FDA Advancing Regulatory Science for Public Health
+$25,000,000 / 50 FTE

The following table displays the budget authority for Advancing Regulatory Science in the FY 2011 President's Budget for FDA programs.

FY 2011 Resource Table

Program[1/2/]	FY 2011 President's Budget Request
Budget Authority:	
Foods	**$2.377**
Center	2.100
Field Activities	0.277
Human Drugs[2]	**$2.752**
Center	2.475
Field Activities	0.277
Biologics [2]	**$1.425**
Center	1.425
Field Activities	0.000
Animal Drugs and Feeds	**$2.150**
Center	2.150
Field Activities	0.000
Devices and Radiological Health[2]	**$4.009**
Center	3.733
Field Activities	0.276
National Center for Toxicological Research[2]	**$1.700**
Headquarters and Office of the Commissioner[2]	**$9.557**
Other Rent and Rent Related	**$0.368**
GSA Rental Payments	**$0.662**
Total, Budget Authority	**$25.000**

[1] FDA has funding for nanotechnology of $6.5 million for FY 2009 and $7.3 million for FY 2010.

[2] Reports to accompany FDA appropriations bills identify the following allocations for the Critical Path Initiative: $16 million for FY 2009 and $18.0 million for FY 2010

1. Initiative Summary:

The Advancing Regulatory Science for Public Health initiative will help FDA build its scientific infrastructure, develop standards for new and emerging technologies, modernize the standards for evaluating products, and accelerate the development of essential medical therapies for the American people.

This initiative will allow FDA to advance regulatory science, which is unique and different from science conducted by industry and academia. Regulatory science focuses on developing tools to properly assess the safety, effectiveness and quality of products that are being developed or are already in the market. Importantly, it focuses on tools and knowledge that help products get from concept to market safely and efficiently. Advancing regulatory science is essential to more efficiently develop foods and medical products.

The Advancing Regulatory Science initiative is the first major effort to address scientific gaps identified in *FDA Science and Mission at Risk*, the 2007 report of the Subcommittee on Science and Technology of the FDA Science Board, which is an Advisory Committee to the FDA Commissioner. This initiative builds on President Obama's commitment to harness the power of science for America's benefit.

2. Why is this funding necessary?

During the past two decades, extraordinary investments have led to revolutionary advances in the biomedical sciences. However, FDA's scientific expertise and infrastructure have not kept pace with these advances. Today, FDA is relying on 20th century regulatory science to evaluate 21st medical products.

In 2007, *FDA Science and Mission at Risk* concluded that FDA is unable fulfill its mission, in part because it lacks modern scientific expertise. The number of new medical products approved by FDA since 1950 has remained constant. Drugs entering phase 1 clinical trials today are no more likely to reach the market than those entering phase 1 trials more than 20 years ago – in part because methods to anticipate product safety and test product efficacy during development are inefficient and outmoded. The lack of core scientific capacities for new and emerging technologies has hampered regulatory review at FDA, delayed the development of promising new therapies, and handicapped FDA's ability to promote and preserve public health.

3. What activities will these funds support?

FDA will use the funds in this initiative for scientific modernization. FDA will begin to build scientific capacity and close the gap in the emerging science and technology areas where FDA is most handicapped. The FY 2011 investment in regulatory science will benefit new product development in areas such as personalized medicine and systems biology — including genomics and other "omics" — nanotechnology, medical imaging, wireless healthcare devices, cell- and tissue-based products, regenerative medicine, and other complex products, such as combination products and biosimilars.

The FY 2011 investment will allow FDA to develop regulatory standards to provide pathways for developing products relying on new and emerging technologies. FDA will support applied science that focuses on product characterization tools and biomarkers for product safety and effectiveness. With this investment, FDA can accelerate the development and evaluation of products that can address unmet health needs.

