What's Next in Parkinson's Disease? A Look at the Pipeline and Clinical Trials

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Parkinson's disease is a progressive neurodegenerative disorder primarily caused by the loss of dopamine-producing neurons in the substantia nigra, a midbrain structure in the brain crucial for motor control, reward, and cognitive functions, leading to manifestation of motor symptoms called parkinsonism like tremors, bradykinesia, rigidity as well as postural instability. It was first described by James Parkinson in 1817.

By the time symptoms appear, 60% or more of these neurons are lost. The disease can also affect norepinephrine production potentially causing non-motor symptoms like fatigue and blood pressure changes.[1] The histological hallmark of Parkinson's disease is the presence of the aggregation of alpha-synuclein into Lewy bodies within neurons.[2]

Parkinson's disease affects 1 to 2 per 1000 individuals, with prevalence increasing to 1% of the population over 60 years. The condition is more common in men, and genetic predisposition is a factor in 5% to 10% of cases. Globally, over 10 million people are estimated to live with Parkinson's disease.[3]

Parkinson's disease is diagnosed clinically, based on patient history, symptoms, and physical examination and an accurate diagnosis in initial stages may be difficult. While no definitive lab or imaging test exists, MRI, Dopamine Transporter Scan, and blood work may support the diagnosis or exclude conditions mimicking Parkinson's disease.[4]

There is an unmet need to develop new treatments that can either alleviate the symptoms of Parkinson's disease or stop its progression.

This article provides an update on current treatment options for Parkinson's disease and outlines the latest development activities including ongoing clinical trials of key experimental drugs with data sourced from IQVIA Pipeline Link and IQVIA Trial Link.

Current treatment options for Parkinson's disease

Although there are no disease-modifying drugs for Parkinson's disease, current pharmacological interventions offer significant symptomatic relief of motor symptoms by increasing endogenous dopamine levels or directly mimicking dopamine's effect on the brain.

The cornerstone of pharmacological therapy remains dopaminergic medications, most notably levodopa, which is typically administered with a peripheral dopa-decarboxylase inhibitor, like carbidopa, to enhance efficacy and reduce side effects. Adjunct therapies such as dopamine agonists (e.g. pramipexole, ropinirole), monoamine oxidase B (MAO-B) inhibitors (e.g. selegiline, rasagiline), and catechol-O-methyltransferase (COMT) inhibitors (e.g. entacapone) are introduced to manage motor fluctuations and reduce "off" periods. Anticholinergic drugs, including procyclidine and trihexyphenidyl, act through non-dopaminergic mechanisms by reducing acetylcholine activity and are primarily used for tremor and rigidity; however, their use is now limited, especially in older adults, due to significant side effects. Amantadine, initially approved for the treatment of influenza, was later found to show efficacy in levodopa-induced dyskinesias and is now used for managing levodopa-induced dyskinesias in patients with Parkinson's disease.[5–7]

While levodopa remains the gold standard, newer formulations like Amneal's CREXONT (extended-release levodopa) and INBRIJA (inhaled levodopa powder) by Acorda Therapeutics offer improved management of "off" episodes and motor functions.[8,9] Additionally, newer dopamine agonists such as UCB's NEUPRO (rotigotine transdermal patch) and Sunovion's KYNMOBI (apomorphine sublingual film) provide alternative delivery routes for continuous symptom control.[10,11]

Emerging therapies targeting non-dopaminergic systems, such as adenosine A2A receptor antagonists like Kyowa Kirin's NOURIANZ (Istradefylline), have shown promise in reducing "off" time when added to levodopa therapy.[12] For advanced cases, Abbvie's DUOPA (levodopa-carbidopa intestinal gel) aims to reduce motor complications.[13]

Recent US FDA approvals include AbbVie's VYALEV (foscarbidopa and foslevodopa) in October 2024. This carbidopa/levodopa prodrug combination, administered as a 24-hour continuous subcutaneous infusion, helps manage motor fluctuations in adults with advanced Parkinson's disease. The approval was based on the pivotal Phase III M15-736 study (NCT04380142) which exhibited VYALEV to have superior improvement in motor fluctuations, with increased "on" time without troublesome dyskinesia and decreased "off" time, compared with oral immediate-release carbidopa/levodopa therapy at 12 weeks in patients with advanced Parkinson's disease.[14]

Supernus Pharmaceutical's ONAPGO is the first subcutaneous apomorphine infusion device to be approved for Parkinson's disease in February 2025 and the approval was based on the results from a Phase III pivotal trial (NCT02006121) which demonstrated that ONAPGO significantly decreased daily "off" time, and this decrease was accompanied by a significant elevation in daily good "on" time. Improvements in both daily "off" time and good "on" time were seen from week 1 and persisted throughout the investigation. Furthermore, ONAPGO-treated patients more frequently reported an improvement in their overall health status, compared with those receiving placebo.[15]

Newer options like Amneal's CREXONT (extended-release) and Acorda's INBRIJA (inhaled powder) are transforming how patients manage "off" episodes and motor symptoms — offering more control, flexibility, and hope.

