# Moving Medical Innovations Forward — New Initiatives from HHS

**U.S. Department of Health and Human Services**
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## A MESSAGE FROM SECRETARY TOMMY THOMPSON

We have entered an exciting new era in American medicine and the advancement of human health. From the moment of conception to the very end of life, we understand more about human health than anyone thought possible only a couple of generations ago. Recent advances in science and technology, such as robotics, nanotechnology, and genomics have created the potential for development of innovative medical products and procedures that can provide new hope and better quality of life for many Americans. More funds are being invested in basic biomedical science in America today than ever before.

Innovative medical technology based on these discoveries — particularly new drugs, devices, and biologics — are giving patients and their families hope for a new chance at life. During the past several decades, medical innovations and new technologies have changed the face of healthcare in the United States. From cholesterol drugs to knee replacements, from PET scans to medical lasers, our medical industry in recent years has brought an amazing number of new treatments to patients and dramatically improved their quality of life.

Notwithstanding these successes, we also have to look ahead — not only to new technologies and treatments themselves, but also to how we can foster the innovation and applied research needed to bring those technologies and treatments forward. We have to reduce the time it takes to get the latest medical breakthroughs and innovative products from the laboratory bench to the bedside.

The Department of Health and Human Services (HHS, or the Department) is an integral part of the nation's robust biomedical innovation infrastructure. From the advancement and application of basic biological and clinical research findings supported by the National Institutes of Health (NIH) to the healthcare quality and efficacy studies supported by the Agency for Healthcare Research and Quality (AHRQ), Congress has charged HHS agencies with key roles in delivering safe new therapies to Americans. Likewise, the Food and Drug Administration's (FDA) safety, efficacy, and quality standards form the gateway for new medical products to reach consumers. Administration of the Medicare Program requires the Centers for Medicare & Medicaid Services (CMS) to determine when and how much the program will pay for new medical technologies. The mission of the Centers for Disease Control and Prevention (CDC) to promote health and quality of life by preventing and controlling disease, injury, and disability helps protect people in all communities from infectious, environmental, terrorist and other human health threats.

As explained elsewhere in this report, each of these agencies is undertaking important activities to promote innovation within their statutory mandates. These agencies are working together and with others in the healthcare sector, in a variety of ways to create an environment that encourages the development of innovative technologies. NIH has begun its Roadmap initiative, which aims to transform the nation's medical research enterprise and help move new discoveries

into clinical testing. FDA has launched its Critical Path Initiative, to modernize the applied sciences on which product development depends. To help speed access to these new technologies, CMS is working on novel ways to better coordinate coverage, payment, and coding decisions for a more timely reimbursement process by working more closely with HHS partners including NIH and FDA. AHRQ has begun the second of its 3-year cooperative research programs regarding the translation of research results into medical practice (the TRIP program). And, CDC has undertaken a broad review of its organization and strategic focus through its Futures Initiative.

To facilitate initiatives underway at HHS agencies and to encourage coordination across the Department, I created the Medical Innovation Task Force in May of this year. I asked this Task Force to report back to me by the end of 2004 on the steps HHS can take to speed the development and availability of new medical technologies. Although each agency's efforts are important, I also wanted to be certain that the Department is not missing any opportunity to promote innovation though improved coordination across the Department.

I am pleased that the Task force has not only met this objective, but gone beyond it to initiate new efforts with other Federal departments that also have important roles to play in facilitating the development of innovative medical technologies.

I would like to take this opportunity to thank the members of the Task Force, CMS Administrator Mark B. McClellan, NIH Director Elias A. Zerhouni, M.D., NCI Director Andrew Von Eschenbach, AHRQ Director Carolyn Clancy, CDC Director Julie Gerberding, and Acting FDA Commissioner Lester M. Crawford, who chaired the Task Force, for their work in moving this new effort forward.

Sincerely,

Tommy Thompson
Secretary, U.S. Department of Health and Human Services

## EXECUTIVE SUMMARY

In May 2004, Secretary Tommy Thompson created the Medical Innovation Task Force and charged the Task Force with identifying steps that the Department of Health and Human Services (HHS, or Department) could take to speed the development and availability of innovative medical technologies. This report is the Task Force's response to that charge.

The Task Force has spent the last 8 months seeking input about existing barriers to the translation of new innovations into medical practice, and soliciting ideas and suggestions for improving the HHS role in the medical product discovery-to-delivery process. The Task Force held discussions with key HHS staff members and solicited comments from the public via the *Federal Register* and a public meeting on innovation at HHS headquarters. Many ideas were brought forward and discussed and a number of excellent short- and long-term suggestions were made. The ideas outlined in this report were selected because they can be implemented relatively quickly without new legislation or regulations. Four of the five selected proposals focus on activities that can be carried out at the Department level and that will improve collaboration among HHS agencies. In addition, given the crucial roles that other Federal entities play in facilitating biomedical innovation, the Task Force explored ways in which HHS could promote innovation through enhanced cooperation with those organizations.

