

# Welcome



## **The Michael J. Fox Foundation**

### **Funding Philosophy and Grant Writing Workshop**

# Discussion Leaders

## **Todd Sherer, PhD**

**Vice President – Research – The Michael J Fox Foundation**

**tsherer@michaeljfox.org**

## **Brian Fiske, PhD**

**Associate Director – Research – The Michael J Fox Foundation**

**bfiske@michaeljfox.org**

## **Mark Frasier, PhD**

**Associate Director- Research – The Michael J Fox Foundation**

**mfrasier@michaeljfox.org**

## **Robert Burke, MD**

**Professor, Columbia University – MJFF Scientific Advisory Board Member**

## **Malu Tansey, PhD**

**Assistant Professor – UT Southwestern**

# **The Michael J. Fox Foundation Strategic Overview**

***Todd Sherer, Vice President -  
Research***



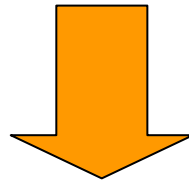
# Topics for Discussion

- **Overview of MJFF mission, organization and approach**
- **Discussion of MJFF funding priorities and programs**
- **Discussion of MJFF review process and criteria**
- **Question and Answer Session**

# MJFF: Dedicated to Finding a Cure for Parkinson's Disease

**The Michael J. Fox Foundation (“MJFF”) was founded in 2000 with very clear objectives**

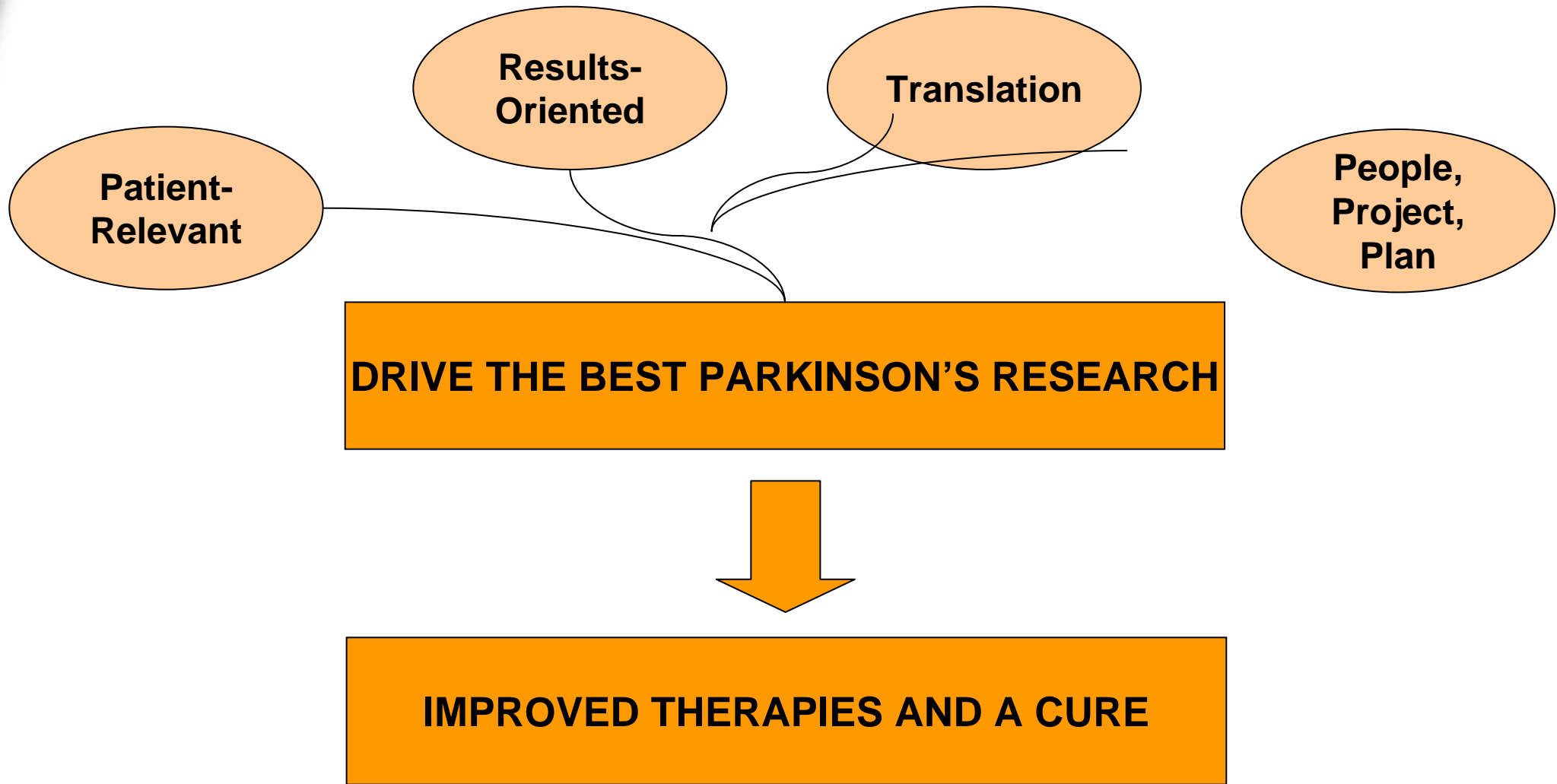
**DRIVE THE BEST PARKINSON'S RESEARCH**



**IMPROVED THERAPIES AND A CURE**

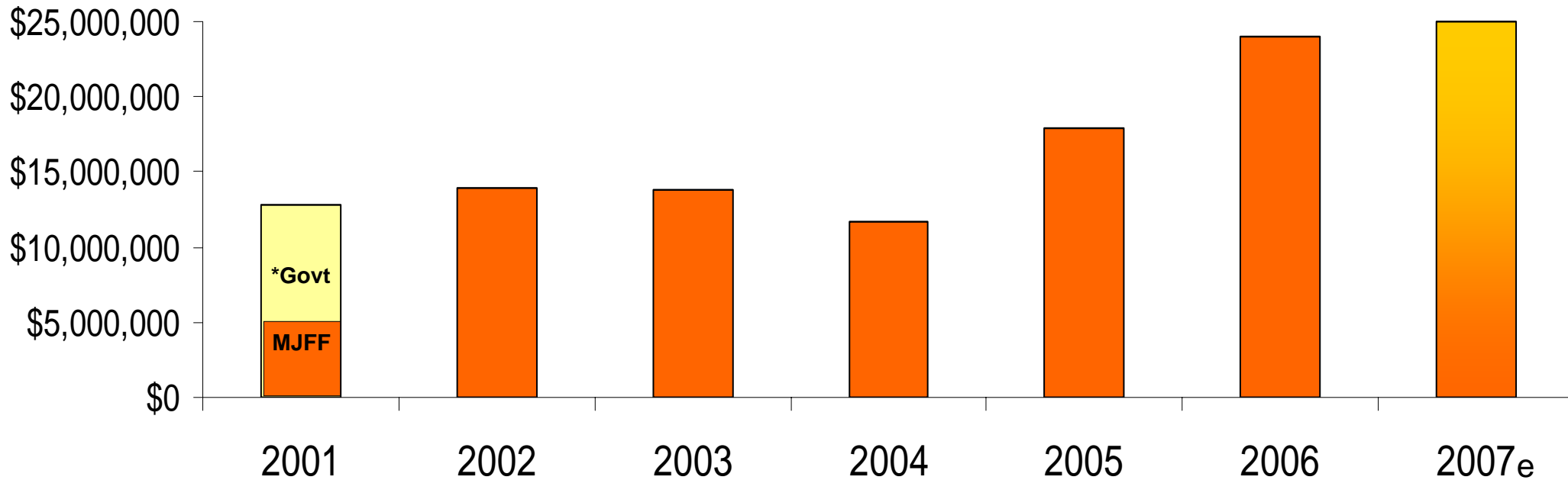
# MJFF: Dedicated to Finding a Cure for Parkinson's Disease

