

FDA Medical Countermeasures Initiative +\$3,510,000 / 7 FTE

The following table displays the FDA budget authority for the Medical Countermeasures Initiative in the FY 2013 Congressional Budget Justification.

Advancing Medical Countermeasures

(Dollars in Millions)

Program	FY 2010 \$170M One-Time Allocation (non-add) ¹	FY 2011 Enacted	FY 2012 Enacted	FY 2013 Request	+/- FY 2012 Enacted
Budget Authority:					
Human Drugs	\$28.017	\$0.000	\$4.756	\$5.596	\$0.840
Center	27.144	0.000	4.756	5.596	0.840
Field Activities	0.873	0.000	0.000	0.000	0.000
Biologics	\$27.362	\$0.000	\$1.974	\$2.226	\$0.252
Center	26.489	0.000	1.974	2.226	0.252
Field Activities	0.873	0.000	0.000	0.000	0.000
Devices and Radiological Health	\$17.099	\$0.000	\$2.997	\$3.720	\$0.723
Center	16.661	0.000	2.997	3.720	0.723
Field Activities	0.438	0.000	0.000	0.000	0.000
FDA Headquarters	\$90.234	\$0.000	\$9.013	10.312	1.299
Other Rent and Rent Related	\$2.603	\$0.000	\$0.472	0.616	0.144
GSA Rental Payments	\$4.685	\$0.000	\$0.826	1.078	0.252
Total Advancing Medical Countermeasures	\$170.000	\$0.000	\$20.038	\$23.548	\$3.510

¹ Under the August 20, 2010, budget amendment and a related announcement by Secretary Sebelius, FDA received \$170 million from amounts appropriated under Public Laws 111-8 and 111-117. Under the terms of Public Law 112-10 (April 15, 2011), FDA can spend the \$170 million on activities related to chemical, biological, radiological and nuclear threats, in addition to the previous authority to spend these funds on emerging infectious diseases.

1. Initiative Summary:

The FDA Medical Countermeasures Initiative (MCMi) is designed to meet America's national security and public health requirements for medical countermeasure (MCM) readiness. In advance of Congress' FY 2012 appropriation for the MCMi, FDA received an allocation of one-time funding at the close of FY 2010 to immediately commence MCMi activities. With these funds, FDA established a base program at its current operating level of 77 FTEs.

The FY 2013 budget contains resources that will allow FDA to sustain the current level of staffing and activities for the MCMi. With these FY 2013 resources, FDA will support partnerships with industry, academia, and government partners to improve MCM development timelines and success rate for MCMs. FDA will also expand technical assistance to developers for the highest priority MCMs.

FDA Medical Countermeasures Initiative

2. Why is this funding necessary?

The FDA plays a vital role in protecting the United States from chemical, biological, radiological, and nuclear (CBRN) threats, and from emerging infectious diseases. FDA is responsible for ensuring that MCMs – such as drugs, vaccines, and diagnostic tests – to counter these threats are safe, effective, and secure. In addition, FDA works closely with Federal partners through the Department of Health and Human Services' (HHS) Public Health Emergency Medical Countermeasures Enterprise (Enterprise) to build and sustain the MCM programs necessary to respond to public health emergencies.

The Threat: According to the U.S. intelligence community, CBRN weapons and emerging infectious diseases present real, substantial and growing threats to the national security of the United States, and will continue to do so for the foreseeable future. For example, the March 2011 unclassified annual threat assessment from the U.S. intelligence community states that:

. . . many of the countries pursuing [weapons of mass destruction] programs will continue to try to improve their capabilities and level of self-sufficiency over the next decade. Nuclear, chemical, and/or biological weapons – or the production technologies and materials necessary to produce them – also may be acquired by states that do not now have such programs. Terrorist or insurgent organizations acting alone or through middlemen may acquire nuclear, chemical, and/or biological weapons and may seek opportunistic networks as service providers.¹

The March 2011 threat assessment echoes a 2009 assessment. According to the U.S. intelligence community, “[o]ver the coming years, [the United States] will continue to face a substantial threat, including in the U.S. Homeland, from terrorists attempting to acquire biological, chemical, and possibly nuclear weapons and use them to conduct large-scale attacks.”² This assessment also stressed that “[i]n particular . . . the terrorist use of biological agents represents a growing threat . . .”

