Executive Summary

The United States is soon to experience a dearth of individuals appropriately trained in the evaluation of regulatory evidence. The emerging field of regulatory science involves the application of scientific methods to improve the development, review and oversight of new drugs, biologics, devices and food products that require regulatory approval. Regulatory science encompasses clinical trial design, quantitative safety assessment, product quality evaluation, quality by design, data mining, pre-clinical toxicology, biomarker development, quantitative sciences including exposure-response assessments, regulatory role of novel technologies, regulatory informatics, data management, and communication of regulatory decisions. These are not traditional topics fully addressed in the current academic curriculum. In order to cultivate future leadership and improve the understanding of the interrelationships of these complex scientific areas, it is critically important that we work toward building an educational consortium to develop an academic platform that provides essential content needed to train future regulatory scientists. A robust regulatory science training consortium would aim to prepare future regulatory scientists working in government, academia, industry and the non-profit arena who all contribute to the integration of regulatory science perspectives in product development, food safety and regulatory oversight.

While there are a variety of academic based programs in the US focused on regulatory affairs, there are very few that take on the challenge of regulatory science education or that have the vision of what that might entail. In most cases, this education is conferred through “on the job training” in regulatory institutions such as the US Food & Drug Administration (FDA), and sometimes in the industry. Further, many individuals do not experience the gamut of medical product development from “bench to bedside,” as very few have exposure to academia, industry, and regulatory environments. As a consequence, there is often a years-wide gap between basic science research, product development, and regulatory approval that hinders all parties. Government may invest in basic research, e.g., through NIH grants, which may then fall into a “black hole” for years before making it through the FDA, which has to come up to speed on the latest scientific research and innovation in order to conduct the regulatory approval process. Greater understanding of regulatory science from all perspectives—academic, industry and government—is needed to address this shortcoming.

A consortium infrastructure is needed to align and coordinate external partners to:

- Develop needed classroom and online module-based curricula.
- Identify, coordinate and administer an academic exchange program for regulatory science trainees, offering exposure to a pathway of product development and regulatory decision making challenges in government, industry and academic settings.
- Implement a sabbatical program in which senior level scientists and physicians work and train within a variety of regulatory science areas.
- Support the FDA fellowship opportunities, including the Commissioner’s Fellowship Program with opportunities at FDA and other regulatory science settings.
The goal for a consortium approach is not to duplicate content that is currently available in the academic setting. Rather it is to supplement existing programs, filling the gaps which are known to exist in traditional educational programs and training modules that do not access, nor fully understand the range of regulatory content that is available. The ultimate objective of the consortium is to identify and prepare future leaders in regulatory science.

Background

FDA defines regulatory science as “the science of developing new tools, standards, and approaches to assess the safety, efficacy, quality, and performance of all FDA-regulated products”. (See FDA’s website on the range of topics: http://www.fda.gov/ScienceResearch/SpecialTopics/RegulatoryScience/default.htm.)

On February 24, 2010, FDA launched its Advancing Regulatory Science Initiative1 (ARS), building on the achievements of existing Agency programs, such as the Critical Path Initiative's2 groundbreaking efforts to transform the way medical products are developed, evaluated, and manufactured. Recognizing the success of the Critical Path model, the ARS expanded its scope to encompass every dimension of regulatory science.

In the October 2010 report, “Advancing Regulatory Science for Public Health – A Framework for FDA's Regulatory Science Initiative,” we speak to the following need:

III. Scientific excellence, professional development, and a learning organization

*FDA will support a culture of and capacity for continuous scientific learning and professional development of our scientific staff. The agency will explore several approaches:*

- **Access to cutting-edge, continuing education and professional development for FDA staff** — through universities and government agencies, for example — as well as policies and resources that support these activities
- **Scientific exchange programs with academic and governmental institutions and with international regulatory counterparts**

While the goals articulated in the report are laudable, the challenge lies in implementation of mechanisms to facilitate a continuous learning environment where the best and brightest are brought to train in academic, industry, and regulatory settings. There is also a need for FDA staff to impart regulatory science knowledge and training to the external community and have the opportunity to keep abreast of the best and most current scientific information available.

