

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

last update: Nov. 28, 2018

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

Compiled by PD research advocates Sue Buff & Gary Rafaloff

Source: ClinicalTrials.gov, The Cure Parkinson's Trust - LCT Programme (CPT-LCT), Michael J. Fox Foundation (MJFF), industry websites, researchers & advocates (Simon Stott: ScienceofParkinsons.com, Kevin McFarthing: The Hope List-Parkinson's Therapies in Development)

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Note 1: Therapies & trials are listed below in no particular order. Similar therapy types are grouped together by color code for easier viewing.

File available at PDTrialTracker.info (Collaboration page)

Note 2: Additional data for listed trials, including **location and eligibility**, is available on ClinicalTrials.gov (ref. Study ID # in table below)

Potential Disease-Modifying Therapies in PD Clinical Trials (includes 53 active Phase 1, 2, 3 trials, as of November 28, 2018)											
Therapy (*=repurposed compound)	Description	Phase	Study Title	Study ID # on ClinicalTrials.gov	Sponsor & Collaborators	Recruiting Status	Study Start Date	Est. Study End Date	Estimated Enrollment	More Info	
Inosine*	Antioxidant / Nutritional supplement	Phase 3	Study of Urate Elevation in Parkinson's Disease, Phase 3 (SURE-PD3)	NCT02642393	M. Schwarzschild, Mass. General Hospital, PSG, MJFF, UofRochester, NINDS	active, not recruiting	Jun-16	Oct-19	270	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)	NCT02168842	Univ. of Rochester, NINDS, MJFF, PSG	active, not recruiting	Sep-14	Mar-19	336	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	NCT02462603	BioElectron (formerly Edison Pharma), CPT-LCT incl. VARI	active, not recruiting	Mar-16	Dec-18	40 (44 actual)	CPT - Linked Clinical Trials Initiative	
UDCA*	Used to treat liver disease. Acts to recover mitochondrial function.	Phase 1	Brain Bioenergetics in Parkinson's Disease and Response to Repeated Oral UDCA Treatment	NCT02967250	Univ. of Minnesota	recruiting	Jun-17	Mar-19	20	More on UDCA at scienceofparkinsons.com	CPT - LCT Initiative planning UDCA trial (est. late '18)
Simvastatin*	Cholesterol-lowering drug	Phase 2	Simvastatin as a Neuroprotective Treatment for Moderate Parkinson's Disease (PD STAT)	NCT02787590	Plymouth Hospitals NHS Trust, CPT-LCT inc. VARI, University of Plymouth, Jon Moulton Charitable Trust	active, not recruiting	Mar-16	May-20	198 (235 actual)	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	NCT03242499	National Taiwan University Hospital,	recruiting	May-17	Dec-19	80		
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)	NCT02655315	University Hospital-Lille, European Commission, ApoPharma	recruiting	Feb-16	Feb-21 (was Feb-18)	338	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)	NCT02728843	ApoPharma,	active, not recruiting	Jul-16	Dec-19 (was Jul-18)	140	CPT - Linked Clinical Trials Initiative	
Exenatide (Bydureon)*	Type 2 Diabetes drug (GLP-1 agonist)	Phase 2 completed.	Trial of Exenatide for Parkinson's Disease (EXENATIDE-PD) (completed) Journal of PD 8.8.17 study commentary	NCT01971242	University College, London, CPT-LCT inc VARI	completed	Jun-14	Aug-16	60	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	NCT03456687	University of Florida, NINDS	recruiting	Jun-18	Apr-21	20	Peptron (Korea) evaluating SR Exenatide PT302 for PD	
Liraglutide*	Type 2 Diabetes drug (GLP-1 agonist)	Phase 2	Safety and Efficacy of Liraglutide in Parkinson's Disease	NCT02953665	Cedars-Sinai Medical Center, CPT - LCT inc VARI, Novo Nordisk A/S	recruiting	Apr-17	Sep-19	57	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)	NCT03439943	University Hospital, Toulouse, CPT - LCT inc VARI, Reseau NS-Park, EUCLID, Sanofi	recruiting	Jun-18	Dec-21	158	CPT - Linked Clinical Trials Initiative	

