

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

last update: 9/4/18

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 31 active trials (17 of which are recruiting), 8 trials in design, and 3 recently completed trials, as of 9/4/18.

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	Recruiting status	Study start date	Est. study end date	Est. Enrollment	Sponsor	Collaborators	Locations & Eligibility: link to 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	active, not recruiting	Mar-16	Dec-18	40 (44 actual)	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)	active, not recruiting	Dec-16	Apr-18	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)	active, not recruiting	Mar-16	May-20	198 (235 actual)	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	
Lovastatin*	Cholesterol-lowering drug	Phase 2	Lovastatin as a Neuroprotective Treatment for Early Stage Parkinson's Disease	recruiting	May-17	Dec-19	80	National Taiwan University Hospital		NCT03242499			
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	completed	Jun-14	Aug-16	60	University College, London		NCT01971242	CPT - Linked Clinical Trials Initiative	MJFF website: Therapies in Development	
Exenatide (Bydureon)*	Type 2 Diabetes drug (GLP-1 agonist)	Phase 1	Effects of Exenatide on Motor Function and the Brain	recruiting	Jun-18	Apr-21	20	University of Florida	NINDS	NCT03456687			
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)	Phase 2	Study to Evaluate the Effect of Lixisenatide in Patients With Parkinson's Disease (LixiPark)	recruiting	Jun-18	Dec-21	158	University Hospital, Toulouse	CPT, Reseau NS- Park, EUCLID, Sanofi	NCT03439943	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	active, not recruiting	Mar-14	Dec-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	Feb-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)	12.20.17 mini- review of PD alpha-syn initiatives

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BIIB054	Alpha-synuclein focus	Phase 2	Evaluating the Safety, Pharmacokinetics, and Pharmacodynamics of BIIB054 in Participants With Parkinson's Disease (SPARK)	recruiting	Dec-17	Jun-22	311	Biogen		NCT03318523	Phase 1 study NCT02459886 completed Nov. 2017.	12.20.17 mini-review of PD alpha-syn initiatives	MJFF Blog 8/14/15: Two-more-alpha-synuclein-drugs-in-clinical-trials
Affitope PD01A	Alpha-synuclein vaccine	Phase 1B completed	Follow-up Study to Assess a Second Boost Immunization With AFFITOPE® PD01A With Regard to Safety and Clinical Activity (AFF008AA) (completed)	completed	Feb-16	Feb-17	26	Affiris AG		NCT02618941	MJFF website: Therapies in Development	ParkinsonsNewsToday 5.18.18 Potential-parkinsons-vaccine-affitope-pd01a-shows-promising-long-term-results	
Affitope PD03A	Alpha-synuclein vaccine	Phase 1 completed	Study Assessing Tolerability and Safety of AFFITOPE® PD03A in Patients With Early Parkinson's Disease (AFF011) (completed)	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)	
Lu AF82422	antibody inhibiting alpha-synuclein	Phase 1	Lu AF82422 in Healthy Non-Japanese and Japanese Subjects and in Patients With Parkinson's Disease	recruiting	Jul-18	Mar-20	44	H. Lundbeck A/S		NCT03611569	Lundbeck 8.7.18 press release re: Phase 1 Lu AF82422		
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	recruiting	Apr-15	Oct-18 / 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	active, not 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	active, not recruiting	Sep-09	Mar-18	60	Sangamo Therapeutics		NCT00985517	AAV2 (CERE-120) for PD (3/13 article)	AAV2-NTN for PD: Lessons Learned (2016 article)	
ProvSavin	Gene therapy	Phase 1/2	Long Term Safety and Efficacy Study of ProSavin in Parkinson's Disease	active, not recruiting	May-11	May-22	15	Oxford BioMedica		NCT01856439			
AAV2-GDNF	Gene therapy / Neurotrophic factors	Phase 1	AAV2-GDNF for Advanced Parkinson's Disease (NT01621581)	active, not recruiting	May-12	Dec-27	actual: 25; est. was 100	NINDS		NCT01621581	enrollment target lowered early 2018		
CDNF (Cerebral Dopamine Neurotrophic Factor)	Gene therapy / 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/	
Nilotinib* (Tasigna)	Leukemia drug (C-Abl Inhibitor)	Phase 2	Impact of Nilotinib on Safety, Tolerability, Pharmacokinetics and Biomarkers in PD (PD Nilotinib)	active, not recruiting	Jan-17	Jul-20	75	Georgetown Univ.		NCT02954978	Nilotinib Phase 1 completed (NCT02281474)		

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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	Dec-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.coimeddev.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	recruiting	Feb-18	Mar-19	60	Dong-A ST Co., Ltd.		NCT03189563			
Nicoderm Patch	Transdermal nicotine	Phase 2 (results expected Q1 2018)	Disease-modifying Potential of Transdermal Nicotine in Early Parkinson's Disease (NIC-PD)	active, not recruiting (as of Sep. '15)	Oct-12	Dec-16	160	James Boyd MD, Univ. of Vermont	MJFF, PSG, GPS, DPG, et al.	NCT01560754	MJFF NIC-PD Grant Information		
Blood Plasma	Blood plasma infusion	Phase 1	The Stanford Parkinson's Disease Plasma (SPDP) Study	active, not recruiting	Nov-16	Dec-18	15	Stanford Univ.		NCT02968433	www.bioportfolio.c om: The-Stanford- Parkinsons-Disease- Plasma-Study	June 2015 TED Talk: "How Young Blood Might Reverse Aging"	
ITI-214	Phosphodiesterase 1 (PDE-1) inhibitor	Phase 1/2	Safety, Tolerability, Pharmacokinetics and Pharmacodynamics of ITI-214 in Parkinson's Disease	recruiting	Sep-17	Aug-18	24	Intra-Cellular Therapies, Inc.		NCT03257046			
EPI-743	Targeted at mitochondrial and other neuro diseases	Phase 2B in design (check with BioElectron/Edison on status)									CPT - Linked Clinical Trials Initiative	Neurology.org April 2016 EPI-743 Phase 2A results	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	More on UDCA at scienceofparkinson s.com	
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/pi peline/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	NAC Phase 1 completed 2012 (NCT01427517)	
NPT200-11	Alpha-synuclein vaccine	Phase 1B planned by UCB/Neuropore									Neuropore Announces Successful Completion of Phase I with NPT200-11, 3.22.16	Phase 1 study completed (NCT02606682).	MJFF update on alpha-syn trials 7/10/17
NPT088	Alpha-synuclein vaccine	Awaiting results from Alzheimer's phase 1B trial NCT03008161 (est. completion 4/19)									Proclara Biosciences - Our Programs (Phase 1B for Alzheimer's NCT03008161)	Ref. MJFF NPT088 research grant	MJFF update on alpha-syn trials 7/10/17
GM608	Neurotrophic factors	Phase 2B planned									GM608 Phase 2A (GAP-PD: NCT01850381) completed.	Genervon.com - Pipeline	
DNL201	Small molecule inhibitor of LRRK2 (LRRK2 inhibitor)	Denali is planning a Phase 1B clinical study in PD patients with and without a LRRK2 mutation by year-end 2018									Denali 8.1.18 press release re: results of DNL201 Phase 1 study	More on LRRK2 at scienceofparkinson s.com	