Appendices

Appendix A: Legal Mandates for this Report

Pub. L. No. 109-482: The National Institutes of Health Reform Act of 2006 (Relevant Provisions)

An Act

To amend title IV of the Public Health Service Act to revise and extend the authorities of the National Institutes of Health, and for other purposes.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE.

This Act may be cited as the "National Institutes of Health Reform Act of 2006".

TITLE I—NIH REFORM

SEC. 102. AUTHORITY OF DIRECTOR OF NIH.

(b) ADDITIONAL AUTHORITIES.—Section 402(b) of the Public Health Service Act, as amended by subsection (a) of this section, is amended by striking paragraphs (2) and (3) and inserting the following:

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(7)(A) shall, through the Division of Program Coordination, Planning, and Strategic Initiatives—

“ (i) identify research that represents important areas of emerging scientific opportunities, rising public health challenges, or knowledge gaps that deserve special emphasis and would benefit from conducting or supporting additional research that involves collaboration between 2 or more national research institutes or national centers, or would otherwise benefit from strategic coordination and planning;

“(ii) include information on such research in reports under section 403;

SEC. 104. REPORTS

(a) REPORT OF DIRECTOR OF NIH.—The Public Health Service Act (42 U.S.C. 201 et seq.), as amended by section 103(a) of this Act, is amended—

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(3) by striking section 403 <<NOTE: 42 USC 283.>> and inserting the following sections:

“SEC. 403. <NOTE: 42 USC 283.> BIENNIAL REPORTS OF DIRECTOR OF NIH.

“(a) IN GENERAL.—The Director of NIH shall submit to the Congress on a biennial basis a report in accordance with this section. The first report shall be submitted not later than 1 year after the date of the enactment of the National Institutes of Health Reform Act of 2006. Each such report shall include the following information:

“(1) An assessment of the state of biomedical and behavioral research.

“(2) A description of the activities conducted or supported by the agencies of the National Institutes of Health and policies respecting the programs of such agencies.

“(3) Classification and justification for the priorities established by the agencies, including a strategic plan and recommendations for future research initiatives to be carried out under section 402(b)(7) through the Division of Program Coordination, Planning, and Strategic Initiatives.

“(4) A catalog of all the research activities of the agencies, prepared in accordance with the following:

“ (A) The catalog shall, for each such activity—

“(i) identify the agency or agencies involved;

“(ii) state whether the activity was carried out directly by the agencies or was supported by the agencies and describe to what extent the agency was involved; and

“(iii) identify whether the activity was carried out through a center of excellence.

“(B) In the case of clinical research, the catalog shall, as appropriate, identify study populations by demographic variables and other variables that contribute to research on minority health and health disparities.

“(C) Research activities listed in the catalog shall include, where applicable, the following:

“(i) Epidemiological studies and longitudinal studies.

“(ii) Disease registries, information clearinghouses, and other data systems.

“(iii) Public education and information campaigns.

“(iv) Training activities, including—

“(I) National Research Service Awards and Clinical Transformation Science Awards;

“(II) graduate medical education programs, including information on the number and type of graduate degrees awarded during the period in which the programs received funding under this title;

“(III) investigator-initiated awards for postdoctoral training;

“(IV) a breakdown by demographic variables and other appropriate categories; and

“(V) an evaluation and comparison of outcomes and effectiveness of various training programs.

“(v) Clinical trials, including a breakdown of participation by study populations and demographic variables and such other information as may be necessary to demonstrate compliance with section 492B (regarding inclusion of women and minorities in clinical research).

“(vi) Translational research activities with other agencies of the Public Health Service.

“(5) A summary of the research activities throughout the agencies, which summary shall be organized by the following categories, where applicable:

“(A) Cancer.
“(B) Neurosciences.
“(C) Life stages, human development, and rehabilitation.
“(D) Organ systems.
“(E) Autoimmune diseases.
“(F) Genomics.
“(G) Molecular biology and basic science.
“(H) Technology development.
“(I) Chronic diseases, including pain and palliative care.
“(J) Infectious diseases and bioterrorism.
“(K) Minority health and health disparities.
“(L) Such additional categories as the Director determines to be appropriate.

“(6) A review of each entity receiving funding under this title in its capacity as a center of excellence (in this paragraph referred to as a `center of excellence'), including the following:

“(A) An evaluation of the performance and research outcomes of each center of excellence.
“(B) Recommendations for promoting coordination of information among the centers of excellence.
“(C) Recommendations for improving the effectiveness, efficiency, and outcomes of the centers of excellence.
“(D) If no additional centers of excellence have been funded under this title since the previous report under this section, an explanation of the reasons for not funding any additional centers.

“(b) Requirement Regarding Disease-Specific Research Activities.— In a report under subsection (a), the Director of NIH, when reporting on research activities relating to a specific disease, disorder, or other adverse health condition, shall—

“(1) present information in a standardized format;
“(2) identify the actual dollar amounts obligated for such activities; and
“(3) include a plan for research on the specific disease, disorder, or other adverse health condition, including a statement of objectives regarding the research, the means for achieving the objectives, a date by which the objectives are expected to be achieved, and justifications for revisions to the plan.

SEC. 106. ENHANCING THE CLINICAL AND TRANSLATIONAL SCIENCE AWARD.

(a) IN GENERAL.—In administering the Clinical and Translational Science Award, the Director of NIH shall establish a mechanism to preserve independent funding and infrastructure for pediatric clinical research centers by—

(b) REPORT.—As part of the biennial report under section 403 of the Public Health Service Act, the Director of NIH shall provide an evaluation and comparison of outcomes and effectiveness of training programs under subsection (a)

Public Law 110-85: The Food and Drug Administration Act of 2007 (Relevant Provisions)

An Act

To amend the Federal Food, Drug, and Cosmetic Act to revise and extend the user-fee programs for prescription drugs and for medical devices, to enhance the post market authorities of the Food and Drug Administration with respect to the safety of drugs, and for other purposes.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE.

This Act may be cited as the "Food and Drug Administration Amendments Act of 2007."

TITLE XI—OTHER PROVISIONS

Subtitle A—In General

SEC. 1104. NIH TECHNICAL AMENDMENTS.

The Public Health Service Act (42 U.S.C. 201 et seq.) is amended—

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(3) in section 403(a)(4)(C)(iv)(III), by inserting "and postdoctoral training funded through research grants" before the semicolon;

Public Law 110-204: The Newborn Screening Saves Lives Act of 2007 (Relevant Provisions)

An Act

To amend the Public Health Service Act to establish grant programs to provide for education and outreach on newborn screening and coordinated follow-up once newborn screening has been conducted, to reauthorize programs under part A of title XI of such Act, and for other purposes.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE.

This Act may be cited as the "Newborn Screening Saves Lives Act of 2007."

SECTION 7. CONTINGENCY PLANNING.

Part A of title XI of the Public Health Service Act (42 U.S.S. 300b-1 et seq.) as amended by section 6, is further amended by adding at the end the following:

"SEC. 1116. HUNTER KELLY RESEARCH PROGRAM.

(a) NEWBORN SCREENING ACTIVITIES.—

"(1) IN GENERAL.—The Secretary , in conjunction with the Director of the National Institutes of Health and taking into consideration the recommendations of the Advisory Committee, may continue carrying out, coordinating, and expanding research in newborn screening (to be known as 'Hunter Kelly Newborn Screening Research Program') including —

"(c) REPORTS.— The Director is encouraged to include information about the activities carried out under this section in the biennial report required under section 403 of the National Institutes of Health Reform Act of 2006.

Appendix B: Priorities and Plans of the Institutes and Centers and the Program Offices in the Office of the Director

This appendix provides brief descriptions of the missions of the NIH Institutes and Centers (ICs) and the program offices in the Office of the Director. Links to strategic plans (or strategic planning Web sites) are embedded in the names of the ICs and offices. The ICs are presented in the order in which they appear on the appropriation table in the Congressional Justification. The mission statements and strategic plans presented here classify and justify NIH priorities.

NIH Institutes and Centers

National Cancer Institute *(NCI)*. NCI leads a national effort to reduce the burden of cancer. The National Cancer Act of 1971 broadened the scope and responsibilities of NCI and created the National Cancer Program, which conducts and supports basic and clinical biomedical research; training; health information dissemination; and other programs with respect to the cause, diagnosis, prevention, and treatment of cancer and HIV/AIDS; rehabilitation from cancer; and the continuing care of cancer patients and their families. NCI aims for a future in which we can prevent cancer before it starts, identify cancers that do develop at the earliest stage, eliminate cancers through innovative treatment interventions, and biologically control those cancers that we cannot eliminate so they become manageable, chronic diseases.

National Heart, Lung, and Blood Institute ***(NHLBI).*** NHLBI provides leadership for a national research program in diseases of the heart, blood vessels, lung, and blood; sleep disorders; and blood resources management. The Institute plans, conducts, fosters, and supports an integrated and coordinated program of basic research, clinical investigations and trials, observational studies, and demonstration and education projects. In addition, NHLBI plans and directs research in the development and evaluation of interventions and devices related to the prevention of diseases and disorders within its purview and the treatment and rehabilitation of patients who suffer from them. Also, the NHLBI oversees management of the NIH Women's Health Initiative.

National Institute of Dental and Craniofacial Research ***(NIDCR).*** NIDCR's mission is to improve oral, dental, and craniofacial health through research, research training, and the dissemination of health information. The Institute accomplishes its mission through basic and clinical research; training and career development programs that ensure an adequate number of talented, well-prepared, and diverse investigators; coordination across all sectors of the research community; and the timely transfer of knowledge gained from research and its implications for health to the public, health professionals, researchers, and policymakers.

National Institute of Diabetes and Digestive and Kidney Diseases ***(NIDDK).*** NIDDK conducts and supports basic and applied research and provides leadership for national programs in diabetes, endocrinology, and metabolic diseases; digestive diseases and nutrition; and kidney, urologic, and hematologic diseases. Several of these diseases are among the leading causes of disability and death and all can seriously affect the quality of life of those who have them.

National Institute of Neurological Disorders and Stroke ***(NINDS).*** NINDS aims to reduce the burden of neurological diseases and disorders. To accomplish this goal, the Institute conducts and supports basic, translational, and clinical research on the normal and diseased nervous system, fosters the training of investigators in the neurosciences, and seeks to better understand, diagnose, treat, and prevent neurological disorders. The NINDS research portfolio encompasses hundreds of neurological disorders, from diseases such as stroke that affect millions of people and are among the leading causes of death and disability, to rare disorders that individually affect a few people but collectively have an enormous impact on patients and families.

National Institute of Allergy and Infectious Diseases ***(NIAID).*** NIAID's mission is to conduct and support research to understand, treat, and prevent infectious and immune-related diseases. Infectious diseases include well-known killers such as HIV/AIDS, tuberculosis, and malaria; emerging or reemerging threats such as influenza and extensively drug-resistant tuberculosis (XDR-TB); and "deliberately emerging" threats from potential agents of bioterrorism. Immune-related disorders include autoimmune diseases such as rheumatoid arthritis as well as asthma, allergies, and problems associated with transplantation.

National Institute of General Medical Sciences ***(NIGMS).*** NIGMS supports basic biomedical research that increases the understanding of life processes and lays the foundation for advances in disease diagnosis, treatment, and prevention. The Institute's programs encompass the areas of cell biology, biophysics, genetics, developmental biology, pharmacology, physiology, biological chemistry, bioinformatics, computational biology, and minority biomedical research and training.

National Institute of Child Health and Human Development ***(NICHD).*** NICHD conducts and supports research on all stages of human development, from preconception to adulthood, to better understand the health of children, adults, families, and communities. This includes research on fertility, pregnancy, growth, developmental disabilities, and medical rehabilitation.

National Eye Institute ***(NEI).*** NEI conducts and supports research that helps prevent and treat eye diseases and other disorders of vision. This research leads to sight-saving treatments, reduces visual impairment and blindness, and improves the quality of life for people of all ages. NEI-supported research has advanced our knowledge of how the eye functions in health and disease.

National Institute of Environmental Health Sciences ***(NIEHS).*** The mission of NIEHS is to reduce the burden of human illness and disability by understanding how the environment influences the development and progression of human disease.