Parkinson's disease clinical trial landscape

As of April 2025, Trial Link listed 60 ongoing clinical trials (active, not recruiting and recruiting), including 38 in Phase II and Phase III development; selected trials are listed in the table below.

DRUG (TARGET/ PRODUCT TYPE)	COMPANY	TRIAL PHASE	ROA	NCT ID	PRIMARY OUTCOME MEASURES	TRIAL START DATE	PRIMARY COMPLETION DATE	PREDICTED LAUNCH DATE*	NUMBER OF SITES (COUNTRIES)
ND0612 (DOPAMINE/ SMALL MOLECULE)	MITSUBISHI TANABE PHARMA	PHASE III	PRE-FILLED SYRINGE	NCT 04006210	"ON" TIME (EFFICACY)	30/9/2019	1/11/2022	Q3 2025	103 (USA+15)
P2B001 (MAOB; DRD2; DRD3; DRD4/SMALL MOLECULE)	PHARMA TWO B	PHASE III	ORAL ORDINARY	NCT 03329508	UPDRS (EFFICACY)	19/1/2018	23/8/2021	Q4 2026	72 (USA+3)
ANVS402 (APP; SNCA; MAPT/SMALL MOLECULE)	ANNOVIS BIO	PHASE III	ORAL ORDINARY	NCT 05357989	"OFF" TIME (EFFICACY); MDS-UPDRS (EFFICACY)	3/8/2022	4/12/2023	Q1 2026	69 (USA+5)
CVL-751 (DRD1; DRD5/ SMALL MOLECULE)	ABBVIE	PHASE III	ORAL ORDINARY	NCT 04223193	UPDRS (EFFICACY)	6/1/2020	1/10/2024	Q4 2026	53 (USA+12)
CVL-751 (DRD1; DRD5/ SMALL MOLECULE)	ABBVIE	PHASE III	ORAL ORDINARY	NCT 04760769	C-SSRS (EFFICACY); EPWORTH SLEEPINESS SCALE (EFFICACY) + 8	24/2/2021	31/1/2026	Q4 2026	140 (USA+13)
CVN424 (GPR6/SMALL MOLECULE)	CEREVANCE	PHASE III	ORAL ORDINARY	NCT 06553027	"OFF" TIME (EFFICACY)	20/9/2024	31/10/2025	Q2 2027	26 (USA+1)
AB-1005 (GDNF/GENE THERAPY)	BAYER	PHASE II	VIAL	NCT 06285643	HAUSER DIARY (EFFICACY)	11/6/2024	30/11/2027	Q3 2032	37 (USA+3)
BIIB122 (LRRK2/SMALL MOLECULE)	BIOGEN	PHASE II	ORAL ORDINARY	NCT 05348785	UPDRS (EFFICACY)	19/4/2022	1/12/2025	Q1 2031	113 (USA+12)
AAV-GAD (GAD1/GENE THERAPY)	MEIRAGTX	PHASE II	VIAL	NCT 05603312	SAFETY (SAFETY); TOLERABILITY (SAFETY)	5/10/2022	6/9/2024	Q2 2030	6 (USA)
ACI-7104.056 (SNCA/ VACCINE)	AC IMMUNE	PHASE II	PRE-FILLED SYRINGE	NCT 06015841	MRI ABNORMALITY (EFFICACY); NEUROLOGICAL EXAMINATION (SAFETY) + 4	24/7/2023	31/1/2028	Q2 2032	12 (UK+2)
NOUVNEU001 (DOPAMINE/ DOPAMINERGIC NEURONAL PRECURSER CELL)	IREGENE	PHASE II	VIAL	NCT 06167681	MOTOR FUNCTION (SAFETY); TOLERABILITY (SAFETY)	17/1/2024	31/12/2025	Q2 2031	2 (CHINA)
HB-ADMSC (ALLOGENIC) (MESENCHYMAL STEM CELL THERAPY)	HOPE BIOSCIENCES	PHASE II	VIAL	NCT 04995081	PROTHROMBIN TIME (PHARMACODYNAMIC); THROMBOEMBOLIC EVENTS (SAFETY) + 16	16/7/2021	15/12/2025	Q1 2031	1 (USA)
TLN-1 (GM1/ LIPOPEPTIDE)	INNOMEDICA	PHASE II	VIAL	NCT 06431971	MDS-UPDRS (EFFICACY)	31/8/2024	30/11/2025	Q1 2031	1 (SWITZER-LAND)
BAN0805 (SNCA/ MONOCLONAL ANTIBODY)	BIOARCTIC	HTT	VIAL	NCT 06671938	ADVERSE EVENTS (SAFETY)	16/3/2026	16/3/2026	Q2 2031	16/3/2026
LY3884961 (GBA1/GENE THERAPY)	LILLY	Gene editing	VIAL	NCT 04127578	GCASE ACTIVITY (EFFICACY); NERVE CONDUCTION STUDY (EFFICACY) + 3	31/12/2030	31/12/2030	Q2 2036	31/12/2030

