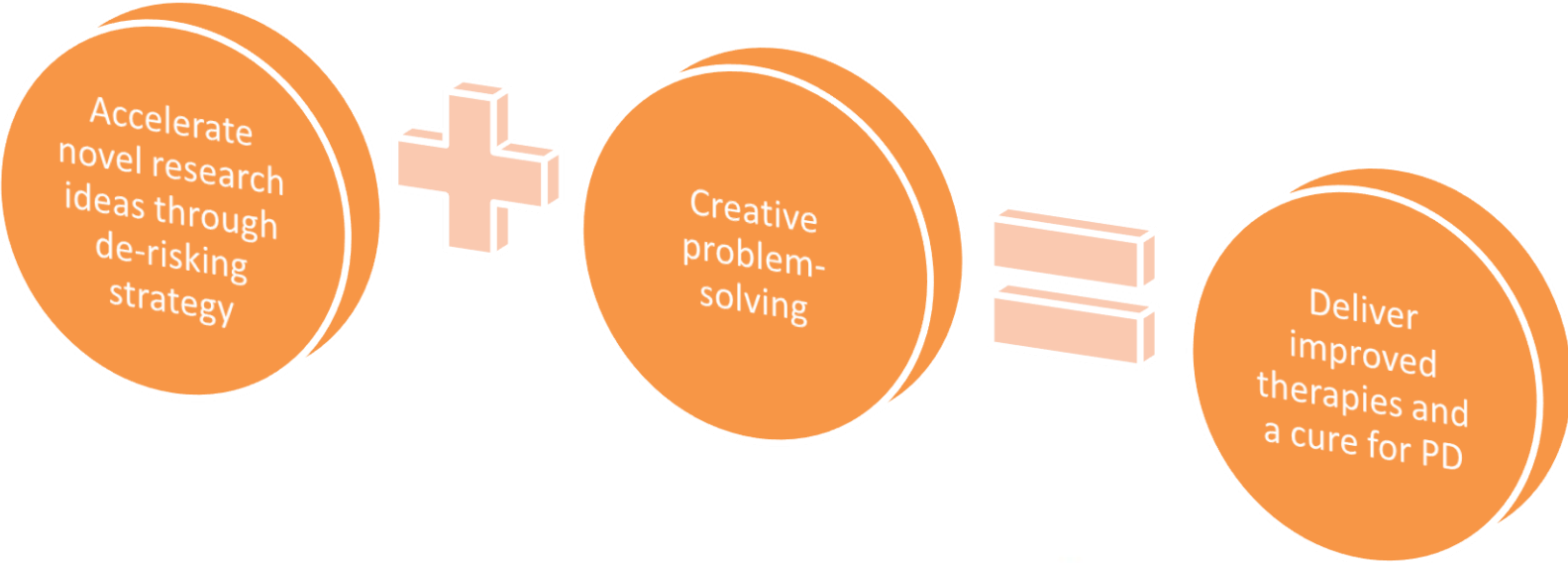


The Michael J. Fox Foundation for Parkinson's Research

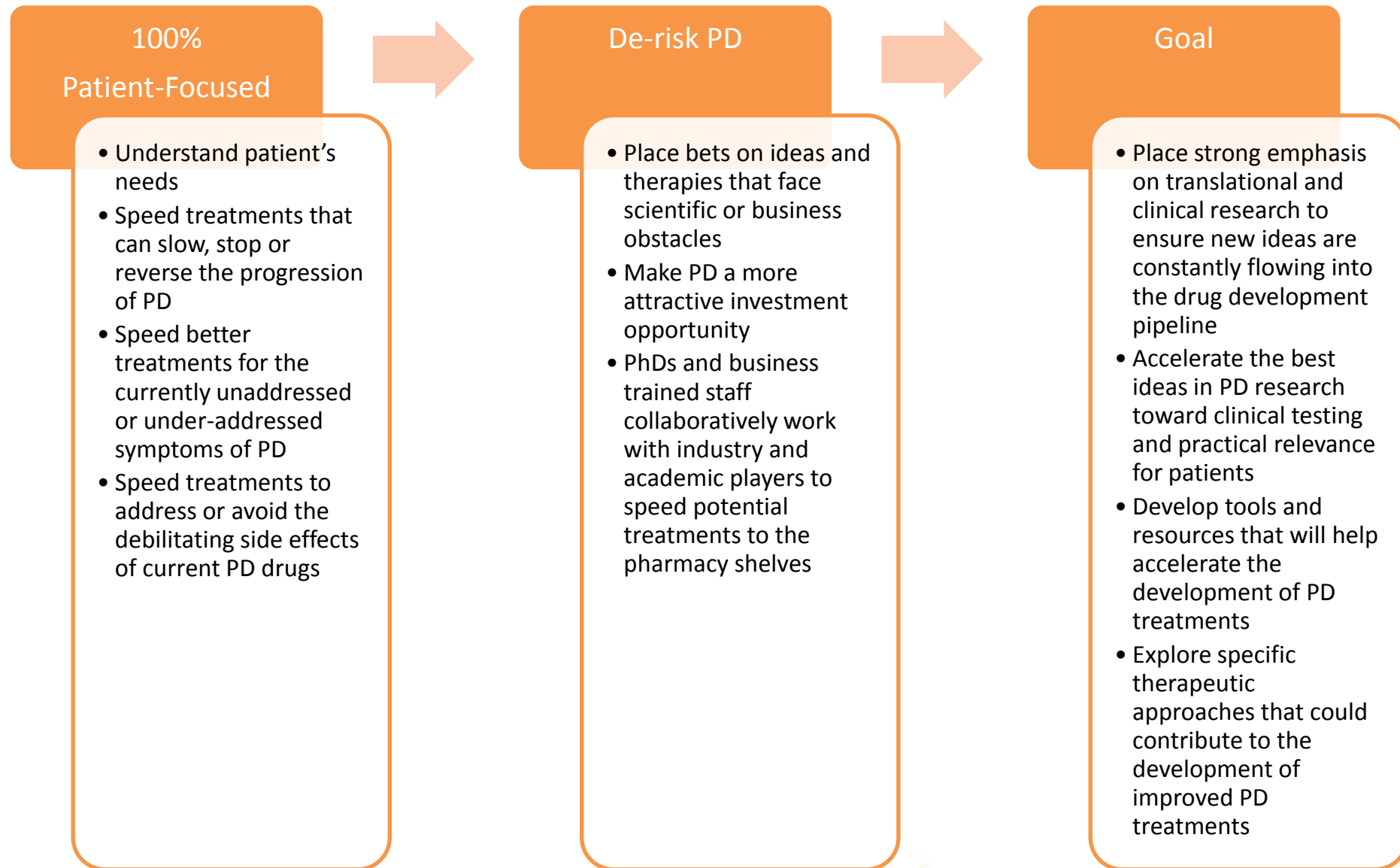
Bridging the gap between basic and
clinical research

December 3, 2012

MJFF Was Founded In 2000 With Clear Objectives



MJFF's Research Strategy



Highlights of Success Stories Through De-risking Strategy

