

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

last update: 10/4/17

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 website, Michael J. Fox Foundation website, 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 27 active trials, 8 trials in design, and 3 recently completed trials, as of 10/4/17.

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	Estimated 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) (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)
Isradapine*	Calcium channel blocker	Phase 3	Efficacy of Isradapine 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 Trial Initiative	
EPI-743	Targeted at mitochondrial and other neuro diseases		Phase 2B in design							Phase 2A completed (NCT01923584)	EdisonPharma.com/about-edison/
UDCA*	Used to treat liver disease. Acts to recover mitochondrial function.		Trial in design							CPT - Linked Clinical Trial Initiative: Trials Pending (seeking funding)	
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 Trial 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 Trial Initiative	Funders: Plymouth Univ., JPM Moulton Trust, CPT
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 Trial 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 Trial Initiative	
Exenatide (Bydureon)*	Type 2 Diabetes drug (GLP-1 agonist)		Trial of Exenatide for Parkinson's Disease (EXENATIDE-PD) (completed)	Jun-14	Aug-16	60	University College, London		NCT01971242	CPT - Linked Clinical Trial 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 Trial Initiative	

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Lixisenatide*	Type 2 Diabetes drug (GLP-1 agonist)		Trial in design							CPT - Linked Clinical Trial Initiative (seeking funding)
MSDC-0160* (Mitoglitazone)	Originally developed to treat Type 2 diabetes. May improve mitochondrial function in PD.		Pre-clinical, Phase 1 in design							CPT - Linked Clinical Trials: VARI, CPT, Metabolic Solutions collaboration (seeking funding) www.msdrx.com/pipeline/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). Cost: \$1.9M							CPT - Linked Clinical Trial Initiative (seeking funding) NAC Phase 1 completed 2012 (NCT01427517)
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)
NPT200-11	Alpha-synuclein vaccine		Phase 2 in design							Neuropore Announces Successful Completion of Phase I with NPT200-11 Phase 1 study completed (NCT02606682)
NPT088, NPT289	Alpha-synuclein vaccine		Pre-clinical, Phase 2 in design							Proclara Biosciences - Our Programs (Phase 1B for Alzheimer's NCT03008161) Ref. MJFF NPT088 research grant
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

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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	
GM608	Neurotrophic factors		Phase 2B in design							GM608 Phase 2A (GAP-PD: NCT01850381) completed. Genervon Biopharmaceuticals Pipeline. See also 1/10/17 press release.
CDNF (Cerebral Dopamine Neurotrophic Factor)	Neurotrophic factors	Phase 1/2	Clinical Study to Test the Safety of CDNF 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/
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) (not yet recruiting)	Sep-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

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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.com/procypra/	www.ncbi.nlm.nih.gov/pmc/articles/PMC3328361/
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) (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.com: The-Stanford-Parkinsons-Disease-Plasma-Study	June 2015 TED Talk: "How Young Blood Might Reverse Aging"