* Source: IQVIA Pipeline Link

Novel therapies in development for Parkinson's disease

Although levodopa (LD) is the preferred drug for Parkinson's disease, due to extracerebral metabolism, a substantial dose of LD is necessary to achieve a therapeutic effect. The administration of this drug inevitably leads to side effects such as gastrointestinal symptoms, involuntary movements, and orthostatic hypotension. LD is effective in managing the motor disturbances associated with Parkinson's disease, extended use of LD is known to cause LD-induced dyskinesia.[16] There is an unmet need to develop new treatments that can either alleviate the symptoms of Parkinson's disease or stop its progression.[17] Current research focuses on alpha-synuclein aggregation, alongside initiatives targeting specific pathways linked to the rarer genetic variants of the disease. Therapies aimed at alpha-synuclein pathology, which contributes to neurodegeneration in Parkinson's disease, hold promise as potential disease-modifying treatments in the near future. When combined with regenerative approaches such as stem cell and gene therapies, these advancements are expected to offer new and effective treatment options in the coming years.

TREATMENT	SUMMARY
ACI-7104	AC Immune's ACI-7104 — a vaccine designed to target alpha-synuclein.
AB-1005	EBayer's AB-1005 — a gene therapy candidate utilizing adeno-associated viral vector serotype 2 (AAV2) to deliver the human glial cell line-derived neurotrophic factor (GDNF) transgene.
Nouv Neu001	iRegene's NouvNeu001 — a chemically induced human dopaminergic neuron precursor from induced pluripotent stem cells (iPSC).
ALY3962681	Lilly's LY3962681 — a novel siRNA (small interfering RNA) therapy being developed for Parkinson's disease.
SLS-004	Seelos' SLS-004 — a gene therapy program aimed at regulating the SNCA Gene, which encodes alpha-synuclein.
BIIB122	Biogen and Denali's BIIB122— a selective and brain-penetrant small molecular inhibitor of LRRK2 (leucine-rich repeat kinase 2).

ACI-7104

AC Immune's ACI-7104 (also known as ACI-7104.056), a vaccine designed to target alpha-synuclein, aims at treating and preventing Parkinson's disease. ACI-7104 specifically targets pathological alpha-synuclein species, including toxic oligomers, whose reduction has been linked to clinical benefits.[18] ACI-7104 is currently under Phase II evaluation for the treatment of patients with early Parkinson's disease. Interim results from the Phase II trial demonstrated that treatment with ACI-7104 resulted in an average 16-fold increase in anti- alpha-synuclein antibodies from baseline from baseline compared to the placebo following three immunizations.[19]

AB-1005

Bayer's AB-1005 (also known as AAV2-GDNF) is a gene therapy candidate utilizing adeno-associated viral vector serotype 2 (AAV2) to deliver the human glial cell line-derived neurotrophic factor (GDNF) transgene. This enables stable and continuous GDNF expression in localized regions of the brain following direct neurosurgical injection. AB-1005 has been granted Fast Track Designation by the US FDA in July 2024. AB-1005 is currently under Phase II development for moderate stage Parkinson's disease, with recruitment ongoing in the REGENERATE-PD Phase II trial (NCT06285643). Data from a Phase I trial demonstrated that administration of AB-1005 was well tolerated with no serious adverse events. Patients with moderate stage Parkinson's disease showed trends for improvement or stability on several motor scales at 36 months, including Movement Disorder Society-Unified Parkinson's Disease Rating Scale (MDS-UPDRS) and motor diaries, and trends in reductions in Parkinson's medications (levodopa-equivalent daily dose) after receiving the study drug.[20]

NouvNeu001

iRegene's NouvNeu001 is a chemically induced human dopaminergic neuron precursor from induced pluripotent stem cells (iPSC). Upon transplantation, it integrates with the body's existing neurons and enhances cellular secretion functions, thereby strengthening the transplanted cells' capacity to repair the original lesion. This process enables it to deliver comprehensive therapeutic benefits.[21] NouvNeu001 is currently being evaluated in a Phase I/II trial (NCT06167681) for mid to late-stage Parkinson's disease with an estimated study completion date of December 2025.