In addition to presenting the five action items proposed for implementation, this report describes some of the various activities already underway at HHS agencies to promote biomedical innovation. The National Institutes of Health (NIH), the Food and Drug Administration (FDA), the Centers for Medicare & Medicaid Services (CMS), the Centers for Disease Control and Prevention (CDC), and the Agency for Healthcare Research and Quality (AHRQ) are each engaged within their respective statutory mandates in activities specifically designed to promote the translation of innovation into medical practice. This report provides an overview of some of their key activities related to medical technology innovation: the NIH Roadmap, the FDA's Critical Path Initiative, CMS's Council on Technology and Innovation and revisions to the national coverage decision process, AHRQ's work to translate research into medical practice (including its Translating Research Into Practice (TRIP) I and II initiatives), and CDC's Futures Initiative. The Task Force believes that the actions recommended in this Report will complement these activities.

The Task Force recommends that HHS undertake or expand the following five activities:

- Enter into new or expanded Memoranda of Understanding with Federal agencies outside of HHS that play an important role in medical technology development, to move technology more rapidly to the bedside. Agencies include the National Science Foundation (NSF), the National Institute of Standards and Technology (NIST/Department of Commerce), the Telemedicine Advanced Technology Research Center (TATRC/Department of Defense, and the National Institute of Disability and Rehabilitation Research (NIDRR/Department of Education). The Task Force also recommends working toward a close collaboration with the Department of Veterans

Affairs. Copies of the MOUs are included in a stand alone attachment accompanying this report.

- Streamline HHS Involvement in Medical Technology: HHS will create a forum to enable investigators and manufacturers to communicate with HHS agencies that influence product development. Such a forum will enable developers to discuss their products with key research, regulatory, and reimbursement agencies.
- Improved collaboration between CMS and FDA: FDA and CMS have agreed to collaborate on four selected activities to facilitate clinical access to innovative medical technologies: a) performing company-requested parallel reviews of new technologies; b) coordinating approval and coverage of Humanitarian Device Exemptions; c) sharing of Summaries of Safety and Effectiveness; and d) analyzing post-marketing surveillance data.
- Support Standardizing E-Clinical Trials: HHS will support the ongoing development of standard formats for electronic clinical trial data, enabling the use of consistent standards for clinical trials and facilitating integration of these data with electronic health records.
- Scientific Education/Training to Speed Medical Innovation: HHS will initiate a program to help educate HHS technology transfer personnel and scientists and engineers about the technology development process. Such a program will improve HHS' ability to contribute to the rapid translation of medical innovations into clinical practice.

The Task Force members look forward to implementing these recommendations and strengthening the Department's ability to facilitate innovation in biomedical technology.

# MOVING MEDICAL INNOVATIONS FORWARD — NEW INITIATIVES FROM HHS

## INTRODUCTION AND BACKGROUND

HHS has an important role to play to foster innovation and the applied research needed to bring new technologies and treatments to patients. This report reflects an 8-month effort to identify steps that the Department of Health and Human Services (HHS or the Department) should take to improve coordination and move forward the many activities already underway at HHS agencies to facilitate the development of innovative medical technologies. This report provides a brief overview of (1) the HHS agencies' current involvement in efforts to facilitate the development and use of medical technologies; (2) the outreach activities undertaken by the Medical Innovations Task Force established by Secretary Tommy Thompson to solicit ideas for better coordinating and streamlining a larger cooperative involvement that includes industry, patient groups, academia, and other stakeholders; and (3) the recommendations of the Task Force for new HHS initiatives to help move medical innovations forward.

## HHS AGENCIES' ROLES IN MEDICAL INNOVATION

HHS is the U.S. government's principal organization for promoting and protecting the health of all Americans and providing essential human services, especially for those who are least able to help themselves. The Department oversees more than 300 programs, covering a wide spectrum of health-related activities. HHS administers more grant dollars than all other Federal agencies combined.[1]

Successful medical innovations are usually the result of large and costly efforts on the part of many people and many organizations. Creative thinkers and researchers in both the public and private sectors make new discoveries in biological sciences, develop new technologies, and conceive new product ideas. Development laboratories assess the ideas, make modifications, and begin scientific evaluation of the concepts; the most promising ideas may be modified further before they reach *candidate product* status. Productive in vitro testing will move into animal testing, and candidate products that appear likely to succeed may graduate to human testing.[2] At the human testing stage, a company must also develop manufacturing methods and facilities that can consistently produce high-quality products at commercial scale.

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[1] For more information, see http://www.hhs.gov/about/whatwedo.html.

[2] Current estimates indicate that most compounds fail during development and never reach the clinical testing stage; of those that do, fewer than one in five is approved for marketing, *Regulatory Affairs Focus*, Sept. 2004, "FDA's Approach to the Pipeline Problem," Janet Woodcock.

