

Testimony of Mary McGuire Richards
Deputy Chief Executive Officer
Parkinson's Action Network
Washington, DC

Outside Witness Hearing for the U.S. House of Representatives
Committee on Appropriations
Subcommittee on Labor, Health and Human Services, Education, and Related Agencies

March 18, 2009

Thank you, Chairman Obey, and Ranking Member Tiahrt for inviting me to testify on behalf of the Parkinson's Action Network regarding the National Institutes of Health. I am the Deputy Chief Executive Officer of the Parkinson's Action Network, also known by our acronym, PAN.

PAN represents the entire Parkinson's community, including the more than 1 million Americans currently fighting Parkinson's disease (PD), the estimated 60,000 newly diagnosed every year, and their families, and all the national Parkinson's organizations, including The Michael J. Fox Foundation for Parkinson's Research, Parkinson's Disease Foundation, National Parkinson Foundation, Parkinson Alliance, and American Parkinson Disease Association.

Parkinson's disease is a chronic, progressive neurological disorder that results from degeneration and premature death of dopamine-producing brain cells. Parkinson's patients experience devastating physical and mental symptoms such as tremors, debilitating slow movements, postural instability (balance problems), sleep disturbances, and a variety of cognitive impairments.

Parkinson's is the second-most common neurodegenerative disease in the United States. The cause of PD is unknown, although research points to a combination of genetic and environmental factors. PD is currently without any known cure.

Today, treatment options only provide relief for only some symptoms of the disease. There is nothing that slows, stops, or reverses progression of the disease or that will ward off ultimate and complete disability. Current state-of-the-art treatment for people with Parkinson's disease is rooted in levodopa and its derivatives. Levodopa was approved almost 40 years ago and, sadly, is still the primary treatment for Parkinson's. Deep Brain Stimulation (DBS) surgery is available for certain patients and treats some symptoms of Parkinson's disease. Yet, levodopa, its derivatives, and DBS only treat some motor symptoms of the disease and are only effective in treating those symptoms for a limited period of time.

Unfortunately, as the disease progresses, the motor and non-motor symptoms worsen and become increasingly difficult to treat. With disease progression, Parkinson's patients experience motor unpredictability and fluctuation. Patients do not know when the medication will "kick in," (resulting in an "on" period) or even if it will (resulting in an "off" or immobile period.)

As Parkinson's progresses, even with treatment, substantial disability -- including the inability to maintain balance, walk, speak, and move -- is inevitable and makes assisted living and nursing home care necessary. Ultimately, PD is a disorder of immobility, where persons suffering from the disease can feel trapped in their own bodies. People living with Parkinson's disease are desperately awaiting an innovative disease modifying therapies that will relieve their pain and halt the disease.

Although the purpose of today's hearing is Fiscal Year (FY) 2010 appropriations for the National Institutes of Health (NIH), I would like to take this opportunity to thank the Committee on behalf of the Parkinson's community for providing \$10 billion for the NIH in the American Recovery and Reinvestment Act, H.R. 1. Thanks to your efforts, this significant investment will facilitate much-needed research and thousands of high paying jobs. Of particular interest to PAN, is ensuring the strategic use of this significant, one-time infusion of biomedical research funding necessary to hasten basic discoveries and development of better therapies and a cure for Parkinson's disease.

Regarding FY 2010 appropriations, PAN continues to work in conjunction with the Ad Hoc Group for Medical Research to promote steady, sustainable increases in funding for NIH. Accordingly, in order to not lose ground in ongoing research and consistent with the President's request, we support the medical research advocacy community's recommendation for at least a seven percent increase above the FY 2009 funding level for the NIH.

However, PAN also continues to focus on how the biomedical research enterprise can engage in science to drive faster treatments and cures for people living with Parkinson's disease. To discern the best and fastest route to curative science, we examine not only how much money is needed but also how those dollars are best spent. To this end, we urge the Committee to provide NIH funding to support NIH Reform implementation as well as the resources necessary to overcome the scientific "Valley of Death."

NIH Reform

An unfortunate outcome of the past several years of flat-funding of the NIH is slowed implementation of many of the reforms called for by the NIH Reform Act of 2006, which was strongly supported by the Parkinson's community. NIH Reform aimed to enhance NIH's transparency, accountability, and strategic planning efforts -- all of which will hasten basic discoveries and their translation into better treatments and cures for Americans facing diseases and disorders.

I would like to first applaud the agency for its implementation of the NIH Reform mandated agency-wide reporting system, which was launched in January of 2009. This reporting tool, called the Research, Condition, and Disease Categorization (RCDC) system, is a comprehensive electronic reporting system that, for the first time, transparently catalogues all of the research activities of the NIH in a standardized format.

Increased transparency of NIH research activities highlights areas of ongoing research to improve research portfolio management, provides greater accountability of research dollars, and spurs creative thinking about new scientific approaches. This information is beneficial for

independent investigators, public advocacy groups, NIH internally, and Congress. The RCDC system is a huge endeavor that has already been fruitful for disease advocacy organizations, including PAN.