The FY 2011 investment will allow the FDA Chief Scientist to establish the Office of Science and Innovation to provide FDA-wide leadership and coordination for cross-cutting efforts in emerging sciences. The Chief Scientist will oversee the efforts to rebuild FDA's core scientific infrastructure.

However, FDA cannot succeed in these efforts alone. Therefore, FDA will also begin to establish a robust collaborative research program in emerging sciences. The scale and range of science issues is just too broad, and in many cases essential expertise lies within academic and government laboratories.

In this initiative, FDA will focus on the emerging technologies and medical products in three broad areas: leadership and coordination, core capacities, and modern product evaluation standards.

In the text that follows, FDA identifies the components that contribute to this initiative. Details about each component appear in the tab in the FY 2011 Budget labeled "Component Papers."

A. Leadership and Coordination: +$2,480,000 / 5 FTE

- **FDA will strengthen scientific leadership.** The Office of the Chief Scientist (OCS) will provide FDA and its centers with dedicated and expert scientific leadership. OCS will work with the centers to define and establish a vision, develop a structure, and prioritize, oversee, support, and coordinate key scientific investments at FDA.

  By establishing the Office of Science and Innovation, OCS will provide scientific support, leadership and coordination. The Office of Science and Innovation will be a core resource of scientific expertise in emerging areas. It will scan the horizon for new technologies, promote innovation in regulatory science and review, and serve as a nucleus for internal and external collaboration.

  OCS will also establish collaborations to support regulatory science research. The collaborations will allow FDA to continuously access expertise in emerging science, harness existing external resources, and strengthen knowledge and skills within the FDA workforce.

  These efforts will focus on emerging technologies and respond to key needs identified by the FDA Science Board. The Chief Scientist will coordinate key professional development programs in emerging sciences and pilot a Challenge Grants program to support scientists in FDA centers who are engaged in peer reviewed, high priority, collaborative, and mission-focused applied regulatory research. This investment begins to respond to concerns of the FDA Science Board, which described FDA research as ". . . critical because it is not conducted by other public or private entities . . . [and is] fundamental to the discharge of FDA's statutory responsibilities to protect and promote the public health."
  Component A-1: +$2.5 million / 5 FTE

B. Core Capacities – Infrastructure, Workforce, Collaboration: +$15,550,000 / 26 FTE

- **FDA will acquire core scientific capacities in emerging technologies.** FDA will build core scientific capacity and expertise in nanotechnology, which holds great promise for advances in medical products and foods. FDA will establish collaborations with scientific institutions and other regulatory agencies and support collaborative regulatory science research in nanotechnology. Nanotechnology efforts will focus on critical product characterization and safety issues given the field's potentially unanswered safety concerns. With this investment, FDA seeks to support innovation while protecting consumers.
  Component A-2: +$7.3 million / 17 FTE

  FDA will support the development and evaluation of products that result from stem cell innovation so that the large Federal investment in stem cell research can transfer from the bench to the bedside.
  Component A-3: +$0.95 million / 2 FTE

- **FDA will recruit next generation scientific staff.** FDA and the Science Board have identified essential areas of emerging science where FDA lacks expertise. The FY 2011 Budget will allow the Center for Devices and Radiological Health to enhance expertise in two forward-looking areas for which there is an urgent and critical need: 1) smart devices for a wide range of applications, including in the development of next generation high-technology orthopedic prosthetic devices and 2) innovative analytic methods that will allow FDA to more efficiently acquire and translate medical data into usable information. FDA will apply these methods to the National Medical Device Registry, *Component A-15*.
  Component A-1: +$0.5 million / 2 FTE

- **FDA will address science issues for creating a National Medical Device Registry.** FDA will begin to link unique device identifiers (UDIs) to health-related electronic data to create a National Medical Device Registry (NMDR) to improve our understanding of the risk-benefit profile of higher risk devices. Additional funding of $1.667 million for the National Medical Device Registry appears in component P-10 of the Protecting Patients Initiative.
  Component A-4: +$2.3 million / 4 FTE

