Meeting Minutes
Department of Health and Human Services
National Institutes of Health
National Institute of Diabetes and Digestive and Kidney Diseases
February 15, 2012

I. CALL TO ORDER
Dr. Rodgers

Dr. Griffin P. Rodgers, Director, NIDDK, called to order the 188th meeting of the National Diabetes and Digestive and Kidney Diseases Advisory Council at 8:30 a.m., Wednesday, February 15, 2012, in the Natcher Conference Center (Building 45), Conference Rooms E1/E2, on the NIH campus in Bethesda, Maryland.

A. ATTENDANCE – COUNCIL MEMBERS PRESENT

Dr. Domenico Accili
Ms. LaVarne Burton
Dr. Judy H. Cho
Dr. Robert C. Flanigan
Dr. Christopher K. Glass
Dr. Gregory J. Gores
Ms. Jane Holt
Ms. Judy M. Hunt
Dr. Thomas R. Insel
Dr. Francine R. Kaufman
Dr. Kenneth Kaushansky
Dr. David M. Klurfeld
Ms. Robin Nwankwo
Dr. Jerry P. Palmer
Dr. Thomas N. Robinson
Dr. Anil K. Rustgi
Dr. John R. Sedor
Dr. Alan R. Shuldiner
Dr. William D Steers
Dr. Robert A. Vigersky
Mr. John W. Walsh
Dr. Mark L. Zeidel

Also Present:
Dr. Griffin Rodgers, Director, NIDDK, and Chairperson, NIDDK Advisory Council
Dr. Gregory Germino, Deputy Director, NIDDK
Dr. Brent Stanfield, Executive Secretary, NIDDK Advisory Council

B. NIDDK STAFF AND GUESTS

Abankwah, Dora – NIDDK
Abraham, Kristin – NIDDK
Agodoa, Lawrence – NIDDK
Akolkar, Beena – NIDDK
Appel, Michael – NIDDK
Arreaza-Rubin, Guillermo – NIDDK
Atkins, Ronald – CSR
Barnard, Michele – NIDDK
Begum, Najma – NIDDK
Bishop, Terry – NIDDK
Bleasdale, John – CSR
Blondel, Olivier – NIDDK
Josephine Briggs – NCATS
Broitman, Marina – NCATS
Brown, Sherry – NIDDK
Camp, Dianne – CSR
Carrington, Jill – NIDDK
Castle, Arthur – NIDDK
Chen, Hui – CSR
Chen, Richard – NIDDK
Christenson, Dane – Pulm. Hyperten. Assoc.
Cleffi, Katie – RTI International

Connaughton, John – NIDDK
Copeland, Randy – NIDDK
Cowie, Catherine – NIDDK
Curtis, Leslie – NIDDK
Dayal, Sandeep – NIDDK
Doherty, Dee – NIDDK
Donohue, Patrick – NIDDK
Doo, Edward – NIDDK
Drew, Devon – NIDDK
Eggerman, Thomas – NIDDK
Eggers, Paul – NIDDK
Ehrhardt, Britt – NIDDK
Evans, Mary – NIDDK
Everhart, James – NIDDK
Fallon, Erica – NIDDK
Farishian, Richard – NIDDK
Feld, Carol – NIDDK
Flessner, Mike – NIDDK
Fonville, Olaf – NIDDK
Fradkin, Judith – NIDDK
Gansheroff, Lisa – NIDDK
Goter-Robinson, Carol – NIDDK
Giambarresi, Leo – AUA
Graves, Reed – CSR
Grey, Michael – NIDDK
Guo, Xiaodu – NIDDK
Haft, Carol – NIDDK
Hamilton, Frank – NIDDK
Hanlon, Mary – NIDDK
Hardy, Dianne – CSR
Hayward, Anthony – NCATS
Hoff, Eleanor – NIDDK
Horlick, Mary – NIDDK
Hoshizaki, Deborah – NIDDK
Hubbard, Van – NIDDK
Kathy Hudson – NCATS
Hunter, Helen – NIDDK
Hyde, James – NIDDK
James, Stephen – NIDDK
Jenkins, Connie – NIDDK
Jones, Teresa – NIDDK
Karp, Robert – NIDDK
Karimbakas, Joanne – NIDDK
Ketchum, Christian – NIDDK
Khan, Mushtaq – CSR
Kim, Sooja – CSR
Kimmel, Paul – NIDDK
Kirkali, Ziya – NIDDK
Kranzfelder, Kathy – NIDDK
Kuczmarski, Robert – NIDDK
Kusek, John – NIDDK
Laughlin, Maren – NIDDK
Lescheck, Ellen – NIDDK
Linder, Barbara – NIDDK
Malik, Karl – NIDDK
Malozowski, Saul – NIDDK
Maruvada, Padma – NIDDK
Margolis, Ronald – NIDDK
Martey, Louis – NIDDK
Mowery, Penny – NIDDK
McBryde, Kevin – NIDDK
McKeon, Catherine – NIDDK
Miller, David – NIDDK
Miller, Megan – NIDDK
Moxey-Mims, Marva – NIDDK
Mullins, Chris – NIDDK
Narva, Andrew – NIDDK
Nelson, Barbara – NCATS
Newman, Eileen – NIDDK
Panniers, Richard – CSR
Pawlyk, Aaron – NIDDK
Pellnitz, Lori – SRI Inter.
Perry-Jones, Aretina – NIDDK
Pike, Robert – NIDDK
Podskalny, Judith – NIDDK
Polglase, Williams – NIDDK
Portnoy, Matt – OER
Rasooly, Rebekah – NIDDK
Reiter, Amy – NIDDK
Ripley, Justin – PWC
Roberts, Tibor – NIDDK
Rys-Sikora, Krystyna – NIDDK
Salaita, Christine –NIDDK
Salomon, Karen – NIDDK
Sahai, Atul – CSR
Sankaran, Lakshmanan – NIDDK
Sanovich, Elena – NIDDK
Sato, Sheryl – NIDDK
Savage, Peter – NIDDK
Serrano, Jose – NIDDK
Sherker, Averell – NIDDK
Shepherd, Aliecia – NIDDK
Silva, Corrine – NIDDK
Smith, Philip – NIDDK
Sheard, Nancy – CSR
Spain, Lisa – NIDDK
Star, Robert – NIDDK
Staten, Myrlene – NIDDK
Steinberg, Jane – NCATS
Tatham, Thomas – NIDDK
Tuncer, Diane – NIDDK
Van Raaphorst, Rebekah – NIDDK
Viswanathan, Mohan – NCATS
Wallace, Julie – NIDDK
Naus, Wendy – Lewis-Burke
Wellner, Robert – NIDDK
Wright, Daniel – NIDDK
Wright, Elizabeth – NIDDK
Yanovski, Susan – NIDDK
Zhang, Guo – NCATS

C. ANNOUNCEMENTS

Dr. Rodgers made the following announcements:

New Council Members

Dr. Rodgers welcomed four new members to the NIDDK National Advisory Council and expressed his gratitude, on behalf of the NIDDK, for their willingness to take time from their busy schedules to advise the Institute.

Joining the Diabetes, Endocrinology and Metabolic Diseases (DEM) Subcouncil are Drs. Alan Shuldiner and Robert Vigersky.