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2 FDA’s Critical Path Initiative: http://www.fda.gov/ScienceResearch/SpecialTopics/CriticalPathInitiative/ucm076689.htm
In that same year, the IOM Drug Forum held a public workshop to explore the state of regulatory science and to examine approaches for building the infrastructure for regulatory science. In the report summarizing the IOM meeting entitled, “Building a National Framework for the Establishment of Regulatory Science for Drug Development”, a number of barriers to enhancing regulatory science as a scientific discipline were noted. Among the barriers discussed were workforce resource constraints at the FDA which often limit the time that FDA scientific staff have for professional development, and systemic barriers across academic medical centers such as lack of financial incentives, prioritizing research based on funding sources and not science, siloed approach to academic research, and aversion to regulation. IOM report also recognized the need for collaborative approaches involving FDA, industry, academia and other stakeholders to overcome challenges to building a robust regulatory science framework. The public-private partnerships created in support of the Critical Path Initiative were referenced as collaborative models that could be built on to enhance the regulatory science infrastructure.

Following that workshop, and building on the efforts of the ARS, IOM convened another meeting in 2011 entitled, “Strengthening a Workforce for Innovative Regulatory Science in Therapeutics Development.” In the summary report from this meeting, stakeholders discussed strategies for addressing gaps in the current landscape of regulatory science education and training as expressed in the 2010 workshop. Recommendations presented at the meeting included, the development of interdisciplinary curricula to address multiple challenges in product development and emerging trends in areas such as clinical trial design, clinical pharmacology and pharmacogenomics; programs to address the different training backgrounds of individuals who seek regulatory science careers; and leveraging existing programs such as the Clinical and Translational Science Awards (CTSAs) and fellowship and training opportunities offered through NIH and FDA.

Snapshot of Current FDA Regulatory Science Training Efforts
FDA is the premier organization for regulatory research and training in the U.S. The agency has created strong training programs for its staff including the Staff Colleges within each Center which offer numerous courses covering a range of regulatory, scientific and professional development topics. The majority of the Centers have also defined core science-based competencies necessary for product reviewers and other scientists working within the agency. FDA offers a learning portal for students, academia, and industry, which provides education and training materials, such as lectures, online courses and case studies, on FDA’s regulatory, product quality, and safety responsibilities. 

In addition, the agency administers several professional development programs including Oak Ridge Institute for Science and Education (ORISE) fellowships and the Commissioner’s

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5 FDA’s Learning Portal for Students, Academia and Industry: http://www.fda.gov/Training/learningportal/default.htm
Fellowship Program (CFP). In 2008, FDA launched the two-year CFP, with goals to: 1) attract top-tier scientists to FDA to address regulatory science issues through mentored projects of high priority to the Agency, 2) train scientists in regulatory science, and 3) serve as a potential recruiting tool.

While the ORISE, CFP and other FDA professional development programs are useful, they are limited in their ability to fully provide the necessary length, appropriate content, and breadth of exposure for trainees in the field of regulatory science.

In 2011, FDA awarded $2 million to launch Centers of Excellence in Regulatory Science and Innovation (CERSIs)\(^7\) at University of Maryland and Georgetown University. In May 2014, FDA expanded its CERSI network, providing funding through a competitive application process to the University of California at San Francisco in a joint effort with Stanford University and Johns Hopkins University. A major aim of the CERSIs is to support educational and training opportunities for students, FDA, academic staff members and faculty in regulatory science. Under memoranda of understanding (MOUs), the FDA and universities will facilitate research collaborations, personnel exchanges between university faculty and FDA staff, joint meetings for education and research, and identify opportunities for FDA personnel to serve as adjunct faculty and advisors at the universities. Overseas, FDA is leading an effort to train international regulators to establish Centers of Excellence (CoE) in their region. The cohorts taught by FDA are expected to train and sustain the staff in best practices for multi-regional clinical trials (MRCT).

Noted above are several examples of ways in which FDA is promoting education and training in regulatory science within the agency, domestically and abroad. However, the onus for fostering a competent regulatory science workforce does not reside totally with the FDA. The development of a comprehensive regulatory science training effort requires collaboration among members of academia, industry, other government agencies, international regulators and patient groups. The proposed regulatory science training consortium would develop necessary content to enhance existing academic and FDA programs in regulatory science and seek to avoid duplication of existing efforts.

**Consortium Approach**

The challenges articulated can be best addressed by establishing a public-private partnership coordinated though a neutral third party convener (501c3). The diagram below demonstrates the “heavy lift” that occurs during the build phase of the partnership. Leveraging an existing consortium model would offer the benefit of minimizing build activities to enable efforts to move quickly into the operations and maintenance phase.