- **FDA will promote scientific collaboration and exchange.** Through enhanced support of partnerships and collaboration, FDA will develop tools to modernize product development and evaluation. Additional investments in FDA's Critical Path Program will position FDA to foster focused partnerships to transform product development and evaluation sciences, advance personalized medicine, and develop novel diagnostic and medical products. The goal is to speed translation of research discoveries into marketed medical products, while reducing risks and costs by identifying ineffective products and potential safety risks early in development.
  Component A-5: +$4.5 million / 1 FTE

C. Update medical product regulatory standards: +$5,300,000 / 16 FTE

- **FDA will update review standards and provide regulatory pathways for new technologies.** FDA will establish regulatory guidance to provide a scientifically sound and safe pathway to better characterize and develop biosimilars.
  Component A-6: +$2.0 million / 4 FTE

  Given the tremendous potential for biotechnology to improve health and reduce illness, FDA will refine its guidance to industry and regulatory pathway for animal biotechnology products.
  Component A-7: +$1.9 million / 10 FTE

- **FDA will promote development of healthy foods and encourage healthy food choices.** FDA will encourage and support food industry efforts to modify food products and give consumers more healthful food choices. FDA will use data from well-designed studies to support a modernized food label to encourage Americans to eat healthier diets and potentially reduce the prevalence of obesity and its associated health care costs in the United States.
  Component A-8: +$1.4 million / 2 FTE

D. Program Support for Advancing Regulatory Science Priorities: +$728,000 / 3 FTE

To ensure that FDA program offices that conduct the Advancing Regulatory Science initiative receive the support necessary to achieve the proposed public health priorities, the initiative includes resources for essential support activities. Support activities include finance and budgeting, human resource assistance, legal counsel, communications, ethics, coordination and related support functions.
Component A-9: +$0.728 million / 3 FTE

4. How does this initiative support important public heath priorities?

The Advancing Regulatory Science Initiative addresses a key Presidential priority to harness the power of science to improve the health of Americans. Advancing Regulatory Science also supports three of the eight guiding principles in the Administration's agenda for health care: investing in prevention and wellness, improving patient safety and quality of care, and quality health care for the American public.

5. What are the risks of not proceeding with this initiative?

Funding the Advancing Regulatory Science Initiative will strengthen FDA's capacity and expertise in mission-critical areas. Specifically, FDA will strengthen the scientific expertise necessary to support product development through strong science leadership and coordination, core support for targeted research at FDA, and strategic collaboration and partnerships through Critical Path and other academic and government partners. This investment will allow FDA to support sound development, evaluation and access to innovative new products that promise to revolutionize medicine and public health. It will

help FDA be better equipped to assess safety and efficacy throughout a product's lifecycle, including post approval safety monitoring.

Failure to fund this initiative will result in the continued erosion of excellence at FDA and deeply affect FDA's core competency and morale. FDA will not be able to attract, recruit or retain the review, laboratory and population scientists needed to help our country harvest the fruits of revolutionary developments in science and informatics. The risks of failures will continue to increase as the gaps between demands on FDA and its capacity to respond continue to grow.

As the Science Board noted, "[t]he imbalance is imposing a significant risk to the integrity of the food, drug, cosmetic and device regulatory system and hence the safety of the public." This initiative begins to plug those gaps. Not funding this initiative will mean that FDA and the U.S. biotechnology, pharmaceutical and device industries will see their global influence wane, with adverse impacts to the U.S. economy and the health and quality of life for all Americans.

6. What will FDA accomplish with this initiative?

This initiative is critical for FDA to be an active participant in 21st century product development and to fulfill its mission to patients and consumers. The Advancing Regulatory Science initiative takes important initial steps to support development of a world-class science workforce and brings much needed core scientific capacities to FDA. With these resources, FDA can better maintain its position as a global leader in regulatory science. As a result, America will continue to be a global leader in biotechnology, food and medical product development.