Therapeutic Development

 Lowering Alpha-Synuclein Vaccine
Project: AFFiRiS AG

 Pioneering Partnership: Jeff Conn,
Vanderbilt University

 Phase II Clinical Testing: Jeff Ostrove,
Ceregene

 Phase IIa Study to Reduce Levodopa:
Addex Pharmaceuticals

Research Tools

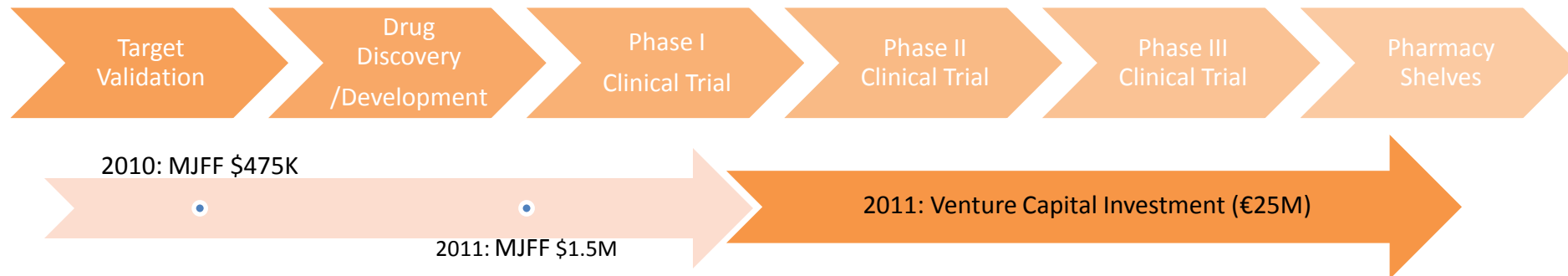
 Parkinson's Progression Markers Initiative
(PPMI)