LY3962681

Lilly's LY3962681 is a novel siRNA (small interfering RNA) therapy being developed for Parkinson's disease. This siRNA therapy is designed to target alpha-synuclein mRNA, aiming to reduce alpha-synuclein levels. The PROSPECT Phase I trial (NCT06565195) is currently ongoing, evaluating LY3962681 in both healthy volunteers and patients with Parkinson's disease.[22]

SLS-004

Seelos' SLS-004 is a gene therapy program aimed at regulating the SNCA Gene, which encodes alpha-synuclein. The system is based on CRISPR-dCas9 combined with the catalytic domain of DNA methyltransferase 3A (DNMT3A), an enzyme responsible for DNA methylation. This method involves hypermethylation of the SNCA genetic code, resulting in a controlled and regulated reduction in alpha-synuclein production. SLS-004 is currently under preclinical evaluation. In vivo data demonstrated that a single dose of SLS-004 resulted in a 27% decrease in alpha-synuclein mRNA levels and a 40% reduction in alpha-synuclein protein expression in a rodent model utilizing CRISPR-dCas9 gene therapy technology. Also, a single dose of SLS-004 significantly enhanced the recovery of tyrosine hydroxylase-positive dopaminergic neurons, which typically degenerate in patients with Parkinson's disease.[23]

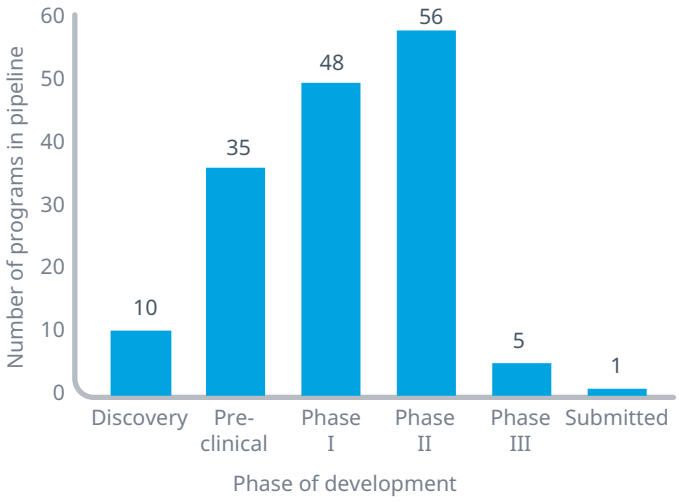
BIIB122

Biogen and Denali's BIIB122 (also known as DNL151) is a selective and brain-penetrant small molecular inhibitor of LRRK2 (leucine-rich repeat kinase 2), the most common genetic risk factor for Parkinson's disease. Elevated LRRK2 levels lead to lysosomal dysfunction, contributing to neurodegeneration and Lewy body formation, which is a key pathological feature of Parkinson's disease. BIIB122 is currently under Phase II evaluation for Parkinson's disease. Two Phase II clinical trials, LUMA and BEACON (NCT05348785 and NCT06602193) are currently investigating BIIB122 with primary completion date of December 2025 and April 2026, respectively.[24]

Parkinson's disease pipeline insights

As of April 2025, IQVIA's Pipeline Link listed 155 programs in development for Parkinson's disease, spanning various phases, with the majority in Phase II (illustrated in Figure 1).