Dr. Alan Shuldiner is the John Whitehurst Professor of Medicine, Associate Dean for Personalized Medicine, Director of the Program in Personalized Medicine, and Head of the Division of Endocrinology, Diabetes and Nutrition at the University of Maryland School of Medicine. He is a very productive researcher whose major research interests are the molecular basis and genetics of type 2 diabetes, obesity, and insulin resistance. His research is supported by the NIH, the American Diabetes Association and the Juvenile Diabetes Foundation. The NIDDK has supported his research since 1996. Since 1999, he has served on nearly 50 peer review panels as either a standing Study Section member or as an *ad hoc* reviewer. Dr. Shuldiner earned his M.D. from Harvard Medical School. He then completed his residency in Medicine at Columbia-Presbyterian Hospital in New York and a Fellowship in Endocrinology within the Diabetes Branch at the NIDDK.

Dr. Robert Vigersky is a Colonel in the Medical Corps at Walter Reed Army Medical Center, and Medical Director of the Diabetes Institute of the Walter Reed Health Care System. He is also a Professor within the Department of Medicine of the Uniformed Services University of the Health Sciences. He is serving as the Department of Defense *Ex Officio* member on the Council. Dr. Vigersky has had a very distinguished academic and medical career as an endocrinologist working at the NIH, within the military, and in private practice. Over the course of his career, he has published extensively in the peer-reviewed literature on topics ranging from reproductive endocrinology, to diabetes management, to telemedicine and e-health. Dr. Vigersky served as President of the Endocrine Society from 2009 to 2010. He earned his M.D. at Boston University and then did his internship and residency work at the Johns Hopkins Hospital.

Joining the Digestive Diseases and Nutrition (DDN) Subcouncil is Mr. John W. Walsh.

Mr. John W. Walsh will serve as a public member of the Council. After being diagnosed with Alpha-1 Antitrypsin Deficiency (Alpha-1) in 1989, Mr. Walsh co-founded the Alpha-1 Foundation, a not-for-profit corporation dedicated to providing the leadership and resources to increase research, improve health, promote worldwide detection, and find a cure for Alpha-1. Mr. Walsh has an extensive background in business management and government relations. He has served three terms on the Department of Health and

Human Service's Advisory Committee on Blood Safety and Availability (1997-2006); is Immediate-Past Chairperson of the National Health Council's Board of Directors (2005-2006); Past Chair and a member of the American Thoracic Society Public Advisory Roundtable; and a Presidential Appointee of the American Thoracic Society's Board of Directors (2004-2005). Mr. Walsh regularly testifies before Congress and advisory groups as a patient advocate. In 2002, Mr. Walsh's contribution to pioneering collaboration in orphan drug development was recognized by the FDA with the Commissioner's Special Citation. In 2006, he was awarded the prestigious Claude Pepper Memorial Award for Healthcare for his outstanding achievements and contributions to the healthcare industry.

Joining the Kidney, Urologic and Hematologic Diseases (KUH) Subcouncil is Dr. Kenneth Kaushansky.

Dr. Kenneth Kaushansky is Dean of the School of Medicine and Senior Vice President of the Health Sciences for Stony Brook University. Prior to assuming that position in 2012, he served as the Helen M. Ranney Professor and Chair of the Department of Medicine at the University of California, San Diego. From 1987 to 1995, he held a series of positions at the University of Washington--Assistant Professor, Associate Professor, and Professor, and he also served as Hematology Section Chief at the University of Washington Medical Center. A member of the Institute of Medicine, Dr. Kaushansky is a leader in hematology research. He has conducted seminal research on the molecular biology of blood cell production. His team cloned several of the genes important in the growth and differentiation of blood cells, including thrombopoietin, a key regulator of stem cell and platelet production. He is also an accomplished clinician, who has championed the need to train more physician-scientists to bridge the gap between the laboratory and the clinical arena. Dr. Kaushansky earned his M.D. from the University of California, Los Angeles, and completed his Internal Medicine Internship, Residency, Chief Medical Residency, and Fellowship in Hematology at the University of Washington.

Dr. Rodgers also announced that the appointment of Dr. Thomas Insel as a temporary *ex officio* Council member.

Dr. Thomas Insel is Director, National Institute of Mental Health, and Acting Director of the new National Center for Advancing Translational Sciences (NCATS) at the NIH. His appointment as a temporary *ex officio* Council member results from a request by the NIH leadership that the NIDDK's Council temporarily assume the second-tier peer review responsibilities for research funded by the NCATS until the Center's own Council is formed. The NCATS was created in statute by the consolidated appropriations law that is funding the Department of Health and Human Services for the remainder of Fiscal Year 2012. The first set of research applications to be considered for funding by the NCATS was transferred to the NIDDK Council for review following the disbanding of the National Center for Research Resources (NCRR), to which they were initially submitted. Dr. Rodgers indicated that these applications would be reviewed in a special Executive Session of the NIDDK Council.

"In Memoriam"

Dr. Wylie Vale, a long-term NIDDK grantee, passed away in early January 2012. He was a Salk Institute professor and a global authority on peptide hormones and growth factors that provide communication between the brain, endocrine, and immune systems. Dr. Vale and his colleagues characterized the peptide known as corticotropin releasing factor (CRF). They demonstrated that the CRF production by certain brain cells triggered many of the body's hormonal, immune and behavioral responses to stressful situations. Dr. Vale's work revealed that an unusual high production of CRF is associated with several disorders; propelled new methods for the diagnosis of pituitary disease; and opened new possibilities for drug development. Dr. Vale was a member of the Institute of Medicine, and the American Academy of Arts and Sciences. He also served as a president of the American Endocrine Society and the International Society of Endocrinology.

NIDDK Staff Changes

Dr. Rodgers welcomed Ms. Camille Hoover as a new NIDDK staff member, and also reported the retirement of Dr. Patricia Robuck.

Ms. Camille Hoover is the NIDDK's new Executive Officer. Ms. Hoover served as Executive Officer of the National Center for Complementary and Alternative Medicine (NCCAM) since 2000. While there, she played a critical role in the creation and organization of the NCCAM. Earlier in her career, she also served at the NIH Clinical Center and within the NCI's Intramural Research Program. Ms. Hoover has extensive experience in addressing administrative management issues, and is a recognized leader in the NIH corporate community, where she has chaired many trans-NIH committees and workshops.

Dr. Patricia Robuck retired in December 2011 from her position as Senior Advisor for Clinical Trials in Digestive and Liver Diseases within the Division of Digestive Diseases and Nutrition. Dr. Robuck spent 11 of her 27 years of federal civil service with the NIDDK, where she managed a portfolio that included significant involvement with fourteen large multi-center clinical consortia. She was instrumental in the leadership of the Institute's Clinical Studies Working Group and was an important NIDDK liaison with the FDA. The NIDDK benefited greatly from her expertise regarding the protection of human subjects in research, as well as her skill in the design and implementation of clinical research and trials.

New Publication

NIDDK's Recent Advances and Emerging Opportunities provides examples of NIDDK-supported research advances published in Fiscal Year 2011, longer-term stories of discovery, profiles of patients, and scientific presentations made at Council meetings. Dr. Rodgers acknowledged the NIDDK's Office of Scientific Program and Policy Analysis for developing the document, and the extramural and intramural scientific staff for their input. The document is posted on the NIDDK website.

http://www2.niddk.nih.gov/AboutNIDDK/ResearchAndPlanning/Advances/FY2012/default.htm

II. CONSIDERATION OF SUMMARY MINUTES OF THE 187th COUNCIL MEETING

Dr. Rodgers

Following a motion that was made and seconded, the Council approved, by voice vote, the Summary Minutes of the 187th Council meeting that had been sent to them earlier for review.