 Laboratory Tools

 Fox Trial Finder



AFFiRiS AG: Lowering Alpha-Synuclein Vaccine Project



- AFFiRiS (Austria-based biotech) is focused on developing vaccines for treatment of various diseases
- Following work on a vaccine for Alzheimer's, AFFiRiS initiated work on Parkinson's disease with the AFFITOPE® PD01 vaccine, achieving preclinical proof of concept in March 2010
- PD01 targets alpha-synuclein, triggering an immune response, allowing the patient's immune system to reduce this protein
- [MJFF provided AFFiRiS funding in 2010](#) to complete preclinical development of PD01; work then advanced to a phase I clinical trial
- A [second grant was awarded to AFFiRiS in 2011](#) for a first-in-humans clinical trial to assess safety and tolerability (study currently on-going)
- Following MJFF's support and financial commitment of approximately \$2M, AFFiRiS received investments from two venture capital firms, Santo VC and MIG Funds, for a combined investment of €25M, with the option to invest an additional €30M combined



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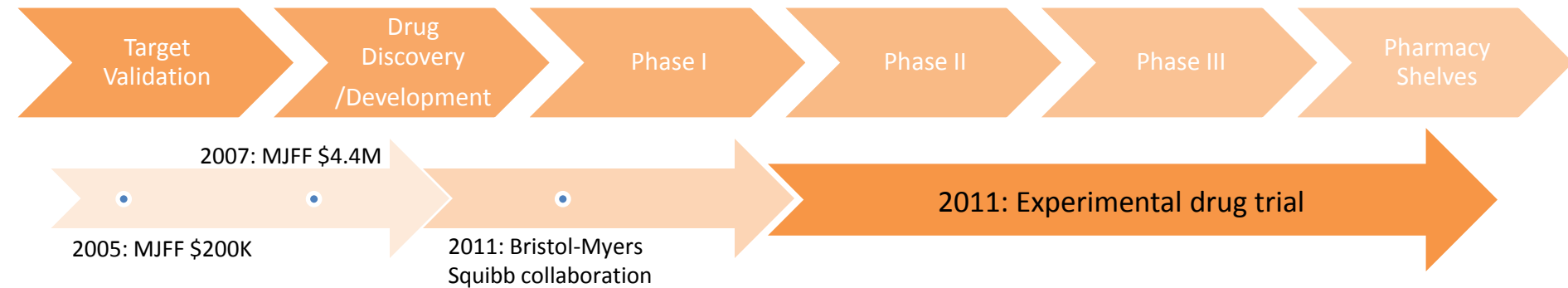
☐ Parkinson's Progression Markers Initiative
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Pioneering Partnership: Jeff Conn, Vanderbilt University



- With over \$5M in funding from MJFF, Conn's team is investigating a specific glutamate receptor called mGluR4
- In 2011 Conn's team brought on a major collaboration with Bristol-Myers Squibb to continue the development of this novel symptomatic treatment
- Experimental drug can possibly enter clinical testing in 2013
- As a part of the initial agreement, MJFF will receive return payments from Vanderbilt to continue funding PD research