National Institute on Aging ***(NIA).*** NIA leads a broad scientific effort to understand the nature of aging and to extend the healthy, active years of life. The Institute provides leadership in aging research, training, health information dissemination, and other programs relevant to aging and older people and serves as the primary Federal agency on Alzheimer's disease research.

National Institute of Arthritis and Musculoskeletal and Skin Diseases ***(NIAMS).*** NIAMS supports research to address the causes, treatment, and prevention of arthritis and musculoskeletal and skin diseases; the training of basic and clinical scientists to carry out this research; and the dissemination of information on research progress in these diseases.

National Institute on Deafness and Other Communication Disorders ***(NIDCD).*** NIDCD conducts and supports biomedical research and research training on normal mechanisms as well as diseases and disorders of hearing, balance, smell, taste, voice, speech, and language. In addition, NIDCD conducts and supports research and research training related to disease prevention and health promotion; addresses special biomedical and behavioral problems associated with persons who have communication impairments or disorders; and supports efforts to create devices that substitute for lost and impaired sensory and communication function.

National Institute of Mental Health ***(NIMH).*** The mission of NIMH is to transform the understanding and treatment of mental illness through basic and clinical research, paving the way for prevention, recovery, and cure. NIMH supports research and research training to fulfill the following four objectives: (1) Promote discovery in the brain and behavioral sciences to fuel research on the causes of mental disorders; (2) Chart mental illness trajectories to determine when, where, and how to intervene; (3) Develop new and better interventions that incorporate the diverse needs and circumstances of people with mental illness; and (4) Strengthen the public health impact of NIMH-supported research.

National Institute on Drug Abuse ***(NIDA).*** NIDA's mission is to lead the Nation in bringing the power of science to bear on drug abuse and addiction. This charge has two critical components. The first is the strategic support and conduct of research across a broad range of disciplines. The second is ensuring the rapid and effective dissemination and use of the results of that research to significantly improve prevention and treatment, and to inform policy as it relates to drug abuse and addiction.

National Institute on Alcohol Abuse and Alcoholism ***(NIAAA).*** NIAAA supports and conducts research focused on improving the treatment and prevention of alcoholism and alcohol-related problems to reduce the enormous health, social, and economic consequences of this disease. NIAAA conducts and supports research in a wide range of scientific areas including genetics, neuroscience, epidemiology, health risks and benefits of alcohol consumption, prevention, and treatment; coordinates and collaborates with international, national, State, and local institutions, organizations, agencies, and programs engaged in alcohol-related work; and communicates research findings to health care providers, researchers, policymakers, and the public.

National Institute of Nursing Research ***(NINR).*** NINR supports clinical and basic research to build the scientific foundation for clinical practice, prevent disease and disability, manage and eliminate symptoms caused by illness, enhance end-of-life and palliative care, and develop the next generation of scientists. The Institute’s scientific focus spans multiple disciplines and unites the biological and behavioral sciences to better understand the complex interactions between the physiological factors of health and disease and an individual’s knowledge, beliefs, and behavior.

National Human Genome Research Institute ***(NHGRI).*** NHGRI's mission has expanded since the initiation of the International Human Genome Project to encompass a broad range of studies aimed at understanding the structure and function of the human genome and its role in health and disease. A critical part of the NHGRI mission continues to be the study of the ethical, legal, and social implications of

genome research. NHGRI also supports the training of investigators and the dissemination of genome-related information to the public and health professionals.

National Institute of Biomedical Imaging and Bioengineering ***(NIBIB).*** NIBIB's mission is to improve health by leading the development and accelerating the application of biomedical technologies. The Institute is committed to integrating the physical and engineering sciences with the life sciences to advance research and medical care.

National Center for Research Resources ***(NCRR).*** NCRR provides laboratory scientists and clinical researchers with the environments and tools needed to make biomedical discoveries, translate these findings to animal-based studies, and then apply them to patient-oriented research. NCRR connects researchers with one another and with patients and communities across the Nation. These connections bring together innovative research teams and the power of shared resources, multiplying the opportunities to improve human health. Together, NCRR's four integrated and complementary divisions biomedical technology, clinical and translational research, comparative medicine, and research infrastructure accelerate and enhance research along the entire continuum of biomedical science.

National Center for Complementary and Alternative Medicine ***(NCCAM).*** NCCAM is dedicated to exploring complementary and alternative healing practices in the context of rigorous science; training complementary and alternative medicine researchers; and disseminating authoritative information to the public and professionals. To fulfill its mission, NCCAM supports a broad-based portfolio of research, research training, and educational grants and contracts, as well as various outreach mechanisms to disseminate information.

National Center on Minority Health and Health Disparities ***(NCMHD).***[1] NCMHD promotes minority health and leads, coordinates, supports, and assesses NIH efforts to reduce and ultimately eliminate health disparities. In this effort, NCMHD supports and partners with other ICs to support basic, clinical, social, and behavioral research; promote research infrastructure and training; foster emerging programs; disseminate health information; and reach out to minority and other communities that suffer from disparities in health.

John E. Fogarty International Center ***(FIC).*** FIC strengthens human and institutional capacity to confront complex global health challenges through innovative and collaborative research and training programs. It builds the knowledge and skills of developing country foreign scientists, identifies crucial gaps in global health research, and supports and advances the NIH mission through international partnerships.

National Library of Medicine ***(NLM).*** NLM is the world's largest research library of the health sciences, serving scientists, health professionals, and the public by collecting, organizing, and providing access to biomedical information. NLM also carries out programs designed to strengthen existing and develop new medical library services in the United States. It conducts research in health communications, supports medical informatics, and provides information services and sophisticated tools in the areas of molecular biology and toxicology/environmental health. NLM creates Web-based services for the general public containing information from NIH and other reliable sources. (Also see "The National Library of Medicine" in the section on "Capitalizing on Discovery," in Chapter 1.)

NIH Clinical Center. The Clinical Center is the NIH facility that provides the patient care, medical services, and environment necessary for NIH scientists to conduct clinical research. Clinical and laboratory research is conducted shoulder-to-shoulder at the Clinical Center and this tandem approach drives all aspects of its operations. (Also see "NIH Clinical Center" in the section on "Extramural and Intramural Research Programs" in Chapter 1)

Center for Information Technology ***(CIT).*** CIT incorporates the power of modern computers into NIH's biomedical and behavioral research programs and administrative procedures by focusing on three primary activities: conducting computational biosciences research, developing computer systems, and providing computer facilities. (Also see "Information and Information Technology" in the section on

"Providing the Platform for Discovery" in Chapter 1.)

Center for Scientific Review ***(CSR).*** CSR carries out peer review of the majority of research and fellowship applications submitted to NIH; serves as the central receipt point for all such Public Health Service applications; makes referrals to scientific review groups for scientific and technical merit review of applications and to funding components for potential award; and develops and implements innovative, flexible ways to conduct referral and review for all grant applications. (Also see "NIH Peer Review Process" under the section on "Extramural and Intramural Research Programs" in Chapter 1.)

Office of the Director

Division of Program Coordination, Planning and Strategic Initiatives (DPCPSI). DPCPSI was established by mandate of the NIH Reform Act of 2006. DPCPSI's role is to identify emerging scientific opportunities, rising public health challenges, and scientific knowledge gaps that merit further research; assist NIH in effectively addressing identified areas; and develop and apply resources (databases, analytic tools, and methodologies) that will support priority setting and analyses of the NIH portfolio. DPCPSI now incorporates the functions of the former Office of Portfolio Analysis and Strategic Initiatives. The primary components within DPCPSI are the Office of Strategic Coordination, which manages the NIH Common Fund (including the Roadmap), and the four OD program offices. DPCPSI also is the locus for NIH planning and reporting required by the Government Performance and Results Act and other government-wide performance assessment endeavors. (Also see the section on *NIH Strategic Planning and the NIH Roadmap and Common Fund* in Chapter 1).

As detailed below, the four OD Program Offices are in the areas of disease prevention; behavioral and social sciences research; women's health; and AIDS research.

- Office of Disease Prevention (ODP). ODP fosters, coordinates, and assesses research related to disease prevention and health promotion, and disseminates related information that aims to improve the health of the U.S. population. ODP advises the NIH Director and collaborates with other Federal agencies, academic institutions, the private sector, nongovernmental organizations, and international organizations in the formulation and implementation of research initiatives and policies that promote public health. There are three additional offices within ODP: Office of Rare Diseases Research (ORDR), Office of Dietary Supplements (ODS), and Office of Medical Applications of Research (OMAR):
  - ORDR stimulates, coordinates, and supports research on rare diseases to advance research opportunities and to respond to the needs of approximately 25 to 30 million people who have one of the approximately 6,500 known rare diseases. (Also see the section on the Rare Diseases Clinical Research Network in Chapter 4, which addresses NIH Centers of Excellence.)
  - ODS promotes and supports, through collaboration with the ICs, basic and clinical research to increase understanding of the impact of dietary supplements (e.g., plant extracts, enzymes, vitamins, minerals, amino acids, hormonal extracts) on disease prevention and health maintenance. The mission is to strengthen knowledge and understanding of dietary supplements by evaluating scientific information, stimulating and supporting research, disseminating research results, and educating the public.
  - OMAR is the focal point for evidence-based assessments of medical practice and state-of-the-science conferences key mechanisms for assessing, translating, and disseminating the results of biomedical research to improve the delivery of health services to the public. The office also conducts an annual course to train journalists on how to critically evaluate and report on medical research.
- Office of Behavioral and Social Sciences Research (OBSSR). OBSSR coordinates and stimulates behavioral and social sciences research throughout the NIH and integrates it more fully into the NIH research enterprise. The Office provides leadership on matters relating to research on the roles of human behavior and the social environment in the development of health, prevention of disease, and therapeutic intervention, as well as in training, continuing education, and dissemination of research

findings to the broader scientific community and the general public.

- Office of Research on Women's Health (ORWH). ORWH serves as the focal point for women's health research at NIH, and promotes, enhances, and expands efforts to improve the health of women through biomedical and behavioral research, including that on sex and gender factors. ORWH ensures compliance with policies on the inclusion of women and minorities in clinical research, and develops and implements NIH programs for the recruitment, retention, reentry, and advancement of women in biomedical careers.
- Office of AIDS Research (OAR). OAR is responsible for the scientific, budgetary, legislative, and policy elements of the NIH AIDS research program. Through its unique and comprehensive trans-NIH planning, budgeting, and portfolio assessment processes, OAR sets trans-NIH scientific priorities, enhances collaboration, and ensures that research dollars are invested in the highest priority areas of scientific opportunity that will lead to new tools in the global fight against AIDS. OAR also supports a number of initiatives to enhance dissemination of research findings to researchers, physicians, institutions, communities, constituency groups, and patients.

[1] With enactment of the Patient Protection and Affordable Care Act, on March 23, 2010, the National Center for Minority Health and Health Disparities became an institute–the National Institute for Minority Health and Health Disparities (NIMHD).

Appendix C: Common Fund Strategic Planning Report, 2009

Common Fund Strategic Planning Report, 2009

National Institutes of Health
Department of Health and Human Services

June 2009

Common Fund Strategic Planning Report, 2009

The National Institutes of Health Reform Act of 2006 requires the Secretary of Health and Human Services (HHS), acting through the Director of the National Institutes of Health (NIH), to submit a report to Congress containing a strategic plan for funding research that, "...represents important areas of emerging scientific opportunities, rising public health challenges, or knowledge gaps that deserve special emphasis and would benefit from conducting or supporting additional research that involves collaboration between 2 or more national research institutes or national centers, or would otherwise benefit from strategic coordination and planning" (42 U.S.C. §282(b)(7)(a)).

To date, the Common Fund has been used to support research initiatives under the NIH Roadmap for Medical Research. The NIH Roadmap is an innovative approach to accelerate fundamental discovery and translation of that knowledge into effective prevention strategies and new treatments. The strategic initiatives funded under the NIH Roadmap address critical roadblocks and knowledge gaps that currently constrain rapid progress in biomedical research. They synergize the work of many NIH Institutes and Centers (ICs), and collectively represent a unique effort that no single or group of ICs or other entity can do, but are the responsibility of the NIH as a whole.

Initiatives under the Roadmap programs are intended to be catalytic in nature and are not expected to receive long-term Common Fund support. The intent with Roadmap programs is to stimulate the development of tools or technologies, acquire fundamental knowledge and data sets, or build critical research resources. The continued use of the tools, data, and resources is to be funded through the ICs.