III. FUTURE COUNCIL DATES

Dr. Rodgers

Dr. Rodgers reminded the Council of future meeting dates.

2012
May 16-17 (Wednesday and Thursday)
September 12-13 (Wednesday and Thursday)
Natcher, Conference Rooms E1/E2, D and F1/F2

2013
February 13-14 (Wednesday and Thursday)
May 15-16 (Wednesday and Thursday)
September 26-27 (Thursday and Friday)*
Building 31, Conference Rooms 10, 6 and 7
* Note divergence from familiar Wednesday and Thursday schedule

The NIDDK expects that most meetings will be a single day. However, Council members were asked to hold two days to ensure flexibility should a situation arise where a longer meeting is required.

IV. ANNOUNCEMENTS

Dr. Stanfield

Confidentiality

Council members were reminded that material furnished for review purposes and discussion during the closed portion of the meeting is considered confidential. The content of discussions taking place during the closed session may be disclosed only by the staff and only under appropriate circumstances. Any communication from investigators to Council members regarding actions on an application must be referred to the Institute. Any attempts by Council members to handle questions from applicants could create difficult or embarrassing situations for the members, the Institute, and/or the investigators.

Conflict of Interest

Advisors and consultants serving as members of public advisory committees, such as the NIDDK Advisory Council, may not participate in situations in which any violation of conflict of interest laws and regulations may occur. Responsible NIDDK staff shall assist Council members to help ensure that the member does not participate in, and is not present during review of applications or projects in which, to the member's knowledge, any of the following has a financial interest: the member, or his or her spouse, minor child, partner--including close professional associates--or an organization with which the member is connected. To ensure that a member does not participate in the discussion of, nor vote on, an application in which he/she is in conflict, a written certification is required. A statement is provided for the signature of the member, and this statement becomes a part of the meeting file. Council members were asked to look at the statement in their folders regarding conflict of interest in their review of applications. Council members were asked to read it carefully, sign it and return it to the NIDDK before leaving.

Council members were reminded that, at Council meetings when applications are reviewed in groups without discussion, that is, "en bloc" action, all Council members may be present and may participate. The vote of an individual member in such instances does not apply to applications for which the member might be in conflict. With respect to multi-campus institutions of higher education: An employee may participate in any particular matter affecting one campus of a multi-campus institution of higher education, if the employee's financial interest is solely employment in a position at a separate campus of the same multi-campus institution, and the employee has no multi-campus responsibilities.

Annual Approval of the Council Operating Procedures

During its annual winter meeting, the NIDDK Council approves its Council Operating Procedures. These Procedures were included for the Council's review in the pre-meeting materials in the Electronic Council Book and were also included in each Council member's folder of materials provided at the meeting. Dr. Stanfield noted that the only change in the operating procedures this year is the inclusion of temporary second-tier review responsibilities for the research applications that will be funded by the new National Center for Advancing Translational Sciences (NCATS). Dr. Stanfield solicited questions or concerns regarding the Council Operating Procedures for 2012, but none were raised. A motion to accept the Council's Operating Procedures for this year was offered, seconded, and approved by voice vote of the Council.

V. REPORT FROM THE NIDDK DIRECTOR
Dr. Rodgers

FY 2012 Appropriations

The Fiscal 2012 budget process was marked by a series of Continuing Resolutions for most federal agencies so that a government shutdown could be avoided. Outstanding funding issues were resolved on December 23, 2011, with the enactment of a consolidated FY 2012 spending package for those agencies that had not received their regular appropriation, including the Department of Health and Human Services. The NIH received $30.8 billion, essentially the same amount as in FY 2011, but about $1 billion less than the President had requested. The final NIDDK appropriation for FY 2012 is $1.797 billion. This amount represents about a 0.3 percent increase over the previous year's funding level for NIDDK, which is similar to the small increase for most Institutes and Centers. For new and competing awards, the NIDDK established a nominal payline of the 13th percentile for Type 1 and Type 2 R01 applications, and a nominal payline of the 18th percentile for Early Stage Investigators (ESIs). ESIs are those New Investigators who are within 10 years of their terminal research degree or medical residency. Grant awards for FY 2012 are subject to programmatic adjustments from the levels approved by the NIDDK National Advisory Council. Dr. Rodgers emphasized that these funding levels pertain to applications to be paid in FY 2012. Many applications submitted in FY 2012 will not be eligible for funding consideration until FY 2013, when new funding policies may apply.

Dr. Rodgers pointed out that the FY 2012 consolidated appropriations act creates in statute a new National Center for Advancing Translational Sciences (NCATS) at the NIH. Initially, this Center will be supported with funds previously targeted for research by the National Center for Research Resources (NCRR), which has been disbanded under the same law. The NCATS is expected to develop new ways of doing translational research. Innovations emerging from the NCATS are intended to reduce the time or expense needed to develop a new drug, or to permit early predictions of the safety and effectiveness of compounds under development. The NIH Director has appointed Dr. Thomas Insel, Director of the National Institute of Mental Health, as the Acting Director of the NCATS. To serve as Acting Deputy Director of the NCATS, he has appointed Dr. Kathy Hudson, Deputy Director for Science, Outreach, and Policy, NIH. They will serve in dual positions while a national search is conducted for the Center's first permanent Director.

FY 2013 Appropriations

The President's Budget Request for Fiscal Year 2013 was released on February 13, 2012 (http://www.whitehouse.gov/omb/budget).

For the NIH, the Request is $30.86 billion, which is essentially the same as the FY 2012 spending level. The request for the NIDDK is about 0.25 percent below the FY 2012 spending level, or approximately $2.8 M less. A Senate hearing on the President's Budget

request for the NIH is set for March 28, 2012. Dr. Rodgers noted that he has been asked to join several other Institute Directors in accompanying the NIH Director to the Senate hearing. The House has not yet scheduled a hearing.

Dr. Rodgers commented on two important points regarding the Fiscal Year 2013 President's Budget request. First, the budget was developed within the context of the Budget Control Act, which incorporates an agreement by the Congress and the President to reduce discretionary spending by $1 trillion over 10 years. Second, this reduction is part of $4 trillion in deficit-reduction proposals that would be implemented over time. It is hoped that such deficit-reduction measures may help federal agencies avoid major across-the-board reductions, which would be triggered by law in January of 2013, because the Select Committee on Deficit Reduction did not produce a 10-year plan for strategically cutting the federal deficit.

Dr. Rodgers said that he was pleased to participate in a briefing on the FY 2013 President's Budget request that was held by the NIH Director for stakeholders on February 14, 2012. Dr. Collins presented an overview of the NIH budget and research emphases, and Institute Directors and representatives discussed research on early human development, AIDS, and Alzheimer's disease. Dr. Rodgers was asked to provide remarks on the NIH's research efforts to combat childhood obesity.

