Continued Evaluation of Clinical Outcomes in Participants With Parkinson's Disease Through 4 Years After Bemdaneprocel Administration

exPDite

Continued Evaluation Study

Scan the QR code to access supplementary content

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Introduction

Bemdaneprocel is an investigational, allogeneic pluripotent stem cell-derived dopaminergic neuron progenitor cell replacement therapy surgically administered to the posterior putamen in people with PD with fluctuating motor symptoms inadequately controlled by standard medications[1]

exPDite was a first-in-human Phase 1 multicenter, open-label trial (NCT04802733) of bemdaneprocel that met its primary objective of safety and tolerability through 12 months post-transplantation[1]

- No TEAEs related to the cell product were reported
- Secondary and exploratory outcomes demonstrated clinical improvement or stability, including improvement in MDS-UPDRS Part III OFF scores through 18 months post-transplantation
  - The high-dose group showed a greater amplitude of change in MDS-UPDRS motor scores and reduction in OFF Times, and an earlier onset of improvement

Following trial completion, all participants enrolled in a Continued Evaluation Study (NCT05897957) for assessment of safety and efficacy through 5 years post-transplantation[2]

Objectives

To report safety and clinical outcomes of the Continued Evaluation Study participants with PD through 4 years after bemdaneprocel administration[2]

Objectives of the Continued Evaluation Study

- **Primary endpoint:** safety and tolerability in participants with PD up to 5 years after receiving bemdaneprocel in exPDite
- **Secondary and exploratory endpoints:** motor and non-motor outcomes, safety, quality of life, and cell survival

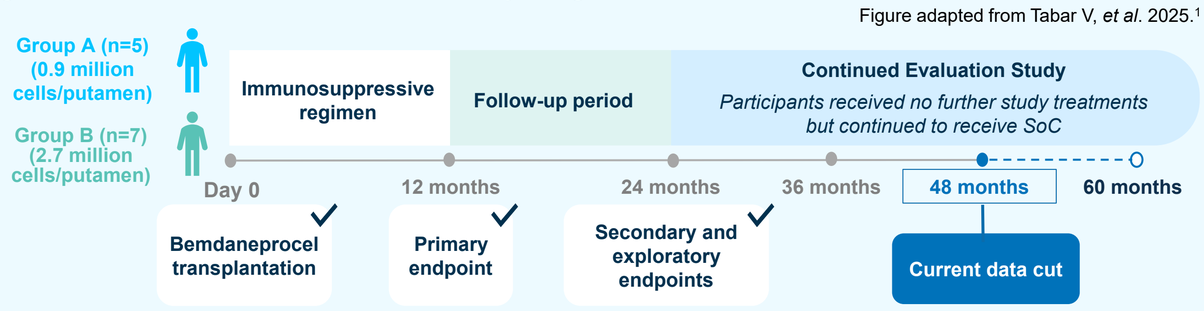
Methods

Participants

- In exPDite, 12 participants with PD enrolled and completed the trial across centers in the USA and Canada to receive either low- or high-dose bemdaneprocel, sequentially; all 12 participants subsequently enrolled in a Continued Evaluation Study **(Figure 1)**[1]
  - **Scan the QR code for participant eligibility criteria**

Figure 1. exPDite and Continued Evaluation Study Design

Figure adapted from Tabar V, *et al*. 2025.[1]



exPDite Surgical Procedure and Immunosuppression[1]

- Bemdaneprocel was administered stereotactically into the posterior putamen bilaterally through a single burr hole on each side in a single session
  - A cannula was used to deliver a total of nine cell deposits along three tracks in each putamen
- Immunosuppression was initiated during transplantation and continued for 1 year post-transplantation
- **Scan the QR code for a diagram of the procedure and the immunosuppression regimen**

Results

Participant Demographics and Baseline Characteristics

- Demographics and baseline characteristics were balanced between the low- and high-dose groups
- All participants had a Hoehn and Yahr score of 2 in the ON state
  - **Scan the QR code for a table of participant demographics and baseline characteristics**

Safety and Tolerability

- Most TEAEs were mild or moderate through 4 years post-transplantation **(Table 1)**
  - TEAEs in the 'Nervous system disorders' and 'Infections and infestations' system organ classes were each reported in 9/12 (75.0%) participants
- No deaths, study discontinuations due to TEAEs, or graft-induced dyskinesias occurred
- No TESAEs were related to study drug delivery device, cell product, or immunosuppression
- No mass lesions or tumors within the brain were observed on MRI