Regulatory agencies oversee and review the initiation and conduct of the human testing phase and the company's data prior to approval at various stages to ensure that the product is safe and effective. Public and private payers evaluate the data to determine whether and, if so, how insurance programs will pay for the product once it reaches the market place. Product development can take years, even decades, and costs millions of dollars, with estimates as high as $800 million.[3] The long time frame and enormous costs of development can be an impediment to the effective use of new biomedical innovations to improve the nation's health.

There are many exciting innovative discoveries on the horizon. New medical therapies, many of which are based on these discoveries, are emerging each year that hold the promise of a cure or improved quality of life. For example, an emerging area of interest in the cardiovascular field is the development of engineered cardiovascular tissue enabling the replacement of diseased blood vessels and heart valves with viable tissue that has a structure and function similar to that of native tissue. There is even the expectation that in children these engineered cardiovascular tissues could grow as the child matures.

Another example of an exciting new technology area is in the field of optical diagnostics and therapeutics. We have already seen the rapid development of medical devices that use minimally invasive optical technologies that enable the diagnosis and treatment of diseases that once required invasive surgery. In addition, many diseases, including certain cancers, can now be diagnosed much earlier, resulting in more effective treatments. One example is a technique that uses light to detect precancers in the colon and lungs. Novel diagnostic approaches based on optical phenomena, such as coherence and fluorescence, are being studied around the world. These approaches, once translated to technologies physicians can use, are likely to have a significant impact on modern medicine.

Emerging technologies such as these will present some unique and challenging technical and regulatory issues. During preparation of this report, public comments were received (in a public docket and during a public meeting held in November) that described barriers that exist at HHS during the medical technology product lifecycle and proposed some ways to address these barriers. For example, one commenter cited "the need for funding and a more substantial governmental role in developing data on the clinical value of new technology, as well as better and more efficient regulatory processes for coverage and reimbursement decisions." (American Clinical Laboratory Association) Another suggested that HHS "facilitate collaboration between FDA, the National Institutes of Health (NIH), industry, and other stakeholders in identifying and validating useful biomarkers." (PhRMA) One company noted that a barrier to innovation resides in data availability and analysis: "data often generated and stored at multiple locations and in multiple formats." (Management Science Associates, Inc.) Several commenters called for government to use information more efficiently than is the case now: "We need to work smartly to develop data for evidence based medicine…. Manufacturers, by working with the government to develop reasonable and practical registries, can provide some of these data. Such registries should be designed to produce needed information in the least burdensome manner." (Cook Group Incorporated). Many related comments suggested improved intra-Department

[3] DiMasi JA, Hansen RW, Grabowski HG. "The price of innovation: new estimates of drug development costs," *J Health Econ*., 2003 Mar;22(2):151-85.

collaboration, for example, "Require greater data-sharing efforts among and within the agencies themselves as well as between the agencies and the broader research community." (Faster Cures) Readers can find details and additional comments in the docket.[4]

Five HHS agencies have key roles to play in the development, assessment, and integration of medical products. These agencies have been working hard to encourage the development and approval of medical technologies with the goal of making such technologies available to patients quickly and cost effectively. In some cases, the agencies have developed cooperative working arrangements with one another and with academia and industry to foster the development of innovative medical technology.

## National Institutes of Health Roadmap

The ***National Institutes of Health*** (NIH) is the Federal focal point for health research. NIH is the steward of medical and behavioral research for the Nation. Its mission is science in pursuit of fundamental knowledge about the nature and behavior of living systems and the application of that knowledge to extend healthy life and reduce the burdens of illness and disability. The research supported by the NIH results in fundamental discoveries on which many innovations are built. From cancer to heart disease, from AIDS to diabetes, laboratory and clinical research at the NIH is one of the engines driving our country's robust biomedical sector.

Beginning May 2002, the NIH convened a series of meetings to chart the ***NIH Roadmap*** for medical research in the 21st century.[5] The purpose was to identify major opportunities and gaps in biomedical research that no single institute at NIH could tackle alone and that the agency as a whole could address to make the biggest impact on the progress of medical research. The NIH is uniquely positioned to catalyze change that can more effectively transform new scientific gains into tangible health benefits. In developing the Roadmap, NIH convened meetings with more than 300 recognized leaders in academia, industry, government, and the public The Roadmap provides a framework for the priorities NIH must address to optimize its entire research portfolio, laying out a vision of a more efficient and productive system of medical research. The most compelling opportunities are grouped in three main areas: new pathways to discovery, research teams of the future, and re-engineering the clinical research enterprise.

## FDA Critical Path and Other Initiatives

The ***Food and Drug Administration*** (FDA) is charged with ensuring the safety and efficacy of pharmaceuticals, biological products, and medical devices and the safety of foods and cosmetics, — products that represent almost 25 cents out of every dollar in U.S. consumer spending. The FDA sets the scientific standards for safety and efficacy that new medical products must meet to reach consumers. The FDA also sets standards for product manufacturing quality. These

[4] See FDA Docket # 2004S-0233

[5] See more on the NIH Roadmap at http://nihroadmap.nih.gov/.

scientific standards not only protect the public, they play a key role in guiding innovators as they turn discoveries and prototypes into new products.