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Semaglutide*	Type 2 Diabetes drug (GLP-1 agonist)	Phase 2	(Semaglutide) GLP1R in Parkinson's Disease (GIPD)	NCT03659682	Oslo Univ. Hospital	not yet recruiting	Jan-19	Dec-24	120	Neuroprotective effects of Semaglutide in MPTP PD mouse model (Neuropeptides, Oct. 18)	
Ambroxol*	Used to prevent build-up of excess mucous in respiratory diseases	Phase 2A	Ambroxol in Disease Modification in Parkinson Disease (Aim-PD)	NCT02941822	University College, London, CPT-LCT inc. VARI, John Black Charitable Trust, PRO.MED.CS Praha a.s - Czech Republic	active, not recruiting	Dec-16	Apr-18	20	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	NCT02445651	Thomas Jefferson Univ., CPT-LCT candidate	active, not recruiting	Mar-14	Dec-18	65		
RO0746015 (PRX002)	Alpha-synuclein vaccine; Immunotherapy (Mab to aSN)	Phase 2	A Study to Evaluate the Efficacy of RO7046015 in Participants With Early Parkinson's Disease (PASADENA)	NCT03100149	Hoffman-La Roche, Prothena Biosciences	recruiting	Jun-17	Feb-21	300	Prothena 4.2.17 press release re: Phase 1b results (NCT02157714)	Prothena 7.5.17 press release re: Phase 2 study (NCT03100149)
BIIB054	Alpha-synuclein focus	Phase 2	Evaluating the Safety, Pharmacokinetics, and Pharmacodynamics of BIIB054 in Participants With Parkinson's Disease (SPARK)	NCT03318523	Biogen - www.biogen.com/en_us/pipeline.html (Phase 1 NCT02459886 completed 11/17)	recruiting	Jan-18	Apr-22	311	12.20.17 mini-review of PD alpha-syn initiatives	MJFF Blog 8/14/15: Two more-alpha-synuclein-drugs-in-clinical-trials
BIIB054	Alpha-synuclein focus	Phase 1	A Study to Evaluate Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of BIIB054 in Japanese Participants With Parkinson's Disease	NCT03716570	Biogen	not yet recruiting	Feb-19	Aug-21	24	https://www.biogen.com/en_us/pipeline.html	
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)	NCT02618941	Affiris AG	completed	Feb-16	Feb-17	26	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)	NCT02267434	Affiris AG	completed	Dec-14	Aug-16	36	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	NCT03611569	H. Lundbeck A/S	recruiting	Jul-18	Mar-20	44	Lundbeck 8.7.18 press release re: Phase 1 Lu AF82422	
MEDI1341	alpha-synuclein antibody	Phase 1	Single Ascending Dose Study of MEDI1341 in Healthy Volunteers	NCT03272165	AstraZeneca, Covance, MMS Holdings, Inc, Catalent	recruiting	Oct-17	Nov-19 (from Sep-19)	40	AstraZeneca 8.29.17 press release re: MEDI1341	
Glycerol Phenylbutyrate	Alpha-synuclein focus	Phase 1	Phenylbutyrate Response as a Biomarker for Alpha-synuclein Clearance From the Brain	NCT02046434	Univ. of Colorado-Denver	active, not recruiting	Jan-14	Sep-18 (was Feb-15)	40	MJFF research grant related to this study	
PBT 434	designed to inhibit aggregation of alpha-synuclein & tau	Phase 1	A Phase 1 Single and Multiple Ascending Dose Study of PBT434 in	not on CT.gov	Prana Biotechnology (has partnership with Takeda)					Prana to-commence-phase-1-clinical-trial-of-pbt434 (6.4.18)	prana-doses-first-patients-in-phase-1 trial (7.5.18)