Over time, a number of characteristics have risen to the top in terms of how we do business



# MJFF: Second Largest Funder Annually of PD Research in the World

- Since 2000, we have **funded over \$95 million in PD research**, and estimate that this will top \$100 million in 2007
- MJFF values efficiency and accountability:  
84 cents of every \$1 spent goes to research efforts



\*Note: FY 2001 funding includes a Government partnership through R21 program

# The Challenges that shape our programmatic strategy

- **Staying at the hub of PD research is required and difficult**
  - Our ability to identify the best research requires incredible effort and manpower
  - Ensuring we have the best staff and the best advisors is key
- **MJFF has no endowment and is committed to a zero-equity model**
  - Money is raised in parallel to programmatic funding creating a sense of urgency
  - Goal to raise \$25 million in 2007 – something we work *very* hard to do!
- **Even with our best efforts, planning, and thinking, investing in science is a high risk endeavor**
  - High failure rate comes with the territory – justification for portfolio approach
  - MJFF must be willing to take risks that other funders will not

## ■ MJFF has no set funding line

- MJFF launches each funding initiative with an estimated budget
- Value on funding high-quality science with potential for significant impact for patients – Primary concern is not a PAYLINE!
  - Final funding may be above or below the initially planned budget
    - If more than anticipated worthy science is reviewed, Board of Directors may increase financial allocation to the program. However, MJFF feels no obligation to dedicate the entire program budget if sufficient projects have not been identified.

# MJFF: Innovative Ways to Make Progress

## ■ MJFF as Funder

- MJFF is only funder in the world uniquely focused on accelerating Parkinson's research
- Driving \$\$ to research quickly

## ■ MJFF as Assessor

- Writing a check is not enough!
- MJFF maintains on-going involvement in monitoring and assessing all our grant programs

## ■ MJFF as Convener/Facilitator

- Gather global leaders across fields and disciplines to debate crucial topics and identify aggressive paths forward
- Facilitate collaboration and resource sharing leads to increased efficiency and generation of innovative research topics
- Examples: PD Subtypes (2006), Stem Cell Summit (February 2007), Drug Discovery (May 2007), Understanding Disease Progression (June 2007)

# **The Michael J. Fox Foundation Funding Priorities and Programs**

***Brian Fiske***  
***Associate Director - Research***



# MJFF Launches Multiple Funding Programs Each Year

## ■ Annual Programs

- Target Validation
- Clinical Discovery Awards
- Rapid Response Innovation Awards Program (always open)

