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Multifaceted Paths Toward Advancing Regulatory Science

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Among the important and lasting contributions of Dr Margaret Hamburg, former Commissioner of the FDA, is a focus on regulatory science. While there has been much discussion about what regulatory science means, ultimately there is a need to provide a strong scientific basis for rational, wise regulatory decision making. Thus, regulatory science provides the underpinning that ensures the role of a regulatory agency to both protect and promote the public health.

By way of full disclosure, I had the honor and privilege of serving as Deputy Commissioner for Medical Products and Tobacco under Dr Hamburg. While I had worked with FDA for many years on advisory committees, particularly in pediatric drug development, served as a member of the Science Board, and interacted with the agency while I was in industry, I did not appreciate the breadth and complexities of FDA's roles, and the range of science needed to support its multiple missions, until I came on board as Deputy.

In this issue of *TIRS*, we have an article by Drs M. Geanacopoulos and R. Barratt from the Office of Translational Sciences, Center for Drug Evaluation and Research (CDER) at FDA.[1] They examine the role of the Critical Path Initiative in supporting regulatory science. They provide insight into 5 diverse projects that FDA undertook to address important scientific questions requiring data to support regulatory decision making, and they discuss the processes of defining the need for data-driven approaches and how such activities can be used in the regulatory milieu.

The diverse nature of the projects reflects the incredible complexity of FDA's activities. There were 2 projects dealing with what many associate with the agency, "getting the dose right" for special populations—children and patients with renal impairment. There also was a project dealing with cardiovascular safety of drugs. In each, the goal was to provide quantitative and validated approaches to ensure scientifically based decisions, and to provide guidance to those who develop and market medical products.

Two other projects hint at the complexities of regulatory enforcement and about the future of drug development. Given the increasing challenges of ensuring high-quality products, both for the active pharmaceutical ingredients (APIs) and other constituents of drugs, dietary supplements, etc, there is a need for standardized, validated screening tools that can be used in the field to examine a wide variety of medical products. The discussion of field-based portable Raman spectrometers is thus highly timely and important in our world of global production and distribution of products. Similarly, as we are introducing more macromolecules into therapeutics, understanding subtle variations in such molecules that might alter clinical performance becomes critical. Hence, the project on protein glycosylation. Since differences in glycosylation may affect pharmacokinetics, organ targeting and distribution, and side effects and efficacy, these efforts are timely indeed.

As we have discussed before, science is changing at an ever-increasing pace. The nature of therapeutic interventions is already different from anything we could have imaged only a few years ago. The prospect of more targeted, more effective, and less toxic medicines is beginning to come to fruition. The need to match that innovation with innovations in regulatory science—from basic chemistry, to genomics (and other *-omics*), to clinical pharmacology, to clinical trials, to postmarket surveillance—is all the greater. Many thanks to Drs Geanacopoulos and Barratt for providing insights into ongoing efforts at FDA, and to Dr Hamburg for having envisioned the path forward.

—Stephen P. Spielberg, MD, PhD
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Reference

1. Geanacopoulos M, Barratt R. How the Critical Path Initiative addresses CDER's regulatory science needs: some illustrative examples. *Therapeutic Innovation & Regulatory Science*. 2015; 49(4):466-472.