

Public Witness Testimony of Amy L. Comstock, Executive Director
Parkinson's Action Network
Washington, DC

Thank you Chairman Regula and distinguished Members of the Committee for inviting me to provide public witness testimony today.

I am the Executive Director of the Parkinson's Action Network, also known by our acronym, PAN. PAN represents the entire Parkinson's community, including the more than one million Americans currently fighting Parkinson's disease (PD), the estimated 60,000 newly diagnosed every year, and their families, and the national Parkinson's organizations, such as 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 the second-most common neurodegenerative disease. It is a chronic, progressive neurological disorder responsible for devastating physical and mental disability in its victims. The causes of PD are unknown, although it is currently believed that a combination of genetic and environmental factors is responsible for most cases. PD is currently without known cure.

Parkinson's patients experience symptoms such as muscle rigidity, debilitating slow movements, tremors, sleep disturbances and pain. Current medications and treatments for this incurable disease can induce disruptive and painful side effects such as often-violent excessive movements known as dyskinesias, cognitive slowing, and psychosis.

Unfortunately, as the disease progresses, the motor and non-motor symptoms worsen and become increasingly difficult to treat. Current drug therapies for PD alleviate some motor symptoms, but no proven means of slowing progression has been found. 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.) Ultimately, PD is a disorder of immobility, where persons suffering from the disease can feel trapped in their own bodies.

Although the vast majority of those affected by PD are over age 65, Parkinson's strikes an increasing number of younger persons, when its victims are in the prime of their working careers. Young onset patients, those diagnosed at age 50 and under, more frequently experience treatment-related motor complications and depression than do older-onset patients. These treatment-related complications often lead to loss of employment, disruption of family life, and greater perceived social stigmatization.

Parkinson's does not discriminate. Interestingly, it has been linked with a number of professions: farmers, welders, and, believe it or not, physicians and teachers, but until we find a cure, we are all vulnerable.

The purpose of today's hearing is NIH appropriations. As I am sure you all you know, PAN was instrumental in helping garner Congressional support for this Subcommittee's doubling of the

NIH budget over five years during the late 1990's and early in this decade. We are proud to have worked along-side many other patient, provider, and research organizations to achieve that significant investment in biomedical research at the world's foremost medical research center.

PAN continues to work in conjunction with the Ad Hoc Group for Medical Research to prevent the proposed freeze in funding for NIH. Flat-funding would, in effect, constitute a significant cut, as the Biomedical Research and Development Price Index (BRDPI) is estimated to have increased by 5.5 percent for FY 2005, and will likely increase by 4.1 percent for FY 2006, and 3.8 percent in FY 2007. Accordingly, in order to not lose ground in ongoing research, we support the medical research advocacy community's recommendation for a 5 percent increase above the FY 2006 funding level for the National Institutes of Health.

I need to be clear, however, that my main message here today is not about the amount of money that is appropriated to NIH. Rather, it is about how it is spent. We believe that NIH has failed to live up to its obligations to the Parkinson's community in terms of its perspective and priorities.

The mission of the National Institutes of Health is to move the public health forward and "extend healthy life and reduce the burdens of illness and disability." More specifically, the mission of the National Institutes of Neurological Disorders and Stroke (NINDS), the lead institute at NIH for Parkinson's disease research, is to reduce the burden of neurological disease. We do not believe NINDS is fulfilling this mission for Parkinson's disease.

As the Parkinson's community has consistently expressed to NINDS, we believe managing research is essential to ensure the NINDS is addressing Parkinson's and American's public health needs. However, as a community, we do not believe that the Parkinson's disease research portfolio at NIH or NINDS is being adequately managed with the GOAL of disease treatments and eradication as the guiding principle, and we see the unfortunate impact of this lack of guiding principle in two areas.

