

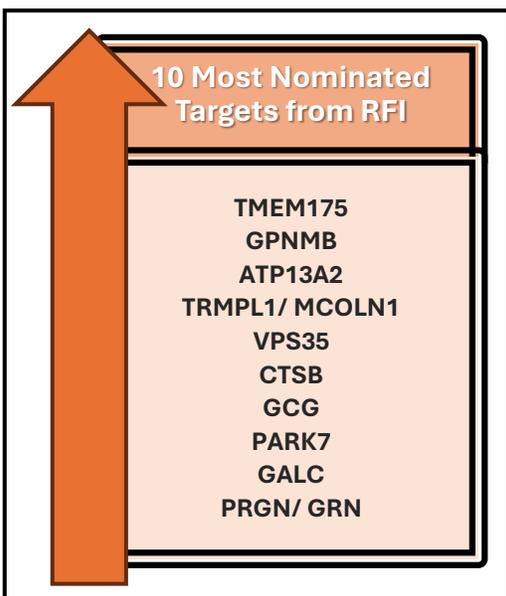
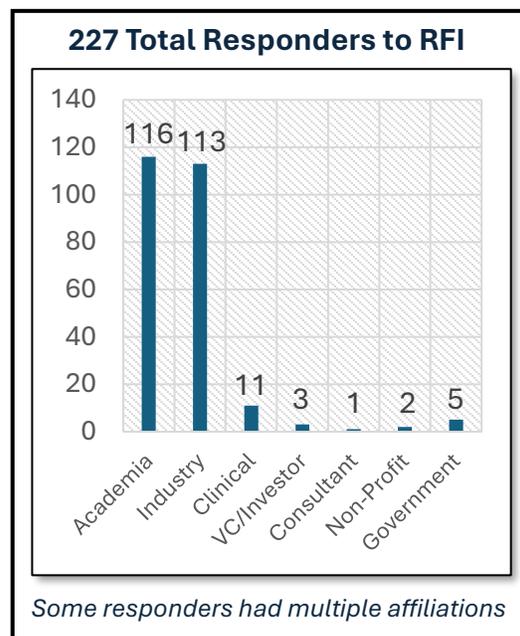
MJFF Target Prioritization Initiative: Latest Updates and Spring 2024 Survey Outcomes

Emerging Targets for Parkinson's Disease – Request for Information

In the spring of 2024, the Michael J. Fox Foundation (MJFF) released a request for information (RFI) for a new collaborative program known as Targets to Therapies (T2T) that aims to select clinically-relevant yet underdeveloped targets for Parkinson's Disease and accelerate their advancement through the target validation pipeline and into therapeutic development.

The findings from this RFI are aiding our efforts surrounding target de-risking and knowledge building relevant to novel or emerging therapeutic targets. This initiative is designed to identify and bolster the validation process for a select group of these promising targets relevant to Parkinson's Disease (excluding well-known genetic targets such as, GBA, LRRK2, Parkin/Pink1, SNCA and NLRP3).

In response to our survey, 227 individuals across diverse areas (top right panel) nominated a total of 210 novel or emerging targets for Parkinson's disease. The 10 highest nominated targets are listed in the graphic below (middle left panel). 139 of the 210 targets were unique to MJFF's existing funding portfolio. In combination with MJFF's current funding programs and these newly recommended targets, a subset of these targets will be prioritized for the next significant wave of validation efforts within MJFF's therapeutic discovery programs.



Program Overview

This RFI is the first step of an exciting pilot initiative launched by MJFF, which builds on the key features of existing programs to proactively prioritize and speed translation of promising biology into and across the therapeutic pipeline. The initiative aims to provide deeper validity packages that link targets to disease pathogenesis with reproducible data and accessible “toolkits” that accelerate the translation of emerging targets. The overarching goal of our new initiative is to increase confidence in targets and disease hypotheses prior to therapeutic development by building a “engine,” in which targets will be prioritized for target validation and toolkit development. The key objectives of these programs will be to:

- Identify, partner, and engage with various stakeholders to guide and inform the strategy for prioritizing targets and advancing target data packages,
- Prioritize the right disease targets, and
- Generate and disseminate target data packages primed to launch and/or advance multiple development programs against a target.

Next Steps

The Prioritization & Selection core committee is the first cog in the de-risking engine (bottom right panel) and provides the framework for the selection of targets entering the pipeline for target validation. This interdisciplinary team includes a diverse group of investors, PD biology-target experts and drug hunters. Together, they are collaboratively sharing knowledge on therapeutic target criteria and utilizing both existing and novel tools and platforms to curate and aggregate data from various sources on emerging PD targets, enabling the prioritization and selection of targets for further validation. The committee's work will result in an updated Target Report to be released in fall 2024, and a comprehensive Target Knowledge Base web portal containing data and resources on validated targets, with further details to be provided in 2025.