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\(^6\) FDA Fellowship, Internship, Graduate and Faculty Programs:
http://www.fda.gov/AboutFDA/WorkingatFDA/FellowshipInternshipGraduateFacultyPrograms/default.htm

\(^7\) Centers of Excellence in Regulatory Science and Innovation (CERSI):
http://www.fda.gov/scienceresearch/specialtopics/regulatoryscience/ucm301667.htm
Consortium Proposal

The goal of the consortium approach would be to:

- Establish a Coordinating Committee comprised of multiple stakeholders to:
  - assess regulatory science education gaps in the existing academic and FDA training programs
  - identify and prioritize training needs critical to establishing an effective regulatory science workforce
  - understand the range of regulatory science educational content that is available
  - develop metrics to assess the usefulness of the consortium approach to address regulatory science training needs
• Develop key training modules to support regulatory science education in key areas including, but not limited to:
  o chemistry manufacturing and controls (CMC), pharmtox, clinical pharmacology, statistics
  o clinical trial design and analysis methods to support the development of biologics, drugs and medical devices
  o patient reported outcome (PRO) development, endpoints to support the development of biologics, drugs and medical devices
  o subtopics of rare diseases
  o ethics
  o pediatrics, elderly, and other vulnerable populations
  o adequate and well-controlled trials
  o drug-device interactions
  o investigator responsibilities (regulatory, legal, ethical)
  o consumer behavior research and risk analysis relating to food safety
  o microbiological, chemical and analytical methods to support food safety
• Develop sabbaticals, fellowships, and academic exchange programs to allow trainees significant dedicated exposure to industry, academic, and regulatory environments. For example, sabbatical opportunities could be established at the FDA to allow senior faculty and midlevel professionals the opportunity to train within FDA review divisions and the Office of Regulatory Affairs.
• Cultivate the development of a pool of future leaders in regulatory science
• Collaborate with FDA to administer the Commissioner’s Fellowship Program. For example, FDA and the Consortium could work in partnership to identify priority projects, sponsor training and development opportunities, and evaluate the program.

Potential Stakeholders

• Academic Training Programs
• Industry
• Reagan-Udall Foundation (RUF)
• NIH, National Center for Advancing Translational Science (NCATS) and Clinical and Translational Science Award (CTSA) institutions
• CERSIs
• The Howard Hughes Medical Institute (HHMI)
• Innovative Medicines Institute (IMI)
• International regulators
• Hamner Institute

Summary

The goal of a consortium would be to address the emerging unmet public health need to develop and train future regulatory scientists who can facilitate the future of efficient medical product development. The proposed consortium approach would offer benefit to all stakeholders
including future regulatory staff, international regulators, industry, academia, and other partners. The consortium could facilitate the development of a comprehensive regulatory science curriculum to supplement courses offered by academia and serve as a resource for all stakeholders. Further, the consortium could administer various fellowship programs including the Commissioner’s Fellowship Program and sabbatical programs devoted to facilitating development of individuals who can train in regulatory settings, academia and industry.

Although this is an ambitious proposal, bold action is necessary to build the infrastructure for a competent and well-trained regulatory science workforce. To assure the success of this project, a staged approach to implementation may be appropriate. Similarly, the different FDA Centers may engage in a phased manner over time. We propose a tiered approach to implementation of the RSTC framework and its four program areas (i.e., curriculum development, fellowships, academic exchange programs and sabbaticals). There are opportunities for the RSTC to achieve quick wins by focusing initial phases of the consortium on curriculum development and fellowship programs. The agency has several existing training and educational programs for which content can be leveraged along with similar resources from other stakeholders to support the curriculum development component. As an example, RUF has partnered with CDER and the Alzheimer’s Association to establish a two-year fellowship program in Alzheimer’s disease. This fellowship as well as the Commissioner’s Fellowship Program would ideally be brought under the auspices of the RSTC to assure efficient administration of these programs, avoid duplication, and take advantage of synergies in training approaches.

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8 With respect to fellowship programs, under Section 770 of the Federal Food, Drug, and Cosmetic Act (FDCA), Congress tasked RUF to “establish polices for funding training fellowships...for scientists, doctors and other professionals..., to foster greater understanding of and expertise in new scientific tools, diagnostics, manufacturing techniques, and potential barriers to translating basic research into clinical and regulatory practice.” Further, “The Commissioner may accept the uncompensated services of Foundation fellows or trainees. Such services shall be considered to be undertaking an activity under contract with the Secretary as described in section 708.” Section 708 of the FDCA permits contractors to have access to information exempt from public disclosure under FOIA exemption 4.
Questions for the Science Board

1) What are the strengths and weaknesses of this proposal?
2) What do you see as priority areas of focus for regulatory science education?
3) Do you envision a role for the CERSI s in this effort? If so, what would be the role?
4) Of the four activity streams proposed (curriculum development, academic exchanges, sabbaticals, fellowships), which area presents an opportunity for quick wins?