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Thank you, Chairman Altmire, and Ranking Member Gohmert for inviting me to testify on behalf of the Parkinson's Action Network regarding the Small Business Innovation Research (SBIR) program. As you know, I am the 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 one 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. It 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 known cure.

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. Today, treatment options only provide some symptomatic relief but are in no way neuroprotective; halting or reversing the progression of the disease. 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. Yet, levodopa and the derivatives only treat the symptoms of the disease and are only effective in treating symptoms for a limited period of time. We still have nothing that will actually slow the progression of Parkinson's or that will ward off ultimate and complete disability. 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. Parkinson's disease sufferers are desperately awaiting an innovative neuroprotective treatment that will relieve their pain and halt the disease.

Before I begin to discuss the Small Business Innovation Research (SBIR) program specifically, it is helpful to understand the context in which PAN views all National Institutes of Health (NIH) programs. As you may know, NIH is the single largest source of Parkinson's disease research funding in the world, and the basic discoveries coming out of NIH are very important, but it is our belief that NIH is not funding enough research that aims to translate basic scientific discoveries into therapies for people living with diseases. As I testified before the House Appropriations Committee in 2006, the primary focus at NIH is on basic research – research that is not necessarily geared towards therapeutic outcomes – rather than research that will advance scientific innovation towards needed therapies.

The drug development process takes many years from beginning to end. At the beginning you have basic research supported by NIH. At the end, one hopes, you have a drug, biologic, or treatment, approved by the FDA, that is available to those afflicted with a particular disease. Unfortunately, between these two bookends of well-understood areas of federal oversight, you have a process that is often-times confusing, inefficient, and not geared toward improving the public health. Currently, no one in the federal government is responsible for ensuring that the scientific baton (promising early NIH-funded research) is passed from basic discovery onto private development, generally a pharmaceutical or biotechnology company, that will pick up the project and see 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."

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." We have suggested that more of the NIH extramural grant program should be focused on potential therapies for particular diseases. Unfortunately, however, due to a lack of funding and in order to maintain basic research grants, NIH has not only 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 un-treated 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.

Having heard our vision of the need for NIH to refocus some of its grantmaking, it should be clear why I and the Parkinson's community are so strongly supportive of the SBIR

program. SBIR grants have a significant role to play in the drug development arena called the "Valley of Death. The Parkinson's Action Network strongly supports the entire NIH SBIR program, but there are several important elements of the program that I will highlight and a few suggestions of areas that would benefit from improvement.

NIH SBIR grants, of course, are awarded to small companies that conduct biomedical research. In order to address the NIH SBIR program from a biomedical perspective, it important to understand in a little more detail, the how these small biomedical companies function. Generally, these companies have one or two lead projects for which they are able to raise funds through private investors. These research projects are investigations into promising products or therapies that investors have determined to be worthy of their money. But, like many things in life, there is often a second tier. The second tier includes research that is also promising, but which, for one reason or another, is not as appealing to private investors. The lack of appeal to investors may occur for a number of reasons – it can be that the science at issue, while worthy, is less certain and more of a risk so private investors are more leery. Or, the lack of appeal to private investors may be because of the size of the potential market. This is a very real issue for a disease like Parkinson's that, while between one and one and a half million people, is not a sizeable market that is appealing for potential profit.

Companies may have trouble attracting private investors to support this second category of projects, which are scientifically valuable research projects but are less certain in terms of the potential return on investment. I cannot emphasize to you enough how troubling it is to a person with Parkinson's or their loved one that there are potentially hundreds of bright ideas out there for better treatments for Parkinson's disease that are not being pursued because our system does not have a process for ensuring that good ideas are not lost. In fact, in a perfect world there should be a way of ensuring that promising ideas move through the pipeline as quickly as the science dictates and the potential benefit to the public health demands. But this is not the case. There is no guarantee that a promising therapy for a disease with a very small population, for example, will move through the pipeline at all. Similarly, there is no guarantee that a risky idea for a disease that affects a larger population, let's say Alzheimer's Disease, with a population of about 4.5 million and growing, will be pursued.

This is where SBIR comes in. The SBIR program supports cutting-edge research where other sources of research are difficult if not impossible to obtain. But when you turn that thought around and look at it from a patient perspective, it is not that this program is about funding, it is that this program makes possible research for many diseases that would not otherwise occur. That is invaluable.

Having stated our strong support for the NIH's SBIR program, however, I do want to offer an important recommendation for the future. As this committee is well aware, the 2003 SBA ruling regarding SBIR eligibility based on majority ownership by "individuals" has had a negative impact on the biomedical research community. It is my understanding that, since that ruling, applications to the NIH SBIR program have dropped precipitously. SBIR applications, we have been told, are down 11.9% in 2005, 14.6% in

2006, and 21% in 2007. And, given the increase in most applications to NIH, it is fair to assume that this drop is a direct result of the eligibility ruling.

From a patient perspective it does not seem logical, and is in fact scary, that we eliminate from eligibility research projects that otherwise merit funding, because of the financial structure of the company. And, the reasoning becomes more muddled when one focuses on the fact that the companies that are being excluded by the SBA rule are the very ones that are doing work that is good enough, for whatever reason, to have attracted venture capital money. The very companies that are doing a good enough job in one area are, because of that success, barred from federal support for other promising research. This policy doesn't penalize companies, it penalizes patients.

Let me give you an example of the impact of this ruling. It is a sad reality that there is a lot we still do not understand about neurological diseases. There are many investigations into compounds that show therapeutic promise in pre-clinical and even early clinical stages in which the research is dropped and no actual therapies are developed. That is why our community is heavily invested in any potential therapy that is beginning to show real promise. We have one now, Spheramine, that is in Phase II clinical trials. Spheramine, quite simply, injects retinal cells into the brain, surgically, to provide a continuous source of dopamine. While this trial is now in Phase II and our community is quite enthusiastic about its promise, the animal research and Phase I research was funded through an SBIR grant. We are fearful that keeping the eligibility ruling as it now stands will keep this kind of promising research from going forward. In fact, who knows what promising therapies are sitting now, unfunded and not moving?

I would like to make one final point in support of revisiting the ruling SBIR ineligibility based on venture capital investment. At NIH, as with SBIR throughout the government, 2.5 percent of the extramural grant monies are set aside for this program. By eliminating a large percentage of private, innovative researchers, we are left with a much smaller pool of applicants from which NIH can draw when funding these grants. And, while all applications are peer-reviewed so, presumably, are all good science, it just seems logical to me that we would want to do everything we could to invite as many applications as possible to go into that peer-review process so that we are assured that what comes out is the best science, with the most promise, that we can fund.

As PAN continues working toward better treatments and cures for Americans, we respectfully seek the Small Business Committee's support for a robust SBIR program at NIH. SBIR is an essential program that provides key funding for patient-oriented research currently languishing in the "Valley of Death" of the biomedical research system. We respectfully request that your support include a revision to not eliminate small companies simply based on their financial structure.

Thank you again for this opportunity to provide testimony. I look forward to working with the Committee on this critical issue for the Parkinson's community, the small business community, and all American families facing disease and disability.