In October 2011, the Honorable Tara O'Toole, Under Secretary for Science and Technology, U.S. Department of Homeland Security in testimony before the Committee on Homeland Security and Governmental Affairs described the growing biological weapons threat:

¹ Clapper, J.R. Statement for the Record on the Worldwide Threat Assessment of the U.S. Intelligence Community for the House Senate Committee on Armed Services. *Annual Hearing to Receive Testimony on the Current and Future Worldwide Threats to the National Security of the United States*, Hearing, March 10, 2011. Available at: http://www.dni.gov/testimonies/20110310_testimony_clapper.pdf. Accessed December 22, 2011.

² Blair, D. Testimony before the Armed Services Committee, United States Senate. *Annual Threat Assessment of the Intelligence Community*, Hearing, March 10, 2009. Available at: http://www.dni.gov/testimonies/20090310_testimony.pdf. Accessed December 22, 2011.

FDA Medical Countermeasures Initiative

We are living in the midst of a biotechnology revolution where the knowledge and tools needed to acquire and disseminate a biological weapon are increasingly accessible. It is possible today to manipulate pathogens' characteristics (e.g. virulence, antibiotic resistance), and even to synthesize viruses from scratch. These procedures will inexorably become simpler and more available across the globe as technology continues to mature . . . Even small-scale attacks could be highly lethal and disruptive, and as has been noted, there is a real possibility of a campaign of bioattacks on multiple targets (the "reload" phenomenon) – because these weapons are self-replicating organisms. Moreover, it is not necessary for a nation-state to maintain a large stockpile of bioweapons to pose a significant asymmetric threat as the development of a significant offensive bioattack capability could occur within weeks or months.³

Numerous U.S. governmental reports have highlighted similar concerns.⁴ For example, the *National Security Strategy* of 2010 notes that "[t]he effective dissemination of a lethal biological agent within a population center would endanger the lives of hundreds of thousands of people and have unprecedented economic, social, and political consequences."⁵

And in a November 2009 report, the National Security Council estimated that the economic cost of a well-executed bioterrorist attack on American soil could exceed one trillion dollars. Such an attack could have profound consequences for our way of life, for trust in government, and for our society and political order.⁶

Naturally occurring emerging infectious diseases also pose a growing threat and could have similar consequences.⁷ For example, in 2006 the Congressional Budget

³ O'Toole, T.J. Testimony before the Homeland Security and Governmental Affairs Committee, United States Senate. Ten Years after 9/11 and the Anthrax Attacks: Protecting against Biological Threats, Hearing, October 18, 2011. Available at http://hsgac.senate.gov/public/index.cfm?FuseAction=Hearings.Hearing&Hearing_ID=1b1b1599-2539-47a0-a2d7-aa9fbb966fb8. Accessed December 22, 2011.

⁴ *U.S. Government Judgments on the Threat of Biological Weapons*. Baltimore, MD: Center for Biosecurity of UPMC. March 2011. Available at http://www.upmc-biosecurity.org/website/resources/publications/2010/pdf/2010-01-19-gov_judgments_BWthreat.pdf. Accessed December 22, 2011.

⁵ *National Security Strategy*. Washington, DC: The White House. May 2010. Available at http://www.whitehouse.gov/sites/default/files/rss_viewer/national_security_strategy.pdf. Accessed December 22, 2011.

⁶ *National Strategy for Countering Biological Threats*. Washington, DC: White House, National Security Council. November, 2009. Available at: http://www.whitehouse.gov/sites/default/files/National_Strategy_for_Countering_BioThreats.pdf. Accessed December 22, 2011.