FDA has long recognized the importance of facilitating the development of innovative medical technology and has undertaken several initiatives in support of these efforts. In January 2003, the FDA launched a broad ***Innovative Technologies Initiative*** to help make innovative medical technologies available sooner and to reduce the costs of developing safe and effective medical products.[6] The agency undertook efforts in three major target areas to:

- Reduce delays and avoidable product development costs by ***enhancing review and improving the quality of applications*** through early communication with industry and other steps;
- Improve the efficiency of the review process by ***adopting a quality systems approach*** to medical product reviews; and
- Facilitate new product development by ***providing clearer up-to-date guidance*** for particular diseases and for emerging technologies.

On March 16, 2004, FDA launched its Critical Path Initiative with a report[7] that calls for academic researchers, product developers, and patient groups to join with FDA to help identify opportunities to modernize the scientific tools used to develop and evaluate innovative medical products. The Critical Path report identifies the problems and some potential solutions to the task of ensuring that scientific breakthroughs are developed into medical products that are demonstrated to be safe and effective for patients as quickly and efficiently as possible. The report illuminates the development path for the three main types of medical products — drugs, biological products, and medical devices — to identify the development problems and formulate steps that could "bring the critical path into the 21st Century." The Initiative focuses on the unique opportunities for FDA to collaborate with academic researchers, product developers, patient groups, and other stakeholders to make the critical path much faster, predictable, more informative, and less costly.

**CMS Council on Innovative Technology**

The ***Centers for Medicare & Medicaid Services*** (CMS) administers the Medicare and Medicaid programs, providing healthcare to about one in every four Americans. CMS evaluates Medicare coverage for new technology based on whether the item or service is reasonable and necessary for the diagnosis or treatment of illness or injury. This determination can significantly affect the use of new technologies.

[6] For more, see http://www.fda.gov/bbs/topics/NEWS/2003/NEW00867.html.

[7] See the full report at http://www.fda.gov/oc/initiatives/criticalpath/whitepaper.html.

CMS has undertaken a number of initiatives that will enhance the development of innovative technology.[8] In December 2003, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (MMA) (Pub. L. 108-173) was enacted. Although CMS was already in the process of making its national coverage determinations more transparent for the public, Section 731 of the MMA required the Secretary to make available to the public the factors that are considered in making national coverage determinations of whether an item or service is reasonable and necessary. The changes were aimed at making the process more efficient and at enhancing CMS' access to relevant information needed to make fully informed decisions.

In August 2004, CMS established the Council on Technology and Innovation to coordinate activities aimed at speeding beneficiaries' timely access to beneficial new medical technologies. The Council, which comprises CMS's senior leadership, is carrying out its specific initiatives through two principal working groups. The **Effective Innovation Working Group** is focusing on making the agency's processes more transparent and more integrated. This group is also working to improve the timeliness and efficiency of the coverage, coding, and payment processes. Specific steps to achieve these objectives include ensuring that the processes are based on the best and latest scientific knowledge, enhancing stakeholder understanding of those processes, and creating enhanced opportunities for stakeholders to communicate with the Agency.

The second group, the **Better Evidence Working Group**, is endeavoring to improve the data that are available for medical decision making. This group is developing and implementing strategies for improving the clinical evidence on which to base decisions relating to coverage, coding and payment.

### AHRQ Translating Research into Practice (TRIP)

The ***Agency for Healthcare Research and Quality*** (AHRQ) supports research on healthcare systems, healthcare quality and cost issues, access to healthcare, and effectiveness of medical treatments, and communicating the most current scientific information to the medical community and consumers. Many of the research evaluation and dissemination efforts undertaken by AHRQ lead to improvements of the healthcare system.

In September 2000, as part of its Translating Research into Practice (TRIP) initiative, AHRQ funded projects to evaluate different strategies for translating research findings into clinical practice.[9] The aim of these 3-year cooperative agreements is to identify sustainable and reproducible health improvement strategies. A total of 27 TRIP projects have been funded since 1999 as part of a major AHRQ initiative to close the gap between knowledge and practice (i.e., between what we know and what we do) to ensure continuing improvements in the quality of the nation's health care. The TRIP II projects include studies to improve pediatric outcomes through chronic care, develop an asthma management model for Head Start, create diabetes education

[8] See, for example, www.cms.hhs.gov/media/press/release.asp?Counter=1244 and 1084.

[9] For more, see www.ahrq.gov/research/trip2fac.htm.

multimedia for vulnerable populations, and improve pain management in nursing homes (see attachment for complete list of TRIP II projects). All of the projects are funded by AHRQ as cooperative demonstration projects that specifically focus on evaluating strategies for translating research into practice through the development of partnerships between researchers and healthcare systems and organizations (e.g., purchaser groups, integrated health service delivery systems, academic health systems, managed care programs including HMOs, practice networks, worksite clinics). The objective of these partnership arrangements is to help accelerate and magnify the impact of the research on clinical practice and patient outcomes in applied settings.