Highlights of Success Stories Through De-risking Strategy

Therapeutic Development

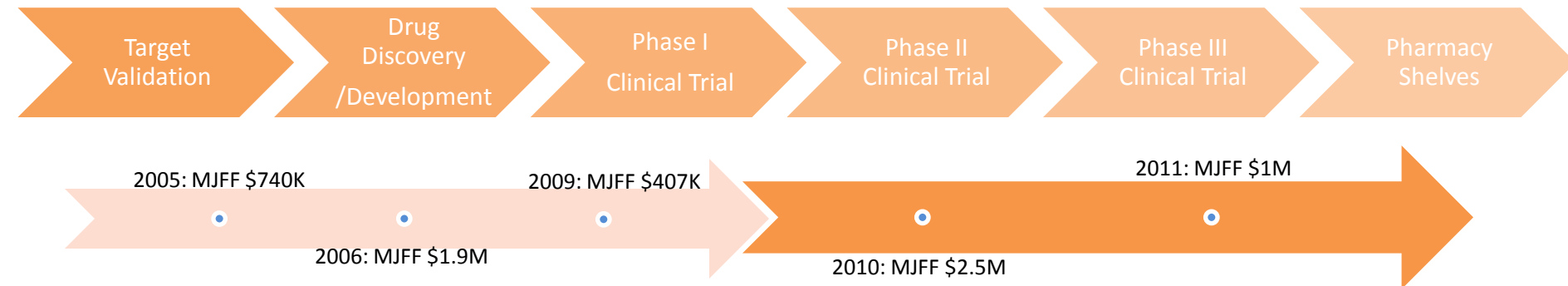
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Phase II Clinical Testing: Jeff Ostrove, Ceregene



- Jeff Ostrove and his team are testing gene therapy for a growth factor called neurturin, as a potential treatment for PD.
- MJFF has been a lead funder of the clinical development of this potential therapy
- Gene therapy has high potential but also high risk – critical role for Foundation support
- Clinical results are expected in 2013

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Phase IIa Study to Reduce Levodopa: Addex Pharmaceuticals



- In a [phase IIa study partially funded by MJFF](#), Addex showed that their novel drug, dipraglurant, was safe and tolerable and proved to effectively reduce dyskinesia in PD patients.
- The company is now developing a strategy to move this drug into later stages of clinical development.
- It is expected that a glutamate-based treatment could be up for regulatory approval by 2016 or 2017.



Highlights of Success Stories Through De-risking Strategy

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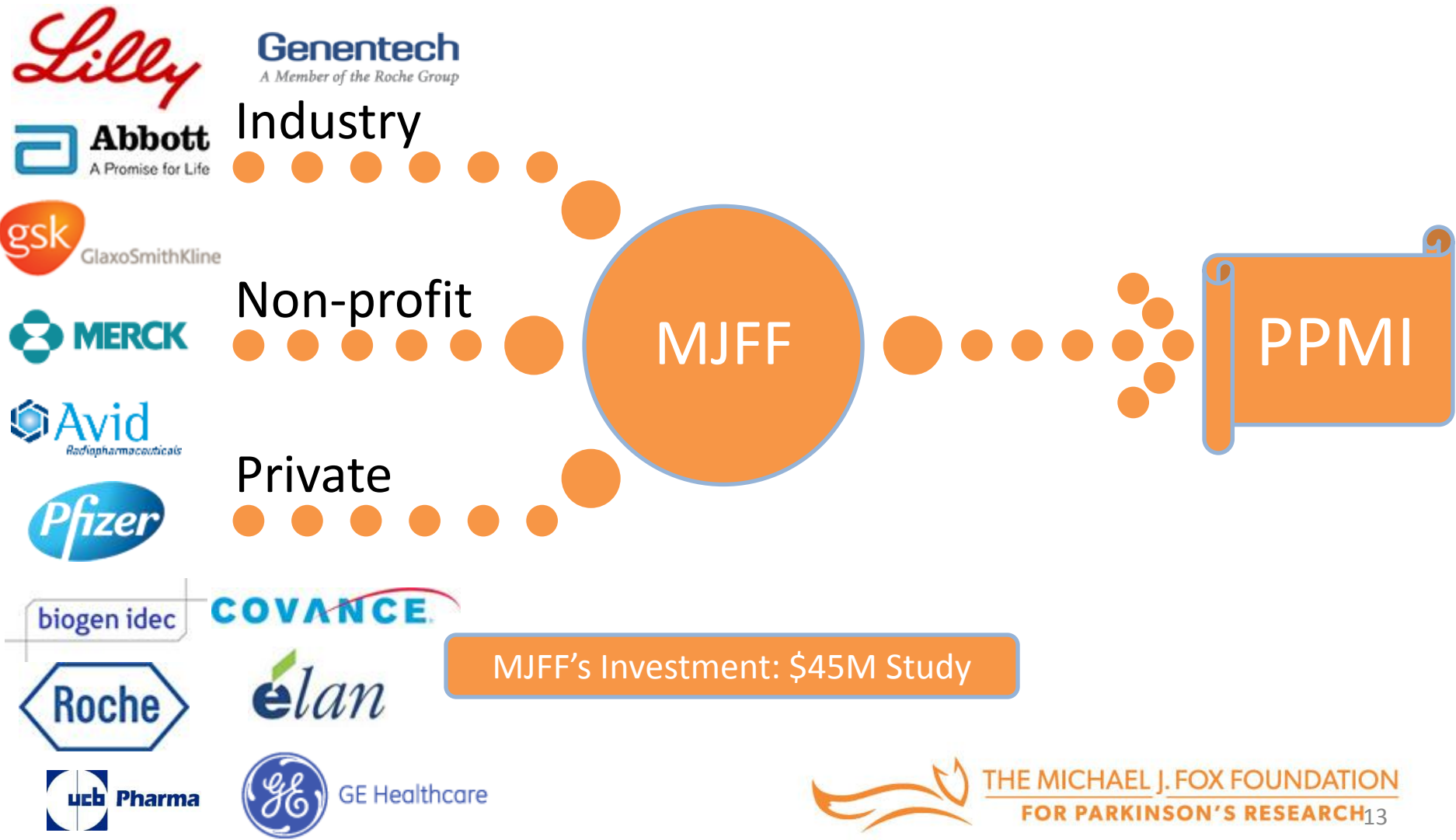
Research Tools

