

Written testimony of The Michael J. Fox Foundation for Parkinson's Research
Submitted to:
Subcommittee on Health
House Committee on Energy and Commerce
Regarding the Hearing: *"Examining Proposals that Provide Access to Care for Patients and Support Research for Rare Diseases"*
June 14, 2023

Chair Rodgers, Chair Guthrie, Ranking Member Pallone, Ranking Member Eshoo, and Members of the House Energy and Commerce Subcommittee on Health, thank you for providing the opportunity to submit testimony in support of today's hearing entitled "Examining Proposals that Provide Access to Care for Patients and Support Research for Rare Diseases." We are thankful for the opportunity to support the National Plan to End Parkinson's Act and highlight the hope it brings to the Parkinson's community.

"The Michael J. Fox Foundation launched with one goal: to end Parkinson's. Since our start in 2000, we've made tremendous progress. We have funded more than \$1.75 billion in research. And the recent discovery of a biomarker is our biggest breakthrough yet. But we're not slowing or stopping to congratulate ourselves — much more work is needed ahead. And we can't do it alone. The Foundation endeavors to partner effectively with the government to leverage federal research investments and keep good ideas moving forward toward patients, and to ensure those living with the disease have access to the care they need. I urge you to keep the National Plan to End Parkinson's Act moving through Congress, and to pass it. We stand ready to work together to make Parkinson's a thing of the past." — Michael J. Fox, actor, advocate, founder of The Michael J. Fox Foundation for Parkinson's Research

The Michael J. Fox Foundation (MJFF) is the world's largest nonprofit funder of Parkinson's research and an organization dedicated to accelerating a cure for Parkinson's and developing improved therapies for those currently living with the disease. MJFF pursues its goals through an aggressively funded, highly targeted research program coupled with active global engagement of scientists, Parkinson's patients and their families, business leaders, clinical trial participants, donors and volunteers. To date, MJFF has funded \$1.75 billion in research and has fundamentally altered the trajectory of progress toward a cure.

On April 12, 2023, MJFF announced an enormous research breakthrough, opening a new chapter for Parkinson's research — with the promise of better drug development and care for all people and families living with the disease.ⁱ The new tool called the α -synuclein seeding amplification assay (α Syn-SAA), validated by the Foundation's landmark brain health study, the Parkinson's Progression Markers Initiative (PPMI), can reveal a key pathology of the disease: abnormal alpha-synuclein — known as the "Parkinson's protein" — in brain and body cells. This new biological test can detect the disease at the molecular level, even before the onset of symptoms. It does so by detecting abnormal alpha-synuclein in spinal fluid not only in people diagnosed with Parkinson's, but also in individuals who have not yet been diagnosed or shown clinical symptoms of the disease but are at a high risk of developing it.ⁱⁱ We have long known that abnormal alpha-synuclein clumps in the brains of people living with Parkinson's through post-mortem analysis, but this is the first time where we can detect it in a living human person.

This breakthrough heralds a new era of research with the promise of speeding faster, cheaper and smarter clinical trials. It also opens a world of opportunities to treat the disease earlier and prevent it altogether.

Parkinson's is a chronic, progressive neurological disorder with no cure or treatment to slow, stop or reverse the progression of the disease. Parkinson's occurs when brain cells that make dopamine, a chemical that coordinates movement, stop working or die. Since Parkinson's can cause tremor, slowness, stiffness, and walking and balance problems, it is referred to as a “movement disorder.” However, there are many non-movement symptoms that can be associated with Parkinson's, such as constipation, depression and dementia. Parkinson's is a lifelong and progressive disease, which means that symptoms worsen over time, and the experience of living with Parkinson's over the course of a lifetime is unique to each person, with symptoms and progression of the disease varying from person to person.

Parkinson's also has the unfortunate distinction of being the fastest-growing neurological disease and second most common after Alzheimer's. Over 1 million Americans, including over 110,000 military veterans, currently live with the disease — a number that is expected to double by 2040.^{iii.iv} To put that into context, there are 90,000 new diagnoses each year in the United States, which equates to 1 person every six minutes. In addition, it costs our country \$52 billion every year to care for people with Parkinson's, half of which is paid by Medicare and Social Security, with the other \$26 billion being paid for by American taxpayers, state and local governments, and through lost wages of patients and caregivers.^v Even more concerning is that, by 2037, the

annual cost and financial burden of Parkinson's is projected to balloon to \$80 billion.^{vi}

However, despite Parkinson's staggering economic toll, the National Institutes of Health (NIH) only invested an estimated \$260 million in annual support for Parkinson's disease research in 2022.^{vii} In other words, the federal government spends approximately 100 times more caring for people with Parkinson's than the NIH spends researching the disease. This is alarming because Parkinson's is the 11th leading cause of death for Americans 65 and older, and disparities in resource allocations for Parkinson's disease become exponentially more concerning as we look to the future.^{viii} The largest risk factor for developing Parkinson's disease is aging, so as the U.S. population ages, many more of our friends and family members will be diagnosed with Parkinson's. We simply cannot afford to wait. We must act now to prevent and cure this disease.