Although the Roadmap programs are currently the only programs funded by the Common Fund, this may not always be the case as new scientific opportunities emerge and the NIH determines how best to respond to new challenges. As the Common Fund grows, the NIH will maintain a continuous effort to be responsive to community needs while providing ongoing support for areas identified through strategic planning endeavors.

This report describes:

- The strategic planning processes undertaken to date to identify program areas currently supported by the Common Fund
- The current status of programs designed to meet the needs articulated through strategic planning
- The plans for future strategic planning efforts

I. Strategic Planning for the Common Fund, 2002-2008: the NIH Roadmap

As described in the Common Fund Strategic Planning Report of 2007, the NIH Roadmap is a series of cross-cutting programs designed to meet criteria established by the NIH Leadership before the Common Fund was established through the 2006

Reform Act. Since these programs were designed to address roadblocks to research, the chief criterion for Roadmap initiatives was that they should be expected to transform the way a broad spectrum of health research is conducted by overcoming specific hurdles or filling defined knowledge gaps. Specific diseases are not targeted through these programs; programs are expected to be relevant to several diseases.

The strategic planning process that was undertaken to identify these hurdles and knowledge gaps had four components:

- Brainstorming with expert panels
- Seeking input from the wider community of NIH stakeholders
- Analyzing the NIH portfolio and research conducted elsewhere to avoid redundancy
- Discussing priorities with the NIH Leadership based on criteria stated in Appendix 1

Ideas for new Roadmap programs have been generated through this process 3 times: in 2002, 2006, and 2008. In each case, roadblocks to research were articulated and programs were developed to overcome these hurdles. The following section describes the problems that have been defined, the goals of the programs that were developed as a response, and the current status of each.

II. Roadblocks Addressed Through the NIH Roadmap Programs and Current Status

The challenges addressed by the NIH Roadmap Programs are shown in Table 1. The budget for each program is provided in Table 2.

The first Roadmap programs were funded in September 2004. Because the immediate goals of most of the Roadmap programs involve development of novel technologies, generation of large datasets, or support of research, the impact of these programs will not likely be appreciated for some time. The real impact of these Roadmap programs will be seen in IC-funded research that uses the tools, technologies, and infrastructure built through the Roadmap.

The continuation of the earliest programs beyond the initial five years followed reviews by external expert panels that provided input on the continued need for the programs, their initial successes, and the continuing priority for the program as a Common Fund/Roadmap program. Each panel also provided input to the NIH about how the programs should be adapted in future years to meet changing needs in the community. The panels also advised the NIH on how the scientific community at large could be made more aware of the opportunities afforded them by the new technologies, tools, and data generated by the programs.

In the paragraphs below, the status of each program is summarized, as are future plans in each area.

Table 1: Roadblocks Addressed by NIH Roadmap Programs

Roadblock:	Program:
1. Clinical and Translational Research Lags Behind Basic Discoveries • Infrastructural support does not integrate necessary components for clinical and translational research. • Numbers of clinical investigators are inadequate. • Basic scientists lack the expertise necessary to move their discoveries beyond the bench. • Improved measures of clinical outcomes are needed. • Interagency regulatory hurdles are overwhelming.	• Clinical and Translational Science Awards (CTSA) • Clinical Research Training Program (CRTP), CTSAs • Rapid Access to Intervention Development (RAID) and CTSAs • Patient Reported Outcomes Measurement Information System (PROMIS) • Clinical Research Policy Analysis and Coordination (CRPAC)
2. Partnerships between NIH and private sector entities can be difficult to establish and cultivate.	Public-Private Partnerships
3. Traditional RO1 application and review process can hamper innovation.	High Risk High Reward
4. Interdisciplinary approaches to complex scientific problems can be difficult to develop.	Interdisciplinary Research
5. Small molecular compounds are needed to explore functions of human genes and to serve as leads for therapeutic compounds that can modify activity.	Molecular Libraries and Imaging
6. A scientific gulf exists between basic nanotechnology research and clinical applications.	Nanomedicine
7. Limited technologies to analyze protein-protein interactions and cellular pathways hinder therapeutic applications.	Building Blocks, Biological Pathways, and Networks
8. Limited technologies for structural analysis of membrane proteins can limit drug development.	Structural Biology of Membrane Proteins
9. Computational tools that allow investigators to mine large datasets need to be developed and combined into an integrated network.	National Computational Biology Centers
10. Knowledge of the contribution of nonpathogenic microbes to human health is rudimentary.	Human Microbiome Project
11. Contributions of higher order DNA structure to human health and disease are poorly understood.	Epigenomics
12. Genome-Wide Association Studies reveal genetic variations that associate with disease, but the molecular effects of the variations are difficult to unravel.	Genotype/Tissue Expression Resource

Table 2: Common Fund/Roadmap Budget Data

Dollars in Millions	FY 2006 Actual B.A.	FY 2007 Actual B.A.	FY 2008 Actual B.A.	FY 2009 Enacted	2010 Request
Institute or Center Roadmap/ Common Fund Contribution	$247.3	$0.0	$0.0	$0.0	$0.0
OD Roadmap/Common Fund Contribution	$85.3	$483.0	$498.2	$541.1	$549.0
Roadmap/Common Fund	**$332.6**	**$483.0**	**$498.2**	**$541.1**	**$549.0**
Roadmap/Common Fund Percent of NIH Labor/HHS Budget Authority[1]	1.2%	1.7%	1.7%	1.8%	1.8%

[1] Adjusted for Type I Diabetes, Global Fund for AIDS, Superfund, and Secretary's transfer authority for NLM.

The status of each of the programs is described in further detail below.

1. Roadblock: Clinical and translational research lags behind basic discoveries.

Programs designed to overcome this problem: Clinical and Translational Science Awards (CTSAs), Clinical Research Training Program (CRTP), Medical Scientist Training Program (MSTP), Rapid Access to Intervention Development (RAID), Patient-Reported Outcomes Measurement Information System (PROMIS), and Clinical Research Policy Analysis and Coordination (CRPAC).

A. Clinical and Translational Science Award Program (CTSAs)

Status: This program supports a national consortium that provides a foundation for clinical and translational science that will catalyze clinical and translational research and allow investigators to move more quickly toward improvements in health. The program is jointly supported by the Common Fund and the National Center for Research Resources. The CTSA program affords the provision of research services and facilities, development of information systems that link clinical research centers nationwide, expansion of the national clinical research enterprise to include community clinics, and training a new generation of clinical investigators. This program, begun in 2006, is transitioning out of the Roadmap to be supported solely by NCRR in 2015.

B. Clinical Research Training Program (CRTP)

Status: As part of the Roadmap's effort to bolster the pipeline of clinical investigators, NIH's CRTP immerses medical students in an intense 12-month research experience during which they acquire the skills necessary to become successful, independent investigators and clinicians. The training environment of

the NIH campus fosters multidisciplinary approaches and provides access to unique patient populations via the largest hospital dedicated to clinical research in the world. This program, begun in FY 2004, is transitioning to full support by the NIH Clinical Center in FY 2014.

C. Rapid Access to Intervention Development (RAID)/Translational Research Core Services

Status: This program makes available, on a competitive basis, certain critical resources that are needed 1) for the development of therapeutic agents and 2) to bridge the gap between discovery and clinical testing to enable more efficient translation of promising discoveries. The RAID program is designed to reduce some of the common barriers that block progress of therapeutic discoveries, especially in cases where efforts involve high risk ideas or therapies for uncommon disorders that cannot attract private sector investment. Where private sector capacity for drug development is limited or not available, the NIH provides the resources needed to facilitate development of promising new therapies for widespread clinical use. By providing investigators with access to drug development resources, as well as expertise in the planning and submission of documents to the Food and Drug Administration (FDA), the RAID Program plays an integral role in fostering the development of novel therapeutics. This program is expected to be continued by IC funds when it transitions out of the Common Fund in FY 2014.

D. Patient-Reported Outcomes Measurement Information System (PROMIS)

Status: PROMIS is a revolutionary effort to enhance the measurement of patient-reported symptoms and functions. In the first phase of the program (FY 2004-2008), PROMIS developed and tested a large survey for measuring patient-reported outcomes and created a computerized adaptive testing system that analyzes all the responses and cross checks them against each other to gain a better understanding of the patient's well being. By analyzing the answers to multiple questions, the computerized adaptive testing system arrives at a more robust, quantifiable measurement of the patient's condition. The PROMIS Program has also created a publicly available, continually updated, Web-based system that allows clinical researchers to access PROMIS-validated items, domains, computerized adaptive testing, and survey forms. Preliminary results demonstrate that brief, 4- to10-question surveys of symptoms and functional states administered by the computerized adaptive testing outperforms today's commonly used, paper-based, self-reporting assessment tools in common health conditions. These results are indicative of the anticipated clinical research advantage of the PROMIS tool, which yields better answers with fewer patients. This program will be supported by the Common Fund through FY 2012, after which it is expected to be supported largely through public-private partnerships in support of clinical studies.

E. Clinical Research Policy, Analysis, and Coordination (CRPAC)

Status: This program was established to help catalyze the harmonization of clinical research policies across U.S. Government agencies. CRPAC engages relevant Federal agencies as well as private sector stakeholders to coordinate, streamline, and optimize policies and requirements for the conduct and oversight of clinical research. The multiple and often inconsistent Federal requirements governing biomedical research present a considerable challenge to the biomedical research community. To address this problem, CRPAC has led a major effort to enhance the consistency of regulatory requirements, facilitate compliance, and optimize the analysis and use of adverse event data. CRPAC has developed a Basal Adverse Event Report (BAER) tool, a single baseline set of medical information for reporting adverse events and unanticipated problems in clinical research that is acceptable to multiple Federal agencies. The BAER includes both pre- and postmarket reporting and complies with national and international standards for data transmission and vocabularies. This program, established through the Office of Science Policy (OSP), NIH Office of the Director (OD), is transitioning out of the Roadmap to become a permanent activity within OSP in FY 2010.

2. Roadblock: Partnerships between NIH and private sector entities can be difficult to cultivate and maintain.

Program designed to overcome this problem: Public-Private Partnerships (PPP)

Status: This program was developed as part of NIH's efforts to facilitate new ways of conducting and supporting research, including the formation of collaborations with pharmaceutical and biotechnology industries, as well as other private entities. The program identifies appropriate partners inside and outside the NIH, as well as develops useful policies to oversee those partnerships. As part of this program, the NIH has established the Biomarkers Consortium, a complex group of related partnerships between the NIH, FDA, industry, and private entities that work to accelerate the development of new drugs by identifying, developing, and qualifying biomarkers, useful indicators of disease progression and effects of therapeutic interventions. This program, established through the OSP in the NIH OD, is transitioning out of the Roadmap to become a permanent activity within OSP in FY 2010.

3. Roadblock: Traditional R01 application and review processes can hamper innovation.

Programs designed to overcome this problem: High Risk High Reward Programs, including the NIH Director's Pioneer Program, NIH Director's New Innovator Program, and Transformative R01 Program.

Status: The High Risk High Reward component of the NIH Roadmap has been built with the intent of finding new ways to foster innovation by piloting new application and review processes. The Common Fund, through these programs, sets aside a small

percentage of the overall NIH budget for transformative research without designating specific funding levels for specific scientific areas. All areas compete, with the most innovative proposals receiving the funds.

A. NIH Director's Pioneer Awards

Status: This program seeks to identify individual investigators with a proven history of innovation and provide them with adequate funding to conduct pioneering research in a new area of investigation. It provides funding for scientists who propose innovative approaches that have the potential to produce an unusually high impact on a broad area of biomedical or behavioral research. The awardees propose to use pioneering and transformative approaches to address major scientific problems and challenge existing paradigms. Since 2004, the program has supported 63 individual investigators. Information about this program, as well as links to awardees by year, can be found at http://nihroadmap.nih.gov/pioneer/. This program has proven to be successful at the identification of outstanding scientists and innovative projects and receives funds from both the Common Fund and various ICs. The NIH Director therefore decided to continue Common Fund support for the program for the foreseeable future.

B. NIH Director's New Innovator Awards

Status: This program supports new investigators who propose research ideas that are unusually creative and highly innovative but lack the preliminary data required to apply for an RO1 grant. Since 2007, this program has supported 61 individual investigators. Information about this program, as well as links to awardees by year, can be found at http://nihroadmap.nih.gov/newinnovator/. Launched in response to Congressional language, this program, like the NIH Director's Pioneer Awards Program, is projected to continue with combined funding from the ICs and the Common Fund for the foreseeable future.