**CDC Futures Initiative**

The ***Centers for Disease Control and Prevention*** (CDC) works with states and other partners to provide a system of health surveillance to monitor and prevent disease outbreaks, implement disease prevention strategies, and maintain national health statistics. Innovative technologies are crucial to the success of CDC efforts.

CDC is undergoing major changes in order to enhance its health impact and to meet the public health challenges of the 21st century. The public health challenges include terrorism preparedness and response; America's aging population; obesity and its dire health consequences; globalization and emerging infectious disease; and the need to modernize our nation's public health workforce and infrastructure.[10]

In selected technology areas, such as development of tests for emerging infectious diseases, CDC plays a key role in the pathway from discovery through delivery. Collaborations with other parts of HHS will enhance their ability to move technology to the bedside.

More than a year ago, CDC created a strategic development process called the *Futures Initiative* that has included hundreds of employees, other agencies, organizations, and the public. Under the Futures Initiative, CDC is aligning its priorities and investments under two overarching health protection goals: Preparedness — people in all communities will be protected from infectious, environmental, and terrorists threats; and Health Promotion and Prevention of Disease, Injury and Disability — all people will achieve their optimal lifespan with the best possible quality of health in every stage of life.

In addition, the agency is developing more targeted goals to ensure an improved impact on health at every stage of life including infants and toddlers, children, adolescents, adults, and older adults.[11]

[10]See www.cdc.gov.

[11] For more information about the CDC Futures Initiative, see www.cdc.gov/futures.

**Interagency Initiatives**

A number of HHS interagency working groups have been established to focus on furthering the development of innovative medical technologies. The actions recommended in this report are intended to support and expand these efforts. Several key examples of the numerous interagency initiatives are described briefly here. With this report, the Department acknowledges the importance of continued close collaboration among agencies within and outside HHS.

- ***Interagency Oncology Task Force*** **(IOTF).** In May 2003, the NIH National Cancer Institute and the FDA announced an agreement to share knowledge and resources to facilitate the development of new cancer drugs and other technologies and speed their delivery to patients. The IOTF[12] has been working in the following areas:
  - Developing markers of clinical benefit (biomarkers) for evaluating new cancer medicines
  - Creating a cancer bioinformatics infrastructure to improve data collection, integration, and analysis for preclinical, pre-approval, and post-approval research across all of the sectors involved in cancer therapy development and delivery
  - Addressing joint technology development issues (e.g., proteomics, diagnostic imaging, molecular targeting)
  - Advancing the development and evaluation of chemoprevention agents
  - Conducting a systematic review of current policies to identify ways in which FDA-NCI collaborations can enhance the development and regulatory process for cancer technologies
  - Improving consumers' awareness of the consequences of their choices about diet and nutrition for cancer prevention
  - Enhancing staff capabilities through collaborative training, joint rotations, and joint appointments
- ***CMS and NIH/NCI.*** CMS and NIH/NCI are collaborating to bring new technologies and more effective treatments to cancer patients. In June 2004,[13] the two agencies executed a joint MOU to address five areas of technology, science, and patient care. The MOU calls for the:
  - Development of a joint process for identifying high-priority clinical questions about the optimal use of new cancer technologies and creation of a process for conducting post-approval studies to address these priority questions
  - Definition of a systematic process for consultations between CMS and NCI experts in the evaluation of new diagnostic and therapeutic cancer technologies for the purposes of payment and coverage decisions

[12] For more, see www.fda.gov/bbs/topics/NEW/2003/NEW00912.html.

[13] For more, see www.cms.hhs.gov/media/press/release.asp?Counter=1084.

- Development of more efficient methods of collecting clinical evidence on new cancer technologies and strategies for making this information more widely available to patients, clinicians, and researchers
- Development of a joint process for the prospective identification and evaluation of emerging technologies, such as molecular imaging, so that reimbursement policies will fully anticipate promising new cancer technologies and help expedite their adoption in the marketplace
- Identification of opportunities for sharing data and resources aimed at improving the quality of care for cancer patients and addressing additional concerns, such as cancer health disparity issues, reducing unwarranted variation in treatment patterns, and improving palliative and end of life care

These two agencies are also working together to develop better evidence to support the best possible treatment decisions for beneficiaries. NCI will help provide the data to demonstrate efficacy of off-label chemotherapies and to expand the availability of advanced imaging, such as positron emission tomography, when such services can improve management and patient quality of care.

- ***CMS, NIH National Heart, Lung, and Blood Institute (NHLBI) and AHRQ.*** The National Emphysema Treatment Trial (NETT), cosponsored by CMS, the NIH NHLBI and AHRQ**,** studied the risks and benefits of lung volume reduction surgery (LVRS), a promising, but potentially risky, treatment for emphysema that had not been studied in controlled trials with long-term follow up. Emphysema is a common lung condition that is a major cause of death and disability in the United States. These agencies undertook NETT to find out what the benefits and risks of the surgery are compared to medical therapy alone, how long the benefits last, and whether the surgery benefits some patients more than others. The results of the NETT trial helped to clarify which patients would benefit from LVRS. CMS used this information to inform its coverage decision regarding LVRS, including selection of facilities to perform LVRS based on the criteria in the NETT trial and specification that patients receive the program of diagnostic and therapeutic services before and after surgery that were provided in the NETT trial. Private insurers, such as Blue Cross Blue Shield of Massachusetts and AETNA, have also used the NETT trial to define criteria for coverage.

