Genetics Society of America

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21st Century Cures Initiative
Committee on Energy and Commerce
U.S. House of Representatives
2125 Rayburn House Office Building
Washington, DC 20515
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Dear Chairman Upton and Colleagues:

We are writing on behalf of the Genetics Society of America (GSA), a professional scientific society with more than 5,000 members working to deepen our understanding of the living world by advancing the field of genetics, from the molecular to the population level.

Thank you for the opportunity to provide input on your deliberations about health research through the 21st Century Cures Initiative. GSA shares your concern about the need to accelerate the pace of cures in America and the critical need to enhance our investment in the research that provides the foundation for medical treatments. Modern approaches to medicine increasingly rely upon an understanding of the complex processes that underpin both health and disease—and which are shared across many living systems.

GSA represents scientists from all 50 states, united by a focus on understanding the operation of living systems. Many of our members do not work directly in humans, but in experimentally accessible model organisms including fruit flies, roundworms, baker's yeast, zebrafish, and many other systems. The fundamental knowledge learned from these non-human systems is *essential* for advancing our understanding of human disease. Indeed, a significant proportion of the genes implicated in human disease were first discovered and characterized in these model systems. As such, studies in model organisms are crucial for the development of new drugs and therapies, even though it may be many years before those treatments come to market. Moreover, the perfect balance of intellectual adventure with uncompromising standards of rigor that comes from training with model organisms hones the skills of the next generation of researchers.

The mission of *21st Century Cures: A Call to Action* states that you plan to look at the full arc of the innovation cycle—from discovery to development to delivery. GSA especially wishes to focus on the importance of discovery. Despite all that we are learning from our previous and ongoing investments in biomedical research, there is much more to discover in the biological sciences that will impact treatments and cures in the future. Although the white paper appropriately includes the challenges related to development and delivery of drugs, we emphasize that *without discovery, the other stages*

will not follow. Translational research simply cannot occur without a base of new knowledge and understanding of underlying biological mechanisms to translate. At a time of exceptional promise, we are concerned that declining investments in foundational research today will lead to a dry pipeline down the road.

The power and potential of basic research is rooted in its unpredictability, since it is impossible to foresee the source of the next major breakthrough.

As an example, the whole of the biotechnology field relies on the use of restriction enzymes—proteins that cut DNA at defined locations. Our understanding of restriction enzymes emerged from foundational research in bacterial systems. Thus, the entire multi-billion-dollar biotechnology industry would not exist without the previous investment in this fundamental work. The discovery of restriction enzymes has also directly led to medical advances, such as the cost-effective and accessible production of insulin, which has revolutionized the treatment of diabetes.

The completion of the Human Genome Project, likewise, has laid the groundwork for understanding the impact of genetic variation on disease, which is critical as researchers work to decode the genetic factors contributing to such complex conditions as Alzheimer's disease, cancer, schizophrenia, diabetes, and Parkinson's disease. Moreover, the Human Genome Project itself led to the development of technologies that have reduced the cost of sequencing a human genome by a factor of nearly one million. The promise of personalized precision medicine is only possible because genome sequencing is becoming an affordable and routine clinical test. The vast amount of data collected as part of the Human Genome Project also spawned entire new fields—including bioinformatics and computational biology—which, when combined with prior developments in population genetics, are now central to the way that we approach the genetics of disease.

Recent developments also demonstrate the contributions of model organisms. For example, the excitement from the ability to edit the human genome precisely, which shows promise in curing individuals of AIDS, is derived directly from genetic studies of recombination in bacteria, yeast, and *Drosophila*—and curiosity-driven research on how certain bacteria handle infection. Similarly, fundamental discoveries in *C. elegans* and plants revealed the unexpected phenomenon of RNA interface, which shows potential for clinical use in regulating gene activity.

These examples demonstrate that it is only through support for a broad spectrum of high quality and promising research, as vetted by experts in the field, that we can lay the foundation for the future.

Although the private sector plays an important role in drug development, basic research necessarily depends upon public investment. Indeed, private industry relies upon the fruits of publicly-funded research. Without robust federal funding from the National Institutes of Health, National Science Foundation, and other federal agencies, the building blocks for innovation will not be available. And

without that foundation, the nation will lose its place in innovation, in the development of medical treatments, in the creation of private-sector biomedical companies, and in the economic return that will result.

Thank you again for the opportunity to participate in your discussions.

Sincerely,

Vicki L. Chandler, PhD
President

Michael Lynch, PhD
Immediate Past President

Jasper Rine, PhD
Vice-President / President-Elect

Genetics Society of America

ABOUT GSA: Founded in 1931, the Genetics Society of America (GSA) is a professional scientific society with more than 5,000 members worldwide working to deepen our understanding of the living world by advancing the field of genetics, from the molecular to the population level. GSA promotes research and fosters communication through a number of GSA-sponsored conferences including regular meetings that focus on particular model organisms. GSA publishes two peer-edited scholarly journals: *GENETICS*, which has published high quality original research across the breadth of the field since 1916, and *G3: Genes|Genomes|Genetics*, an open-access journal launched in 2011 to disseminate high quality foundational research in genetics and genomics. The Society also has a deep commitment to education and fostering the next generation of scholars in the field. For more information about GSA, please visit www.genetics-gsa.org. Also follow GSA on Facebook at facebook.com/GeneticsGSA and on Twitter @GeneticsGSA.