

Potential Disease-Modifying Therapies in the Parkinson's Disease Clinical Trial Pipeline

last update: 11/14/17

Goal: Enable PD patient/family community to more easily **identify funding, staffing, and recruiting needs** for high-priority research towards a cure.

Compiled by PD advocates Sue Buff & Gary Rafaloff

Source: ClinicalTrials.gov, Cure Parkinson's Trust, Michael J. Fox Foundation, industry websites, Parkinson's advocates & researchers

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Note 1: Therapies and trials are listed below in no particular order. Additional data for listed trials is available on ClinicalTrials.gov (trial id# = trial NCT#).

File available at PDTrialTracker.info/Collaboration.html

Note 2: Table below includes 28 active trials, 7 trials in design, and 3 recently completed trials, as of 11/14/17.

Potential Disease-Modifying Therapies in the PD Clinical Trial Pipeline: Active Trials, Trials in Design, Recently Completed Trials

| Therapy (*=repurposed compound) | Description | Phase | Study Title (status) | Study start date | Est. study end date | Est. Enrollment | Sponsor | Collaborators | Study Locations & Eligibility: see ClinicalTrials.gov | More Info | |
|------------------------------------|---|---|---|------------------|---------------------|-----------------|--|--|---|--|--|
| Inosine* | Antioxidant / Nutritional supplement | Phase 3 | Study of Urate Elevation in Parkinson's Disease, Phase 3 (SURE-PD3) (active, not recruiting) | Jun-16 | Aug-20 | 270 | Michael Alan Schwarzschild, Massachusetts General Hospital | PSG, MJFF, UofRochester, NINDS | NCT02642393 | MJFF Blog 9.1.15 | Inosine Phase 2 completed 12/12 (NCT00833690) |
| Isradipine* | Calcium channel blocker | Phase 3 | Efficacy of Isradipine in Early Parkinson Disease (STEADY-PD III) (active, not recruiting) | Sep-14 | Mar-19 | 336 | Univ. of Rochester | NINDS, MJFF, PSG | NCT02168842 | MJFF Blog 6.29.16 | MJFF website: Therapies in Development |
| EPI-589* | Treats childhood mitochondrial diseases | Phase 2A | Safety and Biomarker Study of EPI-589 in Parkinson's Disease (recruiting) | Mar-16 | Jul-18 | 40 | Edison Pharmaceuticals | | NCT02462603 | CPT - Linked Clinical Trials Initiative | |
| Ambroxol* | Used to prevent build-up of excess mucous in respiratory diseases | Phase 2A | Ambroxol in Disease Modification in Parkinson Disease (AiM-PD) (not yet recruiting) | Dec-16 | Dec-17 | 20 | University College, London | CPT, PRO.MED.CS Praha a.s - Czech Republic | NCT02941822 | CPT - Linked Clinical Trials Initiative | |