First, as best as we can tell, there simply is not enough money going towards translational research for Parkinson's disease at NIH right now. The funding numbers that we have for translational research speak volumes about the skewed priorities. The entire NIH budget for FY 2005 is \$28 billion. The NINDS portion of that is \$1.5 billion, with approximately \$128 million going to Parkinson's Disease. Yet, out of that \$1.5 billion, the most recent figure that I have for NINDS' "translational program" is \$22 million. \$22 million out of 1.5 billion for its translational program. That is simply not enough to assure us that what we learn from taxpayer funded basic research is adequately being translated to better treatments and a cure for Parkinson's and other neurological diseases. And while I completely understand that funding levels do not always reflect the potential impact of research, it is a good indicator of priorities. This percentage of translational research funding clearly reflects a low-priority.

In order to advance scientific innovation towards potential relief for current and future Parkinson's patients, a refocus of resources by the NIH is needed to translate basic scientific discoveries into therapies. It's not a difference between basic research and health-oriented research; rather, it's a balance between being stewards for today's generation of patients and stewards for the next generation.

There is a limited amount of translational PD research going on at NIH right now, but even that, we fear, is underfunded. One project currently funded at NINDS is called NET-PD (Neuroprotection Exploratory Trials in Parkinson's disease). NET-PD is a trial to study compounds that may slow the progression of Parkinson's disease. There are four compounds being examined (out of a potential pool of 59) and, so far, two (creatine and the antibiotic minocycline) have been identified as agents worthy of further study.

Our concern, however, is that we have heard from NINDS that regardless of how promising these compounds seem to be at the conclusion of the NET-PD trials, NINDS will only pursue clinical trials of no more than two of the compounds, even if it appears that all four may warrant further research. That arbitrary limitation alone is frustrating for us.

From our perspective, this research is exactly the kind of translational research that NINDS should be aggressively pursuing. Sufficient funding to test in trials ALL the compounds, if the preliminary research is promising, should be a priority. That is NIH's job.

I know that asking NINDS to do more planning in this era of limited resources is not a welcome suggestion, but we believe it is possible for NINDS to immediately focus more translational research, without additional resources or an act of Congress. Shifting priorities does not necessarily demand greater dollars. For example, the intramural, meaning completely within NIH, neurotherapeutics program has been, in the past, an innovative program at NINDS that focused on development of experimental therapies for Parkinson's and other neurological patients. Intramural programs are especially important because the institute has much more control over the direction of the research and they are not subject to the three to five year grant cycle. However, the Parkinson's community is deeply concerned that this innovative, translational program is currently on hold. The apparent halting of this program again illustrates the low priority given to translational research and contributes to a sense that NINDS does not understand the Parkinson's community's sense of urgency.

NINDS could and should reinvigorate the translational work of this intramural program, which would not require any additional funding. Neurotherapeutics is a program through which NINDS can most easily change the "funding as usual" culture and establish a meaningful translational program with the goal of finding better treatments and a cure for Parkinson's.

Second, in addition to our frustration at the simple lack of sufficient translational research being funded by NIH, I want to discuss with you today our unhappiness with the lack of management of the entire Parkinson's disease research portfolio at NIH. We believe that Parkinson's disease research, intramural and extramural, should be conducted based on an action plan or strategic plan that ensures that research which appears promising is pursued. In fact, both the Parkinson's community and the Congress have consistently asked for proactive Parkinson's portfolio management for close to ten years and that request has consistently gone unanswered.

Why are we so focused on process and planning? Because, as a manager myself, I know that results-oriented planning is a necessary step and the fastest route to achieving goals. Reliance on scientific serendipity is not sufficient for Parkinson's patients, caregivers, and their families. No particular amount of funding will ensure that we fully explore promising research and reduce the burden of this disease. A scatter-shot approach may indeed produce results by happenstance, but

we can do better. The cure for Parkinson's should be the goal -- with designated funding and measurable steps toward achieving that goal -- not just a happy accident.

In 1997, some of you may recall, the Morris K. Udall Parkinson's Research Act (Udall Act), was enacted to ensure that NIH-funded research hasten discovery of better treatments and a cure for Parkinson's disease. Among other requirements, the law required that NIH convene planning conferences every two years to assess the progress of and plan the future of PD research. I am sorry to say that NIH has not honored this statutory requirement both in terms of timing and outcome.

