

NEWS

For Immediate Release

FNIH Contact: Charles Pucie
301-435-6248
NIH Contact: NIH Press Office
301-496-5787
FDA Contact: Kristen Neese
301-332-0868
PhRMA Contact: Mark Grayson
202-835-3460

PUBLIC-PRIVATE PARTNERSHIP FORMS *THE BIOMARKERS CONSORTIUM*

TO ADVANCE THE SCIENCE OF PERSONALIZED MEDICINE

Lung Cancer and Lymphoma Set for Initial Investigations; Prospective Projects in Major Depression and Diabetes Cited

WASHINGTON, DC, Oct. 5--The Foundation for the National Institutes of Health (FNIH), the National Institutes of Health (NIH), the Food and Drug Administration (FDA), and the Pharmaceutical Research and Manufacturers of America (PhRMA) today announced the launch of a major public-private biomedical research partnership, *The Biomarkers Consortium*, to search for and validate new biological markers—biomarkers—to accelerate dramatically the delivery of successful new technologies, medicines, and therapies for prevention, early detection, diagnosis, and treatment of disease.

“Rapid realization of the aims of The Biomarkers Consortium is beyond the capacity of any single sector of our nation’s health enterprise, much less the single-institution, or single-investigator, science research approach. This initiative is large-scale and complex. It requires the expertise of all stakeholders—government, industry, patient groups, academia, and other private groups,” said Charles A. Sanders, M.D., chairman of the FNIH board of directors and of The Biomarkers Consortium Executive Committee. “This partnership epitomizes the aim Congress had in mind when it established the Foundation for the National Institutes of Health to support the mission of NIH,” he added.

Biomarkers are molecular, biological, or physical characteristics that indicate a specific underlying physiologic state. Biomarker research already has identified biological indicators that have had immense impact in prevention and treatment of disease. For example, blood pressure and cholesterol biomarkers have enabled diagnostics and therapies which have contributed to a 50 percent decrease in cardiovascular mortality in the U.S. over the last 30 years.

“Today’s announcement of The Biomarkers Consortium is an important landmark. It represents a new step in sharing both the burdens and the fruits of fundamental scientific work and can help identify areas of opportunity, clarify responsibilities, and make important new findings openly available,” said Health and Human Services Secretary Mike Leavitt.

Biomarkers are used to identify risk for disease, to make a diagnosis, to assess the severity of a disease and which organs are involved, and to guide treatment. Biomarkers will greatly accelerate basic and translational research. Among the first projects and project concepts will be several focusing on lymphoma, lung cancer, depression and diabetes.

"The core challenge is to move medicine from a curative model of today to a preemptive era when we can identify and track a disease process as early as possible. The identification of biomarkers is an essential element for the new era of predictive, preemptive, personalized medicine," said Elias A. Zerhouni, M.D., NIH Director. "The consortium enables government, industry, and philanthropy to come together to explore and develop common tools for a common purpose for everyone's benefit."

These markers can be used in clinical studies to assess whether a drug is safe and effective as well as in studies to learn more about health and disease. In addition, the FDA can use biomarkers to determine whether drugs can safely and effectively treat disease. One of the key priority public health challenges singled out in the FDA's *Critical Path to New Medical Products* report is the need for biomarker qualification and standards to support innovation in health care.

"One of our most pressing goals under the FDA's Critical Path Initiative is to improve developmental science and technology to deliver better diagnostics and more personalized treatments to patients more quickly and ultimately improve outcomes," said FDA Acting Commissioner of Food and Drugs Andrew C. von Eschenbach, M.D. "We believe the identification and use of biomarkers in drug development can have a catalytic impact on this mission."

The Foundation for NIH will manage The Biomarkers Consortium under direction of the consortium executive committee, with guidance from leading scientists within and outside the member organizations, plus input from a public representative and funding partners. The principal funding for activities of The Biomarkers Consortium will be by contributions to the Foundation for NIH, manager of the consortium, from private-sector partners representing industry, non-profit and advocacy organizations, and the philanthropic community. Other partners in The Biomarkers Consortium include NIH, FDA, and the Center for Medicaid and Medicare Services (CMS). The foundation is actively seeking additional partners to facilitate funding for the consortium's operations ("funding members") and for individual research projects—each of which will emerge as a distinct scientific initiative under the consortium's administrative umbrella. The Biomarkers Consortium Executive Committee, comprising public and private representatives from the founding partner organizations and other relevant stakeholders, is the primary decision-making body of The Biomarkers Consortium.

To date, \$1.2 million has been committed by the consortium's funding members, which include: the Alzheimer's Association; AstraZeneca; the Biotechnology Industry Organization; Bristol-Myers Squibb; GlaxoSmithKline; The Leukemia & Lymphoma Society; Johnson & Johnson; Eli Lilly & Company; Pfizer Inc; the Pharmaceutical Research and Manufacturers of America; and F. Hoffmann-La Roche.

