2011 RAPS HORIZONS

Regulatory and beyond.

Regulatory on the Horizon

May 2011

Every day, patients around the globe and the healthcare systems that serve them rely on the best available pharmaceuticals, medical devices and biotechnology products to deliver life-saving and life-enhancing care. But even as medical advances have benefited millions, the global healthcare sector today faces profound challenges and change.

The demand for innovative new therapies has grown stronger, while tolerance for patient risk has decreased sharply, and pressure from public and private payers to control costs is on the rise. Unsustainable models have left the current healthcare systems of many nations unable to sufficiently support their populations and incapable of quickly adapting to complex and converging forces. There is ongoing debate over how best to advance good science, good policy and good business practices—and, most importantly, how to meet public health needs.

These challenges are multifaceted and interwoven, and addressing them will require forward thinking and active leadership. To do this, we must fundamentally rethink the processes by which healthcare products move from the laboratory bench to patients' bedsides. Regulatory professionals, who have a hand in every stage of the healthcare product lifecycle, can and *must* play a vital role in solving many of healthcare's most critical challenges.

The 2011 RAPS Horizons event, 7–8 April in Vancouver, brought together regulatory, business, government and academic leaders to explore and debate key issues, including economics, changing science and technology, globalization, and trust and transparency, related to healthcare products, with the goal of examining implications and directions to advance the regulation of these products. The event was the first in a series of ongoing initiatives led by RAPS, and will result in a number of reports, papers and discussions in the coming months. Presented here is a preview of some of the issues that bear further examination.

THE CURRENT LANDSCAPE

Economic issues discussed at Horizons centered on addressing a lack of economic sustainability, systemic disconnects and opportunities to restructure existing healthcare system models to meet the complex demands of patients, payers, manufacturers and regulators. Many of these economic issues were framed around key questions: What is the value of a product to the consumer and society? How do we encourage investment in innovation and simultaneously manage costs? How do we increase beneficial outcomes while satisfying payers?

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Although there are certainly no easy answers, regulatory professionals bring much-needed expertise to the discussion. As RAPS research shows, regulatory professionals are increasingly becoming involved in linking economic perspectives and regulatory strategy through involvement with comparative effectiveness research and health technology assessment initiatives.

Advances in science and technology, including new applications of mobile technology in healthcare, personalized medicine and the use of social media are challenging current regulatory paradigms. Regulation has traditionally lagged behind scientific advances, but the pace of innovation today makes the situation even more acute. Regulatory agencies must quickly develop the necessary understanding and expertise to respond to these advances instantaneously. As innovation progresses, regulators will be called upon not only to address each new technology as it emerges, but also to construct regulatory systems that can effectively adapt to innovation "on the fly." The regulatory profession is firmly grounded in science. Thus, regulatory professionals are in the unique position to help link the latest scientific advancements with sound regulatory approaches.

Globalization presents perhaps the greatest challenge *and* the greatest opportunity for the healthcare product sector. Today, medicinal products, devices and other products are developed, tested, manufactured and marketed globally. The regulatory systems that were established to monitor products nationally or regionally are being forced to adapt to the new reality. While some regulatory standards and approaches are used by regulatory agencies in multiple countries, regulation of healthcare products is not harmonized, which contributes to complexity and costs in the healthcare product sector.

Meanwhile, developing and emerging markets are experiencing economic and population growth that far outpaces that of western and industrialized countries. Many of these countries are developing their regulatory agencies and may seek new or different approaches than those currently used by established regulatory bodies. Will these new agencies create new and perhaps more-efficient models for regulation? Will they seek and utilize harmonized standards? Do they, along with the established regulatory agencies, have the capacity to support a globally connected healthcare product sector? The opportunities for innovators and trailblazers are vast, but so, too, are the challenges. This is another area where regulatory professionals, with their expertise in multiple regulatory systems, can play a crucial role in harmonizing standards and maximizing efficiencies.

Trust and transparency are paramount to the advancement of medical technology and public health. The distrust that often exists on both sides of the regulatory aisle between industry and governmental authorities, and the skepticism of the public and some clinicians aimed at the healthcare industry and regulators, can impede progress toward better health outcomes. Some issues with healthcare products and resulting media coverage have helped fuel this distrust and skepticism. Rebuilding that trust will be difficult, but necessary, in moving toward a future in which communication and transparency are increasingly the expected norm. Regulatory professionals are well-equipped to help various stakeholders better understand the others' concerns by connecting science, regulation and business.

SETTING THE COURSE FOR A NEW HORIZON

Regulatory is at the forefront of these changing forces within healthcare, and it has an important opportunity and duty to lead the way by innovating and developing new regulatory solutions to surmount these challenges. None of these challenges exists on its own. They are uniquely interconnected and so, too, must be the solutions. Regulatory expertise has already had an impact in shaping healthcare's future, but there is more work to do.

The future will depend, in large part, upon driving new models for product development, lifecycle management and regulation. Several considerations for establishing these models were emphasized at the Horizons conference.

First, it will be vital to engage patient groups, clinicians, industry, regulators and payers in the earliest stages of product development. By engaging these stakeholders early in development, it will be possible to identify economic, scientific and regulatory issues and drivers, and to develop

strategies that can address these issues in the development process. Early engagement also will be important in building transparency and trust.

Under new models, clinical testing of products should encompass adaptive design approaches and should seek ways to better link safety, value and economic perspectives. The creation and effective use of data registries is another example of how scientific and technological advancements can be fostered through the identification of need and the sharing of best practices.

New models are likely to include conditional or rolling approvals, in which defined patient groups are given access to products while additional expanded use testing may be undertaken. This approach not only allows patients access to vital products in the earliest phases of development, but may also help minimize risks that may arise in wide-scale use and reduce the economic burden of large-scale clinical trials that can drive up costs and/or prevent studies of some products.

In light of globalization, we cannot lose sight of the critical need to work toward some level of alignment or harmonization among countries and to thoughtfully address the need for building regulatory capacity. By avoiding a "quick fix" regulatory approach, developing and established regulatory agencies can work together to build and/or refine systems that are transparent, understandable and better able to adapt to changes in the scientific, economic and regulatory environment.

THE CALL FOR REGULATORY LEADERSHIP

To be sure, these challenges are not simple or easy, but neither are they abstract problems without solutions. They are highly complex and interrelated issues in need of in-depth discussion among stakeholders to find not *a* way forward, but the *best* way forward, which is not likely to be mere incremental refinements to current models.

Regulatory issues related to cost, global access and supply chains, innovation, and trust and transparency must be addressed to move toward solutions in all facets of healthcare. The regulatory profession is a key player in crafting the way forward, uniquely positioned at the crossroads of several critical knowledge areas and integral to the development of new and sustainable models for healthcare systems across the globe.

We look forward to sharing more of the thinking and discussion that took place during 2011 RAPS Horizons, and examining each of these critical issues more closely in future papers and publications. We stand at an important juncture in healthcare; only by integrating regulatory into the broader approaches of solving some of healthcare's biggest issues will we make progress as a profession that makes better healthcare products possible. Look for more in-depth analysis and Horizons outcomes throughout 2011, with an initial white paper expected in late June.

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