Legislation Reauthorizing Small Business Programs

Dr. Rodgers reported that the Small Business Innovation Research (SBIR) Program and Small Business Technology Transfer (STTR) Program have been reauthorized through 2017. The reauthorization was included in the National Defense Authorization Act of 2012. Importantly, the percentage of extramural research and development funds that must be set aside for these Programs by participating federal agencies will increase over time, starting in FY 2012. Over the period FY 2012 through FY 2017, the funding set-aside for the SBIR Program will gradually increase from the previous level of 2.5 percent to 3.2 percent, while the set-aside for the STTR Program will gradually increase from the previous level of 0.3 percent to 0.45 percent.

VI. "The Patient-Centered Outcomes Research Institute (PCORI)"
Dr. Joseph Selby, Executive Director

Dr. Rodgers introduced Joseph Selby, M.D., M.P.H., the Executive Director of the Patient-Centered Outcomes Research Institute (PCORI). The PCORI was established by law as an independent, non-profit organization. The PCORI's research is intended to help people make informed health care decisions by giving them a better understanding of the prevention, treatment and care options available, and the science that supports those options. Dr. Selby is a family physician, clinical epidemiologist and health services researcher who joined the PCORI from Kaiser Permanente, California, where he was Director of the Division of Research for 13 years. His own research focuses on diabetes outcomes and quality improvement. He was a Commissioned Officer in the U.S. Public Health Service from 1976-1983. He has received a number of awards and honors

including the PHS Commissioned Officer's Award and election to the Institute of Medicine. Dr. Selby received his M.D. from Northwestern University and his Master's in Public Health from the University of California, Berkeley.

Dr. Selby began his presentation by describing the origin and purpose of the PCORI. http://www.pcori.org/
The Institute was established by the 2010 Patient Protection and Affordable Care Act as an independent, non-profit organization. The statute establishing PCORI states: "The purpose of the Institute is to assist patients, clinicians, purchasers, and policy-makers in making informed health decisions by advancing the quality and relevance of evidence concerning the manner in which diseases, disorders, and other health conditions can effectively and appropriately be prevented, diagnosed, treated, monitored, and managed through research and evidence synthesis that considers variations in patient subpopulations and the dissemination of research findings, with respect to the relative health outcomes, clinical effectiveness, and appropriateness of the medical treatments, services, and items…." Dr. Selby noted that the PCORI largely grew out of an interest in comparative effectiveness research, which is intended to provide practical information to patients and their clinicians at the point of decision-making. Thus, conducting and synthesizing research evidence, and disseminating research findings, are crucial components of the PCORI's efforts to foster better health care decisions and outcomes.

The PCORI is not a federal agency. It is governed by a multi-stakeholder, 21-member Board of Governors appointed by the Comptroller General of the United States, Government Accountability Office (GAO). The Board represents the entire health care community, including patients; hospitals, health plans and systems; caregivers, physicians, nurses, and providers; health services researchers; state and local health officials; pharmaceutical, device, and diagnostics manufacturers; and private payers and employers. The PCORI also has a separate, GAO-appointed Methodology Committee composed of national research experts on various aspects of comparative effectiveness research and patient-engaged research. With its focus clearly on patient-centered research, the PCORI and its Board are committed to seeking continuous input from patients and from a broad range of stakeholders to guide its work.

By law, the Institute will sunset at the end of Fiscal Year 2019. Initially funded by an allocation from the Treasury, the PCORI's longer-term funding will be derived from a $1 fee on every Medicare-insured, Medicaid-insured, and employer self-insured enrollees in the U.S. It is expected that annual funding will be approximately $150 million in 2012; $300 million in 2013; and $500 million in 2014 and every year thereafter.

Draft National Research Priorities and Research Agenda

Prior to soliciting and funding any research activities, the PCORI engaged in a five-month interactive process through which it established a proposal for a set of national research priorities and a related national research agenda. The PCORI reviewed the results of other, recent national priority-setting exercises that have been mostly focused on comparative effectiveness research and/or quality improvement in the health arena.

The PCORI then evaluated the results of those exercises in relation to a set of considerations or criteria for PCORI's research investments that were stated in the law establishing the Institute. The considerations include whether research proposals would have: a possible impact on the health of individuals and populations; a likelihood of improving practices and outcomes; inclusiveness of different populations; the potential to address gaps in knowledge and variation in care; a possible impact on health care system performance; the potential to influence decision-making; a commitment to patient-centeredness; rigorous research methods; and efficient use of the PCORI's research resources.

In developing its draft national research priorities and agenda, the PCORI had many informal meetings and interchanges with patients, caregivers, patient advocates and other stakeholders for the purpose of obtaining input and feedback. The PCORI will be obtaining public comments on the draft through several means, including on-line feedback, a national forum of stakeholders on February 27, 2012, and a webcast through which listeners can call in their comments. The public comment period will end on March 15, 2012. Dr. Selby presented the current draft to the Council and elaborated briefly on the priorities.

Draft Research Priority--Assessment of Prevention, Diagnosis and Treatment Options: Comparing the effectiveness and safety of alternative preventive, diagnostic and treatment options, for a wide range of patient-centered outcomes. Draft Research Agenda--Comparisons of alternative clinical options; identifying patient differences in response to therapy; studies of patient preferences for various outcomes. This priority addresses the need to provide evidence-based information to patients and clinicians to drive more effective health care decisions for individuals. Better decisions are expected to lead to better outcomes.

Draft Research Priority--Improving Healthcare Systems: Comparing system-level approaches to improving access, supporting patient self-care, innovative use of health information technology, coordinating care for complex conditions, and deploying the workforce effectively. Draft Research Agenda--Improved support of patient self-management; coordination of care for complex conditions; improvements in the effectiveness and efficiency of care. Because research doesn't always translate immediately into better decision-making, this priority addresses the need for patients and their providers to be supported in making appropriate individual decisions that can lead to better outcomes.

Draft Research Priority--Communication and Dissemination Research: Comparing approaches to providing comparative effectiveness research information and supporting shared decision-making between patients and their providers. Draft Research Agenda--Understanding and enhancing shared decision-making; alternative strategies for dissemination of evidence. This priority addresses the need for improvements in disseminating research information. It is important to know more about the ways that patients and clinicians communicate with each other about research findings and scientific evidence.

Draft Research Priority--Addressing Disparities: Identifying potential differences in treatment effectiveness or preferred outcomes across patient populations and the health care required to achieve best outcomes in each population. Draft Research Agenda--Alternative interventions/strategies to eliminate disparities; improvements in alignment of decisions with preferences. This priority underscores the need to learn more about differences among patient populations. Treatments, preferences for outcomes, and the processing of information about research findings may differ among patient populations and these differences may affect behaviors and study outcomes. The recognition of these differences is a point of similarity between comparative effectiveness research and health disparities research, and to some extent, personalized medicine.

Draft Research Priority--Accelerating Patient-Centered Outcomes Research and Methodological Research: Improving the nation's capacity to conduct patient-centered outcomes research; building data infrastructure; improving analytic methods; training researchers, patients, and other stakeholders. Draft Research Agenda--Improving study designs and analytic methods of patient-centered outcomes research; building and improving clinical data networks; methods for training researchers and patients to participate in patient-centered outcomes research; facilitating the study of rare diseases. This priority underscores the need for a national infrastructure for conducting comparative effectiveness research. This infrastructure should include a data component, training, and ongoing improvements in analytic methods, both experimental and observational, so that the U.S. can monitor outcomes more effectively, more consistently, and more analytically.