C. Transformative R01s

Status: A new program for 2009, this program is intended to allow investigators to articulate pressing needs or areas of opportunity and to fund the most transformative of these projects. Whereas the Pioneer Awards Program emphasizes the past history of the individual investigator, the Transformative R01 Program emphasizes the potential impact that each project may have. It encourages the formation of teams to accomplish the goals of the program and provides adequate funds to support complex projects. This program will pilot a new way of encouraging very high impact research by removing as many administrative barriers as possible. No budget cap is imposed, allowing maximum flexibility to investigators to develop complex approaches that may be beyond the budget of traditional R01s. Award decisions are made by the NIH Director based upon recommendations from a multidisciplinary group of outside experts. Areas of

highlighted need have been identified through the Roadmap Strategic Planning Process, but these awards are open to all areas of investigation and no set-aside dollar figure has been established for any particular topic. If the areas of highlighted need are not adequately addressed through this program, future initiatives on these topics may be developed.

These areas include:

a) Understanding and Facilitating Human Behavior Change
b) Complex 3-Dimensional Tissue Models
c) Formulation of Novel Protein Capture Reagents
d) Providing an Evidence Base for Pharmacogenomics
e) Functional Variation in Mitochondria in Human Disease
f) Transitions from Acute to Chronic Pain

4. Roadblock: Interdisciplinary approaches to complex scientific problems can be difficult to develop.

Program designed to overcome this problem: Interdisciplinary Research (IR) Program

Status: This program overcomes barriers to interdisciplinary research by building research teams, training scientists in multiple disciplines, and changing academic research culture. The program includes initiatives to dissolve academic department boundaries within academic institutions and increase cooperation between institutions, train scientists to cultivate interdisciplinary efforts, and build bridges between the biological sciences and the behavioral and social sciences.

A total of nine IR consortia, managed by teams of NIH staff from multiple ICs, have been funded through this initiative, which represents a new funding mechanism for interdisciplinary research. Through this mechanism, the NIH is piloting a new way to fund projects that cross IC missions and require cooperation among NIH staff to manage the programs. These consortia address complex problems that require novel, interdisciplinary approaches, including aging, fertility in women who undergo cancer therapy, regenerative medicine, Fragile X Syndrome, neuropsychiatric disorders, obesity, genetic engineering strategies, stress and its effects on self control and addiction, and genomics-based drug discovery. Funded in FY 2007, these consortia will be funded by the Common Fund through FY 2011. If the new funding mechanism proves worthwhile, it may continue to be utilized through IC funds for either new projects or for continuation of the existing Common Fund-initiated projects.

As part of the IR Program's efforts, the Interdisciplinary Health Research Training Program enables institutions to develop postdoctoral training programs that provide formal coursework and research training in a new interdisciplinary field to individuals holding advanced degrees in different disciplines. Another IR program, entitled Training for a New Interdisciplinary Workforce, supports scientists at the undergraduate,

graduate, and postdoctoral levels by exposing them to both didactic and research experiences involving interdisciplinary and team approaches to address complex biomedical problems. These training programs, launched in FY 2004, will compete for IC funds with IC-specific training programs beginning in FY 2009.

In an effort to help bridge the gap between medical researchers and behavioral or social scientists, the IR Program also provides exploratory/developmental grants through the Methodological and Technological Innovation in the Behavioral and Social Sciences Program to help facilitate the introduction of new methodologies and technologies to the behavioral and social sciences. These projects, funded in FY 2007, followed workshops held to foster team building in FY 2004 and FY 2006.

Finally, the IR Program encourages changes to administrative practices at NIH in ways that encourage teamwork through recognition and support of team leadership. Working with the NIH Office of Extramural Research, members of the trans-NIH IR Working Group helped design and implement the policy through which NIH now recognizes multiple principal investigators on individual projects. The recognition of multiple principal investigators represents a transformative step through which NIH seeks to foster collaboration and teamwork.

5. Roadblock: Small molecular compounds are needed to explore functions of human genes and to serve as leads for therapeutic compounds that can modify activity.

Program designed to overcome this problem: Molecular Libraries and Imaging Program

Status: This program establishes a national network of centers and supporting technologies for the discovery and development of small molecule probes to interrogate and modify biological pathways. The program currently supports a network of research centers that have identified new "probes"–molecules that are useful for research purposes and could be adapted for therapeutic use. Experts at the centers optimize and perform assays designed by academic researchers and peer-reviewed by the NIH. The centers use advanced technology to screen thousands of small molecules for their ability to bind to or inhibit a protein or protein-mediated activity of interest. In collaboration with the academic scientists who designed the assays, the center validates the "hits" and chooses a subset to improve by chemical modification. To date, the program has assembled a variety of screening assays designed to test small molecules for their ability to target proteins in critical cellular processes such as cellular transport, enzymatic reactions, and protein-protein interactions that become anomalous in multiple diseases. The screening center program moved from its pilot phase to its production phase in FY 2008 and will be funded by the Common Fund through FY 2013, with cofunding from the ICs beginning in FY 2012. It is expected to transition exclusively to IC support beginning in FY 2014.

To support this large scale screening program, several support programs were developed as part of the pilot phase of the program and are continuing during the

production phase. An assay development program, which funds investigators to develop high throughput screening assays for their biological area of interest, has been critical for enabling investigators to take advantage of the resources offered by the screening center. A technology development program enabled improvements to be made to the technical aspects of the high throughput screening process. In addition, an informatics component has been critical for the centralized collection of information about the molecules screened, their structures, and their activities in various assays; for allowing public access to this information; and for development of new informatics methodologies to mine the data. Finally, the library of compounds that the program has developed represents a truly unique and valuable component of the program as a whole. This collection is expected to grow from 300,000 to 500,000 compounds over the next 5 years. These components, which support the screening endeavor, are anticipated to transition to IC funding in FY 2014.

In addition to the small molecule screening effort, this program supports initiatives that are intended to develop novel imaging probes–in part, through adaptation of molecules that could be identified as probes through the screening centers. These initiatives have developed a database of imaging reagents and have supported the development of novel imaging reagents. Common Fund support for the database continues through FY 2013, while future funding for the imaging probe synthesis facility will be determined later this year.

6. Roadblock: A scientific gulf exists between basic nanotechnology research and clinical applications.

Program designed to overcome this problem: Nanomedicine

Status: This program establishes a network of Nanomedicine Centers to determine how cellular machines operate at the nanoscale level and use these design principles to develop and engineer new technologies and devices for repairing tissue, as well as preventing and curing diseases. Launched in FY 2005, the program's first five years were intended to address fundamental basic science questions as a prerequisite to the development of therapeutic strategies. To achieve the targeted goals of this program, the NIH uses flexible authority (Section 214 of the Appropriations Law) to oversee and manage the research. This authority facilitates the movement of funds to the most successful projects within the program. Approved for its second five-year funding period this past year, the program is now planned to continue with Common Fund support through FY 2014. This is a high risk program that expects the goals, if accomplishable, can be achieved within an overall 10-year timeframe of the program.

7. Roadblock: Limited technologies to analyze protein-protein interactions and cellular pathways hinder therapeutic applications.

Program designed to overcome this problem: Building Blocks, Biological Pathways, and Networks

Status: This program consists of two initiatives that are intended to catalyze basic studies of cellular functions by developing tools that will allow basic scientists to study protein-protein interactions and to analyze the consequences of cellular activities through examination of cellular metabolites.

The Technology Centers for Networks and Pathways develop and apply technologies to detect transient protein-protein interactions that control the cellular functions. Five centers were established in FY 2005 to develop innovative tools to enable researchers to determine, in real time, the amounts, locations, and interactions of large numbers of individual proteins within a single cell. These fundamental needs are still pressing and unsolved, so these centers will receive additional support through the Common Fund through FY 2013.

The Metabolomics initiative was established in FY 2004 to support the development of technologies that will allow investigators to monitor cellular processes more accurately through analysis of by-products (metabolites) generated by the processes. This program was developed as a 5-year program that has been jointly funded by the Common Fund and the ICs. FY 2008 was the last year of Common Fund support for this initiative, as IC-funded investigators can now use the technologies developed, and further technical advances are being funded through the ICs.

8. Roadblock: Limited technologies for structural analysis of membrane proteins can limit drug development.

Program designed to overcome this problem: Structural Biology of Membrane Proteins

Status: This program establishes centers for Innovation in Membrane Protein Production as well as individual research projects that aim 1) to formulate new methods for producing ample quantities of cellular membrane proteins that are of a quality suitable for structural and functional studies and 2) to develop and improve technologies and methods for structural analysis. The program develops novel approaches for the production and stabilization of membrane proteins to enable determination of their structures at high resolution. These approaches are paying off, as increasing numbers of membrane-associated protein structures are being determined and facilitating drug development. The success of the protein and continued need for technology development in this area prompted the decision to fund this program for an additional 5 years through the Common Fund; funding is now expected to continue through FY 2013. After that point, the community at large is expected to use the new technologies to analyze membrane proteins and use this knowledge to design novel therapies.

9. Roadblock: Computational tools that allow investigators to mine large datasets need to be developed and combined into an integrated network.

Program designed to overcome this problem: National Centers for Biomedical Computing (NCBCs)

Status: This program was established in 2004 to develop computational tools intended to catalyze research in the basic and clinical sciences. The centers create innovative software programs and other tools that arm the biomedical community with the methods needed to integrate, analyze, model, simulate, and share data relevant to human health and disease. Each center also works with members of the research community to develop informatics needs targeted toward specific disease areas. These "driving biological problems" include Huntington's Disease, Hypertension, Cardiovascular Disease, Alzheimer's Disease, Diabetes, Schizophrenia and Bipolar Disorder, HIV, Prostate Cancer, and heritable disorders. The set of disease areas targeted by these efforts is dynamic and responsive to needs of the community. The need for informatics is so broad and cross-cutting that this program is continuing with Common Fund support through FY 2014.

10. Roadblock: Knowledge of the contribution of nonpathogenic microbes to human health is rudimentary but could potentially transform our understanding of health and disease.

Program designed to overcome this problem: Human Microbiome Project

Status: This program develops tools and generates resources to facilitate characterization of the human microbiome and analysis of its role in human health and disease. The program establishes links between the human microbiome and states of health and disease through several integrated initiatives.

The first was launched in FY 2007 to "jumpstart" the effort by sequencing a reference set of genomes from cultured microbes. These reference sequences will facilitate the analysis of complex mixtures of microbes to be obtained from human body sites. Beginning in FY 2009, samples from 5 body sites (skin, nose, mouth, gastrointestinal tract, and vagina) will begin to be collected from more than 100 individuals. By analyzing microbial populations at multiple body sites in normal, healthy individuals, the program builds the foundation for an advanced understanding of the degree of microbial diversity that may exist among individuals. A series of demonstration projects will build upon this foundation to analyze the microbiome in individuals with varying diseases or conditions to determine whether changes in our microbiome correlate with changes in health status.

In addition to the sequencing effort, this program supports the development of technological improvements that will enable the effort to proceed faster and with reduced costs. NIH is also working with researchers from several countries to establish the International Human Microbiome Consortium. This consortium will provide a forum for data sharing and information exchange relevant to the program. In addition, the vast amount of sequence data generated by this program will be deposited in a publicly accessible database and the sequences of the bacterial strains studied will be made available for future studies. Finally, the Ethical, Legal, and Social Implications (ELSI) of the microbiome project are being studied through a dedicated initiative.

The Human Microbiome Project will be funded by the Common Fund through FY 2012. During this timeframe, a foundation will be laid to allow the continued exploration of the human microbiome through investigator-initiated projects funded by the ICs.

11. Roadblock: Contributions of higher order DNA structure to human health and disease are poorly understood.

Program designed to overcome this problem: Epigenomics of Human Health and Disease

Status: This program seeks to help define the relationship between the modifications to DNA that alter its three-dimensional structure (the epigenome) and human health and disease. Like the Human Microbiome Project, a series of integrated initiatives has been established to achieve this goal.