The initiative will also generate significant benefits for public health in the United States. It will allow for faster product development and availability, decreased product development costs, and new, more efficient regulatory pathways for emerging technologies.

This initiative will benefit every American by increasing access to new medical technologies that treat serious illnesses and improve quality of life. It will increase the accuracy and efficiency of FDA review, reducing adverse health events, regulatory costs, and the time-to-market for new medical technologies. As a result, U.S. consumers and the U.S. health care system will benefit from lower medical costs. Populations in other countries will also benefit, as the FDA remains the international "gold standard" for regulatory science.

In general terms, this investment will support:

- Strong scientific leadership and coordination
- Establishment of core science capacities at FDA and support for scientific partnerships and collaboration with academia
- New regulatory science tools, pathways and guidance for new and emerging technologies
- Improved accuracy and efficiency of FDA review
- Faster access to personalized medicine and new life-saving therapies.

FY 2011 Advancing Regulatory Science Performance Table:

The following table contains information about performance commitments associated with FDA's proposed increase for Advancing Regulatory Science, including accomplishments achieved during Fiscal Year 2009 and expected in Fiscal Year 2010, and proposed program outputs to be achieved in Fiscal Year 2011. For more information regarding the alignment of FDA's Strategic Action Plan goals and objectives with each Center's or Office's Subprogram areas and associated annual performance goals, see the information on strategic alignment in the Performance Materials Tab of this budget document.

Leadership and Coordination

Program Outputs	FY 2009 Actual	FY 2010 Appropriation	FY 2011 Request	FY 2011 +/- FY 2010 PB
Establish organizational resources for strengthening science at FDA	Established the Office of Chief Scientist (OCS), which includes a new Office of Science and Innovation	Develop staffing plans for OCS; conduct comprehensive assessment to identify critical science gaps and scientific areas for coordinating across Centers; evaluate existing Center programs for strengthening science	Key staff hired; OCS coordinate key professional development programs in emerging sciences; establish Cross-Center Working Groups; Establish Scientific Challenge Grant programs for collaborative, mission critical, applied regulatory science research	Cross-Center Working Groups operational; key professional development programs operational, new challenge grants scientific leadership and coordination efforts established

Core Capacities – Infrastructure, Workforce, and Collaboration

Program Outputs	FY 2009 Actual	FY 2010 Appropriation	FY 2011 Request	FY 2011 +/- FY 2010 PB
Enhance scientific capabilities in nanotechnology	Complete the initial setup of the NCTR/ORA Nanotechnology Core Facility; limited scale effort at Centers to	Identify core needs to build laboratory capacity Establish goals of training programs in	Acquire critically needed equipment and technical staff to conduct product assessment and safety research in support of regulatory decision	Establish a professional development program in nanotechnology Establish

Program Outputs	FY 2009 Actual	FY 2010 Appropriation	FY 2011 Request	FY 2011 +/- FY 2010 PB
	characterize nanotechnology-based products as they relate to human health Provide science based guidance to those seeking to use nanomaterials in FDA regulated products	nanotechnology to enhance expertise in nanotechnology Hold public workshop(s) to inform regulatory research needs in nanotechnology Identify potential partners and models for collaboration to conduct interdisciplinary research to address product characterization and safety	making Establish a professional development program in nanotechnology Establish collaborative programs in nanotechnology with universities, government agencies and international regulatory counterparts	collaborative programs in nanotechnology with universities, government agencies and international regulatory counterparts
Strengthen FDA's scientific capacity to regulate products resulting from stem cell innovation	Development of MRI imaging methods to track neural stem cells after transplantation into mouse brains	Identify and preclinically test biomarkers that correlate with mesenchymal stem cell safety and effectiveness Finalize the guidance for industry, "Potency Tests for Cellular and Gene Therapy Products" Draft guidance on initiation and conduct of early phase clinical trials using cellular and gene therapies Draft guidance on the preclinical safety assessment of cell and gene therapy products	Develop analytic tests for characterization of stem cells products Conduct outreach activities to ensure the scientific community is aware of regulatory requirements	Develop analytic tests for characterization of stem cells products Conduct outreach activities to ensure the scientific community is aware of regulatory requirements