⁷ See for example *Strategic Implications of Global Health* (ICA 2008-10D) [Washington, DC: National Intelligence Council; December 2008. Available at: http://www.dni.gov/nic/PDF_GIF_otherprod/ICA_Global_Health_2008.pdf (accessed December 22, 2011)] which assessed that while numerous infectious and noninfectious health conditions can potentially impact U.S. strategic interests, "...for the foreseeable future [infectious diseases] will remain the top health-related threat to U.S. national security..." noting that the U.S. population "...will

FDA Medical Countermeasures Initiative

Office estimated that in the year following a severe influenza pandemic, U.S. gross domestic product could decline by 4.25 percent, a loss of approximately \$645 billion to the U.S. economy in current dollars.⁸

The FDA MCMi: In August 2010, HHS Secretary Sebelius released the results of year-long review of the Enterprise. This review assessed U.S. readiness to reduce the impact of a future public health emergency and improve the nation's capacity to respond quickly and effectively to CBRN and emerging infectious disease threats.⁹ The *Enterprise Review* revealed that regulatory uncertainties associated with MCM development are among the most significant obstacles to successful MCM development.¹⁰

The *Enterprise Review* identified key steps that the Federal government must take to modernize the Enterprise. In particular, the report highlighted how critical FDA is to the success of the Enterprise. The report also called for greater investment in regulatory innovation and MCM regulatory science and for FDA to take an even more active role in fostering the development and facilitating the availability of MCMs.

In response, FDA immediately launched its MCMi to enhance FDA's regulatory processes, to foster clear regulatory pathways for MCMs and to facilitate the timely use of available MCMs. The MCMi is a comprehensive program to address key challenges in three areas:

- enhancing the regulatory review process for the highest priority MCMs and related technologies
- advancing regulatory science for MCM development
- modernizing the regulatory and legal framework.

The FY 2013 investment will contribute to sustaining the MCMi and to protecting the United States from potentially catastrophic CBRN and emerging infectious disease threats. The MCMi is essential to reduce the slow pace of development and reverse the high failure rates associated with MCM development. In addition, the MCMi is essential to helping transform the Enterprise so it can respond faster and more

continue to be vulnerable to emerging infectious diseases – many of which will originate overseas (e.g., HIV/AIDS, West Nile, and dengue fever) – including a potential influenza pandemic or an outbreak of a “mystery” disease (e.g., SARS.)”

⁸ *A Potential Influenza Pandemic: Possible Macroeconomic Effects and Policy Issues*. Washington, DC, Congressional Budget Office. December 8, 2005; revised July 27, 2006. Available at: <http://www.cbo.gov/ftpdocs/69xx/doc6946/12-08-BirdFlu.pdf>. Accessed December 22, 2011.

⁹ Sebelius, K., Speech before the American Medical Association Third National Congress on Health System Readiness. Washington, DC: US Department of Health and Human Services; December 1, 2009. <http://www.hhs.gov/secretary/speeches/sp20091201.html>. Accessed December 22, 2011.

¹⁰ *The Public Health Emergency Medical Countermeasures Enterprise Review – Transforming the Enterprise to Meet Long-Range National Needs*. Washington, DC: US Department of Health and Human Services. August 2010. Available at: <https://www.medicalcountermeasures.gov/documents/MCMReviewFinalcover-508.pdf>. Accessed December 22, 2011.

FDA Medical Countermeasures Initiative

nimbly to “...any attack or threat, known or unknown...” as envisioned in the *Enterprise Review*.

3. What has this program accomplished?

Since the announcement of the FDA MCMi in August 2010, FDA and its drug, device and biologics programs have worked aggressively to ensure that the United States has access to high-priority MCMs to respond to CBRN and emerging infectious disease threats, such as pandemic influenza.