Studies in experimental animal models has established that diet, environmental exposures, and aging can significantly alter genetic activity by producing chemical modifications to DNA that alter the coiled structure that DNA assumes in different cell types. However, very little information is available about the way that DNA coils in normal, healthy human cells, so it is difficult to know the extent to which human disease may result from changes to this structure. To clarify this, the Epigenomics Program is enabling the development of comprehensive reference maps of the human epigenome from many different cell types. It also fosters new technologies for epigenomic analysis, an integrated Data Coordinating Center, and novel regulators of epigenomic structure.

An understanding of the human epigenome has the potential to transform knowledge about disease onset and progression, as well as to lead to novel therapeutic approaches. Together with several international partners, the NIH is working to establish an International Consortium to foster collaboration and information exchange worldwide in this endeavor. The fundamental knowledge obtained through this program will catalyze research in all areas of medicine and increase our understanding of the genetic basis of health and disease. This program was launched with "jumpstart" funds in FY 2007 but major funding began in FY 2008. It is slated to receive Common Fund support through FY 2015.

12. Roadblock: Genome-Wide Association Studies reveal genetic variations that associate with disease, but the molecular effects of the variations is difficult to unravel.

Program designed to overcome this problem: Genotype/Tissue Expression (GTEx) Resource

Status: Genome-Wide Association Studies are revealing increasing numbers of genetic variations that result in susceptibility to disease. However, using this information to intercede before disease develops will require an understanding of the molecular consequences of the genetic variation. This is very difficult to unravel, since

a change in DNA sequence may alter a part of the chromosome involved in regulating a gene (or genes) far removed from the sequence variant itself, and it could influence the regulation of genes in many tissues.

To overcome this problem, the GTEx program, to begin in FY 2010, will correlate genetic variability with variability in expression of many genes in many tissues. To do this, samples from 320 donors (either surgical donors or autopsy donors) will be acquired from several tissues, the genotypes determined for each, and then a gene expression profile obtained for each tissue. The genes expressed and the level at which they are expressed can then be correlated with genetic variations of the donors.

Common Fund support of this program will allow the feasibility of the approach to be determined through a two-year period of support. Analysis of the data from the initial two years will determine whether further investment to scale up the approach is warranted.

III. Looking to the Future: Strategic Planning for the Common Fund

The Common Fund was established by the 2006 Reform Act to encourage strategic planning for research that crosses IC borders and coordination in program management. These core principles for the Common Fund are sufficiently broad that they provide the NIH with flexibility to determine the most pressing needs and to respond corporately to these challenges.

Through the Roadmap Programs, the NIH addresses fundamental, cross-cutting challenges that influence virtually every disease area and have potential for exceptionally high impact. Future planning for Roadmap programs will continue to involve heavy input from the public to identify common bottlenecks and to articulate cross-cutting areas of exceptional opportunity. However, as the Common Fund grows, additional types of programs may be supported that serve the stated mission of the Common Fund to encourage multi-IC planning and coordination but do not address the criteria established for the Roadmap.

While growth and diversification of Common Fund programs will depend on growth of the Fund itself, the planning strategies for all types of Common Fund programs will share the requirement that multiple ICs and their respective communities are served by each program. This will require staff from multiple ICs to interact, share information, and bring their communities together to identify gaps in knowledge, brainstorm, and articulate programmatic needs.

Facilitated by the Office of Strategic Coordination within the Division of Program Coordination, Planning, and Strategic Initiatives, these planning activities will involve gathering input at multiple levels to establish priorities for Common Fund dollars. Data concerning the NIH research portfolio, research conducted elsewhere, and the research needs vocalized by the community will also be used to help establish these priorities.

Appendix 1: Criteria for NIH Roadmap Programs

The overarching goal of all Roadmap initiatives is to accelerate the discovery and translation of scientific knowledge into public health benefits. Roadmap is conceived of as a five- to ten-year "incubator space" for NIH initiatives that meet all of the following criteria:

Is the proposed initiative truly transforming–could it dramatically affect how biomedical and/or behavioral research is conducted over the next decade?

Will the outcomes from the proposed initiatives synergistically promote and advance the individual missions of the ICs to benefit health?

Does the proposed initiative require participation from NIH as a whole and/or does it address an area(s) of science that does not clearly fall within the mission of any one IC or OD program office?

Is the proposed initiative something that no other entity is likely or able to do, and is there a public health benefit to having the results of the research in the public domain?

Appendix D: Monitoring Adherence to the NIH Policy on the Inclusion of Women and Minorities as Subjects in Clinical Research (excerpt)

Link to on-line version of full report

Department of Health and Human Services
National Institutes of Health

MONITORING ADHERENCE TO THE NIH POLICY ON THE INCLUSION OF WOMEN AND MINORITIES AS SUBJECTS IN CLINICAL RESEARCH

Comprehensive Report: Tracking of Human Subjects Research
As Reported in Fiscal Year 2007 and Fiscal Year 2008

NIH Tracking/Inclusion Committee

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Carl Roth, Ph.D., LL.M., Co-Chair
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2009

Preface

To demonstrate effective implementation of Public Law 103-43 and implementation of NIH policies on the tracking and inclusion of women and minorities in clinical research, the Office of Research of Women's Health (ORWH), in collaboration with the Office of Extramural Research (OER) and the Office of Intramural Research (OIR), has led monitoring efforts for compliance including convening a trans-NIH Tracking and Inclusion Committee. Monitoring efforts have included the documentation of the numbers of males and females by race and ethnicity enrolled in clinical studies funded by NIH, as well as biennial statements from each Institute and Center (IC) Advisory Councils to confirm compliance with NIH policies. These and other efforts serve to ensure that NIH procedures comply with the NIH policy on the inclusion of women and minorities in clinical studies.

Data monitoring for the magnitude and diversity of clinical studies funded by NIH is not a simple task. There has been an extensive and dedicated effort to provide accurate and reproducible data. However, transitions in information system software at the NIH have introduced the need for modifications for monitoring inclusion and data collection.

The Acting Director of NIH has now established a task force to be co-chaired by the Director of ORWH, the Director of the National Center for Minority Health and Health Disparities (NCMHD), and an IC Director to examine the entire process for evaluating and monitoring the inclusion of women and minorities in clinical research funded by NIH; and the results of the deliberations of this task force should be reflected in the next biennial report. However, the FY2007 and FY2008 data in this document reflect the data reporting and current trends of the inclusion of participants in NIH clinical research.

This report would not be possible without the efforts of the members of the NIH Tracking and Inclusion Committee and the eRA Population Tracking User Group (ePTUG) who have each provided many hours addressing a multiplicity of issues related to data entry, reconciliation of grants, contracts and cooperative agreements, and other related issues or concerns.

Monitoring Adherence to the NIH Policy on the Inclusion of Women and Minorities as Subjects in Clinical Research Summary Report of NIH Inclusion Data

NIH AGGREGATE POPULATION DATA REPORTED IN FY2007 and FY2008

Because new clinical research studies begin each year while other studies may be ending, the inclusion figures will vary from year to year due to the scientific topics under study and the prevalence of those conditions within each individual study. These data help to establish trends on the inclusion of women and minorities as subjects in clinical research. Data on inclusion are tabulated from human subject populations in NIH-defined Phase III clinical trials and other human subject research studies and are based on self identification by the participants. NIH clinical research studies are determined in accordance with the NIH definition of clinical research to include, for example, non-intervention clinical research, non Phase III clinical trials, epidemiological studies, behavioral studies, and database studies.

Analysis of aggregate NIH data on inclusion for FY2007 and FY2008 documents participants of all ages, that substantial numbers of women and men, non-minority men, and minorities of all ages have been included as research subjects in NIH clinical trials and other human subject research studies during these fiscal years. However, caution should be utilized to avoid over-interpreting the figures that are provided. The NIH Tracking and Inclusion Committee have provided for the reader's interest conclusions that can be reasonably drawn from the data.

Previous inclusion reports and aggregate enrollment figures for women, men and minority groups for FY1994 to the present can be found on the ORWH website at http://orwh.od.nih.gov/inclusion.html.

NIH CLINICAL RESEARCH: Fiscal Years 2007 and 2008

In FY2007 there were 15,567 extramural and intramural clinical research protocols, including Phase III and other clinical studies, of which 10,914 protocols reported human subject participation as noted in this report's trend summary tables *(Table 1A)*. Of these, 95.9% were domestic protocols and 4.1% were foreign protocols. *(Table 1E)* Approximately 17.4 million participants were enrolled in extramural and intramural research protocols of which 92.7% were domestic participants and 7.3% were foreign participants. Of the 17.4 million participants, 58.2% were women, 39.5% were men and 2.3% did not provide sex identification.*(Table 1A)* Further, 29.9% of the total participants, and 26.5% of the Domestic-only participants, were reported as minorities following the current OMB categories for race and ethnicity. (*Table 1F and 2C)*

Correspondingly, in FY2008 there were 15,598 extramural and intramural clinical research protocols, including Phase III and other clinical studies, of which 11,045 protocols reported human subject participation as noted in this report's trend summary tables.*(Table 1A)* Of these, 95.5% were domestic protocols and 4.5% were foreign protocols.*(Table 1E)* Approximately15.4 million participants were enrolled in extramural and intramural research protocols of which 91.7% were domestic participants and 8.3% were foreign participants. Of the 15.4 million participants, 60.0% were women, 38.9% were men and 1.1% did not provide sex identification.*(Table 1A)* Further 28.6% of the total participants, and 24.9% of the Domestic-protocol participants, were reported as minorities following the current OMB categories for race and ethnicity. (*Table 1F & Table 3C*)

While the number of participants in all extramural and intramural clinical research decreased (17.4M in FY2007 and 15.4M in FY2008), there was no significant change in the proportion of women and men (58.2%F and 39.5%M in FY2007; and 60.0%F and 38.9%M in FY2008). *(Table 1A)*

NIH Defined Phase III Clinical Research: FY2007 and FY2008

In FY2007 there were 749 extramural and intramural Phase III clinical research protocols, of which 653 protocols reported human subject participation as noted in this report's trend summary tables.*(Tables 4A and 5A)* Of these, 93.3% were domestic protocols and 6.7% were foreign protocols. Clinical studies not included in this analysis are those studies that have just begun and have not reported enrollment data or have not begun recruiting patients. *(Table 4E)* A total of 591,159 participants were enrolled in extramural and intramural Phase III research protocols of which 72.5% were domestic participants and 27.5% were foreign participants. Of the 591,159 participants, 54.9% were women, 42.2% were men and 2.8% did not provide sex identification. *(Table 4A)* Further, 41.4% of the total participants, and 20.6% of Domestic-protocol participants, in Phase III clinical research were reported as minorities following the current OMB categories for race and ethnicity. (*Table 5C*)

Of the 197 extramural and intramural Phase III research protocols that report following the former OMB standards in FY2007, minority representation was highest for Blacks (not Hispanic) at 10.3% and lowest for American Indian/Alaska Natives at 0.4%. Hispanics represented approximately 4.5%, Asian/Pacific Islanders were 1.9% and Whites (not Hispanic) 81.0% of the participants. The categories *Hawaiian/Pacific Islander* and *More Than One Race* were not designations with the former OMB standards. (*Table 4B*)

Moreover, in FY 2007, there were 424 extramural and intramural Phase III research protocols reporting data following the current OMB standards for reporting by both race and ethnicity. Accordingly, minority representation by race was highest for Blacks at 22.1% and lowest for Hawaiian/Pacific Islanders 0.1%. Asians represented 12.4%, American Indian/Alaska Natives 2.5% and Whites 34.9% of participants. Participants identifying as *More Than One* Race were 1.1% of the total number of participants. In addition, 26.9% did not identify a race category.*(Table 4C)* Of the 424 extramural and intramural Phase III research protocols designating an ethnicity in FY2007, 66.8 % of total participants identified as "Not Hispanic", 18.8 % of the total participants identified as "Hispanic or Latino", and 14.5% of the total participants did not identify an ethnicity category. The racial distribution of the "Hispanic or Latino" participants is also provided separately. (*Table 4D*)