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DNL201	LRRK2 inhibitor / Lysosomal function enhancer	Phase 1B	Study to Evaluate DNL201 in Subjects With Parkinson's Disease	NCT03710707	Denali Therapeutics (www.denalitherapeutics.com/develop/#pipeline)	recruiting	Nov-18	Sep-19	30	Denali 8.1.18 press release on results of DNL201 Phase 1 (mentions active Phase 1 for DNL151, not on CT.gov) More on LRRK2 at scienceofparkinsons.com
LTI-291	Gcase enzyme activator	Phase 1b	Trial to test safety and tolerability of LTI-291 in Parkinson's patients with a heterozygous mutation in the GBA1 gene	not on CT.gov	Lysosomal Therapeutics, Allergan. Study conducted in the Netherlands					www.lysosomaltx.com/patients www.allergan.com - Allergan Option Arrangement with Lysosomal (LTI), 1.9.17
GZ/SAR402671	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)	NCT02906020	Genzyme, a Sanofi Company	recruiting	Dec-16	Apr-22	243	Sanofi Initiates Phase 2 Clinical Trial to Evaluate Therapy for Genetic Form of Parkinson's Disease (2/14/17)
AAV-hAADC-2	Gene therapy	Phase 1/2	AADC Gene Therapy for Parkinson's Disease	NCT02418598	Jichi Medical University, Takara Bio Inc., Gene Therapy Research Institution Co.	recruiting	Apr-15	Oct-18 / Jan-22	6	
VY-AADC01	Gene therapy	Phase 1	Safety Study of AADC Gene Therapy (VY-AADC01) for Parkinson's Disease	NCT01973543	Voyager Therapeutics, UCSF, Veristat, Feinstein Institute, Oregon Health and Science U.	active, not recruiting	Oct-13	Dec-19	15	MJFF grant info on this study Preliminary positive Phase 1 results announced 9/17.
VY-AADC01	Gene therapy	Phase 1	Safety and Efficacy Study of VY-AADC01 for Advanced Parkinson's Disease	NCT03065192	Voyager Therapeutics	active, not recruiting	May-17	Dec-21	16	www.voyagertherapeutics.com/our-approach-programs/pipeline/
VY-AADC02	Gene therapy	Phase 2	VY-AADC02 for Parkinson's Disease With Motor Fluctuations	NCT03562494	Voyager Therapeutics	recruiting	Jun-18	Dec-20	42	parkinsonsnewstoday.com, 2018/07/20, parkinsons gene therapy vy-aadc in phase 2 trial
CERE-120 (AA2-NTN)	Gene therapy	Phase 1/2	Safety and Efficacy of CERE-120 in Subjects with Parkinson's Disease	NCT00985517	Sangamo Therapeutics	active, not recruiting	Sep-09	Mar-18	60	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	NCT01856439	Oxford BioMedica	active, not recruiting	May-11	May-22	15	
AAV2-GDNF	Gene therapy / Neurotrophic factors	Phase 1	AAV2-GDNF for Advanced Parkinson's Disease	NCT01621581	NINDS	active, not recruiting	May-12	Dec-27	actual: 25; est. was 100	enrollment target lowered early 2018
CDNF (Cerebral Dopamine Neurotropic 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	NCT03295786	Herantis Pharma Pl., Renishaw Plc.	recruiting	Sep-17	Nov-19	18	Study website (TreatER project. Funding from EU Horizon 20/20 Program) herantis.com/pipeline/cdnf-in-parkinsons-disease/
GDNF	Gene therapy	Phase 2 completed	GDNF in Idiopathic PD	NCT03652363	North Bristol NHS Trust	completed	Oct-12	Apr-16	42 actual	medgenesis.com - Feb. '18 press release; planning Phase 3 Medgenesis GDNF program received grants from MJFF, Parkinsons UK
OXB-102 (AXO-Lenti-PD)	Gene therapy	Phase 1/2	Study of OXB-102 (AXO-Lenti-PD) in Patients With Bilateral, Idiopathic Parkinson's Disease	NCT03720418	Oxford BioMedica, Axovant Sciences	recruiting	Oct-18	Jun-22/Dec-31	30	Axovant-Licenses- Investigational- Gene- Therapy-for-PD 6.6.18
Nilotinib* (Tasigna)	Leukemia drug (C-Abl Inhibitor)	Phase 2	Impact of Nilotinib on Safety, Tolerability, Pharmacokinetics and Biomarkers in PD (PD Nilotinib)	NCT02954978	Georgetown Univ.	active, not recruiting	Jan-17	Jul-20	75	Nilotinib Phase 1 completed (NCT0281474)

