What Lies Ahead?

2015

Trends to Watch: Health Care Product Development in North America

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"What Lies Ahead?" for 2015

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

Thought leaders from industry, academia, government, and patient organizations were asked to choose the trends they foresee shaping the world of health care product development and access during the coming year. "Our experts viewed this as a tough task this year," said Susan Cantrell, Senior VP and Managing Director, DIA Americas. "With so much rapid change in the science, technology, policy, economic, and social factors relating to health care product development and access, it was difficult to narrow down to those trends that may have the most impact." The results of their careful consideration are presented in the 2015 report, and the complexity of today's health care landscape is evident in the selections.

Though some of the 2015 trends appeared in the 2014 report, a closer look reveals how much the health care product environment is changing. In 2015, the trendy aspect of collaboration is that it has become the norm. In fact, collaboration could be viewed as a theme running through the Top 10 trends themselves. That all stakeholders, including patients, industry, policy makers, regulators, payers, and others, are working together in some way to effect change is evident. Clinical trial data transparency and sharing of data was the sixth trend in 2014 and rises to a tie for the second trend of 2015. Sharing the second spot is the growing importance of oncology as a therapeutic class, and important advances in this therapeutic area have resulted from collaborative projects such as the Lung-MAP study.

The top trend for 2015, the rising influence of payers in discussions of value and cost of medical products, signals the growing interdependence of stakeholder efforts to advance the availability of new and innovative products to meet medical needs. Many of the Top 10 trends center on multi-stakeholder approaches to improve cost-effectiveness and to modernize and streamline development and approval processes.

"Our thought leaders' insights have been quite accurate year over 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 2015 report."

What Lies Ahead for Therapeutic Innovation & Regulatory Science in 2015:

1. The Rising Influence of Payers: Driving the Need for Evidence-Based Outcomes Information and Creating a Challenging Pricing Environment for Targeted and New Therapies.

Observational studies and comparative effectiveness research have become critical means to describe clinical practice and to measure safety and value, and conducting real-world studies to demonstrate value in the formulary is fast becoming part of the cost of doing business in the US. Further, payers' focus on controlling costs along with changes to provider reimbursement systems are placing the cost of medicines under intense scrutiny. Pricing issues that were seen in 2014 for insulin and Hepatitis C therapies may begin to affect coverage of specialty therapies, including existing and new therapies for cancer, among other drugs. These forces will create pressure for increased transparency in formulary decision making criteria so that sponsors can determine how to best collect and assess supportive data, and for

collaboration between payers and medical product developers in studying real-world outcomes. The growing pressure on industry to demonstrate product efficacy and value over the current standard of care, safety, and economics will have increasing impacts on R&D, health economics, and marketing decisions.

2. A Two-Way Tie for the Second Most Influential Trend of 2015:

Growing Importance of Oncology as a Therapeutic Class. There is huge unmet need in the 200 or so diseases collectively referred to as cancer. In the US alone, the number of newly diagnosed cases in 2025 is expected to increase by 42% over today's rates, in part because of the aging of the population. Over the next two years, oncology drugs are expected to account for 12% of sales globally, making them the largest therapeutic category. According to a report from the Pharmaceutical Research and Manufacturers of America (PhRMA), in 2014 there were 771 cancer medications and vaccines in trials or awaiting FDA review. Active areas of oncology research include targeted therapies, made possible by advances in genomics, immunotherapy, and combination therapies.

High Visibility for Clinical Trial Data Transparency. The European Medicines Agency (EMA) clinical trial data sharing policy is in place, initially requiring publication only of clinical trial reports for all newly approved drugs. A second phase review of various aspects in relation to individual patient data (IPD) will include finding the most appropriate way to make IPD available, in compliance with privacy and data protection laws. In the US, Health and Human Services (HHS) has proposed new rules requiring clinical trial sponsors to report the summary results of all clinical trials, not just those for products receiving FDA approval. The OIG (Office of the Inspector General) 2015 Work Plan includes oversight of the extent to which clinical trials comply with the reporting requirements set forth by the FDA Amendments Act (FDAAA) and the way in which FDA is ensuring that these requirements are met. Large pharma and Project Data Sphere, the Yale University Open Data Access project, and other programs are opening previously closed data sets to the public for use. The Open FDA initiative allows access to a public database for analyzing drug and device adverse events, recalls, and labeling information. 2015 will be an important year for clinical trial data transparency and sharing, with implementation activities in full swing.

