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Committee on Small Business
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Thank you, Chairman Graves and Ranking Member Velázquez 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, after Alzheimer's. The cause of PD is unknown, although research points to a combination of genetic and environmental factors. PD is currently without any 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. Unfortunately, today's treatment options provide only some symptomatic relief; there are currently no treatments that halt or reverse 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 more than 40 years ago and, sadly, is still the primary treatment for Parkinson's. Levodopa and the derivatives only treat the symptoms of the disease and are only effective in treating symptoms for a limited period of time. There is 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.

I am here today because PAN, like many patient advocacy organizations, strongly supports the Small Business Innovation Research (SBIR) program. At PAN, we are most familiar with SBIR as it operates at the National Institutes of Health (NIH). As you may know, NIH is the single largest source of Parkinson's disease research funding in the world.

To understand why the SBIR program is so important, it is helpful to understand something very fundamental about how medical research is conducted: The basic scientific discoveries coming out of NIH are very important; but it is also important to "translate" those basic scientific discoveries into therapies for people living with diseases. Let me say that again: both basic scientific research AND the research needed to translate those discoveries into new drugs and therapies are crucial.

The drug development process takes many years from beginning to end – for neurological diseases like Parkinson's, the process can take 15 years or more. At the beginning of this process 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 and inefficient; promising discoveries can be lost because no one is ensuring that they are "translated" or carried through to test their therapeutic potential. This place where basic discoveries often languish is referred to as the "Valley of Death."

It is disconcerting for people living with Parkinson's and other untreated or under-treated conditions to know that many potential therapies or disease-understanding breakthroughs are lost in the "Valley of Death" simply because there is not enough funding to move basic research to product development. This translational 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.

This is where SBIR grants come in. SBIR grants have a significant role to play in the drug development arena. In FY2010, NIH awarded \$616 million in SBIR grants to hundreds of small businesses across the country. NIH SBIR grants are awarded to small companies that can bridge the divide between a basic discovery from which we may have learned more about a disease and the hard work of testing that discovery for its commercial and, from my perspective, therapeutic potential. Most often it is the small start-up biotech companies that are the true innovators of medical cures and treatments.

Historically, these small companies have raised their needed capital from private investors. But, in recent years we have seen a dramatic and harmful shift away from investment funds in biomedical research. The lack of appeal to investors may occur for a number of reasons – biomedical research takes a very long time so the return on investment may not be soon enough. Also, for a disease like Parkinson's the lack of

appeal to private investors may be because of the size of the potential market and ultimately profit (we are only one million or so in this country) and the greater risk involved with testing therapies for a disease of the brain.

The SBIR program supports and is focused on cutting-edge research where other sources of research are difficult, if not impossible, to obtain. And the SBIR grants, though relatively small, can make all the difference in whether that cutting-edge research is pursued or not. SBIR will provide Phase I funding of up to \$150,000 for six months to examine the technical merit, feasibility, and potential for commercialization of the proposed research effort. A meritorious project can then get Phase II funding of up to \$1,000,000 in total costs for two years to continue the research and development project.

I am certain that without SBIR funding, many of these small companies pursuing only one or two projects at a time, would simply not exist and some very promising research efforts would not be pursued. That is why, when you look at the SBIR program from a patient perspective, this program is not just about funding, it is about pursuing possible treatments and cures for many diseases.

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 even for a disease that affects a larger population, let's say Alzheimer's Disease, with a population over 5 million and growing, will be pursued.

I also want to offer our thoughts on the long-standing issue of whether to allow majority venture capital owned firms in the SBIR program. After the 2003 SBA ruling regarding SBIR eligibility based on majority ownership by "individuals," there was a precipitous drop in applications to the NIH SBIR. Given the increase in most applications to NIH, it is fair to assume that the drop was 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 small company. In fact, venture capital dollars are often the only source of capital that is willing to fund long-term risky biotech start-ups companies. And, the reasoning becomes even more frightening 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 even in this very challenging financial climate. 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 just penalize companies, it penalizes patients.

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. It just seems logical to me that we would want to do everything we could to invite as many applications as possible into that peer-review process so we are assured that what comes out is the best science, with the most promise, that we can fund.

Small companies have always been a vital piece of innovative biomedical research and advances in the country. Small companies conduct so much of the critical translational research that needs to be done in the “Valley of Death” – the middle of that development pipeline between NIH basic science on the front-end and drug development by big pharmaceutical companies on the back end. And the SBIR program is critical to helping those small companies play their key role in developing the new drugs and therapies of tomorrow.

As PAN continues working toward better treatments and cures for millions of 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 so that small companies are not eliminated based on their financial structure.

PAN supports the Committees efforts to move SBIR Reauthorization legislation expeditiously through the House and a bill can get signed into law before the next reauthorization deadline of May 31, 2011.

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.