Correspondingly, in FY2008 there were 726 extramural and intramural Phase III clinical research protocols, of which 639 protocols reported human subject participation as noted in this report's trend summary tables. *(Tables48A and 6A)* Of these, 91.5% were domestic protocols and 8.5% were foreign protocols. Clinical studies not included in this analysis are those studies that have just begun and have not reported enrollment data or have not begun recruiting patients. A total of 792,578 participants were enrolled in extramural and intramural Phase III research protocols of which 74.6% were domestic participants and 25.4% were foreign participants.*(Table 4E)* Of the 792,578 participants, 57.5% were women, 40.3% were men and 2.2% did not provide sex identification.*(Table 4A)* Further, 38.9% of the total participants, and 20.2% of Domestic-only participants, in Phase III clinical research were reported as minorities following the current OMB categories for race and ethnicity. *(Table 6C*)

Of the 164 extramural and intramural Phase III research protocols that report following the former OMB standards in FY2008, minority representation was highest for Blacks (not Hispanic) at 9.7% and lowest for American Indian/Alaska Natives at 0.4%. Hispanics represented approximately 4.1%, Asian/Pacific Islanders were 2.0% and Whites (not Hispanic) 82.0% of the participants. The categories *Hawaiian/Pacific Islander* and *More Than One Race* were not designations with the former OMB standards. (*Table 4B*)

Moreover, in FY 2008, there were 475 extramural and intramural Phase III research protocols reporting data following the current OMB standards for reporting by both race and ethnicity. Accordingly, minority representation by race was highest for Blacks at 18.4% and lowest for Hawaiian/Pacific Islanders 0.1%. Asians represented 17%, American Indian/Alaska Natives 2.7% and Whites 50.2% of participants. Participants identifying as *More Than One* Race were 2.2% of the total number of participants. In addition, 9.4% did not identify a race category. Of the 475 extramural and intramural Phase III research protocols designating an ethnicity in FY2008, 82.3 % of total participants identified as "Not Hispanic", 11.5 % of the total participants identified as "Hispanic or Latino", and 6.2% of the total participants did not identify an ethnicity category. The racial distribution of the "Hispanic or Latino" participants is also provided separately. (*Table 4C*)

While the number of participants in Phase III extramural and intramural clinical research increased (591,159 in FY2007 and 792,578 in FY2008), there was a slight change in the proportions of women and men (54.9%F and 42.2% in FY2007 and 57.5%F and 40.3%M in FY2008). *(Table 4A)*

The following sections provide data on extramural research and intramural research separately.

EXTRAMURAL CLINICAL RESEARCH: Fiscal Years 2007 and 2008

In FY2007, there were 13,719 extramural clinical research protocols, including Phase III and other clinical studies, of which 9,362 protocols reported human subject participation. Of these, 82.3% were domestic protocols and 3.5% were foreign protocols. *(Table 7A)* Approximately 13.9 million participants were enrolled in extramural research protocols of which 92.8% of the total enrollment is domestic participants and 7.2% of the total enrollment is foreign participants. *(Table 7B)* Of the 13.9 million participants, 61.80% were women, 35.54% were men and 2.62% did not provide sex identification. Further, 31.44% of the total participants were reported as minorities following the current OMB categories for race and ethnicity. *(Table 8A)*

Correspondingly, in FY2008, there were 11,045 extramural clinical research protocols, including Phase III and other clinical studies, of which 9,381 protocols reported human subject participation. Of these, 81.2% were domestic protocols and 3.7% were foreign protocols. *(Table 9A)* Approximately 12.6 million participants were enrolled in extramural research protocols of which 91.7% of the total enrollment is domestic participants and 8.3% of the total enrollment is foreign participants. *(Table 9B)* Of the 12.6 million participants, 63.84% were women, 35.04% were men and 1.12% did not provide sex identification. Further, 29.4% of the total participants were reported as minorities following the current OMB categories for race and ethnicity. *(Table 10A)*

While the number of participants in extramural clinical research protocols decreased (13.9 million in FY2007 and 12.6 million in FY2008), there was no significant change in the proportions of women and men (61.8%F and 35.5%M in FY2007 and 63.8%F and 35.0%M in FY2008). *(Table 8A and Table 10A)* However, when sex-specific studies were excluded, the proportions of women and men in all extramural clinical research reported in FY2008 were similar to the proportions in the general population reported in FY2009 (from 46.5% to 45.61% for females and 49.8% to 52.6% for males. *(Table 11A and Table 12A)*

<u>NIH Defined Phase III Extramural Clinical Research: FY2007 and FY2008</u>

In FY2007 there were 711extramural Phase III clinical research protocols, of which 617 protocols reported human subject participation. *(Table 13A)* A total of 547,687 participants were enrolled in extramural Phase III research protocols of which 55.10% were women, 41.83% were men and 3.07% did not provide sex identification. *(Table 14A)*

In FY2007 there were 399 extramural Phase III research protocols reporting data following the current OMB standards for reporting race and ethnicity. Minority representation by race was highest for Blacks at 23.21% and lowest for Hawaiian/Pacific Islanders 0.13%. Asians represented 13.09%, American Indian/Alaska Natives 2.59% and Whites 34.29% of participants. Participants identifying as *More Than One* Race were 1.02% of the total number of participants. In addition, 25.7 % did not identify a race category. Of the 399 extramural Phase III research protocols designating an ethnicity in FY 2007, 67.77 % of total participants identified as "Not Hispanic", 17.78% of the total participants identified as "Hispanic or Latino", and 14.44 % of the total participants did not identify an ethnicity category. The racial distribution of the "Hispanic or Latino" participants is also provided separately. (*Table 14B*)

In FY2008 there were 696 extramural Phase III clinical research protocols, of which 602 protocols reported human subject participation. *(Table 15A)* A total of 776,034 participants were enrolled in extramural Phase III research protocols of which 57.22% were women, 40.58% were men and 2.2% did not provide sex identification. *(Table 16A)*

Correspondingly in FY2008, there were 452 extramural Phase III research protocols reporting data following the current OMB standards for reporting race and ethnicity. Minority representation by race was highest for Blacks at 18.68% and lowest for Hawaiian/Pacific Islanders 0.12%. Asians represented 17.41%, American Indian/Alaska Natives 2.74% and Whites 51.22% of participants. Participants identifying as *More Than One* Race were 2.22% of the total number of participants. In addition, 7.62 % did not identify a race category. Of the 452 extramural Phase III research protocols designating an ethnicity in FY 2008, 83.84 % of total participants identified as "Not Hispanic", 10.38% of the total participants identified as "Hispanic or Latino", and 5.78 % of the total participants did not identify an ethnicity category. The racial distribution of the "Hispanic or Latino" participants is also provided separately. (*Table 16B*)

While the number of extramural Phase III clinical research protocols decreased (711 in FY2007 and 696 in FY2008) *(Table 13A and Table 15A)*, there was a slight increase in the proportion of women (55.1 %F and 41.8%M in FY2007 and 57.2%F and 40.6%M in FY2008). *(Tables 14A and 16A)*

INTRAMURAL CLINICAL RESEARCH: Fiscal Years 2007 and 2008

In FY2007 there were 1,848 intramural clinical research protocols, including Phase III and other clinical studies, of which 1,552 protocols reported human subject participation. *(Table 7A)* Approximately 3.5 million participants were enrolled in intramural research protocols of which 43.39% were women, 55.42% were men and 1.20% did not provide sex identification. (*Table 16A*)

In FY2007, approximately 3.5 million participants were reported in all intramural research including Phase III clinical trials, and other clinical studies. Of the 449 intramural research protocols that report data following the former OMB standards, minority representation was highest for Blacks (not-Hispanic) at 17.6% and lowest for American Indian/Alaska Natives at 0.2%. Asian/Pacific Islanders represented 3.65%, Hispanics 4.31%; and Whites (not Hispanic) 73.16% of the intramural research study population. The categories *Hawaiian/Pacific Islander* and *More Than One Race* were not designations with the former OMB standards. (*Table 17C*)

For the 1,103 intramural clinical research studies that reported data following the current OMB standards in FY 2007, the largest racial minority group was Blacks at 9.72 % and the smallest racial minority group was Hawaiian/Pacific Islanders at 0.16%. Asian represented 7.66%, American Indian/Alaska Natives 0.89% and Whites 69.85% of participants in all intramural clinical research. Approximately 0.56% of participants reported *More Than One Race* as their racial category. In addition, 11.16 % did not identify a

race category. Of the 1,103 intramural research protocols following the current OMB standards designating an ethnicity in FY2007, 85.50 % of total participants identified as "Not Hispanic", 4.19 % of the total participants identified as "Hispanic or Latino", and 10.31 % of the total participants did not identify an ethnicity category. The racial distribution of the "Hispanic or Latino" participants is also provided separately. (*Table 17B)*

Correspondingly, in FY2008 there were 1,873 intramural clinical research protocols, including Phase III and other clinical studies, of which 1,664 protocols reported human subject participation. *(Table 9A)* Approximately 2.8 million participants were enrolled in intramural research protocols of which 42.82% were women, 55.93% were men and 1.25% did not provide sex identification. (*Table 18A*)

In FY 2008, approximately 2.8 million participants were reported in all intramural research including Phase III clinical trials, and other clinical studies. Of the 413 intramural research protocols that report data following the former OMB standards, minority representation was highest for Blacks (not-Hispanic) at 30.34% and lowest for American Indian/Alaska Natives at 0.15%. Asian/Pacific Islanders represented 3.38%, Hispanics 4.0%; and Whites (not Hispanic) 60.73% of the intramural research study population. The categories *Hawaiian/Pacific Islander* and *More Than One Race* were not designations with the former OMB standards. (*Table 18C*)

For the 1,251 intramural clinical research studies that reported data following the current OMB standards in FY 2008, the largest racial minority group was Asians at 9.8 % and the smallest racial minority group was Hawaiian/Pacific Islanders at 0.2%. Blacks represented 9.4%, American Indian/Alaska Natives 0.81% and Whites 67.92% of participants in all intramural clinical research. Approximately 0.61% of participants reported *More Than One Race* as their racial category. In addition, 11.30 % did not identify a race category. Of the 1,251 intramural research protocols following the current OMB standards designating an ethnicity in FY2008, 85.30 % of total participants identified as "Not Hispanic", 4.07% of the total participants identified as "Hispanic or Latino", and 10.62 % of the total participants did not identify an ethnicity category. The racial distribution of the "Hispanic or Latino" participants is also provided separately. (*Table 18B*)

While the number of participants specifically in Phase III intramural clinical research protocols significantly decreased (3.5M in FY2007 and 2.8M in FY2008), there was no substantive change in the proportions of women and men (43.4%F and 55.4% M in FY2007 and 42.8%F and 55.9%M in FY2008). *(Tables 17A and Table 18A)*

NIH Defined Phase III Intramural Clinical Research: FY2007 and FY2008

In FY2007 there were 38 intramural Phase III clinical research protocols, of which 36 protocols reported human subject participation. Of these, 88.8%, of the total number of protocols are domestic and 11.1% of the total number of protocols is foreign. *(Table 13A)* A total of 43,472 participants were enrolled in intramural Phase III research protocols of which 77.1 are domestic participants and 22.9% are foreign participants.*(Table 13B)* Of the 43,472 participants, 52.8% were women, 47.2% were men and 0% did not provide sex identification. *(Table 19A)* Further, 27.3% of total participants in Phase III intramural clinical research protocols were reported as minorities following the current OMB categories for race and ethnicity. (*Table 13C*)

Correspondingly, in FY2008 there were 39 intramural Phase III clinical research protocols, of which 37 protocols reported human subject participation. Of these, 89.1% of the total number of protocols is domestic and 10.8% of the total number of protocols is foreign. *(Table 15A)* A total of 16,544 participants were enrolled in intramural Phase III research protocols of which 36.7% of the total enrollment is

domestic participants and 63.2% are foreign participants. *(Table 15B)* Of the 16,544 participants, 69.71% were women, 28.92% were men and 1.37% did not provide sex identification. Further, 56.67% of total participants in Phase III clinical research protocols were reported as minorities following the current OMB categories for race and ethnicity. (*Table 20A*)

While the number of participants specifically in Phase III intramural clinical research protocols significantly decreased (43,472 in FY2007 and 16,544 in FY2008), there was a substantial increase in the proportions of women (52.8%F and 47.2% M in FY2007 and 69.7%F and 28.9%M in FY2008). *(Tables 19A and Table 20A)*

TREND REPORT ON NIH AGGREGATE POPULATION DATA: FY1995 – FY2008

Trend data vary over time because the data for each year represent the net total of data resulting from: (1) studies continuing from the prior year; (2) the addition of new studies reported; and (3) the subtraction of studies that are no longer reported.