Clinical Outcomes

- Trends toward improvement in clinician- and patient-reported measures of motor function were observed, primarily in the high-dose group, through 4 years post-transplantation **(Figure 2)**
- Motor experiences of daily living scores (mean [SD] CFB at 4 years post-transplantation) were stable in the low-dose (4.0 [6.2]) group and improved in the high-dose group (-2.3 [2.9])

Antiparkinsonian Medication Use

- Mean (SD) CFB in LEDD at 4 years post-transplantation was 74.0 mg (370.3) and 331.0 mg (476.1) in the low- and high-dose group, respectively

Regional Dopaminergic Activity

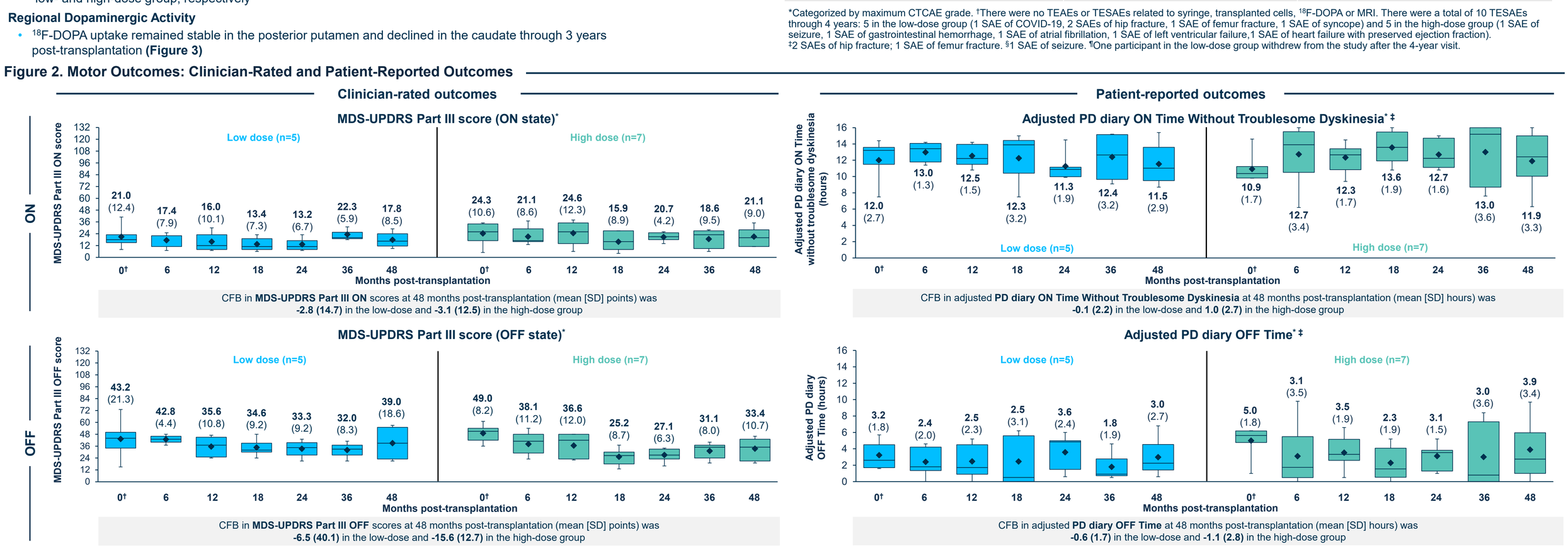
- ^{18}F-DOPA uptake remained stable in the posterior putamen and declined in the caudate through 3 years post-transplantation **(Figure 3)**

Table 1. Safety and Tolerability

Participants Reporting, n (%), [Number of Events]	Low Dose (n=5)	High Dose (n=7)
≥1 TEAEs*		
Mild	1 (20.0) [34]	2 (28.6) [48]
Moderate	2 (40.0) [23]	3 (42.9) [14]
Severe	2 (40.0) [9]	2 (28.6) [4]
TEAEs by assessed relationship†		
Related to cannula	1 (20.0) [1]	0
Related to surgery	0	3 (42.9) [3]
Related to immunosuppressive drugs	3 (60.0) [16]	5 (71.4) [12]
Related to disease	3 (60.0) [22]	6 (85.7) [10]
≥1 TESAEs†		
Related to surgery	0	1 (14.3) [1]§
Related to disease	2 (40.0) [3]‡	0
Deaths	0	0
Study discontinuations due to TEAEs	0¶	0