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- ☐ Fox Trial Finder



Parkinson's Progression Markers Initiative (PPMI)

MJFF has bridged the gap between industry players, non-profit organizations, and private individuals to fund PPMI.



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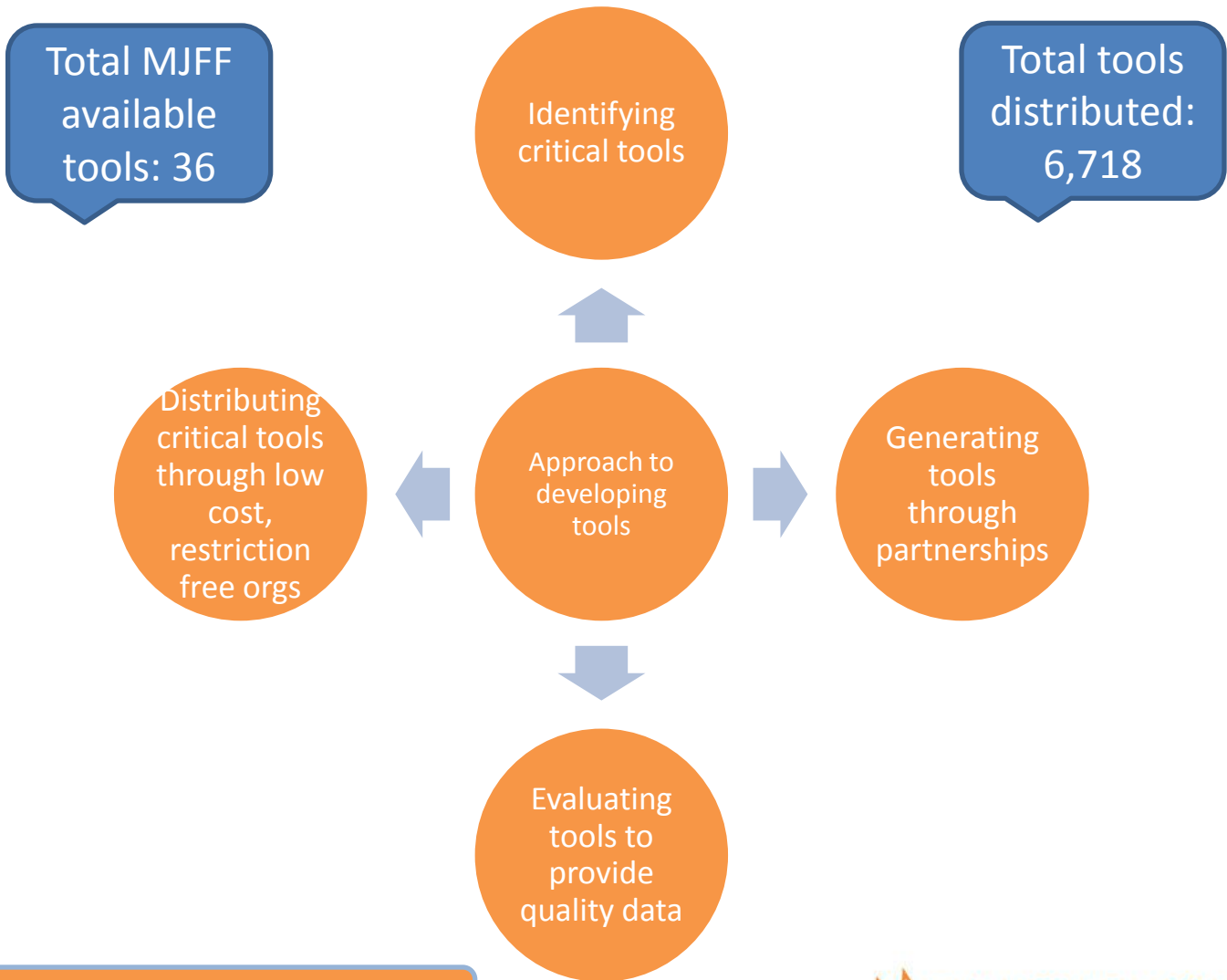
☐ Laboratory Tools