Table 21 is a fourteen year summary report showing a steady increase in the number of protocols and enrollment. The number of protocols with enrollment increased from 3,188 in FY1995 to 11,045 in FY2008 – a 3.5 fold increase. Reported enrollment increased from approximately 1.0 million (FY1995) to 15.4 million (FY2008) – a 15.1 fold increase; minority enrollment increased from approximately 0.4 million (FY1995) to 4.3 million (FY2008) – an 11.7 fold increase in minority representation in NIH clinical research. *(Table 21A)* The total number of protocols reported with enrollment data has increased such that, since FY2003 the number is in excess of 10,000 protocols per year. *(Table 21B)*

With the deployment of an updated population tracking system in 2002 and the OMB requirement to report data using the current format, NIH was able to report domestic and foreign data in a better way. Thus, trend data are available for domestic and foreign protocols and participation beginning in FY2002. Domestic enrollment increased from 10.2 million (FY2002) to 14.1 million (FY2008) – a 1.4 fold increase. Foreign enrollment increased from 0.9 million (FY2002) to 1.3 million (FY2008) – a 1.4 fold increase. *(Table 21A)* Overall, the total enrollment has increased with domestic participation ranging between 75.9-92.7% and foreign participation ranging between 7.3-24.1%. In FY2008, domestic and foreign enrollment was 91.7% and 8.3% respectively. *(Table 21C)*

Table 1 is a summary report of all extramural and intramural clinical research by sex/gender and minority representation following the old and new data formats for domestic and foreign studies. The report demonstrates that female participation in all extramural and intramural research generally ranged between 51.7% and 63.9%, male participation in all extramural and intramural research ranged between 34.0% and 45.0%. *(Table 1A)* Overall minority participation in all extramural and intramural clinical research ranged between 28.6% and 43.1%. *(Tables 1B-D)Table 1E* provides a comparison of domestic and foreign participation between FY2002 and FY2008. The vast majority of protocols are domestic (~94%-96%) of the total clinical research protocols. While the number of foreign protocols has increased, they incorporate only about 4%-6% of the total clinical research protocols with enrollment. *Table 1F* shows domestic and foreign enrollment for the seven-year period. Domestic minority enrollment varied between 24.1% and 28.9% of total domestic participation, while foreign minority enrollment varied between 67.7% and 90.9% of total foreign participation.

Table 4 is a summary of NIH-funded Phase III extramural and intramural clinical research by sex/gender and minority enrollment following the old and new data reporting formats for domestic and foreign studies. This table demonstrates that female participation in NIH funded Phase III extramural and intramural clinical research generally ranged between 54.1% and 74.8% and male participation in NIH-funded Phase III extramural and intramural clinical research ranged between 24.3% and 44.6%. *(Table 4A)* Overall minority participation in NIH-funded Phase III extramural and intramural clinical research ranged from 26.9% to 41.4%. *(Tables 4B-D) Table 4E* provides a comparison of domestic and foreign participation between FY2002 and FY2008. The vast majority of protocols are domestic, ranging from 75.5% and 95.8% of the total clinical research protocols. While the number of foreign protocols has decreased, they incorporate only about 4.2%-9.6% of the total clinical research protocols with enrollment in the last seven years. *Table 4F* shows domestic and foreign enrollment for the seven-year period. Domestic minority enrollment varied between 20.2% and 25.4% of total domestic participation, while foreign minority enrollment in NIH-funded Phase III clinical research varied between 48.4% and 96.2% of total foreign participation. In comparing both domestic and foreign Phase III enrollment over the seven

year period shows that the small percentage of foreign protocols in FY2008 account for a significant proportion of the total foreign enrollment.

Tables 22-25 summarize domestic and foreign participation for NIH funded clinical research and NIH-funded Phase III clinical research. For extramural and intramural clinical research, domestic participants enrolled in domestic protocols, female participation ranged between 58.1% and 67.3% while male participation ranged between 31.2 and 39.5%. (*Table 22A)* For NIH-funded Phase III extramural and intramural clinical research, domestic participants enrolled in domestic protocols, female participation ranged between 53.3 and 64.6% while male participation ranged between 34.4 and 44.8%. *(Table 23A)* For all extramural and intramural clinical research, foreign participants enrolled in foreign protocols, female participation varied from 39.2% to 59.5% while male participation varied from 39.3% to 60.4%. (*Table 24A)* For NIH-funded Phase III extramural and intramural clinical research, foreign participants enrolled in foreign protocols, female participation varied from 47.4% to 59.2% while male participation varied from 40.4% to 52.5%. *(Table 25A)*

Appendix E: Research Training and Graduate Medical Education Data

NATIONAL RESEARCH SERVICE AWARD (NRSA) AND NATIONAL LIBRARY OF MEDICINE (NLM) RESEARCH TRAINING PROGRAMS

Ph.D.s Awarded to NIH Trainees and Fellows		
Field of Study	FY 2007	FY 2008
Life Sciences	**2,192**	**2,369**
Biological/Biomedical Sciences	*1,943*	*2,120*
Biochemistry	188	218
Biomedical Sciences	68	72
Biophysics	64	49
Biotechnology	4	2
Bacteriology	8	4
Plant Genetics	5	7
Botany/Plant Biology	6	4
Anatomy	2	2
Bioinformatics	14	25
Biometrics & Biostatistics	25	19
Cell/Cellular Biology and Histology	131	133
Cancer Biology	43	121
Ecology	15	1
Developmental Biology/Embryology	77	69
Endocrinology	5	6
Entomology	2	2
Immunology	160	183
Molecular Biology	203	202
Microbiology	150	161
Neuroscience	327	359
Nutritional Sciences	24	19
Parasitology	6	9
Toxicology	41	46

Genetics, Human & Animal	137	147
Pathology, Human & Animal	21	37
Pharmacology, Human & Animal	96	100
Physiology, Human & Animal	64	59
Zoology, Other	1	3
Biology/Biological Sciences, General	37	28
Biology/Biomedical Sciences, Other	15	14
Health Sciences	*238*	*240*
Speech-Language Pathology & Audiology	9	12
Environmental Health	4	10
Environmental Toxicology	2	7
Health Systems/Service Administration	5	3
Public Health	39	35
Epidemiology	60	65
Kinesiology/Exercise Sciences	8	13
Nursing Science	56	60
Rehabilitation/Therapeutic Services	4	2
Veterinary Medicine	4	4
Health Sciences, General	4	3
Health Sciences, Other	22	11
Agricultural Sciences/Natural Resources	*11*	*9*
Agricultural Economics	1	1
Agricultural Science, Other	1	0
Plant Sciences, Other	0	2
Environmental Science	0	3
Forest/Resources Management	1	
Social Sciences	**300**	**288**
Psychology	*235*	*224*
Clinical	81	96
Cognitive & Psycholinguistics	28	16
Counseling	2	6
Developmental & Child	24	26

Human Development & Family Studies	6	5
Experimental	14	10
Educational	2	
Industrial & Organizational	1	1
Personality	10	1
Physiological/Psychobiology	26	14
Psychometrics & Quantitative	7	0
School	1	2
Social	19	24
Psychology, General	9	12
Psychology, Other	7	10
Social Sciences	*65*	*64*
Anthropology	13	5
Demography/Population Studies	3	6
Economics	8	14
Public Policy Analysis	5	7
Sociology	23	20
Social Sciences, Other	5	0
Physical Sciences	**115**	**139**
Chemistry	*67*	*85*
Analytical	15	12
Inorganic	5	5
Organic	24	31
Medicinal/Pharmaceutical	1	0
Physical	6	10
Polymer	1	1
Theoretical	2	4
Chemistry, General	3	7
Chemistry, Other	10	15
Computer Sciences	*11*	*11*
Computer Science	10	8
Computer & Information Sciences, Other	1	2

Geological & Earth Sciences	*1*	
Geology	1	
Mathematics	*12*	*12*
Applied Mathematics	1	4
Geometry/Geometric Analysis	3	0
Statistics	5	6
Mathematics/Statistics, General	1	
Mathematics/Statistics, Other	0	1
Ocean/Marine Sciences	*2*	*0*
Marine Sciences	1	0
Oceanography, Chemical and Physical	1	0
Physics	*23*	*31*
Optics/Phototonics	1	1
Polymer Physics	1	0
Condensed Matter/Low Temperature	2	1
Physics, General	1	1
Physics, Other	3	4
Engineering	**143**	**174**
Aerospace, Aeronautical & Astronautical	2	1
Bioengineering & Biomedical	99	130
Chemical	27	30
Computer	0	1
Electrical, Electronics and Communications	7	6
Environmental Health Engineering	3	1
Industrial & Manufacturing	1	0
Materials Science	1	
Mechanical	3	2
Education	**18**	**13**
Humanities	**7**	**3**
Other Fields	**26**	**17**
TOTAL	2,801	3,003
Note: Detailed field data are provided only for broad fields with ≥ 100 Ph.D. recipients		

Sources: NIH IMPAC II and the Doctorate Records File.

Demographic Characteristics* of NRSA Participants		
Gender	FY 2007	FY 2008
Female	51.5%	51.5%
Male	45.8%	45.9%
Unreported	2.6%	2.6%
Race/Ethnicity		
White	67.2%	66.0%
Asian	15.0%	14.9%
Hispanic	6.7%	7.0%
African American	8.3%	7.6%
Native American	1.0%	1.0%
Pacific Islander	0.7%	0.7%
Unreported	5.9%	7.9%

Sources: NIH IMPAC II
*Reporting personal information such as sex, race, and ethnicity is voluntary.

GRADUATE MEDICAL EDUCATION: NIH-SPONSORED, ACGME-ACCREDITED, RESIDENCY AND SUBSPECIALTY TRAINING PROGRAMS

Successfully Completed Residency and Subspecialty Training By Academic Year		
NIH Clinical Center Program Specialty	Successfully Completed	
	2007/2008	2008/2009
Allergy and Immunology	4	3
Medical Genetics	4	1
Medical Biochemical Genetics	0	2
Critical Care Medicine	4	3
Endocrinology, Diabetes, and Metabolism	6	4
Hematology	3	4
Infectious Disease	3	4
Oncology	13	9
Rheumatology	2	4
Pathology-Anatomic and Clinical	4	3

Blood Banking/Transfusion Medicine	1	2
Cytopathology	1	1
Hematology (Pathology)	1	2
NICHD/Georgetown University Hospital Program / Pediatric Endocrinology*	2	3
Psychiatry	1	2
Total	49	47

*Cosponsored by NICHD and Georgetown University Hospital
Source: AAMC GME Track Database

Appendix F: Report of the Advisory Committee on Research on Women's Health, FYs 2007-2008 (excerpt)

Link to on-line version of full report

Report of the Advisory
Commitee on Research
on Women's Health

FISCAL YEARS
2007–2008

Office of Research on Women's Health & NIH Support for Research on Women's Health Issues

Preface

The Advisory Committee on Research on Women's Health (ACRWH), in concert with the Office of Research on Women's Health (ORWH) and the Coordinating Committee on Research on Women's Health (CCRWH), submits to the Director of the National Institutes of Health (NIH) this Biennial Report for fiscal years (FYs) 2007 and 2008. The report describes the comprehensive and coordinated efforts of the ORWH and the NIH Institutes, Centers (ICs), and Offices to address women's health issues through research and related activities in accordance with the NIH Revitalization Act of 1993. The information in this Biennial Report was prepared by the ORWH and by each of the NIH ICs and Offices to highlight significant research studies and other achievements and initiatives that have contributed to an increased knowledge of women's health. Using criteria supplied by the NIH Office of Financial Management (OFM) and the U.S. Department of Health and Human Services Office on Women's Health, and based on budget data provided by NIH ICs, this report also contains information on NIH budget allocations for women's health research during FY 2007 and FY 2008. In addition, the report contains information obtained from the NIH ICs and Offices documenting the inclusion of women and minorities in NIH-funded clinical research during the same time period.

The ACRWH has reviewed the information contained herein and believes that this Biennial Report accurately reflects the breadth and depth of research and related activities through which the NIH, in FY 2007 and FY 2008, has fulfilled its mandate from the U.S. Congress to address women's health issues and women's inclusion in research.