| Simvastatin* | Cholesterol-lowering drug | Phase 2 | Simvastatin as a Neuroprotective Treatment for Moderate Parkinson's Disease (PD STAT) (recruiting) | Sep-15 | Dec-19 | 198 | Plymouth Hospitals NHS Trust | University of Plymouth | NCT02787590 | CPT - Linked Clinical Trials Initiative | Journal of PD article 11.1.17 on rationale for Simvastatin trial |
| Deferiprone* | Used to reduce iron levels in blood after transfusions | Phase 2 | Conservative Iron Chelation as a Disease-modifying Strategy in Parkinson's Disease (FAIRPARKII) (recruiting) | Feb-16 | Dec-18 | 338 | University Hospital, Lille | European Commission, ApoPharma | NCT02655315 | CPT - Linked Clinical Trials Initiative | Funding received from EU Horizon 2020 research and innovation program. |
| Deferiprone* | Used to reduce iron levels in blood after transfusions | Phase 2 | Study of Parkinson's Early Stage with Deferiprone (SKY) (recruiting) | Jul-16 | Jul-18 | 140 | ApoPharma | | NCT02728843 | CPT - Linked Clinical Trials Initiative | |
| Exenatide (Bydureon)* | Type 2 Diabetes drug (GLP-1 agonist) | Phase 2 completed. View results: CPT site & Lancet. | Trial of Exenatide for Parkinson's Disease (EXENATIDE-PD) (completed) Journal of PD 8.8.17 study commentary | Jun-14 | Aug-16 | 60 | University College, London | | NCT01971242 | CPT - Linked Clinical Trials Initiative | MJFF website: Therapies in Development |
| Liraglutide* | Type 2 Diabetes drug (GLP-1 agonist) | Phase 2 | Safety and Efficacy of Liraglutide in Parkinson's Disease (recruiting) | Apr-17 | Sep-19 | 57 | Cedars-Sinai Medical Center | CPT, Novo Nordisk A/S | NCT02953665 | CPT - Linked Clinical Trials Initiative | |
| Lixisenatide* | Type 2 Diabetes drug (GLP-1 agonist) | Launched in April 2017. (20 hospitals in France) | | Apr-17 | | | Sanofi | | | CPT - Linked Clinical Trials Initiative | |
| NAC (n-Acetyl Cysteine)* | Treats acetaminophen overdose. Used to loosen mucous in cystic fibrosis & COPD. | phase unspecified | Physiological Effects of Nutritional Support in Patients with PD (enrolling by invitation only) | Mar-14 | Mar-18 | 65 | Thomas Jefferson Univ. | | NCT02445651 | | |
| RO0746015 (PRX002) | Alpha-synuclein vaccine | Phase 2 | A Study to Evaluate the Efficacy of RO7046015 in Participants With Early Parkinson's Disease (PASADENA) (recruiting) | Jun-17 | Jun-21 | 300 | Hoffman-La Roche | Prothena Biosciences | NCT03100149 | Prothena 4.2.17 press release re: Phase 1b results (NCT02157714) | Prothena 7.5.17 press release re: Phase 2 study (NCT03100149) |