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Nilotinib* (Tasigna)	Leukemia drug (C-Abl Inhibitor)	Phase 2	Nilotinib in Parkinson's Disease (NILO- PD) (patients involved in design)	NCT03205488	Northwestern Univ. Feinberg School of Medicine, Univ. of Rochester, Univ. of Iowa, MJFF, CPT-LCT inc VARI	recruiting	Oct-17	Oct-20	135	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	NCT02452723	Cyto Therapeutics, a subsidiary of Int'l Stem Cell Corp.	recruiting	Mar-16	Dec-19	12	Int'l Stem Cell Corp. 6.11.18 press release re: ISC-hpNSC	Trial article in San Diego Union Tribune 3/7/2016
NTCELL	Stem cell therapy	Phase 2b	Investigation of the Safety and Efficacy of NTCELL in Patients With Parkinson's Disease	NCT02683629	Living Cell Technologies,	active, not recruiting	Feb-16	Apr-19	18	lctglobal.com - development-to-date	Phase 1/2a (NCT01734733) completed June 2015. 4 patients
OK99	Stem cell therapy	Phase 1	OK99 Stem Cells to Treat PD	not on CT.gov	Celavie Biosciences					www.celavie.com/celav ie/product- development/	3 year Phase 1 taking place in Mexico
Neural Stem Cell- Derived Neurons	Stem cell therapy	Phase 1/2	Transplantation of Neural Stem Cell- Derived Neurons for Parkinson's Disease	NCT03309514	Neurogeneration	not yet recruiting	Feb-18	Jun-19	12	http://www.neurogene ration.com	
fetal stem cell transplant	Stem cell therapy	Phase 1	TRANSEURO Open Label Transplant Study in Parkinson's Disease (TRANSEURO)	NCT01898390	Univ. of Cambridge, TRANSEURO European Research Consortium CPT	active, not recruiting	May-12	Dec-19	40	http://www.transeuro. org.uk/index.html	
human neural stem cells (ANGE- S003)	Stem cell therapy	Phase 2/3	A Study To Evaluate the Safety and Efficacy of Human Neural Stem Cells for Parkinson's Disease Patient (hNSCPD)	NCT03128450	Second Affiliated Hosp. of Sochow University, Shanghai Angecon Biotechnology Cooperate	enrolling by invitation	Apr-17	Nov-18	12		
Dopamine Neuronal Precursor Cells	Stem cell therapy	Phase 1/2	Evaluation of Safety and Tolerability of Fetal Mesencephalic Dopamine Neuronal Precursor Cells for PD	NCT01860794	Bundang CHA Hospital	recruiting	May-13	Apr-22	15		
Neural Precursor Cells	Stem cell therapy	Phase 1/2	Safety and Efficacy Study of Human ESC-derived Neural Precursor Cells in the Treatment of PD	NCT03119636	Chinese Academy of Sciences, 1st Affiliated Hosp. of Zhengzhou Univ.	recruiting	May-17	Dec-20	50		
Umbilical cord derived mesenchymal stem cells	Stem cell therapy	Phase 1/2	Use of Mesenchymal Stem Cells in Parkinson Disease (PD)	NCT03684122	Univ. of Jordan	recruiting	Jun-18	Apr-19	12	APDA: Understanding stem cell therapy in PD treatment	
Cu(II)ATSM	Releases copper ("Cu") in cells with damaged mitochondrial electron transport chains.	Phase 1	Phase 1 Dose Escalation Study of Cu(II)ATSM in Parkinson's Disease	NCT03204929	Collaborative Medicinal Development Pty Limited,	recruiting	Jul-17	Mar-19	38	www.colmeddev.com/ procvpra/	www.ncbi.nlm.nih.gov/ pmc/articles/PMC33283 61/
DA-9805	Botanical drug product composed of 3 main raw herbal materials	Phase 2	Safety and Efficacy of DA-9805 for Parkinson's Disease	NCT03189563	Dong-A ST Co., Ltd.	recruiting	Feb-18	Mar-19	60		
Lingzhi (Ganoderma)	medicinal mushroom	Phase 3	Effects of Lingzhi on Disease Progression in Patients With Untreated Early PD	NCT03594656	Xuanwu Hospital, Beijing	not yet recruiting	Jul-18	Dec-20	288	Pharmacological effects of natural Ganoderma and its extracts on neurological diseases	www.ncbi.nlm.nih.gov/ pubmed/22593926
Nicoderm Patch	Transdermal nicotine	Phase 2 (results expected Q1 2018)	Disease-modifying Potential of Transdermal Nicotine in Early Parkinson's Disease (NIC-PD)	NCT01560754	James Boyd MD, Univ. of Vermont, MJFF, PSG, GPS, DPG, et al.	active, not recruiting (as of Sep. '15)	Oct-12	Dec-16	160	MJFF NIC-PD Grant Information	
Blood Plasma	Blood plasma infusion	Phase 1	The Stanford Parkinson's Disease Plasma (SPDP) Study	NCT02968433	Stanford Univ.	active, not recruiting	Nov-16	Dec-18	15	www.bioportfolio.com: The-Stanford- Parkinsons-Disease- Plasma-Study	June 2015 TED Talk: "How Young Blood Might Reverse Aging"

