

Appropriations Subcommittee Statement on the Fiscal Year 2013 Budget

Open Statement for the Record House Subcommittee on Labor-HHS-Education Appropriations configuration options

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Mr. Chairman and Members of the Committee:

I am pleased to present the President's budget for the National Institute of General Medical Sciences (NIGMS) of the National Institutes of Health (NIH). The FY 2013 budget of \$2,378,835,000 includes a decrease of \$48,354,000 below the comparable FY 2012 level of \$2,427,189,000.

This year, in 2012, the National Institute of General Medical Sciences (NIGMS) celebrates its 50-year anniversary as NIH's "basic research institute." Since 1962, NIGMS has continuously supported highly creative people committed to building a broad and deep foundation of discovery. The findings are used and applied by scientists everywhere, leading to new diagnostics, new therapies, and new ways to prevent a wide range of diseases.

Model Systems Illuminate Human Health

Laboratory-animal versions of disease are a staple of basic biomedical and behavioral research. Because fruit flies, worms, mice, and other animals are easy and relatively inexpensive to work with—and have most of the same genes and many of the same behaviors as we do—they are valuable tools for biomedical discovery. Sometimes, though, results with animal models do not hold up in human studies, in part because organisms studied in the laboratory lack the genetic diversity of people. NIGMS has addressed this problem through its support of the Collaborative Cross, a large-scale mouse-breeding project that significantly expands the genetic diversity of mice. This project has made its resources widely available to scientists everywhere—helping to fast-track important discoveries about genetics and human disease.

Other recent studies with model systems, in this case worms, have pointed to new information about a group of neurological diseases that have a common molecular defect: the inability of normal cellular proteins to fold themselves into their proper three-dimensional shapes. Misfolded proteins are implicated in Alzheimer's, Parkinson's and Huntington's diseases, amyotrophic lateral sclerosis, cancer, cystic fibrosis, and type 2 diabetes. The recent work identified new genes and signaling pathways that keep proteins

folded properly and prevent toxic clumping. The researchers also extended their findings by identifying small molecules that appear to repair misfolded proteins.

All Systems Go

While animal models offer key clues to understanding human disease, other studies that investigate large, interacting systems are an essential avenue for learning about health and disease. Systems biology approaches, which promote a more thorough grasp of the intricate and dynamic workings of how molecular and cellular parts interact to make a whole, is a robust area of NIGMS-funded biomedical research.

Human behavior is one example of an enormously complicated system—not just for an individual, but also between individuals and within and between populations. Systems biology research employing mathematical models can draw connections among a vast number of inputs, uncovering new connections and making new predictions. NIGMS has joined forces with the NIH Office of Behavioral and Social Sciences Research to identify opportunities, challenges, and gaps in knowledge needed to develop useful models of social behavior. This past fall, NIGMS issued a call for funding research that models social behavior. The new program has generated substantial interest in the research community, and the Institute is looking forward to the results that are likely to have broad application.

Another scientific area of great complexity, even though the subject of study is microscopic, is the interactions between viruses and their hosts. For many years, NIGMS has funded the AIDS-Related Structural Biology Program to obtain the three-dimensional structures of HIV proteins. Representing the culmination of hundreds of studies, researchers have just published a map of nearly 500 physical interactions between components of HIV and those in human cells. The research provides a gold mine for further studies into new drugs and vaccines against HIV.

Accelerating the Pace of Discovery

As our world has flattened due to increased human travel and expanded commercial trade among many international partners, a number of new diseases have emerged and infected people around the world. To help the nation and the world understand and prepare for contagious outbreaks, NIGMS funds the Models of Infectious Disease Agent Study (MIDAS). This international effort continues to add new research expertise to increase its capacity to simulate disease spread, evaluate different intervention strategies, and help inform public health officials and policymakers. In 2011, MIDAS scientists used whole-genome sequencing to trace the path of the *E. coli* outbreak that made thousands of people ill and killed over 50 people in Germany and France. The project demonstrates the power of

modeling and is one of the first uses of genetic detective work to study the dynamics of a food-borne outbreak.

The NIGMS investment accelerates the pace of discovery through its support of chemistry projects that enable biologists to study cells and organisms using state-of-the-art chemical tools; help clarify the chemical reactions that underlie human metabolism; and provide new strategies for drug development. NIGMS-supported chemists recently made two new discoveries that should enhance the manufacture of key drugs. In the first study, scientists made significant progress toward a simpler, more efficient way to synthesize Taxol, an important cancer drug used routinely to treat ovarian, breast, lung, liver, and other cancers. In a second study, NIGMS-funded chemists unveiled the working parts of the commonly used anti-fungal medicine amphotericin B, nicknamed by physicians “ampho-terrible” for its harsh side effects. The new work opens up possibilities for designing similar anti-fungal medicines that are just as effective but easier on the body.

Investing in the Future of Discovery

The Institute believes that a strong biomedical research workforce is essential for the tandem goals of improving health and maintaining global competitiveness. In 2011, NIGMS published “Investing in the Future: the NIGMS Strategic Plan for Biomedical and Behavioral Research Training.” Implementation of this plan is now in full swing. Going forward, NIGMS has articulated clearly that research training is a partnership between the NIH and the academic community and continues to engage actively with its full range of stakeholders. Key foci include the importance of excellent mentoring, a continuing emphasis on diversity, and the need to recognize a full menu of career options beyond academic research for newly trained scientists.

NIGMS has also recently established a new organizational component, the Division of Training, Workforce Development, and Diversity, which integrates training, diversity, and capacity-building activities across Institute programs. This new component also oversees the Institutional Development Award (IDeA) program that broadens the geographic distribution of NIH funding. A new component of this effort is the IDeA Program Infrastructure for Clinical and Translational Research initiative, which encourages applications from IDeA states to develop infrastructure and capacity to conduct clinical and translational research on diseases that affect medically underserved populations and/or diseases prevalent in these 23 states and territories traditionally underfunded by the NIH.

Extending the Reach of Basic Research

Within the clinical realm, NIGMS continues to support the NIH Pharmacogenetics Research Network (PGRN), now in its 12th year of funding. This endeavor has yielded a bounty of medically relevant knowledge, including how genetic information can help predict how heart drugs, cancer medicines, nicotine patches, and a range of other treatments are likely to work in a particular person. One PGRN project is now partnering with the Electronic Medical Records and Genomics (eMERGE) Consortium to test samples from people whose electronic medical records are also available to the researchers. The goal is to demonstrate that DNA differences can be useful for decision-making about drug type and dosage, and ultimately to improve medication safety and efficacy.

Thank you, Mr. Chairman. I would be pleased to answer any questions that the Committee may have.