Building the Research Portfolio

Dr. Selby emphasized that the PCORI's initial research agenda is very broad, without a focus on any specific health conditions. The Institute remains open to funding compelling patient-oriented research that meets its priorities and agenda. The PCORI recognizes that a variety of study designs and approaches may contribute valid new knowledge about the comparative clinical effectiveness of specific strategies. Dr. Selby gave some examples of the types of studies the PCORI might fund. Studies could include comparing the effectiveness of two or more strategies for prevention, treatment, screening, diagnosis, or surveillance that have not been adequately studied and where better evidence would be helpful to support decision-making by patients, caregivers, and health care professionals. It would be important that research studies generate data that does not come exclusively from traditionally designed clinical trials conducted in very targeted, narrowly defined patient populations, with exclusionary criteria. Rather, a goal would be to generate data from real-world clinical practice situations, with the consideration of the full range of patient-centered outcomes, and close attention to the possibility that treatment effects may differ across patient sub-populations. Studies that examine individual differences in patient values and preferences could support shared decision-making. To develop these types of studies will require advances in analytic methods, novel approaches, and ways to increase patient enrollment in studies that can be performed in settings based on real-world clinical practice. Randomization can be achieved, but probably through a

"clustered" approach, rather than at the level of individual patients. Dr. Selby expressed his hope that a variety of study designs and approaches can be undertaken to tailor therapies to patient needs.

As the PCORI builds its research portfolio, Dr. Selby expects that some of its activities will focus on targeted strategic areas, including disease areas, whereas other activities will be pursued through an open-ended process in which researchers can submit their ideas. After the public comment period ends, the PCORI's Board will accept a final version of the national research priorities and agenda. Then, the PCORI will pursue a joint strategy of bottom-up and top-down research initiatives that are aligned with its stated research priorities and agenda.

The PCORI will seek funding partnerships with other organizations as appropriate, but will also seek to distinguish itself through independent work. With specific regard to contracts, Dr. Selby noted that the law establishing the Institute authorizes contracts for the management of funding and the conduct of research in accordance with appropriate agencies and instrumentalities of the Federal Government and appropriate academic research, private sector research, or study-conducting entities. The law also says that preference in entering into contracts shall be given to the Agency for Healthcare Research and Quality (AHRQ) and the NIH.

Dr. Selby expects that the PCORI's first standing research announcements, similar to NIH Program Announcements, will be issued in mid-May 2012, with one for each of the five priorities. The first research applications in response to those announcements will be accepted in mid-July and every four months thereafter, and the PCORI will partner with the NIH Center for Scientific Review in assessing them. The PCORI expects to fund approximately $90 million in research through this broad initiative, with funding commitments made by the end of 2012. In parallel strategic efforts, the Institute will also begin issuing conference grants, convening brainstorming workshops, and forming advisory groups for the purpose of developing a more refined, targeted research agenda and subsequent *ad hoc* funding announcements that may be issued as early as August 2012. The Board and Methodology Committee may also generate some additional research initiatives.

Council Questions and Discussion

Prevention: *In the PCORI's interactions with patient groups, was the topic of disease prevention efforts by individuals brought up, or is this concept centered in the arena of the health care system?* Dr. Selby replied that prevention was mentioned by stakeholders and throughout the legislation that established the PCORI. The very first national research priority incorporates the assessment of prevention strategies. Because the PCORI is charged with doing comparative clinical effectiveness research, it is likely that the PCORI's prevention efforts will focus on illnesses, interventions, and outcomes that are being addressed within the medical care system. However, the PCORI has not drawn any boundaries concerning the types of research that it will or will not support.

Evaluation: *How is the PCORI handling the definition, goals and expectations for its success so that the Congress can evaluate whether the Institute should be extended beyond its current sunset date of 2019? Given the limited budget that the PCORI has to accomplish a very broad mission, what will be the PCORI's primary measures of success against which it can be evaluated when it sunsets--changes in decision-making, outcomes, or costs?* Dr. Selby replied that the PCORI wanted to have a very broad research agenda initially, but realizes that it will have to develop a more targeted agenda, starting in 2012, through a consensus-building process with stakeholders. The size of the PCORI budget means that it will not be possible for the Institute to tackle every issue and health condition, and therefore, it may be criticized by those who feel that their concerns are not being addressed. However, the PCORI hopes to forge consensus through workshops, advisory panels, standing committees, and other means of engaging stakeholders in a deliberative process. With respect to outcome measures for the PCORI, Dr. Selby said that cost issues are not the PCORI's primary concern. Rather, the Board and the staff seek to affect the way that research is conducted by providing a heightened awareness of the importance of engaging patients, clinicians, clinical organizations, and groups that develop clinical guidelines. By funding and reporting on methodological research advances, the PCORI will demonstrate that engagement of stakeholders can significantly change the kind of research performed and also aid in the translation of that research.

Types of Studies: *What would distinguish a grant that goes to PCORI versus one that goes to the NIH or to the Agency for Healthcare Research and Quality (AHRQ)?* Dr. Selby said that the PCORI will be in a better position to answer that question following the receipt of stakeholder input on its draft national research priorities and agenda. However, funding announcements will definitely solicit proposals for both randomized and observational studies along the lines of the five draft priorities. As for distinguishing features, applications to the PCORI will need to demonstrate that there is a need to be addressed, and that patients or other relevant stakeholders were engaged in developing the research application and will be engaged during performance of the research. Also, applications will likely need to have a plan for implementing or disseminating the research findings. The PCORI applications will also have distinguishing characteristics in the review process. The Study Sections that review them will have three members who are non-scientist patients or other stakeholders and who have been trained to represent and articulate stakeholder perspectives. Moreover, in addition to being reviewed on the five current NIH peer review criteria, the PCORI applications will be subject to a sixth review criterion called "Patient and Stakeholder Engagement," which will be built into the scoring system.

Stakeholder Involvement: *How is the PCORI planning to continue stakeholder involvement, especially among patients with multiple chronic conditions that negatively affect quality of life and pose a huge cost burden?* Dr. Selby responded that the PCORI's commitment to patient-centered research is reflected in its hiring decisions. Four of the current 15 employees are dedicated to patient engagement. They plan to build a community of patients willing to serve on working groups, advisory panels, and Study Sections. The PCORI will also engage this patient community for surveys and ongoing

dialogue. Patients with multiple chronic conditions will play a very important role in the PCORI's efforts to further patient-centered research because of the complex therapeutic challenges they present.

Existing Research Base: *How does the PCORI plan to capitalize on the AHRQ and NIH portfolio of comparative effectiveness research that is currently funded and available?* Dr. Selby noted that the NIH Director and the Director of the Agency for Healthcare Research and Quality (AHRQ) are on the PCORI's Board. They are both very interested in ensuring that the PCORI's activities complement the efforts of their agencies at the levels of infrastructure, evidence synthesis, and comparative clinical effectiveness research.

Clinical Trials: *If the PCORI is to achieve its goal of delivering better health care to patients, isn't it essential to give high priority to improving the design of clinical trials so that issues surrounding end-points, patient selection, patient information, and patient compliance don't impede meaningful results?* Dr. Selby noted that there will be opportunities to partner with the NIH to make contributions regarding patient-centered design of clinical research and trials, and analyses of the comparative benefits of clinical interventions.