We thank the Subcommittee for its consideration of H.R.2365, the National Plan to End Parkinson's Act, and urge you to advance the bill through Committee and to the House floor. Introduced by Representative Gus Bilirakis (R-FL) and Representative Paul Tonko (D-NY), this bipartisan, no-cost legislation will, for the first time, unite the federal government and non-federal partners in a mission to treat, prevent and cure Parkinson's, alleviate financial and health burdens on American families, and reduce government spending over time. This legislation establishes an advisory council comprising representatives from federal agencies supporting Parkinson's-related research and services, as well as patients, care partners, researchers, clinicians, and

other non-federal experts, all charged with creating a national plan to coordinate and maximize efforts to prevent and cure Parkinson's disease.

The advisory council will host meetings throughout the year and provide biannual reports to Congress and the Secretary of Health and Human Services containing evaluations of all federally funded programs related to Parkinson's. The reports will include recommendations on priority actions to prevent and cure Parkinson's, improve health outcomes, reduce the financial impact of the disease on patients and the federal government, and limit exposures to environmental risk factors. The goal of the advisory council is to ensure efficient and effective coordination among all federal entities with responsibility for managing, treating, and curing Parkinson's disease. Importantly, this coordination of efforts across the federal government and non-federal stakeholders will create efficiencies by maximizing research opportunities, avoiding duplicative efforts, and allocating resources and expertise where they will be the most impactful.

The National Plan to End Parkinson's Act is a game-changing piece of legislation that provides the pathway for federal agencies, private organizations, the medical community, people and families living with Parkinson's and caregivers to unite in a singular mission to end Parkinson's, alleviate its medical, financial, and emotional burdens on American families, and ease the pressure on public programs like Medicare and Social Security. By advancing this critical legislation, you are supporting the first step in what will be a truly historic and transformative period for those living with Parkinson's disease and their loved ones.

On behalf of the Parkinson's community, we thank the Subcommittee for its support and for the opportunity to submit this testimony. As the Subcommittee looks to advance legislation in the 118th Congress, we urge you to prioritize the National Plan to End Parkinson's Act to find a cure for Parkinson's and improve the quality of life of millions of Americans. On behalf of the more than one million people in the United States living with the disease, thank you for your time and attention. We hope you will consider The Michael J. Fox Foundation a trusted resource as the Subcommittee continues its important work this Congress.

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ⁱ Michael J. Fox Foundation. Breaking News: Parkinson's Disease Biomarker Found. April 13, 2023. Available at: <https://www.michaeljfox.org/news/breaking-news-parkinsons-disease-biomarker-found>

ⁱⁱ Siderowf A, Concha-Marambio L, Lafontant DE, Farris CM, Ma Y, Urenia PA, Nguyen H, Alcalay RN, Chahine LM, Foroud T, Galasko D, Kieburz K, Merchant K, Mollenhauer B, Poston KL, Seibyl J, Simuni T, Tanner CM, Weintraub D, Videnovic A, Choi SH, Kurth R, Caspell-Garcia C, Coffey CS, Frasier M, Oliveira LMA, Hutten SJ, Sherer T, Marek K, Soto C; Parkinson's Progression Markers Initiative. Assessment of heterogeneity among participants in the Parkinson's Progression Markers Initiative cohort using α -synuclein seed amplification: a cross-sectional study. *Lancet Neurol.* 2023 May;22(5):407-417. doi: 10.1016/S1474-4422(23)00109-6. PMID: 37059509.

ⁱⁱⁱ Parkinson's Foundation. Statistics. Available at: <https://www.parkinson.org/Understanding-Parkinsons/Statistics#:~:text=Approximately%2060%2C000%20Americans%20are%20diagnosed,are%20diagnosed%20before%20age%2050.>

^{iv} Mantri S, Duda JE, Morley JF. Early and Accurate Identification of Parkinson Disease Among US Veterans. *Fed Pract.* 2019 Jun;36(Suppl 4):S18-S23. PMID: 31296979; PMCID: PMC6604980

^v Yang, W., Hamilton, J.L., Kopil, C. et al. Current and projected future economic burden of Parkinson's disease in the U.S.. *npj Parkinsons Dis.* 6, 15 (2020). <https://doi.org/10.1038/s41531-020-0117-1>

^{vi} Ibid.

^{vii} National Institutes of Health. Focus On Parkinson's Disease Research. Available at: <https://www.ninds.nih.gov/current-research/focus-disorders/focus-parkinsons-disease-research>

^{viii} Centers for Disease Control and Prevention, National Center for Health Statistics. National Vital Statistics System, Mortality 1999-2020 on CDC WONDER Online Database, released in 2021. Data are from Multiple Cause of Death Files, 1999-2020, as compiled from data provided by the 57 vital statistics jurisdictions through the Vital Statistics Cooperative Program. Available at: <https://wonder.cdc.gov/ucd-icd10.html>.