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|--|-------------------------------------|---|--|------------------|---------------------|-----------------|---------------------------|---|---|--|
| BIIB054 | Alpha-synuclein focus | Phase 1 | Single-Ascending Dose Study of BIIB054 in Healthy Participants and Early Parkinson's Disease (active, not recruiting) | Jul-15 | Nov-17 | 66 | Biogen | | NCT02459886 | MJFF Blog 8/14/15: Two-more-alpha-synuclein-drugs-in-clinical-trials |
| Affitope PD01A | Alpha-synuclein vaccine | Phase 1B completed (results Q4 2017) | Follow-up Study to Assess a Second Boost Immunization With AFFITOPE® PD01A With Regard to Safety and Clinical Activity (AFF008AA) (completed) | Feb-16 | Feb-17 | 26 | Affiris AG | | NCT02618941 | MJFF website: Therapies in Development See Affiris 6.7.17 press release re: PD03A and PD01A |
| Affitope PD03A | Alpha-synuclein vaccine | Phase 1 completed (final results Q4 2017) | Study Assessing Tolerability and Safety of AFFITOPE® PD03A in Patients With Early Parkinson's Disease (AFF011) (completed) | Dec-14 | Aug-16 | 36 | Affiris AG | | NCT02267434 | ParkinsonsNewsToday 6.15.17 Parkinson's vaccine triggers solid immune response. Phase 1 clinical trial shows Affiris press release 6.7.17 re: results of clinical study using AFFITOPE PD03A (SYMPATH funded) |
| Glycerol Phenylbutyrate | Alpha-synuclein focus | Phase 1 | Phenylbutyrate Response as a Biomarker for Alpha-synuclein Clearance From the Brain (active, not recruiting) | Jan-14 | Feb-15 | 40 | Univ. of Colorado-Denver | | NCT02046434 | MJFF research grant related to this study |
| RO0746015 (PRX002) | Gene therapy | Phase 2 | A Global Study to Assess the Drug Dynamics, Efficacy, and Safety of GZ/SAR402671 in Parkinson's Disease Patients Carrying a GBA Mutation (MOVES-PD) (recruiting) | Dec-16 | Apr-22 | 243 | Genzyme, a Sanofi Company | | NCT02906020 | Sanofi Initiates Phase 2 Clinical Trial to Evaluate Therapy for Genetic Form of Parkinson's Disease (2/14/17) |
| AADC Gene Therapy | Gene therapy | Phase 1/2 | AADC Gene Therapy for Parkinson's Disease (NCT02418598) (recruiting) | Apr-15 | Oct -17 / Jan-22 | 6 | Jichi Medical University | Takara Bio Inc., Gene Therapy Research Institution Co. | NCT02418598 | |
| AADC Gene Therapy | Gene therapy | Phase 1 | Safety Study of AADC Gene Therapy (VY-AADC01) for Parkinson's Disease (active, not recruiting) | Oct-13 | Dec-19 | 15 | Voyager Therapeutics | UCSF, Veristat, Feinstein Institute, Oregon Health and Science U. | NCT01973543 | MJFF grant info on this study Preliminary positive Phase 1 results announced 9/17. |
| AADC Gene Therapy | Gene therapy | Phase 1 | Safety and Efficacy Study of VY-AADC01 for Advanced Parkinson's Disease (NCT03065192) (recruiting) | May-17 | Dec-21 | 16 | Voyager Therapeutics | | NCT03065192 | |
| CERE-120 (AA2-NTN) | Gene therapy | Phase 1/2 | Safety and Efficacy of CERE-120 in Subjects with Parkinson's Disease (NCT00985517) (active, not recruiting) | Sep-09 | Mar-18 | 60 | Sangamo Therapeutics | | NCT00985517 | |
| ProvSavin | Gene therapy | Phase 1/2 | Long Term Safety and Efficacy Study of ProSavin in Parkinson's Disease (NCT01856439) (active, not recruiting) | May-11 | May-22 | 15 | Oxford BioMedica | | NCT01856439 | |
| AAV2-GDNF | Neurotrophic factors / Gene therapy | Phase 1 | AAV2-GDNF for Advanced Parkinson's Disease (NT01621581) (recruiting) | May-12 | Dec-27 | 100 | NINDS | | NCT01621581 | |
| CDNF (Cerebral Dopamine Neurotrophic Factor) | Neurotrophic factors | Phase 1/2 | Clinical Study to Test the Safety of CDFN by Brain Infusion in Patients With Parkinson's Disease (recruiting) | Sep-17 | Nov-19 | 18 | Herantis Pharma Plc. | Renishaw Plc. | NCT03295786 | Study website (TreatER project. Funding from EU Horizon 20/20 Program) herantis.com/pipeline/cdnf-in-parkinsons-disease/ |