Disease Diversity: *What will the PCORI do to address the challenge of the mounting diversity of human disease and the diversity of humans who have disease?* Dr. Selby noted that this issue is of great concern to the PCORI Methodology Committee, which may pursue better ways to analyze clinical trial data. Moreover, it is possible that treatment heterogeneity among patients may be informed through analyses of large observational data sets that have gene expression tests. While the insights gained would not be definitive, they could help generate testable hypotheses about patient subgroups and their characteristics.

Dissemination of Results: *It will be imperative to disseminate PCORI's research results; is there a dissemination plan and a plan for training future investigators in the methodologies that have been developed?* Dr. Selby said that dissemination of results is extremely important to the PCORI. Currently, 16 percent of the PCORI's funds are earmarked for dissemination and translation efforts to be undertaken by the Agency for Healthcare Research and Quality (AHRQ), and four percent is earmarked for the Department of Health and Human Services. One of the questions the PCORI is now considering is whether even more of its resources need to be targeted to dissemination. It is likely that the AHRQ will continue to disseminate the PCORI's research results if the Institute is not extended beyond its current 2019 sunset date.

Partnerships with the NIH: *Will the PCORI partner with NIH Institutes and Centers in joint Requests for Applications and other research solicitations in areas of shared research interest and methodologies, and if so, what will that process be like?* Dr. Selby replied that both the PCORI and the NIH leadership are interested in exploring possible partnerships. Meetings will be held between the PCORI and the NIH to discuss the

framing of those kinds of opportunities. For example, the PCORI may be able to make a patient-centered or analytic contribution to NIH clinical trials.

VII. ADVISORY COUNCIL FORUM: "The NIH Small Business Innovative Research (SBIR) Program and Small Business Technology Transfer (STTR) Program"

Dr. Rodgers introduced the Council Forum. He said that the NIDDK is seeking the Council's advice regarding ways that the Institute can make the most effective use of funds that are legislatively mandated for the Small Business Innovative Research (SBIR) Program and the Small Business Technology Transfer (STTR) Program. Both Programs are intended to support innovative research, with the potential for commercialization, which is conducted by small businesses. They are "set-aside" Programs whose funding, as specified in statute, is a percentage of the extramural research and development funds of 11 federal agencies, including the NIH. Thus, while funds for these Programs are set-aside from the NIH budget, the SBIR-STTR applications compete against each other within their own pool of separate funds. They don't compete directly with applications in other NIH Programs.

A. "The NIH SBIR-STTR Program and Reauthorization"
Dr. Matthew Portnoy, Coordinator, NIH SBIR and STTR Programs, and Director, Division of Special Programs, Office of Extramural Research, NIH

Dr. Portnoy presented an overview of the NIH Small Business Innovation Research (SBIR) Program and the Small Business Technology Transfer (STTR) Program. http://grants.nih.gov/grants/funding/sbir.htm

Dr. Portnoy noted that both Programs have recently been reauthorized through 2017, as part of the National Defense Authorization Act for FY 2012. The reauthorization provides the most sweeping changes to the Programs since the legislative establishment of the SBIR Program in 1982 and the STTR Program in 1992. Dr. Portnoy pointed out that both Programs are overseen and coordinated by the Small Business Administration (SBA) for the NIH and other participating federal agencies. The SBA is developing draft documents for public comment regarding implementation of the reauthorization changes. Subsequently, the SBA will issue new regulations and policy directives outlining the specific ways that changes made by the law will be effected. Until that occurs, the Programs will continue to operate under existing regulations and procedures, consistent with the SBA's interim guidance.

Goals and Phases of Programs

Dr. Portnoy emphasized that the reauthorization does not change the Congressional intent for the Programs. The SBIR Program will continue to foster cooperative research between federal agencies and small businesses. It will continue to pursue its goals of stimulating technological innovation; using small businesses to meet federal Research and Development (R&D) needs; fostering and encouraging participation by minority and

disadvantaged persons in technological innovation; and increasing private-sector commercialization of innovations derived from Federal research and development investments. The STTR Program has very similar goals, except that it fosters cooperative research and technology transfer between a small business, which is the applicant, and a not-for-profit research institution, which is typically but not necessarily a university.

The Programs will also continue to have a phased approach. A Phase I award supports a feasibility or proof-of-concept study that is relatively small in funding and duration. Phase II is a full research and development project aimed toward commercializing a specific product or technology. Currently, it is not possible to apply for a Phase II award without having completed a Phase I project; however, that may change as a result of the reauthorization. Phase III of the SBIR Program is the commercialization stage for a prototype or product that has been developed in Phase II. It is a point at which regulatory approval, manufacturing or other steps are necessary before marketing. It is congressionally prohibited to use SBIR-STTR funding for Phase III; however, federal agencies can use other funds to support this phase if they wish.

Examples of Changes Made by Reauthorization

Some of the main elements of the reauthorization were described by Dr. Portnoy. Perhaps the most significant change is that the percentage which participating agencies must set aside from their extramural research and development funds to support these Programs will increase gradually from FY 2012 through Fiscal Year 2017. In FY 2011, the set-asides for the SBIR and STTR Programs, respectively, were 2.5 percent and 0.30 percent. In FY 2012, the respective set-asides will be 2.6 percent and 0.35 percent. By 2017, the respective set-asides will reach 3.2 percent and 0.45 percent. Under the new law, the guidelines regarding funding amounts will be made consistent for both SBIR and STTR awards--at $150,000 in total costs for Phase I and $1,000,000 in total costs for Phase II. Moreover, new "hard" funding limits or "caps" will preclude awards for either Program that are greater than 50 percent above the guidelines, unless a waiver is granted by the SBA. This is an important change for the NIH, which currently tends to exceed existing, flexible funding guidelines because of the cost of clinical research. Other facets of the new law include some changes in eligibility criteria for SBIR applicants; expanded opportunities for venture capital participation in SBIR; an increase in the amount of funding to support technical assistance for both SBIR and STTR Programs; a new authority to use a percentage of SBIR funds for administration, outreach and program management; and continued study of the Programs by the National Academies of Science.

Features of NIH Programs

Dr. Portnoy described some of the current features of the NIH SBIR-STTR Programs. With respect to funding, the combined FY 2011 budget for NIH SBIR and STTR Programs was $682 million--$609 million for SBIR and $73 million for STTR. Of the approximate total of $2.5 billion set aside for these Programs across the federal government, the NIH amount was the second largest, following the $1.4 billion spent by

the Department of Defense. About 95 percent of NIH SBIR-STTR funds are spent through the research grant mechanism and the remainder through research contracts. Within the NIH, the SBIR-STTR set-aside is allocated to the participating NIH components in a way that is proportional to their budgets. The NIDDK is usually the fourth largest participant. Dr. Portnoy noted that the SBIR-STTR budgetary breakouts for FY 2012 and beyond will be complicated because the amount of the set-aside is increasing under the reauthorization law, and in addition, new parts of the NIH will have these Programs.

Dr. Portnoy presented FY 2011 data on the NIH SBIR-STTR Programs. The NIH received over 6,400 SBIR-STTR applications and made about 900 awards for an overall 14 percent success rate. For the SBIR Phase I, the success rate was about 11.5 percent, which tends to be fairly comparable to other participating federal agencies. For the SBIR Phase II, for which applicants must have completed a Phase I project, the FY 2011 success rate was close to 30 percent. Success rates were higher for the STTR Program, which has a much smaller set-aside of funds and a much smaller pool of applicants.