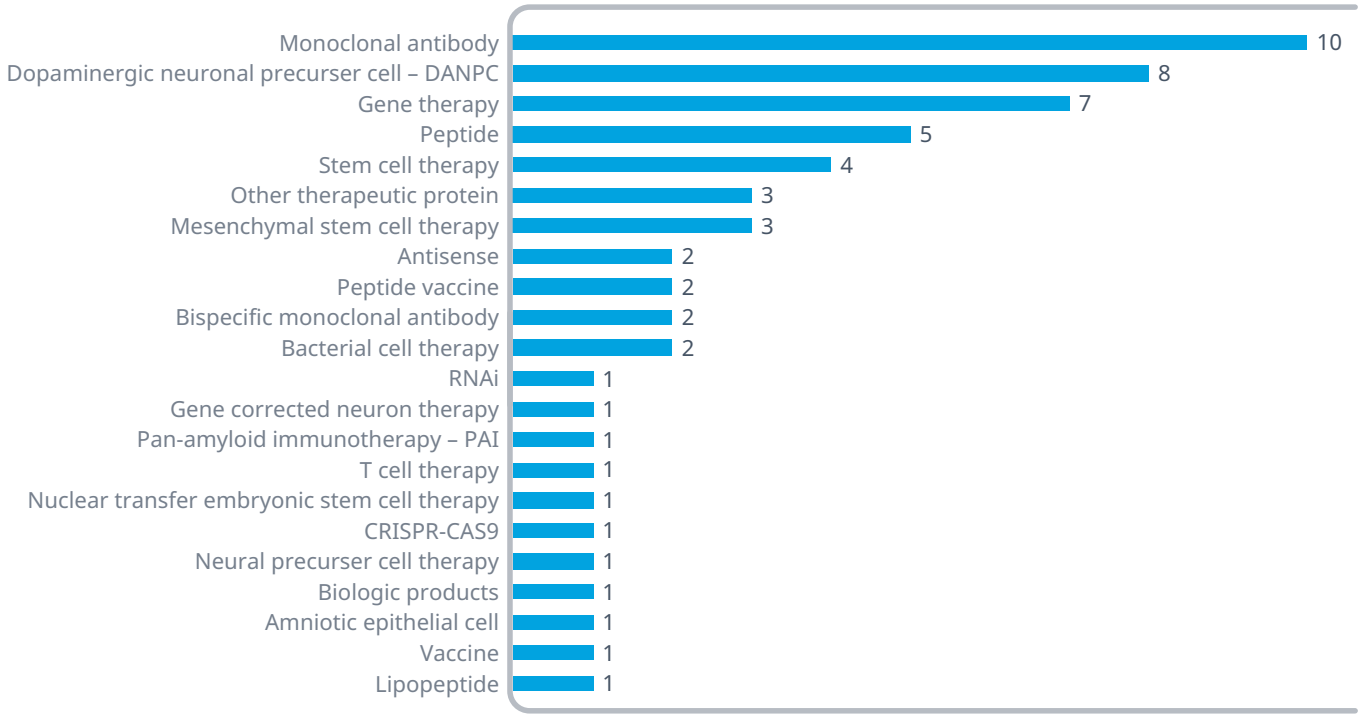
Figure 1: Phase-wise distribution of Parkinson's disease pipeline assets



Source: IQVIA Pipeline Link/Trial Link.

The most advanced candidate in the pipeline is Mitsubishi Tanabe Pharma's ND0612, which is in submitted phase. The new drug application (NDA) for ND0612 was submitted to the US FDA in 2023, and the company announced receiving a complete response letter (CRL) in June 2024. While the FDA found no concerns regarding the efficacy of ND0612, the CRL highlighted additional safety information on the carbidopa component of ND0612, along with further information on product quality, device specifications, and manufacturing site inspections. The company aims to resubmit the NDA by mid-2025.[25] On 20 February 2025 the European Medicines Agency accepted for review the marketing authorization application for ND0612 for motor fluctuations in Parkinson's disease.[26] ND0612 is a drug-device combination therapy that provides a continuous 24-hour subcutaneous infusion of liquid levodopa/carbidopa (LD/CD) to treat motor fluctuations in individuals with Parkinson's disease. ND0612 is designed to minimize motor fluctuations in patients with Parkinson's disease by enhancing the pharmacokinetics of the drugs and maintaining stable, continuous therapeutic levodopa plasma concentrations through a continuous subcutaneous infusion of liquid LD/CD compared to oral LD/CD treatments. The program is predicted to launch in Q3 2025, according to IQVIA Pipeline Link.

Figure 2: Various biologic programs in development for Parkinson's disease



While all submitted and Phase III candidates are small molecules, there are 59 biologic products in development, spanning from discovery to Phase II. These include cell therapies, RNA interference (RNAi), monoclonal antibodies, gene therapies, vaccines, and more (illustrated in Figure 2).

Summary

The pipeline and clinical trial landscape for Parkinson's disease is highly dynamic, with numerous ongoing trials focused on enhancing patient outcomes.

However, currently approved therapies for Parkinson's disease primarily address symptom control, particularly motor dysfunction, but do not impact the underlying neurodegenerative process. Increasing research efforts are now focused on disease-modifying strategies, including gene therapies to enhance dopamine production, monoclonal antibodies targeting alpha-synuclein aggregation, and biologics aimed at preserving neuronal function.

These emerging approaches offer hope for slowing or halting disease progression.

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