- ***FDA and CDC.*** FDA and CDC have been working closely to protect the blood supply from contamination from West Nile Virus. The first domestically acquired human cases of West Nile Virus infection were documented in 1999, in the New York City area. In 2002, West Nile Virus infections were identified in 44 states and the District of Columbia, involving more than 4,000 human cases. According to the CDC, the outbreak was the largest recognized arboviral meningoencephalitic epidemic in the Western Hemisphere. FDA's Center for Biologics Evaluation and Research (CBER) and the CDC recognized that blood donations from infected but asypmptomatic donors posed a threat to the blood supply. CBER and the CDC alerted physicians, the blood community, and the public to the potential threat, while emphasizing that the benefits of medically indicated transfusions still far outweighed the risks, and that West Nile Virus could not be acquired by donating blood.

As a result of this collaborative effort within 8 months of identifying the risk of West Nile Virus exposure from blood and before the start of the 2003 infection season, universal investigational blood donor screening for West Nile Virus was made available. The screening prevented the introduction of over 1,000 units of potentially infectious blood into the blood supply. More than 12 million blood donations are now screened for West Nile each year. At least 800 transfusion-associated human West Nile Virus-infections are believed to have been prevented in 2003 because of the blood screening protocols currently in place.

The cooperative effort has also provided a new model for rapidly addressing emerging infectious disease threats to blood safety. CDC's surveillance system, by enabling early identification of asymptomatic West Nile Virus infection in humans, has provided a unique new real-time contribution of blood screening to acute infectious disease surveillance and control. For its part, CBER is continuing to work with multiple partners to evaluate and implement effective West Nile Virus donor testing and is also involved in facilitating other efforts relevant to West Nile Virus, including innovative vaccine development and potential immune therapies.

- ***CDC, FDA, and CMS.*** CDC, FDA, and CMS have worked collaboratively on the OraQuick test, the first rapid HIV test to be approved by FDA. Two months after FDA licensed OraQuick in November 2002 and after determining that the test was easy to use and had little risk of an incorrect result, FDA classified the test as waived under Clinical Laboratory Improvement Amendments. As a waived test, the OraQuick test can be used at wide variety testing sites, including at community and outreach settings.

  The CDC has estimated that one-fourth of the approximately 900,000 HIV-infected people in the United States are unaware that they are infected. Because of the potential public health benefits of rapid HIV testing, CDC and CMS have worked with State and other health officials to make the test widely available and to offer technical assistance and training for its use. For instance, CDC sponsored twenty three-day training courses on rapid HIV testing in 2003 and 2004; an additional 20 courses plus four train-the-trainer courses are scheduled for 2004 and 2005. Additionally, CDC is conducting surveillance in 14 health departments to monitor rapid HIV antibody test implementation and client acceptability and has purchased and distributed 500,000 rapid test kits to state health departments and community-based organizations.

## ACTIVITIES OF THE TASK FORCE

The Task Force sought input from industry, academia, patient groups, and other stakeholders regarding the value of these types of collaborative efforts and the benefit of fostering coordination among ongoing activities of HHS agencies. First, the Task Force requested comments from the public via an open docket in the *Federal Register*, published May 24, 2004.[14] Approximately 25 comments were received from individuals, companies, and organizations regarding their experiences and insights in translation of new innovations into medical practice.

Second, the Task Force held a public meeting on November 8, 2004, to discuss critical issues.[15] At that meeting, eight formal presentations were made by representatives of pharmaceutical companies and associations. These speakers identified hurdles to innovative product development and made recommendations about what steps HHS can take to create or enhance coordination across its agencies to stimulate the development of new technologies. In addition, several members of the audience gave presentations on related issues.

Third, the Task Force analyzed the input and developed recommendations for actions that are within the purview of the Department and that can be implemented within a short- to medium-term time frame. Some of the suggestions involved actions that can be implemented immediately, while other comments, not addressed in this report, refer to long-term projects that would require additional planning and funding. Although the Task force recognizes the broader array of policy issues that can affect medical innovation, the activities of the Task Force and the recommendations in this report are responsive to the Secretary's request to identify immediate action items.

## TASK FORCE INITIATIVES

The Task Force is recommending that five initiatives be expanded or implemented beginning in 2005. Providing enhanced coordination will enhance activities already underway at HHS agencies while creating a broad departmental environment that encourages the development of innovative technologies. The activities listed here can be implemented over the short term without statutory or regulatory changes.