## ■ Biennial Programs

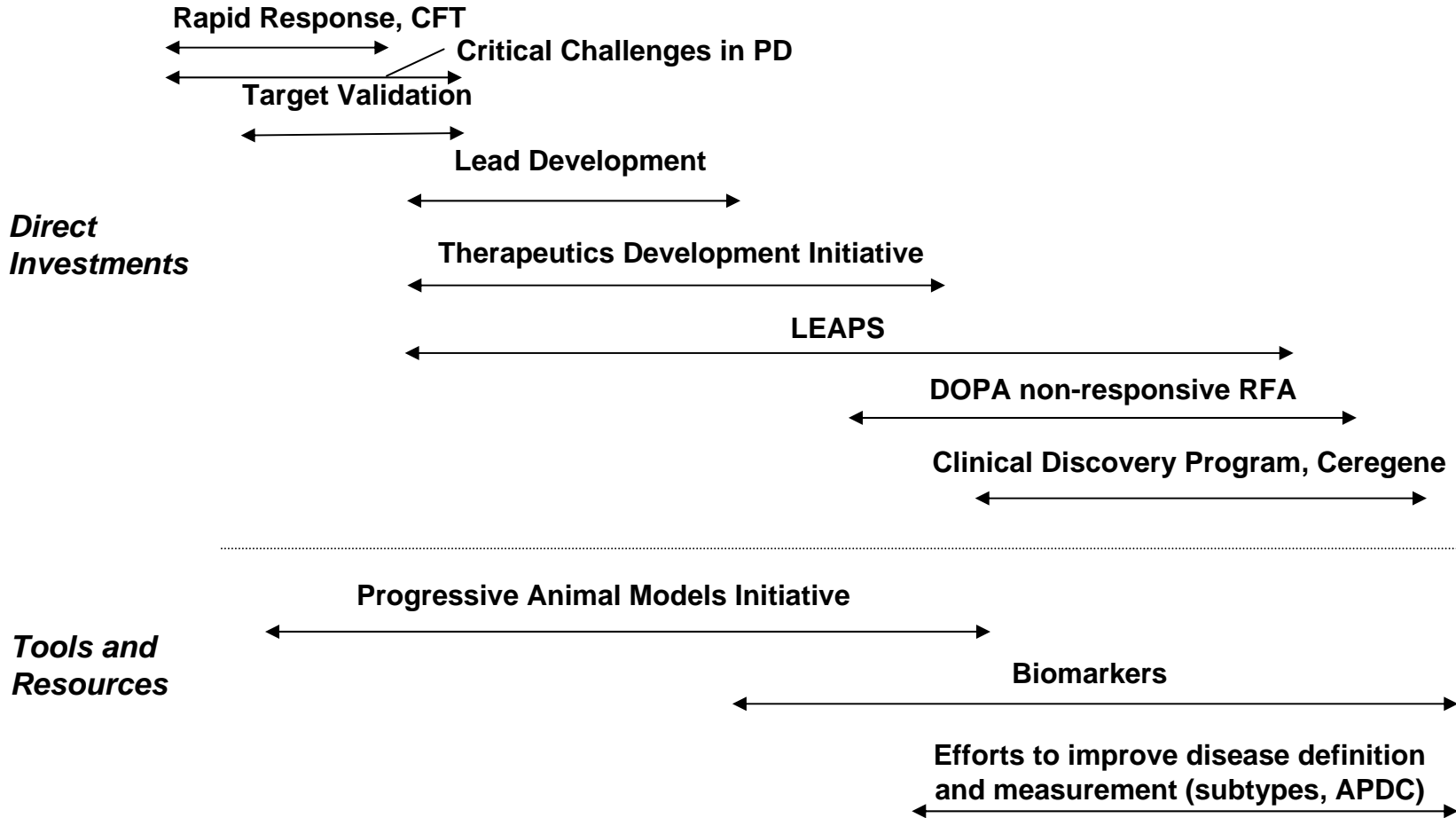
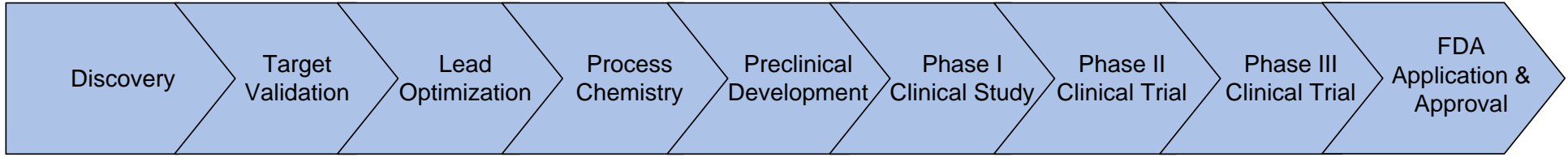
- LEAPS
- Therapeutics Development Initiative

## ■ Field Stimulation Programs

- Biomarkers
- ‘Unmet Needs’ (e.g., DOPA Non-responsive Symptoms, Cognitive Program)
- Critical Challenges in PD (2007 experiment—if successful could become more frequent)
- Topic-specific ‘one-off’ programs (e.g., PD Subtypes, Progressive Animal Models, etc.)

