benefit versus the patient safety risk, a set of common elements has emerged that is integral to meaningful Benefit-risk analysis. The industry and regulatory agencies are also growing in their understanding of the variable importance of different risk elements at different stages of the product development and regulatory review. FDA continues to work out the details of its more qualitative Benefit-risk assessment framework, with input from industry and other stakeholders. The methods and best practices for successful communication of the assessment to the patient and the health care provider will continue to evolve.

There is no doubt that determining how to best assess Benefit-risk will be a priority in 2014, with a shifting focus to the meaningful communication of Benefit-risk information to patients and practitioners. One can envision the day that these concepts will become so fundamental to the development and use of medical therapies that they are no longer considered "trends."