Although the Parkinson's community is pleased with the progress that has already been made, we are also aware that many of the NIH Reform implementation efforts should be further along than they are today. In fact, RCDC system's development was slowed, in part, due to a lack of sufficient funding. Without the commitment of resources to implement these Reform activities, the struggle between new efforts and the need to continue funding new ideas in research is increasingly difficult. In order for NIH to implement the reforms recently passed by Congress, appropriations for NIH must, at a minimum, keep pace with biomedical inflation.

The Scientific Management Review Board was to issue its first report to Congress within a year and a half of enactment of the bill. However, it is lagging behind schedule -- its members were only named in September of 2008 and no report has yet been issued. The creation of the Board was to be significant because the reform legislation made it responsible for making recommendations regarding changes in NIH organizational structure. The Board is also tasked with reviewing the current research portfolio and making strategic planning recommendations and ensures that NIH maximizes scientific opportunities that impact public health. This provision was important to disease advocacy organizations as it aimed to drive patient-oriented outcomes, but we are concerned about early signs that it is behind schedule.

Although proper and timely implementation of NIH Reform efforts is necessary, these reform provisions only constitute a modest and incremental approach to creating patient-oriented outcomes at the agency. Flat-funding at the agency also harms on-going efforts to enhance translational and clinical research needed to hasten better treatments and cures for Parkinson's disease and many others.

Valley of Death

It is helpful to understand the context in which PAN views all NIH programs. As you may know, NIH is the single largest source of Parkinson's and other biomedical research funding in the world. As such, it is charged with developing new knowledge to improve the health of Americans living with diseases and disabilities.

NIH funds world-class basic science, but a coordinated, large-scale effort within the agency is needed to support this emerging type of science. Handing off publicly-financed research to private industry for product development is one of the most difficult steps in crossing the Valley of Death, and a coordinated, well-supported translational research enterprise at NIH will help researchers in moving their work along.

NIH must rethink how to support the unique needs of translational science and provide the expertise, leadership, and training necessary to tackle complicated issues that prevent or slow research from moving into potential therapies. Intellectual Property and FDA expertise is essential to this part of the research endeavor, and existing efforts at the Institute must be bolstered or remodeled. NIH must develop a unique infrastructure as well as systems that are

needed to support translational science and hasten discovery of new treatment and cures for all Americans.

It is widely agreed, however, that there are major obstacles in the research endeavor that are slowing, if not blocking, our way to better treatments and cures for untreated and under-treated diseases. The issue is the scientific Valley of Death where promising therapies and treatments fail to connect from research to products.

Currently, no one in the federal government is responsible for ensuring that the scientific baton is passed from basic discovery onto private therapy development. The drug development pipeline in this country generally depends upon pharmaceutical or biotechnology companies picking up a discovery and seeing it through to the end of the FDA approval process. This middle part of the process, where promising drugs can be lost and no one is ensuring that good ideas in the lab are “translated” into real possibilities for patients, is referred to as the “Valley of Death.”

In addition, over the last 15 years, science has become more sophisticated as we better understand the complexities of human biology and disease; however, a consequence has been moving researchers who conduct basic science farther away from patients who might benefit from the application of such science. A new model is required that will fill the gap, or the Valley of Death, between basic discoveries and potential therapies for disease.

It has been the position of the Parkinson’s community for quite some time that NIH should focus on patient-oriented outcomes by doing more to combat the Valley of Death. Unfortunately, however, due to a lack of funding and in order to maintain basic research grants, NIH not only has not focused more on translational research, but has actually cut these programs. As Dr. Zerhouni said in his Senate Labor, Health and Human Services, and Education Appropriations Subcommittee testimony on March 19, 2007, “the impact [of NIH budget cuts] is primarily in our ability to translate from the laboratory to the clinic to the bedside into the community what we need to do to prevent diseases.”

It is disconcerting for people living with Parkinson’s and other untreated or under-treated conditions to know that many potential drugs are languishing in the “Valley of Death” simply because there is not enough funding to move basic research to product development. This science is some of the most difficult and costly research needed to develop therapies and meet the public health need, including developing pre-human testing, efficacy trials, production design and a range of other steps needed to determine whether a drug will be safe and effective. It is also essential for reducing the burden of disease and disability for millions of Americans.

The research that will bridge the Valley of Death is translational science, which aims to move basic discoveries into therapies for people living with diseases. Without translational research, the development of our best solutions to major public health needs – better treatments and cures – are slowed if not stopped altogether.

Let me be clear that PAN continues to support basic research discoveries coming out of NIH, which are very important. Robust research at the beginning of the pipeline is essential for continuing to grow our knowledge of biological and disease processes as well as to provide a

feeding ground for new, novel ideas in science. Of course, should novel ideas show promise, additional funding should be directed at translating these discoveries into treatments to alleviate the suffering of people living with disease.

Today, the Parkinson's Action Network again seeks your support for NIH funding sufficient to make gains in NIH Reform implementation and overcoming the Valley of Death. PAN continues to support basic science, but, as a patient advocacy organization, we are ultimately concerned with improving the health of people living with Parkinson's disease. We ask the committee to ensure that the NIH maintains the dedicated funding, resources, and systems necessary to support patient-oriented research essential to finding a cure.

On behalf of the Parkinson's community, I thank you for your continued interest in Parkinson's disease issues and your support for better treatments and a cure for Parkinson's. I would be happy to answer any questions you may have.