☐ Fox Trial Finder



Our Investment in Laboratory Tools

MJFF prioritizes the generation and availability of critical research tools to accelerate therapeutic development for PD patients.



MJFF's Investment to date: \$12M

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Fox Trial Finder: A Clinical Trial Matching Tool



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INFO FOR VOLUNTEERS INFO FOR TRIAL TEAMS ABOUT FOX TRIAL FINDER **FIND TRIALS** MY FOX TRIAL FINDER



FIND A TRIAL

☐ I have Parkinson's or am registering for someone who does

☐ I do not have Parkinson's but would like to volunteer for a trial

I am located in

United States Postal Code

My birthday is

Month Year

GET STARTED or see [Quick Search](#) results

WHY ARE CLINICAL TRIALS IMPORTANT?



Clinical trial recruitment is a challenge across all diseases, and Parkinson's disease is no exception. Thirty percent of all clinical trials fail to recruit a single subject and 85 percent of trials finish late due to recruitment troubles. Despite a willingness to participate, less than 10 percent of PD patients take part in trials. Patients pay the ultimate price for underenrollment in clinical research, as it means longer time horizons to treatment breakthroughs.



With an estimated 1 million people living with Parkinson's in the U.S. alone, the PD community is poised to take steps to transform their willingness into action — and results. Fox Trial Finder is one solution to help patients and their loved ones get involved in research by making it easier to find trials that are right for them.

FROM MICHAEL J. FOX



TRIAL TEAMS



Do you need volunteers for your clinical trial? Find them with Fox Trial Finder!

LEARN MORE

JOIN THE 10,982 CLINICAL TRIAL VOLUNTEERS WHO HAVE ALREADY STEPPED UP.

- Matches volunteers— PD and controls— to trials they may qualify for
- Matching is based on demographic characteristics, medication history, diagnosis duration, etc. in their area
- Volunteers can then connect directly – online and anonymously – with members of a trial team through secure messaging to learn more

Questions? Contact us at support@foxtrialfinder.org

Conclusion

- Pharmaceutical companies are open to novel models for risk sharing therapeutic development.
 - Development of these models takes time and flexibility
- There is a big need for changing strategies around research tool development and accessibility.
 - Restrictive use licenses limit standardization and discourage field-wide use
- Data availability is also key – but efforts need to be focused given high costs and labor involved
- Foundations can play important roles in direct investments in therapeutic development or in utilizing their neutral convening power to accelerate research