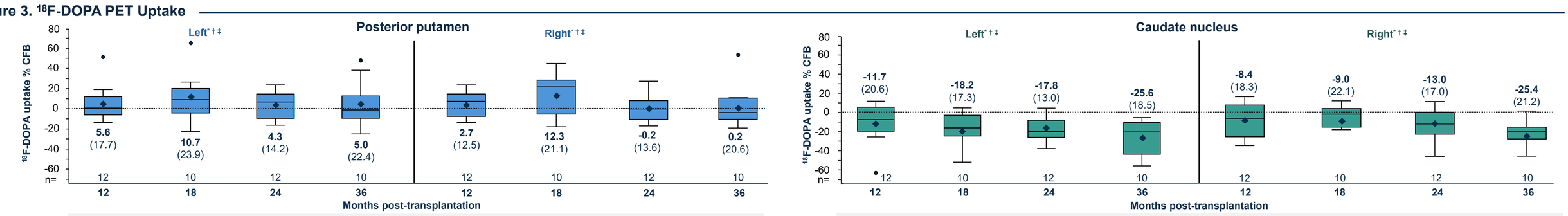
*Categorized by maximum CTCAE grade. †There were no TEAEs or TESAEs related to syringe, transplanted cells, ^{18}F-DOPA or MRI. There were a total of 10 TESAEs through 4 years: 5 in the low-dose group (1 SAE of COVID-19, 2 SAEs of hip fracture, 1 SAE of femur fracture, 1 SAE of syncope) and 5 in the high-dose group (1 SAE of seizure, 1 SAE of gastrointestinal hemorrhage, 1 SAE of atrial fibrillation, 1 SAE of left ventricular failure,1 SAE of heart failure with preserved ejection fraction). ‡2 SAEs of hip fracture; 1 SAE of femur fracture. §1 SAE of seizure. ¶One participant in the low-dose group withdrew from the study after the 4-year visit.

Figure 2. Motor Outcomes: Clinician-Rated and Patient-Reported Outcomes



*Horizontal lines, median; diamonds, mean; boxes, quartiles 1 and 3; whiskers, extreme values; annotations on graphs are mean (SD). In each analysis, a minority of time points were based on n−1 participants. †The baseline value is defined as the last recorded value prior to the first dose of immunosuppressive drugs or cell transplant surgery. ‡PD diary time (hours) is adjusted for a 16-hour waking day.

Figure 3. ^{18}F-DOPA PET Uptake



4-year data will be presented at a future congress. *Horizontal lines, median; diamonds, mean; boxes, quartiles 1 and 3; whiskers, extreme values; dots, outliers; annotations on graphs are mean (SD). †Includes participants in both the high- and low-dose groups. ‡The baseline value is defined as the last recorded value prior to the first dose of immunosuppressive drugs or cell transplant surgery.

Conclusions

- **Bemdaneprocel continued to demonstrate a favorable safety profile through 4 years; there were no deaths or discontinuations due to TEAEs**
  - There were no TEAEs related to bemdaneprocel or of graft-induced dyskinesia, and no evidence of mass lesions or tumors within the brain
- **Motor outcomes demonstrated sustained favorable effects compared with baseline through 4 years**
  - MDS-UPDRS Part III OFF scores were stable and continued to show trends of improvement from baseline; in some patient populations with PD, Part III OFF scores may increase by ~12–18 points over 4 years, depending on disease duration and treatment status[3–5]
  - ON Time Without Troublesome Dyskinesia was stable in the low dose group, and increased from baseline in the high-dose group
  - MDS-UPDRS Part II continued to show a reduced score from baseline in the high-dose group
- **^{18}F-DOPA PET findings supported durable dopaminergic signal preservation in the posterior putamen, while caudate uptake continued to decline through 3 years**
- **These data should be interpreted carefully given the small study population and open-label design**
- **The efficacy and safety of bemdaneprocel are being evaluated further in exPDite-2 (NCT06944522), a Phase 3, randomized, surgery-controlled, double-blind trial in approximately 102 participants with PD aged 45–75 years with motor symptoms inadequately controlled by standard treatments[6]**

Bemdaneprocel is investigational and has not been approved for treatment by any health authority.