The ACRWH acknowledges the valuable contributions to this report of the CCRWH, which is made up of the directors of each of the ICs and Offices or their designated representatives. We are also grateful to the many NIH staff members who prepared and reviewed the reports of their ICs or Offices. We appreciate the work of the NIH Tracking and Inclusion Committee in preparing information on the inclusion of women and minorities in NIH-funded research and the work of the NIH OFM in collecting and tabulating the budgetary data published in this report.

Finally, the ACRWH wishes to acknowledge the work of ORWH staff. This Biennial Report reflects the achievements of the ORWH in fulfilling all aspects of its core mission in strengthening and enhancing research related to diseases and conditions that affect women; ensuring the appropriate representation of women in NIH research; supporting the advancement of women in biomedical careers; and building programs to ensure the development of a cadre of researchers, both women and men, in the field of interdisciplinary women's health research.

(For a full listing of ACRWH members for FY 2009, please see pages iv–vi.)

Introduction to the Biennial Report

As directed in the National Institutes of Health (NIH) Revitalization Act of 1993,[1] the Advisory Committee on Research on Women's Health (ACRWH) submits to the NIH Director a report describing the activities of the Committee and its findings related to the mandates and funding for women's health. This report includes coordinated efforts of the NIH Institutes, Centers (ICs), and Offices to address women's health issues through research and related activities. As the 20th anniversary of the establishment of the Office of Research on Women's Health (ORWH) approaches, this FY 2007–2008 Biennial Report bears witness to the phenomenal growth in women's health research and related programs that has occurred since the formation of the Office in 1990. This report reflects major FY 2007–2008 ORWH research programs, initiatives, and activities, as well as highlights that were reported through the Coordinating Committee on Research on Women's Health (CCRWH) from the NIH ICs and Offices. This report is not a comprehensive listing of all NIH research on women's health, which would necessarily be encyclopedic; however, the report does serve to summarize, under a single cover, examples of the wealth of NIH advances in women's health research. This Biennial Report also provides information on and analysis of support for women's health research and related activities. During FY 2007–2008, NIH spent approximately $3.5 billion per year on research specifically related to women's health and approximately $23 billion on research relevant to both women and men.

The Biennial Report is divided into two major parts. Part One is based on ORWH programs and describes ORWH scientific, interdisciplinary, research, career development, and research dissemination and outreach programs. Data are also reported on the inclusion of women and minorities in NIH-funded clinical research as provided from the Office of Extramural Research. Many ORWH programs reflect the Office's roles in coordinating trans-NIH activities. Most ORWH programs are conducted in collaboration with NIH ICs and Offices. Other ORWH activities are conducted in collaboration with Federal agencies and/or with public and private partners. Part Two of the Biennial Report provides the individual reports on women's health research from 20 NIH Institutes, 4 Centers, and 7 Offices, which include highlights of some of their most promising research programs.

Office of Research on Women's Health

Information about ORWH programs is organized into six sections covering the following areas: ORWH Research; ORWH Interdisciplinary Research and Career Development Programs; ORWH Biomedical Career Development Activities; ORWH Research Dissemination and Outreach; Monitoring Adherence to the NIH Policy on the Inclusion of Women and Minorities as Subjects in Clinical Research; and NIH Budget for Women's Health Research.

Section I describes FY 2007–2008 NIH women's health research priorities, developed in coordination with the CCRWH and reviewed by the ACRWH. It also provides a table of ORWH-funded projects grouped by diseases and conditions. It also provides examples of special ORWH research initiatives in FY 2007–2008 and highlights of ORWH-cofunded research projects and research workshops and conferences. A strategic planning effort, begun in 2008, is described in Section I. The effort, which will update the 1999 *Agenda for Research on Women's Health for the 21st Century*, is currently ongoing. It is anticipated that the updated research agenda will be completed in time for the 20th anniversary of the founding of ORWH in September 2010.

The Agenda for Research on Women's Health for the 21st Century recognized that women's health research is an inherently broad interdisciplinary field of endeavor, encompassing a full range of science. Since 1999, ORWH has been working to provide institutional support for interdisciplinary research and interdisciplinary research career development. Section II highlights major ORWH efforts to catalyze interdisciplinary women's health research and career development through two programs:

[1] The NIH Revitalization Act of 1993, P.L.103-43,107, Stat. 22 [codified at 42 U.S.C. 289(a)(1)] [Sec. 486(287d)(d)].

the Specialized Centers of Research (SCOR) on Sex and Gender Factors Affecting Women's Health, and the Building Interdisciplinary Research Careers in Women's Health (BIRCWH) Institutional Mentored Career Development Program. Section II also describes ORWH efforts to catalyze NIH interdisciplinary research and IC collaboration to advance understanding of a specific multifactorial condition predominantly affecting women, namely chronic fatigue syndrome.

Since its inception in 1990, the mandate of ORWH has included women's career development and the development of women's health researchers. The BIRCWH program is a major example of a highly successful mentored career development program that was developed and implemented by ORWH in 1999. Section III provides information on a number of other programs through which ORWH works to promote women's biomedical career development and the development of careers in research on women's health and sex/gender factors. Section III reports on the collaborative efforts of ORWH and the *Eunice Kennedy Shriver* National Institute of Child Health and Human Development (NICHD) in supporting the Women's Reproductive Health Research Career Development program, and on the ORWH-initiated trans-NIH Reentry into Biomedical and Behavioral Careers Research Supplement Program.

Section III describes the activities of the NIH Director's Working Group on Women in Biomedical Careers to provide an NIH response to the challenges to Federal agencies posed in the 2007 National Academy of Sciences report, *Beyond Bias and Barriers: Fulfilling the Potential of Women in Academic Science and Engineering*. Section III also highlights FY 2007–2008 ORWH/NIH Intramural Women's Health Research programs, ranging from a summer research program for high school students interested in science to negotiation skills for tenure-track women scientists. The section ends with a summary of a wide range of other ORWH activities to promote the career development of women, some of which involve partnering with professional societies.

Section IV on research dissemination and outreach provides information on new ORWH Internet-based health information initiatives, including a collaborative effort with the NIH National Library of Medicine to create an online resource for information on women's health research; a Web-based course cosponsored with the Food and Drug Administration (FDA) on *The Science of Sex and Gender in Human Health*; and a multimedia approach to communicate advances being made from past and current women's health research. ORWH strives to ensure that the information generated from the NIH investment in research on women's health informs future research efforts and improves women's health care. Thus, outreach to the largest possible population of clinicians and researchers, women, healthcare providers, and others interested in women's health is a very important part of its mandate. Section IV describes ORWH outreach activities, including the Women's Health Seminar series and the Vulvodynia Awareness campaign.

Section V details NIH efforts to monitor the inclusion of women and minorities in NIH-funded clinical research, including data by ICs as well as NIH aggregate figures. Section VI provides information on NIH expenditures on women's health research, including a breakdown of expenditures by disease category and other major categories of interest (e.g., aging research).

NIH IC Support for Research on Women's Health

Part Two of the Biennial Report is composed of individual reports from each of 20 NIH Institutes, 4 Centers, and 7 Offices located within the Office of the Director, NIH. These IC and Office reports summarize their major initiatives and activities and provide highlights of their funded research related to women's health and sex/gender research, consistent with their specific missions.

You are invited to read this in-depth report to become acquainted with the tremendous advances in women's health research that have taken place during this 2-year period and to appreciate the promise for even greater advances in the future, not just for women's health, but also for men's health and for careers in women's health research for both men and women.

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Office of Research on Women's Health (ORWH)

Introduction to ORWH Programs

In 1983, the Assistant Secretary for Health, Dr. Edward N. Brandt, established the U.S. Public Health Service Task Force on Women's Health Issues, in recognition of the paucity of data related to women's health. The Task Force produced a 1985 report, *Women's Health: Report of the Public Health Service Task Force on Women's Health Issues, Volume I.*[2] The report delineated a series of criteria for differentiating a health problem, condition, or disease as a woman's issue. The criteria included the following:

- Diseases or conditions unique to women or some subgroup of women;
- Diseases or conditions more prevalent in women or some subgroup of women;
- Diseases or conditions more serious in women or some subgroup of women;
- Diseases or conditions for which risk factors are different for women or some subgroup of women; or
- Diseases or conditions for which interventions are different in women or some subgroup of women.

The report also recommended that "biomedical and behavioral research should be expanded to ensure emphasis on conditions and diseases unique to, or more prevalent in, women in all age groups."

Following the issuance of the Task Force report, the National Institutes of Health (NIH) established a policy for the inclusion of women in clinical research. This policy, which urged the inclusion of women, was first published in the *NIH Guide to Grants and Contracts* in 1987.[3] Later that year, minority scientists and other researchers at NIH recognized the need to address the inclusion of minority populations. As a result, a subsequent version of the NIH Guide published for the first time a policy encouraging the inclusion of minorities in clinical studies.[4]

In 1990, the Congressional Caucus for Women's Issues requested that the General Accounting Office (GAO), now known as the Government Accountability Office, conduct an investigation into the implementation of the guidelines for the inclusion of women by NIH. This report, included in congressional testimony, indicated that the implementation of the policy for the inclusion of women was slow and not well communicated, that gender analysis was not being performed routinely, and that the impact of this policy could not be determined.[5] The GAO testimony also indicated that there were differences in the implementation of the policy recommending the inclusion of minorities, and that not all Institutes and Centers (ICs) factored adherence to these policies into scientific merit review. GAO findings concerning the lack of consistent implementation of policies for inclusion of women in NIH clinical trials led NIH to establish the ORWH within the Office of the

[2] U.S. Public Health Service. Women's health: Report of the Public Health Service Task Force on women's health issues. *Public Health Reports* 100(1):74-106, 1985.

[3] Division of Research Grants. Inclusion of women in study populations. *NIH Guide to Grants and Contracts* 16(3):2, 1987.

[4] Division of Research Grants. Inclusion of minorities in study populations. *NIH Guide to Grants and Contracts* 16(32):3-4, 1987.

[5] *National Institutes of Health: Problems in Implementing Policy on Women Study Populations* (GAO/T-HRD-90-38, 1990). Washington, DC: *U.S. Government Accountability Office.*

NIH Director in September 1990. Since its establishment, ORWH has served as the focal point for women's health research at NIH. The responsibilities of the Director, ORWH, include the following:

(1) Advises the NIH Director and staff on matters relating to research on women's health;

(2) Strengthens and enhances research related to diseases, disorders, and conditions that affect women;

(3) Ensures that research conducted and supported by NIH adequately addresses issues regarding women's health;

(4) Ensures that women are appropriately represented in biomedical and biobehavioral research studies supported by NIH;

(5) Develops opportunities for and supports recruitment, retention, reentry, and advancement of women in biomedical careers; and

(6) Supports research on women's health issues. ORWH works in partnership with the NIH Institutes and Centers to ensure that women's health research is part of the scientific framework at NIH and throughout the scientific community.

ORWH was established in statute in the NIH Revitalization Act of 1993.[6] An Advisory Committee on Research on Women's Health (ACRWH), composed of non-Federal members, was also statutorily mandated in the Revitalization Act as a mechanism for eliciting advice and recommendations on priority issues affecting women's health research. This Committee provides leadership to ORWH by advising the ORWH Director on appropriate research activities in women's health. ACRWH members are chosen from among health practitioners, advocates, research scientists, educators, and other professionals. Committee members are actively involved in reviewing and advising on matters related to research priorities, the women's health research portfolio for NIH, career development, inclusion of women and minorities in NIH-funded clinical research, and other ORWH or NIH programs related to women's health.

ORWH also benefits from the advice of a Coordinating Committee on Research on Women's Health (CCRWH). The CCRWH was also established in statute in the 1993 NIH Revitalization Act and is composed of Institute and Center Directors or their designees as a direct liaison for ORWH with NIH ICs. Both the ACRWH and the CCRWH provide valuable guidance, collaboration, and support for activities of ORWH in women's health research, career programs, and outreach efforts.

ORWH programs and efforts have expanded in breadth and depth over the years. The research funded or cofunded by ORWH is based on collaborative efforts with the ICs and supports peer-reviewed, science-driven initiatives. These collaborations are benefiting the health of all Americans across the lifespan, men as well as women, and all racial and ethnic groups.

[6] The NIH Revitalization Act of 1993, P.L. 103-43, 107, Stat. 22 [codified at 42 U.S.C. 289(a)(1)] [Sec. 486(287d)(d)].