MCMi Accomplishments: During its first year, FDA made substantial progress to implement the MCMi using the one-time funding that HHS transferred to FDA. The *MCMi Year 1 Status Report* summarizes FDA’s achievements as of September 2011¹¹ For example, FDA:

- Established Public Health and Security Action Teams for multiplex *in vitro* diagnostic tests and for therapies and diagnostics for acute radiation syndrome
- Launched a rigorous MCM regulatory science program that identified more than 80 intramural research projects for funding
- Sponsored an Institute of Medicine workshop to obtain authoritative guidance for the MCM regulatory science program FDA’s regulatory science program for MCMs
- Published a request for information to solicit science recommendations for the extramural component of the MCM regulatory science program
- Established a partnership with the Defense Advanced Research Projects Agency (DARPA) to collaborate on regulatory science research
- Issued an umbrella Emergency Use Authorization (EUA) for doxycycline post-exposure prophylaxis to support pre- and post-event activities for mass distribution and dispensing efforts to address an anthrax event
- Participated in an analysis on the feasibility of expanding the existing shelf-life extension program to include State and local MCM stockpiles
- Hosted a meeting of state and local public health leaders to share information on MCM legal issues related to emergency preparedness and response
- Launched the MCMi professional development program, which includes threat briefings by experts to ensure that FDA reviewers are fully aware of the threats – and therefore the risks – as they conduct risk-benefit analyses on MCM products.

¹¹ *FDA’s Medical Countermeasures Initiative Year-1 Status Report*. Washington, DC: US Food and Drug Administration. September 2011. Available at <http://www.fda.gov/downloads/EmergencyPreparedness/MedicalCountermeasures/UCM270750.pdf>. Accessed December 22, 2011.

Since the release of the *MCMi Year 1 Status Report*, FDA:

- Issued a 5-Year strategic plan for the MCMi¹²
- Launched Public Health and Security Action Teams for warfighter trauma care and to address pediatric, pregnancy, and special population issues
- Held workshops on developing and evaluating next-generation smallpox vaccines and regulating multiplex *in vitro* diagnostic tests
- Held an advisory committee meeting on smallpox drugs
- Published a concept paper on a novel regulatory approach for multiplex diagnostic tests
- Initiated a portfolio review and gap analysis of the intramural MCM regulatory science program to inform future MCM regulatory science investments
- Launched a program to qualify animal models as drug development tools
- Developed legislative proposals to enhance emergency preparedness and response that were submitted to Congress
- Issued an amendment to the EUA for doxycycline emergency kits for United States Postal Service employees who volunteer to support implementing the National Postal Model for emergency response.

MCM Activities Funded with the FY 2012 Appropriation: For FY 2012, Congress appropriated \$20,038,000 to provide a base of funding for FDA's MCMi. The FY 2012 appropriation allows FDA to sustain 70 of its 77 MCMi FTE and supports an investment in MCM regulatory science (\$327,000).

With FY 2012 funding approved by Congress, FDA will conduct the following MCMi activities:

- Sustain Public Health and Action Teams for warfighter trauma care, acute radiation syndrome, pediatric, pregnancy, and special population issues, and *in vitro* diagnostics
- Establish a Public Health and Action Team for next-generation assessment of MCM safety and efficacy during public health emergencies
- Finalize analysis of the regulatory gaps associated with the use of stockpiled MCMs to identify data needs to support the continued development of pre-EUA packages, with special focus on at-risk populations such as children
- Provide technical assistance to the developers of the highest-priority MCMs (MCMs procured by the U.S. government) to foster effective development and to support regulatory review

¹² Available at

<http://www.fda.gov/downloads/EmergencyPreparedness/MedicalCountermeasures/UCM286201.pdf>

- Strengthen extramural MCM regulatory science partnerships with NIH and DoD, focusing on tools to assess efficacy, MCM product quality, and advanced diagnostics
- Work with Enterprise partners to fill data needs associated with the development of pre-EUA packages for stockpiled MCMs
- Implement a program to qualify animal models as drug development tools
- Issue revised guidance on *Animal Models—Essential Elements to Address Efficacy Under the Animal Rule*
- Foster MCM development through agreements with Enterprise partners that facilitate MCM collaboration, communication, and information sharing
- Identify and communicate best review practices for interfacing with and supporting MCM sponsors
- Enhance rapid deployment and pre-event planning and positioning of MCMs.