Abbreviations: CFB, change from baseline; COVID-19, Coronavirus disease-2019; CTCAE, Common Terminology Criteria for Adverse Events; ^{18}F-DOPA, ^{18}F-DOPA, 6-[^{18}F]fluoro-L-3,4-dihydroxyphenylalanine; LEDD, levodopa-equivalent daily dose; MDS-UPDRS, Movement Disorder Society–Unified Parkinson's Disease Rating Scale; MRI, magnetic resonance imaging; PD, Parkinson's disease; PET, positron emission tomography; SAE, serious adverse event; SD, standard deviation; SoC, standard of care; TEAE, treatment-emergent adverse event; TESAE, treatment-emergent serious adverse event.

References: 1. Tabar V, *et al. Nature*. 2025;641(8064):978–983; 2. clinicaltrials.gov. NCT05897957. Updated March 2024. Available at: clinicaltrials.gov/study/NCT05897957 (Accessed: October 2026); 3. Simuni T, *et al. Mov Disord*. 2018;33(5):771–782; 4. Kempster PA. *BMJ Neurol Open*. 2025;7(2):e001215; 5. Holden SK, *et al. Mov Disord Clin Pract*. 2018;5(1):47–53; 6. clinicaltrials.gov. NCT06944522. Updated August 2026. Available at: clinicaltrials.gov/study/NCT06944522 (Accessed: October 2026).

Disclosures: A Fasano: has stock ownership in InBrain Pharma and has received payments as consultant and/or speaker from Abbott, AbbVie, Boston Scientific, CereGate, Dompé Farmaceutici, InBrain Neuroelectronics, Iota, Ipsen, Medtronic, Merz, Paladin Labs, Sunovion, Syneos Health, and UCB; has received research support from AbbVie, BlueRock Therapeutics, Boston Scientific, ES, Medtronic, and Praxis; and receives royalties from Springer. **C Henchcliffe**: has received honoraria for consulting from AskBio and Certara; stock options for scientific advisory board participation from Axent Biosciences; honoraria for scientific advisory board participation from AbbVie, Bayer AG, Canary Health Technologies, and ProJenX; honoraria for DSMB meetings from MeiraGTx; research support from BlueRock Therapeutics and Weston Brain Institute; and honoraria for presentations from the American Academy of Neurology, ASGCT, the Parkinson Study Group, Cleveland Clinic Foundation, Stanford, and UCLA. **H Sarva:** has received clinical trial support from Bial, Biogen, BlueRock Therapeutics, Covance, Genentech, Insightec, National Institutes of Health (NIH), NeuroDerm, Novo Nordisk, Bukwang, UCB, Cerevance, MeiraGTx, and Prevail Therapeutics; and has received honoraria for consulting from NeuroDerm and Novo Nordisk. **A Lozano:** has nothing to disclose. **S Kalia:** has received consultant and/or speaker honoraria from Abbott, Boston Scientific, Medtronic, and Novo Nordisk. **KKH Yu:** has nothing to disclose. **C Brennan:** has nothing to disclose. **M Louie-Gao** and **G Belfort:** are employees of BlueRock Therapeutics. **V Tabar:** is a scientific advisor for BlueRock Therapeutics.

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BlueRock THERAPEUTICS

Presented at the 31st World Congress on Parkinson's Disease and Related Disorders; November 11–14, 2026; Milan, Italy.