# MJFF: Snapshot of Current Portfolio

**\$100M by end of 2007**



# Target Validation

- Supports up to two years and \$250K for projects focused on validating promising PD targets
- Work should seek to directly determine whether modulation of a potential target results in disease-relevant effects in a whole-animal, mammalian model.
  - Target must be clearly defined
  - Compound screening may be considered in cases where no clear and reasonable strategies exist to modulate a target, but in general are not appropriate as the primary focus of a project
  - Applicants testing small molecule strategies must provide sufficient bioavailability and pharmacokinetic data to justify compound use and dosing in vivo.
  - Cell culture and non-vertebrate studies are allowed as intermediate aims but must be justified and the final project deliverable should still involve testing in a mammalian model.
- Target Validation is launched annually
  - Target Validation 2008 has just been launched
  - Pre-proposal deadline: December 18, 2007
  - Funding decisions expected by May 2008

# Clinical Discovery Awards

- Supports up to three years and \$750K for investigators to conduct novel or critical clinical research seeking to improve treatment of PD and its complications.
- Focus on patient-oriented and well-designed clinical research projects of potentially high-impact approaches that could lead to improved treatments and/or support pivotal clinical testing
  - Must include active recruitment and participation of consenting human subjects
  - Projects solely focused on the identification of risk factors (genetic or environmental) are generally not appropriate.
- The Clinical Discovery Awards program is launched annually
  - Next Round expected to launch in Spring 2008 and funding decisions made by Fall 2008

# Rapid Response Innovation Awards

- Supports up to one year and \$75K for investigators to conduct innovative research focused on the cause and cure of PD
- Focus on critical scientific questions/next steps that can launch new directions for PD therapy development
  - Can be basic, preclinical and/or clinical research
  - Preliminary data not essential (although good rationale is)
  - Focus on clear and testable hypotheses within one-year timeframe
  - Investigator teams must demonstrate expertise and laboratory feasibility to perform work within the year timeframe (i.e., no training grants)
- The Rapid Response Innovation Awards program is open year round
  - Proposals can be submitted at any time
  - Proposals are evaluated by internal MJFF scientific staff and external reviewers with funding decisions made within six weeks from submission
  - Postdocs can apply as project PIs! (But must include the Laboratory head as the Administrative PI)

# Critical Challenges in Parkinson's Disease

- Supports projects focused on specific challenges or hypotheses that are critical barriers to field progress.
- Proposals must seek to directly address the specific challenge
  - Challenges are pre-defined by MJFF and its advisors as areas of emerging need or issues that have been a significant challenge to the field.
  - 2007 program focused on testing specific LRRK2 and a-syn properties that might lead to downstream pathology
  - Funding limits and project duration are defined by the challenge (e.g., 2007 program will fund projects up to \$400K for two years)
- Critical Challenges may become an annual program with new challenges prioritized each year.