Program Outputs	FY 2009 Actual	FY 2010 Appropriation	FY 2011 Request	FY 2011 +/- FY 2010 PB
Improved tools for modernizing medical product assessment in areas such as clinical trial design and data analysis, and more accurate predictors of product safety and efficacy using biomarkers	Engaged public-private partnerships to identify and qualify new biomarkers (e.g., C-Path Institute) and to improve detection of serious adverse events in clinical trials (e.g., Duke University)	Initiate new projects Conduct review of critical path initiative, evaluate high priority areas for further investments	Fund projects to validate new biomarkers, enable personalized medicine, modernize and increase the efficiency of the clinical trial enterprise, improve tools to predict safety and effectiveness of medical products, modernize the methods used in toxicology studies	New projects to validate new biomarkers, enable personalized medicine, modernize and increase the efficiency of the clinical trial enterprise, improve tools to predict safety and effectiveness of medical products, modernize the methods used in toxicology studies
Enhanced continuing education and professional development programs for scientific staff, increased scientific exchanges	Continued improvement of Center-specific professional development programs and enhanced online opportunities	Conduct needs assessment for scientific professional development; establish baseline for measures of participation and set FY 2011 targets	Increase participation in continuing education, professional development, and scientific collaborative programs (target to be set in FY 2010); targeted increases in performance incentives	Increase participation in continuing education, professional development, and scientific collaborative programs

Update Medical Products Regulatory Standards

Program Outputs	FY 2009 Actual	FY 2010 Appropriation	FY 2011 Request	FY 2011 +/- FY 2010 PB
Enhance capabilities to efficiently regulate new animal biotechnology products	Develop baseline performance	Meet "real time" review[1] goals by completing reviews of existing submissions and continuing to work with Sponsors on earlier stage submissions Draft intra-agency Biopharm Animal Guidance	Hire and train staff to improve the knowledge base and expertise to facilitate review and potential approval of animal biotechnology products	Hire and train staff to improve the knowledge base and expertise to facilitate review and potential approval of animal biotechnology products
Increase number of regulatory standards established to guide drug innovators in the application of technologies such as genomics, proteomics and medical imaging to	Provide scientific and strategic input to the Predictive Safety Testing Consortium to qualify biomarkers for drug-induced kidney toxicity in	Provide scientific and regulatory advice in biomarker qualification for cancer, Alzheimer's, Parkinson's, and cardiovascular disease, with The Biomarker Consortium, the	Develop regulatory policy to clarify FDA expectations and requirements surrounding submission of applications for biosimilar products	Expand the scope of regulatory preparations to include biosimilar products

[1] "Real-time" review refers to a submission review system developed by CVM to work closely with producers of GE animals that plans, protocols, studies, etc. be evaluated as they are developed (i.e., in real-time).

drug discovery and development	humans	Coalition against Major Diseases, and other major consortia Provide scientific advice for neglected diseases to stakeholders such as the Gates foundation, C-Path and other coalitions	Begin acquiring necessary equipment and expertise to evaluate biosimilar applications	
Improve public health and reduce chronic disease by modernizing the food label, increasing consumer awareness, and reducing intake of sodium and trans fats	Program evaluation of nutrition labeling education for children ages 9-13	Continue nutrition labeling education campaign, including program targeted to middle and high school teachers	Develop nutritional criteria for labeling on the front of food packages that consumers can rely on to make informed choices for healthy eating	Increased use of nutrition labeling and standardized front of package labeling to enable food choices that help consumers lower their risk of chronic disease