4. What activities will FY 2013 funding support?

With this FY 2013 increase, FDA will support 7 FTE that are performing MCM activities. Currently, FDA is supporting the 7 FTE with the one-time funding allocated to FDA under Public Laws 111-8 and 111-117. The FY 2013 budget increase will allow FDA to sustain its full, current MCMi operating level of 77 FTE and to conduct the following MCMi activities.

A. Medical Countermeasures (+\$3,510,000 / 7 FTE)

FDA MCMi Objective 1 – Enhance the Review and Approval Processes for MCMs (+\$1,081,000 / 4 FTE)

FDA will operationalize Public Health and Security Action Teams for pediatric, pregnancy, and special population issues and teams for the next-generation assessment of MCM safety and efficacy during public health emergencies.

FDA will foster the development and deployment of MCMs by strengthening its program of technical assistance – including the development of regulatory management plans – for the developers of the highest-priority MCMs. FDA will also ready MCMs for use under an EUA in advance of an emergency.

<i>CDER</i>	+ \$840,000 / 3 FTE
<i>CDRH</i>	+ \$241,000 / 1 FTE

FDA Medical Countermeasures Initiative

FDA MCM Objective 2 – Advance Regulatory Science for MCM Development and Evaluation (+\$1,792,000 / 2 FTE)

FDA will sustain its MCM regulatory science program, relying heavily on partnerships with industry, academia and U.S. government partners that enable FDA to harness cutting-edge science and apply innovative approaches to the regulatory process to improve MCM development timelines and success rates. In particular, FDA will focus investments in regulatory science on:

- developing and qualifying tools to assess efficacy, such as animal and biomimetic models
- developing methods to assess product quality and assays to support the release of MCMs
- developing and assessing advanced diagnostic tests
- developing novel manufacturing platforms.

CBER + \$252,000 / 1 FTE
CDRH + \$241,000 / 1 FTE
FDA HQ +\$1,299,000 / 0 FTE

FDA MCM Objective 3 – Modernize the Legal, Regulatory, and Policy Framework for Effective Public Health Response (+\$241,000 / 1 FTE)

FDA will continue to work collaboratively with HHS to examine the legal framework and the regulatory and policy approaches for MCM development and availability to ensure these adequately support emergency preparedness and response. These efforts include strengthening FDA’s program to support rapid deployment and pre-event planning and positioning of MCMs.

CDRH + \$241,000 / 1 FTE

B. Rent Activities for Advancing Medical Countermeasures Initiative (+\$396,000 / 0 FTE)

The \$396,000 increase in budget authority will enable FDA to pay GSA Rent and Other Rent and Rent-Related costs for employees supported by the FY 2013 MCMi increase. Funding these rent activities will reduce the need to redirect resources from core, mission-critical public health activities to pay rent costs.

FDA Medical Countermeasures Initiative

5. How does this initiative support important public health priorities?

The FDA MCMi supports important national security and public health priorities. Through the MCMi, FDA is helping to ensure that Americans have access to the medicines and vaccines they need to counter a deliberate CBRN attack or a naturally occurring epidemic.

The FY 2013 budget request for MCMi supports the need for “rapid and reliable development of medical countermeasures to respond to public health threats,” as articulated in the National Security Strategy of 2010. FDA’s MCMi will also protect American’s health and foster resilience in response to emergencies.

The FY 2013 funding will also help implement FDA priorities articulated in the HHS *Enterprise Review*, released on August 19, 2010.¹³ As recommended by the review, FDA will promote MCM development by:

- supporting robust engagement with sponsors and government partners to facilitate the development of critical MCM products
- establishing clear regulatory pathways for developing MCMs
- advancing FDA MCM regulatory science to identify and resolve gaps that prevent successful MCM development and approval
- modernizing the legal, regulatory, and policy framework to foster the application of advances in regulatory science to the regulatory review process and supporting preparedness for and response to CBRN threats and emerging infectious disease threats with through the availability of MCMs.

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

Not approving the FY 2013 MCMi budget request poses genuine risks for the health of Americans and the security of the United States:

- The Nation’s ability to respond to natural or deliberate infectious disease outbreaks and CBRN threats will remain limited and insufficient.
- FDA will not be able to sustain the MCMi program at the level necessary to support the priorities in the *Enterprise Review*.
- The Federal government will not be able to fulfill its responsibility to protect the nation’s health and keep Americans safe during public health emergencies.