### *Action 1.* Memoranda of Understanding to Facilitate Cooperation

To establish a general framework for cooperation between the HHS agencies and other Federal departments, HHS has established memoranda of understanding (MOUs) with several other relevant departments in support of innovative medical technology. The cooperating departments propose to work together to expedite the development and availability of innovative technologies. The following MOUs or letters of agreement are included in this report:

- Department of Health and Human Services and the National Science Foundation (NSF)

[14] *Federal Register*, vol. 69, p. 29544.

[15] *Federal Register*, vol. 69, p. 61018.

- Department of Health and Human Services and Department of Education's, National Institute on Disability and Rehabilitation Research
- Department of Health and Human Services and the U.S. Army Medical Research and Materiel Command, Telemedicine and Advanced Technology Research Center
- Department of Health and Human Services and the Department of Commerce, National Institute of Standards and Technology

Through these MOUs, the organizations agree to work together to:

- Coordinate strategies, policies, and programs
- Streamline the pathway from discovery to delivery
- Launch educational and informational initiatives
- Create research initiatives in areas of common interest

These agreements will provide encouragement and a framework within which to develop collaborations that focus on bringing innovative technologies to the patient bedside. Copies of the MOUs are included as a stand alone document accompanying this report.

Additionally, the Department of Veterans Affairs and HHS have agreed to collaborate in similar areas.

### *Action 2:* Streamline HHS Involvement in Medical Technology Using the ICBIO Model

As a new medical technology moves through the various stages (from development, to approval, to use), each stage brings new decision-makers who often seek different types of information to make their decisions. In large part, this occurs because the statutory missions of each of the major agencies in HHS that deal with new medical technology are fundamentally different. Comments from the public docket suggest this creates confusion for sponsors and technology developers and can stall progress. Improved cooperation among the agencies and communication about the various requirements of HHS constituent agencies can assist the discovery-to-delivery trajectory.

One outstanding example of such cooperation among HHS agencies is the Interagency Council on Biomedical Imaging in Oncology (ICBIO), formed under the administrative leadership of the Division of Cancer Treatment and Diagnosis (DCTD) in the National Cancer Institute (NCI).[16] The ICBIO, created in 2000, is a multi-agency group designed to serve as a sounding board for investigators and manufacturers attempting to take emerging medical imaging technology to the marketplace. The ICBIO meetings are confidential and include staff from NIH, FDA, and CMS.

Within the next six months, the FDA's Center for Devices and Radiological Health, along with support from the NIH's NCI and National Institute of Biomedical Imaging and Bioengineering (NIBIB), will create a forum based on the ICBIO model for discussion of the use of image-guided interventions. While NCI will take part of the lead, the area for collaboration will not

[16] For details on the ICBIO see www.cancer.gov/dctd/icbio.

exclusively focus on oncology; other critical areas such as cardiovascular disease will be part of this forum. The evaluation of these technologies is not straightforward, and many technologies are being developed that involve new clinical or mathematical approaches that need to be evaluated. This new forum will complement ongoing initiatives (e.g., the IOTF) that facilitate sharing expertise, working collaboratively across disciplines, and working across agencies to bring emerging technologies from discovery to the marketplace.

In the future, the agencies will consider creating additional councils in other technology areas. Two examples suggested by the Task Force include pediatric devices and in-vitro diagnostics for oncology. In the area of pediatric devices, agencies within HHS have been meeting with each other and with key private sector organizations. AHRQ has expertise in evaluation of health outcomes and medical evidence that would be necessary and complementary addition to the current ICBIO and the future councils based on this model. The NIH Biomedical Engineering Consortium (BECON) has also recently begun to consider this topic.

### *Action 3*: NIH/FDA Initiative to Standardize E-Clinical Trials

The costs, time, and effort of conducting clinical trials create a significant barrier to the clinical testing of innovative technologies. A significant cost in these trials is the use of time-consuming paper-based documentation or the transfer of electronic records manually from one electronic system (e.g., clinical lab data) to a trial data set. Electronic data capture and management have the potential to significantly decrease the cost of medical product development while enabling real time safety analysis and early trend detection, thus improving patient safety. However ,a standardized electronic environment must be available so that users don't have to create a new electronic format each time a trial is set up, and so that electronic data will be recorded consistently across clinical research and clinical care databases.

In the past, harmonization of research data and medical records has been hampered by the existence of different approaches to standardizing these electronic records. FDA and NIH have already begun a number of important initiatives to address this critical problem. These efforts include, among others, adoption of standards developed by the Clinical Data Interchanges Standards Consortium for capturing observations made during clinical studies; adopting standards agreed to by the Consolidated Health Informatics initiative; development of electronic data exchange messages in Health Level Seven that would be interoperable with the Electronic Health Record also being developed in Health Level Seven; and reengineering the clinical research enterprise through the NIH Roadmap and other initiatives.