4. A Cluster of Key Trends Tied at Number Four:

Thought leaders will be considering improvements for the next PDUFA cycle to streamline and accelerate drug development and approval, including innovations proposed in the 21st Century Cures and related legislative initiatives. With the fifth authorization of the Prescription Drug User Fee Act (PDUFA V) set to expire in September, 2017, discussion of key issues for PDUFA VI will begin during 2015. These discussions, and those about the next authorizations of MDUFA (Medical Device User Fee Amendments), GDUFA (Generic Drug User Fee Amendments), and BsUFA (Biosimilars User Fee Amendments), will be catalyzed by the expectation that pending 21st Century Cures legislation will impose new requirements on FDA aimed at modernizing the conduct of clinical trials and streamlining regulatory approval pathways. Collection of input and discussion of PDUFA VI requirements has already begun and will intensify in the spring of 2015, while the 21st Century Cures draft legislation itself is discussed and debated.

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Continuing Evolution of Patient/Consumer Engagement. The goal of therapies is to improve patient health outcomes, and the patient/consumer has been recognized as an important stakeholder in health care product development. Industry has evolved rapidly in its understanding of the full potential of patient involvement, recognizing that patients through their input can accelerate the development of effective therapies in a variety of ways. Implementation of patient engagement has uncovered its complexities, and developers, regulators, and patients alike are seeking best practices for engaging together to achieve the best outcomes for patients.

Increasing Recognition of the Value of Big Data and Skill in Its Utilization. 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. The number of life sciences companies maintaining dedicated big data teams is growing, but because analytic approaches for big data sets are different from traditional approaches, many companies still outsource to specialized vendors and consultants for large scale studies. Regardless of the source of analytic expertise, the use of big data by the health care products industry will increase in 2015.

Focus on Unmet Medical Needs. The focus on unmet medical needs in the search for innovation will intensify, driven by pressure from the regulatory approval process, health technology assessment, payers, patients and advocates. Products that duplicate available treatments are no longer valued unless they are meaningfully superior or lower in cost, because resources are limited and the number of diseases and conditions for which treatments are needed is immense. In mature markets, there is unmet medical need in diseases such as Alzheimer's, Parkinson's, cancers, and rare diseases, which represent a large collective disease burden.

A Three-Way Tie Rounds out the Top Ten:

Collaboration is the New Norm. Collaboration among and within health care stakeholder groups was considered cutting edge as recently as two years ago – TransCelerate Biopharma celebrated its 2nd birthday in September 2014 – as a means of breaking down barriers and sharing resources to facilitate innovation. In a short period of time, new models of collaboration have been established to improve all aspects of the health care product life cycle. No less important today, collaboration is the norm, not a market differentiator. Stakeholders must work together to be successful and fill knowledge gaps if the needs of patients are to be met in a sustainable manner.

Regulatory Agency Support of Innovation that will Improve Patient Outcomes. In 2014, FDA leveraged improvements in regulatory science and approval pathways to approve innovative new medications that are expected to advance the level of clinical care in a number of therapeutic areas. These included orphan drugs for rare diseases, breakthrough therapies, and novel new antibiotics. FDA will continue to make significant investments in regulatory science to help translate new technologies and basic science tools into real-world diagnostics, treatments, and cures; and importantly, the Agency will continue to make strides in incorporating patient perspectives to meet patient needs while balancing safety considerations.

Changes at FDA: At the top of our 2015 watch list will be the changes taking place at FDA in the wake of the resignation of Dr. Margaret Hamburg as FDA Commissioner and the appointment of Dr. Robert Califf as Deputy Commissioner for Medical Products & Tobacco.

The Growth of Biosimilars. Recently, an FDA Advisory Committee recommended approval of the first biosimilar to be marketed in this country. FDA's approach to the consideration of data packages for biosimilar review is becoming more clear through this case example, and the FDA plan for 2015 guidances includes several that will focus on biosimilars, including labeling for biosimilar products, considerations in demonstrating interchangeability, statistical approaches to evaluating analytical similarity data, and additional questions and answers regarding the Biologics Price Competition and Innovation Act of 2009. The coming year will see a number of new applications for biosimilars approval and the entry of an increasing number of biosimilar products to the US market.

An important trend that just missed the Top 10 deserves mention:

11. Personalized Medicine/Tailored Therapies and Companion Diagnostics Focus on Policy and Regulation.

Scientific advances allowing us to understand the molecular basis of disease and growing ability to apply these in clinical care are resulting in the rapid advancement of personalized medicine/tailored therapies. The simultaneous concern with controlling health care costs is creating a need for policy and regulation to address cost concerns while fostering innovation. This balance will be critical to the advancement of personalized medicine and will be addressed in multiple policy forums, including President Obama's Precision Medicine Initiative, aimed at moving precision medicine into every day clinical practice, and the 21st Century Cures initiative which aims to streamline innovative development and its applications.