"This is a pioneering effort by America's pharmaceutical companies to partner with the other health research sectors. This effort will advance biomarker research and development as never before. This research is critically important to the development of new cures, treatments, and diagnostics addressing the most pressing diseases facing patients today," said Billy Tauzin, president and CEO of PhRMA which represents the country's leading pharmaceutical research and biotechnology companies.

Biomarkers serve a wide range of purposes in drug development, clinical trials, and therapeutic assessment strategies and can be used to measure the natural history of a disease, indicate and

compare the effectiveness of new pharmaceutical treatments, or define the state of a given disease or condition. Information and results from consortium projects will be used broadly by researchers to promote the appropriate use of biomarkers to improve the public health worldwide.

The most useful biomarkers known today took several decades to develop. Moreover, harmonization to arrive at common approaches to biomarker assessment and evaluation has not been accomplished. FNIH, CMS, FDA, NIH, and the pharmaceutical, biotechnology, diagnostics, and medical device industries recognize the critical need for a coordinated cross-sector partnership effort to more rapidly identify and qualify biomarkers to support basic and translational research, guide clinical practice and, ultimately, support the development of safe and effective medicines and treatments for a wide range of diseases. The Biomarkers Consortium will harmonize approaches to identifying viable biomarkers, verifying their individual value, and formalizing their use in research and regulatory approval.

Promising project concepts that to date have been approved in principle for further development are:

Lung Cancer and Non-Hodgkin's Lymphoma Projects

Two of the consortium's initial projects, which will be conducted by the National Cancer Institute (NCI), will assess the use of Fluorodeoxyglucose-Positron Emission Tomography (FDG-PET) as a potential biomarker for clinical trials conducted in non-Hodgkin's lymphoma and non-small cell lung cancer. The use of FDG-PET, a promising imaging technology, will be evaluated as a potential predictive biomarker of tumor response and patient outcome. These FDG-PET studies could have enormous impact on patient management by validating a tool that can measure responses to treatments and enable more rapid drug development.

The rationale for using FDG-PET relies on the fact that tumor cells require substantial energy to survive and spread. Cells metabolize glucose for energy, and, in tumor cells, this metabolism is generally enhanced. FDG-PET is an effective way to measure glucose metabolism. This imaging procedure involves first injecting a patient with FDG that is attached to a radioactive tracer (F18), followed by imaging of the patient in a PET machine. Tumor cells consume significantly larger amounts of the FDG than normal cells, which enables this imaging technique to detect tumors as small as 1 cm. In both of the planned FDG-PET clinical trials, the levels of "tumor uptake" of FDG are measured/imaged before treatment and again at intervals thereafter. A decrease in uptake of FDG would indicate a decline in the number of tumor cells.

The FDG-PET lung and lymphoma clinical trials, which emerged from the Oncology Biomarker Qualification Initiative (OBQI), an interagency collaboration, were announced by NCI, FDA, and CMS in February 2006. The OBQI was designed through the NCI-FDA Interagency Oncology Task Force (IOTF) to evaluate specific biomarkers in cancer clinical trials for potential incorporation by the FDA as outcome measures for parameters such as treatment response. It is anticipated that these first two trials to evaluate FDG-PET in non-Hodgkin's lymphoma and lung cancer will inform both the regulatory review process for these cancers and also provide CMS with evidence-based measures to make informed reimbursement decisions.

The Foundation for NIH is seeking to raise \$7.5 million in private funds for these two projects. Thus far, \$3.75 million in commitments have been received from Amgen, AstraZeneca, Bristol-Myers Squibb, Pfizer Inc, and Merck & Co., Inc., and additional funders are anticipated to join the effort. Public funds devoted to these projects include NCI funding of approximately \$1.5 million for the non-small cell lung study and \$2.25 million for the non-Hodgkin's lymphoma study.

“Although we are early in the development of imaging tools as biomarkers, this research holds the potential, over time, to be used not only in the diagnosis of cancer, but in monitoring and predicting response to therapy,” said NCI Director John E. Niederhuber, M.D.

Whole Genome Association in Major Depressive Disorder: Identifying Genomic Biomarkers for Treatment Response

This project would be an extension of the National Institute of Mental Health Sequenced Treatment Alternatives to Relieve Depression (STAR*D) Study, a nationwide public health clinical trial to determine the effectiveness of different treatments for people with major depressive disorder. This study would expand the search for genes associated with effective outcomes in the treatment of the disorder.