# LEAPS (Linked Efforts to Accelerate Parkinson's Solutions)

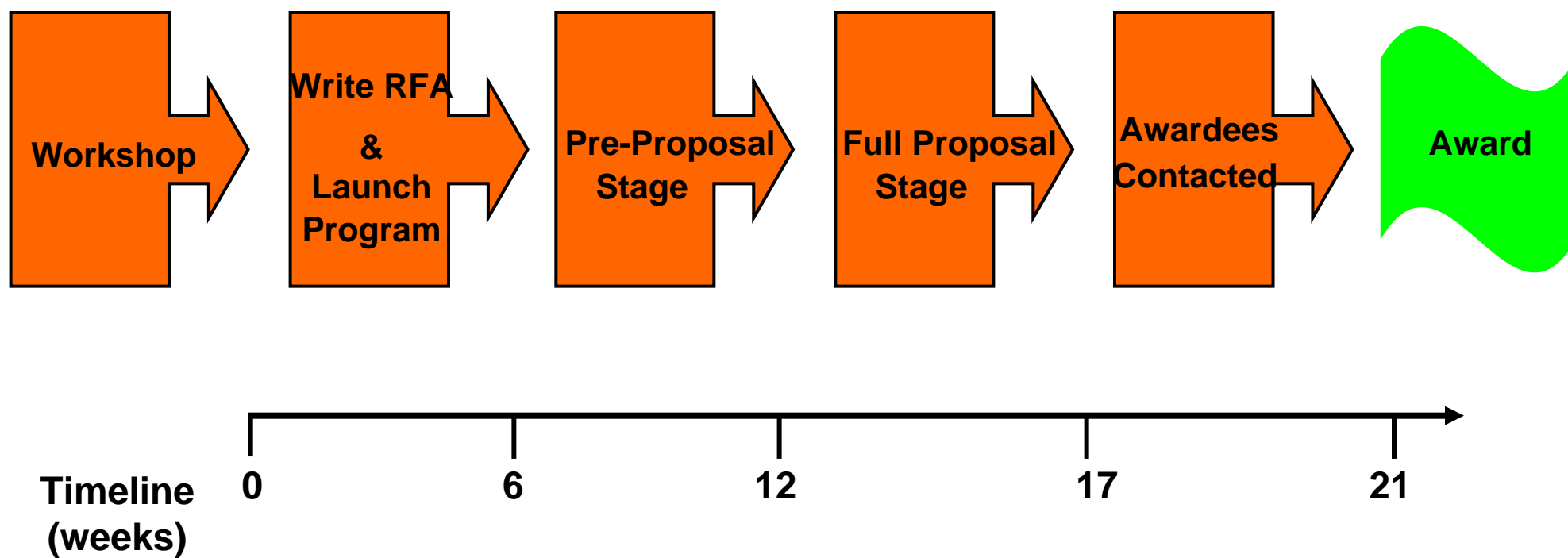
- Supports multi-year, multi-million dollar grants for collaborative, milestone-driven efforts to move a therapeutic program significantly closer to the clinic
- LEAPS is an intentional departure from conventional research grants and requires:
  - Development of a multi-phase research blueprint in advance – project management is key!
  - Structuring of simultaneous experiments and anticipation of handoff points
  - Collaborative sharing of information at every stage
  - Flexibility in project workplan, timeline and budget to allow for unanticipated barriers
  - MJFF objective is to inspire a collaborative, pathway focused and goal-oriented approach to research to accelerate the pace of progress
  - MJFF is a true partner in the research efforts and not just a funder
- LEAPS have historically been launched every other year (next round possible in 2009)

# **The Michael J. Fox Foundation Review and Award Process**

***Mark Frasier***  
***Associate Director - Research***



# MJFF: Programmatic Process



# MJFF: Post Award Process

- MJFF funds the project, not the laboratory
  - There is an expectation of the investigator to adhere to awarded aims
  - Milestones and deliverables are written into the award letter
  - Any changes to the milestones or budget must be discussed with MJFF
  - Check-in calls and in-person assessment meetings are part of every project to ensure progress against awarded aims is made
- If promising results and clear “next steps” supplemental funding is always possible

# MJFF: Criteria for a Successful Application

- Scientific Idea
  - Impact on PD (is it the “critical next step?”), Relevance to goals of the RFA, MJFF portfolio
- Research Plan
  - Testable hypothesis: Is it a “yes/no” question or open-ended (“understand the mechanism”)
    - Will the studies proposed directly test the stated hypothesis?
  - Clear deliverables
  - Achievable in the stated timeframe
- Team
  - Is there appropriate expertise to carry out the proposed experiments?

# Example of Successful Aims

A gene was identified in a *C. elegans* screen that could protect against alpha-synuclein toxicity

Specific Aim 1. Determine the particular protein domains responsible for its protective actions in *C. elegans* by using targeted deletion.

Specific Aim 2. Determine whether the target is protective in a mammalian cell culture model of PD

Specific Aim 3. Determine whether genetic alteration of the target in a mouse affects the susceptibility to a neurotoxin model of PD

Specific Aim 4. Determine whether manipulation of the target protects against a mammalian model of alpha-synuclein over-expression

*Target Validation 2007 Program*

# The Michael J. Fox Foundation Question and Answer

## *MJFF Staff and Advisors*