The NIH takes several steps to build its SBIR-STTR Programs. About 70 percent of SBIR-STTR awards are made to applications in response to the NIH omnibus Funding Opportunity Announcement. The NIH accepts SBIR-STTR applications in response to the FOA during three standard receipt dates each year. Investigators are also encouraged to apply to targeted Program Announcements, Requests for Applications, or Requests for Contracts. The NIH SBIR-STTR also features some specialized technical assistance. For example a "Niche Assessment" can give Phase I SBIR applicants a market analysis of their work. In addition, the NIH also offers technical assistance to applicants who are trying to move from Phase II to Phase III--a transition point often called the "Valley of Death" because many ideas die due to difficulties in commercialization. For Phase II SBIRs, a "Commercialization Assistance Program" (CAP) provides applicants with hands-on training about business and strategic planning. There is a competing renewal opportunity for Phase II SBIR awardees in recognition that pre-clinical work or early-stage clinical trials are extremely expensive, time-consuming, and likely to involve regulatory requirements. The NIH is also encouraging joint efforts between small businesses and NIH intramural laboratories to speed technology transfer to the marketplace. Typically, the NIH does not procure products or technology developed by its SBIR-STTR awardees. Rather, the NIH usually wants the results of the innovative research to move directly into the open market place. Thus, the NIH generally assesses the success of the SBIR-STTR Programs by asking: "Is the company selling the product or technology? What is the return on investment?" The NIH tracks the outcomes of the SBIR Program through an integrated, flexible database, the "Performance Outcomes and Data Systems," or PODs. Currently for internal use, PODs will soon be opened up to companies to upload their own data so that program analyses can be performed.

Dr. Portnoy's office, located within the NIH Office of Extramural Research, coordinates policies with the Institutes and Centers, the SBA, and other agencies for which the NIH provides centralized administrative services for these two Programs. His office is responsible for policy reporting, the development of centralized ("parent") funding

announcements, and outreach efforts. The Institutes and Centers are given as much administrative flexibility as possible in operating the Programs, provided that they stay within the confines of the legislation. Most Institutes and Centers, including the NIDDK, follow a distributed model in which organization's SBIR-STTR portfolio is spread among Program Directors who manage larger portfolios; one or more of these Program Directors may have a "lead" role in coordinating the Programs.

Dr. Portnoy closed by stating that, following receipt of the SBA's policy directives regarding implementation of the new reauthorization, his office will work closely with the Institutes and Centers to implement necessary changes in the NIH SBIR-STTR Programs.

B. "NIDDK SBIR-STTR Programs"
Dr. Marva Moxey-Mims, Deputy Director, Division of Kidney, Urologic, and Hematologic Diseases, and Division Coordinator for SBIR-STTR Programs

Dr. Moxey-Mims said that the NIDDK works closely with the NIH SBIR-STTR Coordinator, Dr. Portnoy, to clarify policies, procedures and other program requirements and to harmonize activities. The NIDDK also participates in several trans-NIH SBIR-STTR efforts, including collection of outcomes data, technical assistance programs such as the "Commercialization Assistance Program" (CAP), annual conferences, and trans-NIH Funding Opportunity Announcements. Dr. Moxey-Mims presented a list of some of the trans-NIH solicitations in which the NIDDK has participated. She noted that the NIDDK also participates in the Phase II competitive renewal program when the Institute believes that a product or technology is very close to commercialization and the NIDDK investment could make a difference.

Profile of the Programs

With a few exceptions, the NIDDK general strategy for its SBIR-STTR Programs is the same one it employs for its regular research grant (R01 grant) portfolio, that is, to fund investigator-initiated research grants, rather than to issue targeted research solicitations. Other NIH components vary in their approaches; for example, the National Cancer Institute takes a targeted approach through extensive use of research and development contracts.

Dr. Moxey-Mims presented data on several aspects of the NIDDK SBIR-STTR Programs.

- ***Types of Applicants:*** Data averaged over the FY 2009 through FY 2011 time period show that the majority of applications and awards are for the Phase I SBIR Program. The main reason for the small numbers of Phase II SBIR applications is that II applicants must have completed a Phase I project. Over the FY 2006 through FY 2010 period, 63 percent of SBIR-STTR applicants held Ph.D.s, compared with 58 percent of R01 grant applicants. The percentages of M.D. and M.D./Ph.D. applicants were much lower for the SBIR-STTR Programs than for the R01 Program. About twenty-one

percent of the SBIR-STTR applicants either had other degrees, or did not provide information about their degree status.

- ***Funding Rates:*** Over time, the funding success of the NIDDK's SBIR-STTR applications has been reasonably comparable to data on the NIDDK R01 Program and the NIH-wide SBIR-STTR Program. Data for the FY 2009 through FY 2011 period show that more than 15 percent of the NIDDK's Phase I SBIR-STTR applications were funded, and more than 35 percent of Phase II SBIR-STTR applications were funded.

- ***Size of Awards:*** Dr. Moxey-Mims said that the NIDDK has used the current flexibility afforded by the Programs to fund some applications at levels higher than the guidelines. Phase II awards are the largest. In FY 2011, the average total cost of a Phase I SBIR award was well over $400,000 and the average total cost of a Phase II STTR award was approximately $1,000,000.

- ***Types of Research Projects:*** Dr. Moxey-Mims showed data on applications and awards to seven categories of research (Behavior, Biologic, Device, Diagnostic, Drug, IT and Tool) and noted that the proportional numbers of awards made in a given research category generally paralleled the numbers of applications submitted to that category. Although there is some overlap among research categories, the largest one is device development, which accounts for nearly 35 percent of the NIDDK SBIR-STTR applications and awards. The second largest category is drug development. The next major categories are the development of tools and biologics.

- ***Progression to Phase II:*** From FY 2000 through FY 2009, 54 percent of the SBIR Phase I awardees applied for a Phase II award, and 31 percent of those who applied were successful. Comparable data on the STTR Program show that 45 percent of the Phase I awardees applied for a Phase II award, and 30 percent were successful.

Dr. Moxey-Mims gave several examples of successful products that have been generated with support of the NIDDK SBIR-STTR Programs. These examples include: a system to generate images of motor function in the gastrointestinal tract; a monitor of caloric expenditure via an audio ear bud; recombinant thyroid stimulating hormone; continuous glucose monitors; a "smart insulin" polymer that senses glucose and releases insulin; an interactive computer education program to teach self-management skills to children with diabetes; improved dialysis catheters; methods for blood flow measurement in dialysis access; and a dissolvable surgical gel to stem blood flow temporarily during surgery that requires the joining of blood vessels. Dr. Moxey-Mims pointed out that there are many information-technology applications funded through the SBIR-STTR Programs that would be difficult to fund through other research mechanisms.

Challenges and Options for Program Development

The NIDDK has encountered some challenges with its SBIR-STTR Programs. Dr. Moxey-Mims said that these challenges include: inexperienced applicants who lack knowledge of grantsmanship; the lack of details in applications due to the applicant's

concerns about proprietary rights; difficulties in assessing the commercial potential of proposals; the lack of metrics for meaningful evaluation of the portfolio; applicants who repeatedly apply for and receive Phase I awards but never progress to the next phase of the Program; and the special challenges of Phase II, including the inexperience of applicants in dealing with FDA regulatory procedures for clinical research.