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| Nilotinib* (Tasigna) | Leukemia drug (C-Abl Inhibitor) | Phase 2 | Impact of Nilotinib on Safety, Tolerability, Pharmacokinetics and Biomarkers in PD (PD Nilotinib) (recruiting) | Jan-17 | Jul-20 | 75 | Georgetown Univ. | | NCT02954978 | Nilotinib Phase 1 completed (NCT02281474) | |
| Nilotinib* (Tasigna) | Leukemia drug (C-Abl Inhibitor) | Phase 2 | Nilotinib in Parkinson's Disease (NILO-PD) (patients involved in design) (recruiting) | Oct-17 | Oct-20 | 135 | Northwestern Univ. Feinberg School of Medicine | Univ. of Rochester, Univ. of Iowa, MJFF | NCT03205488 | MJFF, VARI and CPT collaborate to assess Nilotinib | MJFF Blog 3.15.17 |
| ISC-hpNSC | Stem cell therapy | Phase 1 | A Study to Evaluate the Safety of Neural Stem Cells in Patients With Parkinson's Disease (recruiting) | Mar-16 | Mar-19 | 12 | Cyto Therapeutics, a subsidiary of Int'l Stem Cell Corp. | | NCT02452723 | Trial article in San Diego Union Tribune 3/7/2016 | Trial update in San Diego Tribune 11/14/16 |
| Cu(II)ATSM | | Phase 1 | Phase 1 Dose Escalation Study of Cu(II)ATSM in Parkinson's Disease (recruiting) | Jul-17 | Mar-19 | 38 | Collaborative Medicinal Development Pty Limited | | NCT03204929 | www.colmeddev.co m/procypra/ | www.ncbi.nlm.nih. gov/pmc/articles/P MC3328361/ |
| DA-9805 | Botanical drug product composed of 3 main raw herbal materials | Phase 2 | Safety and Efficacy of DA-9805 for Parkinson's Disease (not yet recruiting) | Sep-17 | Mar-19 | 60 | Dong-A ST Co., Ltd. | | NCT03189563 | | |
| Nicoderm Patch | Transdermal nicotine | Phase 2 (results expected early 2017) | Disease-modifying Potential of Transdermal NiCotine in Early Parkinson's Disease (NIC-PD) (active, not recruiting) | Oct-12 | Dec-16 | 160 | James Boyd MD, Univ. of Vermont | MJFF, PSG, GPS, DPG, et al. | NCT01560754 | MJFF website: Therapies in Development | |
| Blood Plasma | Blood plasma infusion | Phase 1 | The Stanford Parkinson's Disease Plasma (SPDP) Study (active, not recruiting) | Nov-16 | Dec-18 | 5 | Stanford Univ. | | NCT02968433 | www.bioportfolio.c om: The-Stanford- Parkinsons-Disease- Plasma-Study | June 2015 TED Talk: "How Young Blood Might Reverse Aging" |
| EPI-743 | Targeted at mitochondrial and other neuro diseases | Phase 2B in design (check with BioElectron/Edison on status) | | | | | | | | CPT - Linked Clinical Trials Initiative | Phase 2A completed (NCT01923584) |
| UDCA* | Used to treat liver disease. Acts to recover mitochondrial function. | Trial in design | | | | | | | | CPT - Linked Clinical Trials Initiative: Trials Pending (seeking funding) | |
| MSDC-0160* (Mitoglitazone) | Originally developed to treat Type 2 diabetes. May improve mitochondrial function in PD. | Pre-clinical, Phase 1/Phase 2 in design | | | | | | | | CPT - Linked Clinical Trials: VARI, CPT, Metabolic Solutions collaboration | www.msdrx.com/p ipeline/msdc-0160 |
| NAC (n-Acetyl Cysteine)* | Treats acetaminophen overdose. Used to loosen mucous in cystic fibrosis & COPD. | Trial in design (CPT: NAC Trial-The Facts) | | | | | | | | CPT - Linked Clinical Trials Initiative (seeking funding) | NAC Phase 1 completed 2012 (NCT01427517) |
| NPT200-11 | Alpha-synuclein vaccine | Phase 2 in design | | | | | | | | Neuropore Announces Successful Completion of Phase I with NPT200-11, 3.22.16 | Phase 1 study completed (NCT02606682). MJFF funded. |
| NPT088 | Alpha-synuclein vaccine | Awaiting results from Alzheimer's phase 1B trial NCT03008161 | | | | | | | | Proclara Biosciences - Our Programs (Phase 1B for Alzheimer's NCT03008161) | Ref. MJFF NPT088 research grant |
| GM608 | Neurotrophic factors | Phase 2B in design | | | | | | | | GM608 Phase 2A (GAP-PD: NCT01850381) completed. | Genervon Pipeline. See also 1/10/17 press release. |