FDA and NIH will co-lead a new HHS-sponsored initiative,[17] which also involves the Consolidated Health Informatics initiative and the National Committee on Vital and Health Statistics. The National Institute of Standards and Technology (NIST), which plays an important role in federal information technology security, will collaborate. The goal will be to capture and manage electronically the data collected during clinical trials. To move this initiative forward, FDA and NIH will enlist other HHS agencies to participate. Other government agencies that are involved in the conduct of clinical trials and many private sector partners will also be involved, as will standard-setting bodies. This collaboration will identify the gaps in the existing standards

[17] Oversight by the Office of the National Coordinator for Health Information Technology (ONCHIT).

between current methods and more advanced electronic data systems. We expect to make rapid progress over the next 2 years to develop usable standards for this initiative.

### *Action 4:* Collaboration Between FDA and CMS

Currently, medical product development and coverage/reimbursement of most new therapies and diagnostics have been approached as activities that occur in a serial manner. After new products are developed and tested, data and information on the product are submitted for review by FDA. If the product meets the relevant approval standards, FDA will approve the product for commercial marketing. Next, the company seeks reimbursement for the product from health insurers, health plans and other third party payers, which increases its availability to patients. Once on the market, non-proprietary data collected by FDA is added to a growing body of information about the safe use of that product and is used to inform the regulatory review of future therapeutic and diagnostic medical products.

Greater collaboration between FDA and CMS can provide important opportunities to: speed patient access to new, innovative medical products; create resource and time efficiencies in moving medical products from the bench to the bedside; spur new, innovative medical product development; and better inform consumers and health care providers.

FDA and CMS have agreed to collaborate on the following four activities to facilitate patient access to innovative medical technologies.

**Parallel review.** At the request of an applicant, and with the concurrence of both agencies, FDA regulatory review and CMS review would be conducted in parallel to minimize delays between marketing approval and reimbursement.

**Humanitarian Device Exemptions.** FDA and CMS will work together to coordinate marketing approval, postmarket evaluations, and reimbursement approvals for humanitarian device exemptions(HDE).

The Safe Medical Devices Act of 1990 provided for HDEs to encourage the discovery and use of devices that benefit a relatively small number of individuals. This provision allows FDA to grant an exemption from the effectiveness requirements for devices if the device is designed to treat or diagnose a disease or condition that affects fewer than 4,000 individuals per year in the United States, there is no comparable device available, and the probable benefits to health from using the device outweigh the risks of illness or injury from its use. Coordination between FDA and CMS could be uniquely helpful in developing an expedited coverage pathway for technologies that have received HDEs from the FDA.

**Summaries of Safety and Effectiveness.** Once a medical product has been approved, FDA may draft a document, such as a *Summary of Safety and Effectiveness* (SSE), describing the evidence reviewed by the Agency to reach its decision, its analysis of the evidence, and the basis for its conclusion. After several weeks, to allow time for redaction of trade secret, confidential commercial, and other information protected by law from public disclosure, FDA makes SSEs available to the public on the Agency's website. To reduce the time between a premarket

approval decision and a postmarket coverage or payment determination for a new medical product, FDA will expedite the redaction of select SSEs for CMS, upon request, if that product is eligible for coverage/payment by CMS or its contractors.

**Postmarket Surveillance Data.** At present, FDA use of CMS data for postmarket surveillance activities is limited. Greater use of existing information and mechanisms (consistent with the Health Insurance Portability and Accountability Act privacy rule), and the collection of new data through implementation of the Part D prescription drug benefit provisions of the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, could provide FDA, CMS, industry, and, ultimately, the public, with invaluable information regarding the use of medical products. Because many products have a different risk-benefit profile in "real world" use than they do in a clinical study, such information may enable those products to be used more safely and effectively than provided for in the original label. Moreover, this data could also shed light on the safety and effectiveness of off-label use. FDA and CMS will initiate several pilot projects this year to collect safety information on approved medical products.

### *Action 5:* Interagency Cross Training Effort

Technology transfer office personnel and scientists in all HHS agencies could benefit from a better understanding of the product development process, including FDA regulatory process and its relationship to medical reimbursement through CMS. Not only could existing training efforts be more strategically targeted, but additional training efforts may be necessary, given the complexity of the subject matter and the different educational backgrounds and experiences of HHS staff.

The HHS Interagency Technology Transfer Council will support an extensive interagency cross training effort to educate technology transfer staff, managers, and scientists/engineers about the need for and impact of rapid translation of medical innovations into patient and population practice.

We believe that this plan could be implemented in a matter of months. Much of the training material is already available in individual agencies. The subjects covered will include what kinds of data FDA needs to approve new products; intellectual property issues; and how to collaborate among public and private sector entities to move innovations along the development pipeline. These curricula now used to train new employees within HHS agencies, will be used to cross-train throughout the Department.

## CONCLUSIONS

The initiatives described here are designed to complement and augment existing efforts on the part of HHS agencies that are working to improve the development and use of new medical innovations. These efforts can be implemented immediately, and will help improve coordination and efficiency both within HHS and between HHS and other Federal agencies. We recognize the enormous challenge ahead. This report and its actions recommended here represent one step along the continuum in improving the discovery to delivery cycle for innovative medical technology. We hope that such improvements placed into practice will result in improved health and quality of life for all Americans.