The NIDDK is seeking the Council's advice regarding ways that it can maximize the set-aside funding for SBIR-STTR Programs--funding that is required by legislation and that will be increasing over the next few years. Dr. Moxey-Mims outlined some of the questions the NIDDK is trying to answer. For example, what strategies can the Institute use to generate opportunities in these Programs for the NIDDK patient and scientific communities? Should the NIDDK continue an investigator-initiated approach or take a more targeted approach? How can the NIDDK assess when a specific area is ripe for a targeted opportunity? Should there be more of a focus on Requests for Applications or the contract mechanism? What is the appropriate balance of funding and flexibility between Phase I and Phase II projects? How can these Programs and portfolios be meaningfully evaluated in a timely manner at the NIDDK and NIH levels--and by what metrics? Are there ways to incentivize productive partnerships that have not yet been explored with industry and/or investors?

Dr. Moxey-Mims thanked other NIDDK Program staff who contributed to the presentation, including Guillermo Arreaza-Rubin, Christine Densmore, Teresa Lindquist, and Karl Malik.

Council Questions and Discussion

Venture Capitalists: *Investors may be funding the most promising or easy-to-commercialize ideas before they can make it into SBIR-STTR applications. As a result, the remaining concepts may be considered too risky or not of sufficient promise for venture capitalists to pursue through the SBIR-STTR Programs.* Dr. Portnoy noted that the reauthorization makes changes in the SBIR-STTR programs that may make them more appealing to venture capitalists. However, concerns were expressed during development of the new legislation that very well-funded companies could drive out small companies that may be spin-offs of university research. For that reason, there remain certain limitations on the participation of venture capitalists.

Evaluation: *The central NIH database, PODs, could be an important evaluation tool. Using PODS, it may be possible to determine success rates for the SBIR Program since its inception. Data on Phase II studies could indicate which concepts became commercialized and profitable, and whether they originated with targeted or unsolicited research. The NIH could also draw upon the expertise of MBAs in promoting and evaluating business success.* Dr. Portnoy said that centralized tracking of the outcomes of the SBIR-STTR Programs is now more important than ever for the NIH, and that the SBA is also interested in centralizing government-wide data on commercialization. Currently, the PODs database captures data only on companies that go through the Commercialization Assessment Program (CAP), but it can be expanded. However, it is

challenging to incentivize companies to enter their data into PODs and there are also OMB requirements that must be met regarding data collection. Another issue is that many companies funded years ago may no longer exist. These are some of the impediments that will need to be overcome, and it may be possible to use the Internet and other information in the public domain to help surmount them. Dr. Moxey-Mims noted that she submitted a concept to capitalize on the business expertise of students in the Capstone Program at Johns Hopkins. Now, a group of MBA students there is working to develop a guide by which laboratory investigators with good ideas, but no marketing experience, can use the SBIR-STTR Programs to achieve commercialization.

Examples of Success: *Given that grantsmanship experience is generally lacking among the SBIR-STTR applicants, would it be useful to provide investigators and reviewers with narrative examples of research products and technologies that moved successfully into the market place? Such examples would also help to demonstrate the success of the Programs to the Congress, and to the public via the NIH website. However, because they would be anecdotal, such success stories also need to be complemented by rigorous studies of return on investment.* Dr. Portnoy responded that his office is revamping the NIH SBIR-STTR website and plans to include examples of success stories, as well as sample applications. He noted that, each year, fully one-third of all applications and awards involve brand new companies that need to learn about the process for writing applications, particularly the information that needs to be included for reviewers to make fair assessments of the proposed research.

Council members also offered the following comments:

Program Balance: *A greater use of targeted research solicitations may be necessary and beneficial in certain areas. Given that the amount of the set-aside is increasing, it may be an appropriate time to give greater emphasis to targeted solicitations that could be aimed at filling unmet strategic goals and needs. Perhaps the NIDDK could issue targeted Program Announcements and Requests for Applications focused on common diseases within the mission of the NIDDK, and thus likely to produce benefits in terms of health prevention and disease therapy. A targeted approach could also benefit research on orphan diseases.*

VIII. SCIENTIFIC PRESENTATION: "Preventing Childhood Obesity"

Dr. Thomas Robinson, Irving Schulman Endowed Professor in Child Health and Professor of Pediatrics and of Medicine, Division of General Pediatrics at Stanford University School of Medicine; and Director of the Center for Healthy Weight, Stanford University

A member of the NIDDK Advisory Council, Dr. Robinson focuses on "solution-oriented" research, that is, developing and evaluating health promotion and disease prevention interventions for children, adolescents and their families to directly inform medical and public health practice and policy. His research is largely experimental in design, including the conduct of randomized controlled trials that are based in the school, family and community. Dr. Robinson received his M.D. from Stanford University and his M.P.H.

in Maternal and Child Health from the University of California, Berkeley. He completed his internship and residency in Pediatrics at Children's Hospital, Boston, and Harvard Medical School, and then returned to Stanford for post-doctoral training as a Robert Wood Johnson Clinical Scholar.

A total of 1456 grant applications, requesting support of $381,502,039 were reviewed for consideration at the February 15, 2012 meeting. Funding for these applications was recommended at the Scientific Review Group recommended level. Prior to the Advisory Council meeting, an additional 1227 applications requesting $317,223,541 received second-level review through expedited concurrence. All of the expedited concurrence applications were recommended for funding at the Scientific Review Group recommended level. The expedited concurrence actions were reported to the full Advisory Council at the February 15, 2012 meeting.

IX. CONSIDERATION OF REVIEW OF GRANT APPLICATIONS

NIDDK Applications

A total of 1456 grant applications, requesting support of $381,502,039 were reviewed for consideration at the February 15, 2012 meeting. Funding for these applications was recommended at the Scientific Review Group recommended level. Prior to the Advisory Council meeting, an additional 1227 applications requesting $317,223,541 received second-level review through expedited concurrence. All of the expedited concurrence applications were recommended for funding at the Scientific Review Group recommended level. The expedited concurrence actions were reported to the full Advisory Council at the February 15, 2012 meeting.

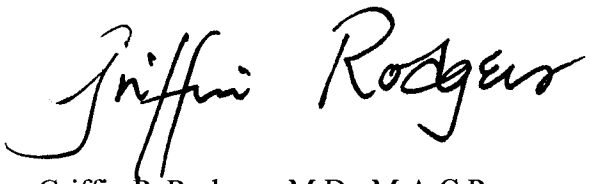
NCATS Applications

A total of 42 grant applications, requesting support of $115,063,430 were reviewed for consideration at the February 15, 2012 meeting. Funding for these applications was recommended at the Scientific Review Group recommended level.

X. ADJOURNMENT

Dr. Rodgers expressed appreciation to all the presenters. He thanked the Council members for their attendance and valuable discussion. There being no other business, the 188th meeting of the NIDDK Advisory Council was adjourned at 4:30 p.m., February 15, 2012.

I hereby certify that, to the best of my knowledge, the foregoing summary minutes are accurate and complete.



Griffin P. Rodgers, M.D., M.A.C.P.
Director, National Institute of Diabetes and Digestive and Kidney Diseases, and
Chairman, National Diabetes and Digestive and Kidney Diseases Advisory Council