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Supplementary Methods

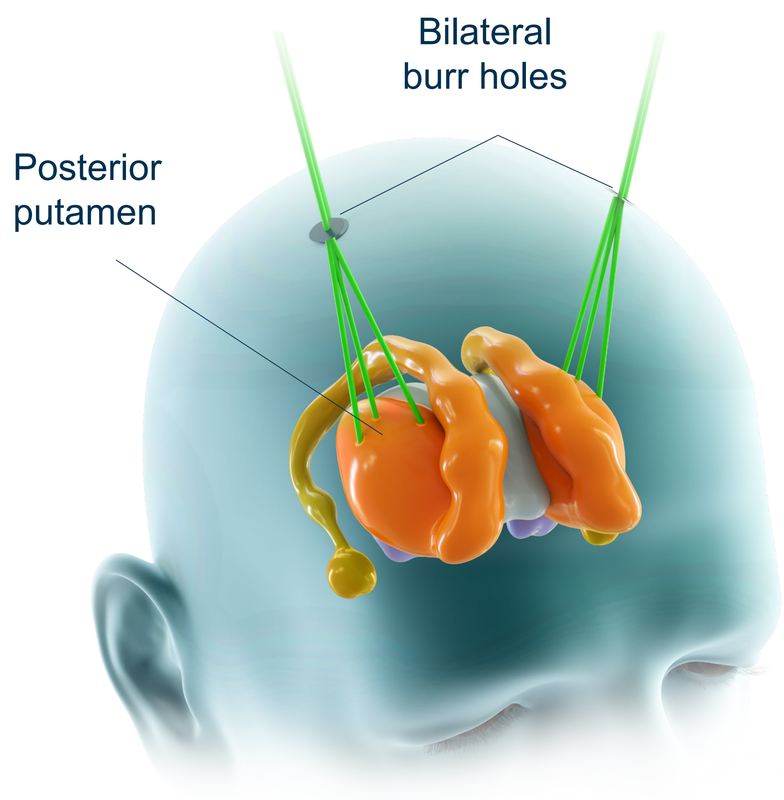
Key Eligibility Criteria for Participants in the exPDite Study[1,2]

- Idiopathic PD for ≥5 to ≤15 years, based on modified UK Parkinson's Disease Society Brain Bank criteria
- Levodopa responsive, defined as ≥30% improvement in MDS-UPDRS Part III
- Aged 50 to 78 years at time of enrollment
- Hoehn and Yahr score of 0−3 in ON state (Canada) or 0−2 in ON state and 3−4 in OFF state (USA)

exPDite Surgical Procedure and Immunosuppression[1]

- Bemdaneprocel was administered stereotactically into the posterior putamen bilaterally through a single burr hole on each side in a single session **(Figure 1)**
  - A cannula was used to deliver a total of nine cell deposits along three tracks in each putamen
- The 1-year immunosuppression regimen consisted of:
  - Basiliximab (20 mg, IV), administered intraoperatively and on postoperative Day 4
  - Methylprednisolone (500 mg, IV), initiated on Day 0, tapered to prednisone 5 mg (PO) and continued at 5 mg QD for 1 year
  - Tacrolimus (PO, BID), initiated on postoperative Day 1 and adjusted to a target trough blood level of 4−7 ng/mL for 1 year

Figure 1. Surgical Procedure



Supplementary Results

Table 1. Participant Demographics and Baseline Characteristics

Parameter	Low Dose (n=5)	High Dose (n=7)
Age, years		
Mean (SD)	66.8 (6.8)	66.7 (4.8)
Sex, n (%)		
Male	4 (80.0)	5 (71.4)
Female	1 (20.0)	2 (28.6)
Race, n (%)		
Asian	0	1 (14.3)
White	3 (60.0)	5 (71.4)
Other	1 (20.0)	1 (14.3)
Multiple	1 (20.0)	0
Time since PD diagnosis, years		
Mean (SD)	11.0 (3.7)	7.8 (2.2)
Hoehn and Yahr score in the ON state, n (%)		
2	5 (100.0)	7 (100.0)
3	0	0
Hoehn and Yahr score in the OFF state, n (%)		
2	1 (20.0)	0
3	4 (80.0)	7 (100.0)

Bemdaneprocel is investigational and has not been approved for treatment by any health authority.

Abbreviations: BID, twice daily; CFB, change from baseline; IV, intravenous; MDS-UPDRS, Movement Disorder Society–Unified Parkinson's Disease Rating Scale; PD, Parkinson's disease; PO, orally; QD, once daily; SD, standard deviation.

References: 1. Tabar V, *et al. Nature.* 2025;641(8064):978–983; 2. clinicaltrials.gov. NCT05897957. Updated March 2024. Available at: clinicaltrials.gov/study/NCT05897957 (Accessed: October 2026).

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