Depression is a serious illness affecting millions of Americans, some who have depression as well as their families, friends, and co-workers. The cost of depression in human suffering, lost work, and medical expenditures rises every year. Anti-depressants help many patients, but no one medicine helps everyone, and it may take weeks before knowing whether the medicine is working. This study will seek genetic markers of treatment response, thereby providing a potential for a simple test to predict who will respond to which medicine—shortening response time, diminishing suffering, and helping patients function better at home and at work.

Diabetes and Pre-Diabetes Biomarker Project

This project would build upon an existing National Institute of Diabetes and Digestive and Kidney Diseases pilot study, and would seek to discover new biomarkers related to type II diabetes and pre-diabetes that could be used in developing a new assay and speed up and improve the translation of this assay from the bench to the bedside. This new methodology of diagnosis could lead to a more reliable, cheaper, and faster diabetes test.

Diabetes mellitus is a devastating disease affecting not only blood sugar levels, but also causing kidney disease, blindness, heart disease, and peripheral vascular disease. It usually begins with elevated blood sugars that may be present for months or years before a diagnosis is made. To determine if a patient has diabetes, the glucose tolerance test is done, but is inconvenient and not terribly reliable. This project will seek to identify biomarkers of diabetes from before the disease really begins to the time of full-blown diabetes. This can allow earlier treatment, better monitoring, and ultimately reduce morbidity and mortality from diabetes as well as providing the potential for huge savings in health care costs.

Evaluate the Utility of Adiponectin as a Biomarker Predictive of Glycemic Efficacy by Pooling Existing Clinical Trial Data from Previously Conducted Studies

The primary objective of this study would be to determine whether a soluble protein, adiponectin, has utility as a predictive biomarker of glycemic control in normal non-diabetic subjects and patients with type II diabetes following treatment with a novel and promising new class of compounds, Peroxisome Proliferator-Activated Receptor (PPAR) agonists.

The Foundation for the National Institutes of Health was established by the United States Congress to support the mission of the National Institutes of Health – improving health through scientific discovery. The foundation identifies and develops opportunities for innovative public-private partnerships involving industry, academia, and the philanthropic community. A non-profit, 501(c)(3) corporation, the Foundation raises private-sector funds for a broad portfolio of unique programs that complement and enhance NIH priorities and activities. The foundation’s Web site address is www.fnih.org.

The Pharmaceutical Research and Manufacturers of America (PhRMA) represents the country's leading pharmaceutical research and biotechnology companies, which are devoted to inventing medicines that allow patients to live longer, healthier, and more productive lives. PhRMA companies are leading the way in the search for new cures. PhRMA members alone invested an estimated \$39.4 billion in 2005 in discovering and developing new medicines. Industry wide research and investment reached a record \$51.3 billion in 2005. PhRMA Internet address: www.phrma.org.

The U. S. Food and Drug Administration (FDA) is responsible for protecting the public health by assuring the safety, efficacy, and security of human and veterinary drugs, biological products, medical devices, our nation's food supply, cosmetics, and products that emit radiation. The FDA is also responsible for advancing the public health by helping to speed innovations that make medicines and foods more effective, safer, and more affordable; and helping the public get the accurate, science-based information they need to use medicines and foods to improve their health. The FDA Web site is at: www.fda.gov.

The National Institutes of Health (NIH) -- "The Nation's Medical Research Agency" -- includes 27 Institutes and Centers and is a component of the U. S. Department of Health and Human Services. It is the primary Federal agency for conducting and supporting basic, clinical, and translational medical research, and it investigates the causes, treatments, and cures for both common and rare diseases. For more information about NIH and its programs, visit www.nih.gov.

The National Cancer Institute (NCI) is a component of NIH. The NCI coordinates the National Cancer Program, which conducts and supports research, training, health information dissemination, and other programs with respect to the cause, diagnosis, prevention, and treatment of cancer, rehabilitation from cancer, and the continuing care of cancer patients and the families of cancer patients. Additional information about NCI can be found at its Web site, www.cancer.gov.

The National Institute of Mental Health (NIMH) is one of 27 components of the National Institutes of Health (NIH), the Federal government's principal biomedical and behavioral research agency. NIH is part of the U.S. Department of Health and Human Services. The NIMH is the lead Federal agency for research on mental and behavioral disorders. Its mission is to reduce the burden of mental illness and behavioral disorders through research on mind, brain, and behavior. For more information visit: www.nimh.nih.gov.

The National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK) conducts and supports research in diabetes and other endocrine and metabolic diseases; digestive diseases, nutrition, and obesity; and kidney, urologic and hematologic diseases. Spanning the full spectrum of medicine and afflicting people of all ages and ethnic groups, these diseases encompass some of the most common, severe, and disabling conditions affecting Americans. For more information about NIDDK and its programs, see www.niddk.nih.gov.

#####

10.5.2006