Last year, after close examination of the implementation of the original Udall Act, the Parkinson's community along with the Congressional Working Group and Caucus also concluded that many essential components of the legislation have not been implemented per the 1997 Act, including the overall coordination and planning of Parkinson's research funds. Therefore, the Congressional Working Group on Parkinson's disease introduced legislation to insist NIH manage federal research dollars to focus on discovery of better treatments and a cure. The bill is called the Morris K. Udall Parkinson's Disease Research Act Amendments of 2005.

The bill does not ask for more money. Rather, because a strategic planning document to guide Parkinson's research grants has never been fully developed and implemented, the bill insists, with greater specificity, that NIH not only create and implement such a plan but also extensively report to Congress to ensure accountability. Although we would be fools to not wish for more money for PD research, an increase in federal dollars for Parkinson's research is not our message today. As Michael J. Fox recently said during the opening ceremonies of the World Parkinson Congress, "it is naïve to think that another billion dollars, thrown at this disease in the same old way, will yield different, translatable results." Regardless of funding, without better management of current dollars we believe the search for the cure will be significantly lengthened. This is painful for our community because time is not neutral for Parkinson's patients and their families.

Let me give you an example, the most recent example, of poor research planning at NIH.

In June of 2005, the NINDS hosted a Parkinson's Summit with Parkinson's researchers and representatives from the Parkinson's community organizations in attendance. Prior to the Summit, the Parkinson's organizations suggested that the Summit be used to develop a Parkinson's research investment plan. In an era of limited funding, we must maximize the impact of those funds with proper planning. Therefore, we hoped that this Summit would result in a plan (as the current law requires) that contained measurable goals against which progress could be assessed, which would have also fulfilled statutory planning requirements. Sadly, this has not happened.

I was at this meeting and I can tell you that, at best, what occurred was minimal compliance with the current law. As far as I can tell, after spending two days together discussing current PD research, no planning document, analysis, summary, or guidance has resulted from that meeting. The only follow-up documentation so far has been a partial list of topics discussed at the Summit without any analysis or recommendations. The listed initiatives had no priority or weight according to criteria such as current state of research, feasibility, relative importance, timeline,

knowledge gaps, or cost. In a letter signed by every Parkinson's community organization, we expressed our concern that, without additional analysis from NINDS, responses from participants would likely be of little value in the strategic planning process necessary for NINDS to fulfill its mission. Frankly, it is hard to see how this Summit will ultimately be considered anything but a failure.

In fact, this Committee, in reports accompanying the last several years' annual appropriations bills has encouraged that NIH develop and implement a research investment plan – insisting that the goal of better treatments and a cure is considered as a part of the research funding process. The Fiscal Year 2006 report language indeed called for a report from the NIH to this Committee on the establishment of a Parkinson's research plan six months after the Parkinson's Summit, which would have been in early December of 2005. To date, as far as we know, this report has not yet been issued.

In conclusion, we believe that there is hope for today's Parkinson's disease patients and their families. There are emerging therapies that should be pursued – even therapies that could potentially reverse the progression of the disease. These are the neuro-restorative therapies, such as neural growth factors, gene therapies, and tissue transplants including stem cells, which ultimately may restore function in patients suffering from Parkinson's disease as well as other neurodegenerative disorders.

But, without overall planning to ensure this promising research is followed-up on, it may take many more years than necessary to determine if this hopeful research may become much-needed therapies. Because a strategic plan for funding Parkinson's disease research, including translational research, is absent, we must continue to push NIH for a proactive approach to curing disease that includes a research plan with the central goal of turning these **potential** therapies into **actual** therapies.

Today, the Parkinson's Action Network again seeks your support for Parkinson's research albeit in a slightly different manner. Strategic planning could provide the key that will release many of your constituents, along with others around the country, from the physical and emotional pain and crippling financial consequences of Parkinson's disease. Please understand that funding cuts would have a devastating effect on Parkinson's research, and PAN continues to support a five percent increase in NIH funding. However, we know that focus on results-oriented research is equally, if not more, important 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.