¹³ *The Public Health Emergency Medical Countermeasures Enterprise Review – Transforming the Enterprise to Meet Long-Range National Needs*. Washington, DC: US Department of Health and Human Services. August 2010. Available at: <https://www.medicalcountermeasures.gov/documents/MCMReviewFinalcover-508.pdf>. Accessed December 22, 2011.

- The United States will not be able to realize the return on the multibillion-dollar investments it has made in biodefense during the past decade.

7. What will FDA accomplish with the initiative?

Funding this initiative will support:

- a highly interactive review process for MCMs and related technologies
- a strong FDA workforce with enhanced expertise in CBRN issues
- active FDA engagement and collaboration with Federal MCM partners
- clear, well-defined and appropriate regulatory and scientific plans for HHS' highest priority MCMs
- an MCM regulatory science program to foster MCM development
- an improved legal framework and improved regulatory and policy approaches to MCM development and use
- faster development and availability of MCMs
- a more resilient Nation that is better able to cope with the CBRN and infectious disease threats
- job creation and economic development; every bioscience job creates 5.8 additional jobs
- stronger national security.

FY 2013 Medical Countermeasures Performance Table:

FDA is using FDA-TRACK, our agency-wide performance management system, to track, analyze, and report monthly and quarterly performance measures, progress and accomplishments for FDA's most important initiatives. These initiatives include ongoing efforts as well as new efforts as showcased in the following FY 2013 performance tables. Upon finalization and receipt of the FY 2013 request, FDA will be developing performance measures and/or key project milestones for the funded initiatives. You will find these measures, milestones, and progress on the FDA-TRACK website - www.fda.gov/fdatrack.

The following tables contain performance items associated with this initiative.

FDA Medical Countermeasures Initiative

Performance Measures	FY 2012 Enacted Performance Level	FY 2013 Performance Level +/- FY 2012 Enacted	Most Recent Actual
<p>Enhance development, evaluation, approval, and surveillance processes for high-priority MCMs and platform technologies; create Public Health and Security Action Teams to analyze processes, identify gaps and hurdles, and propose recommendations for improvement</p>	<ul style="list-style-type: none"> Establish Public Health and Action Team for next-generation assessment of MCM safety and efficacy during public health emergencies Increase technical assistance to the developers of the highest-priority MCMs (i.e., MCMs that have been procured by the US government) to foster effective development and support regulatory review 	<ul style="list-style-type: none"> Operationalize Public Health and Action Teams for pediatric, pregnancy and special population issues and next-generation assessment of MCM safety and efficacy during public health emergencies Foster the development and deployment of MCMs by: (1) strengthening its program to provide technical assistance—including the development of regulatory management plans—to the developers of the highest-priority MCMs; and (2) readying MCMs for use under an EUA in advance of an emergency review 	<p>N/A</p>
<p>Support MCM development and evaluation by establishing regulatory science programs for MCM products based on extramural, collaborative research programs</p>	<ul style="list-style-type: none"> Strengthen extramural MCM regulatory science partnerships with NIH and DoD with a focus on tools to assess efficacy, MCM product quality, and advanced diagnostics 	<ul style="list-style-type: none"> Sustain MCM regulatory science program that relies heavily on partnerships with industry, academia and additional U.S. government partners with a focus on developing tools to assess efficacy, MCM product quality, advanced diagnostics, and novel manufacturing platforms 	<p>NA</p>
<p>Modernize the legal, regulatory, and policy framework for efficient preparedness and response by assessing current laws and regulations and proposing changes that will facilitate an efficient response to public health emergencies</p>	<ul style="list-style-type: none"> Enhance rapid deployment and pre-event planning and positioning of MCMs 	<ul style="list-style-type: none"> Strengthen program to support rapid deployment and pre-event planning and positioning of MCMs 	<p>NA</p>

FDA Medical Countermeasures Initiative