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ITI-214	Phosphodiesterase 1 (PDE 1) inhibitor	Phase 1/2	Safety, Tolerability, Pharmacokinetics and Pharmacodynamics of ITI-214 in Parkinson's Disease	NCT03257046	Intra-Cellular Therapies, Inc.	recruiting	Sep-17	Aug-18	40	Intracellulartherapies.com news-release April 2018	
KM-819	FAF-q inhibitor	Phase 1	KM-819 for Patients With Parkinson's Disease	NCT03022799	Kainos Medicine	recruiting	Oct-16	Oct-17	88	www.kainosmedicine.com pipeline	
IRX4204	RXR agonist (nuclear retinoid receptors)	Phase 1 completed	Study of IRX4204 for Treatment of Early Parkinson's Disease	NCT02438215	IO Therapeutics	completed	Aug-14	May-15	15	io- therapeutics.com/programs/ 4204-2/	NCT02438215 = Phase 1 for PD, completed, publication not found.
K0706	Abl & Fin kinase inhibitor /potential alpha-syn affect	Phase 2	A Phase 2 Study of K0706 for Early Parkinson's Disease	NCT03655236	Sun Pharma (SPARC)	not yet recruiting	Dec-18	Feb-21	504	Seekingalpha - SPARC slideshow Sept. 2018, ref. slides 19 - 22	Sun Pharma plans Phase 2 of of SCC-138 (Abl &Fin kinase inhibitor) in Q3 2019
Nicotinamide riboside	dietary supplement	N/A	A Randomized Controlled Trial of Nicotinamide Supplementation in Early PD: the NOPARK Study	NCT03568968	Haukeland University Hospital	not yet recruiting	Oct-18	Oct-21	200		