exPDite-2: A Phase 3, Randomized, Sham-Controlled, Double-Blind Trial to Evaluate Bemdaneprocel, a Midbrain Dopaminergic Neuron Progenitor Cell Therapy for Parkinson's Disease

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Introduction

- Bemdaneprocel is an investigational cell therapy composed of human embryonic stem cell-derived midbrain dopaminergic neuron progenitor cells under development for the treatment of Parkinson's disease (PD)
  - These cells, surgically delivered to the posterior putamen, are designed to replace degenerated striatal dopaminergic input and restore function
  - Preclinical studies in rodents support safety and proof-of-concept[1–3]
- The Phase 1 exPDite trial (NCT04802733) was an open-label, multicenter, multisite study evaluating two doses of bemdaneprocel that achieved its predefined primary objective of safety and tolerability 12 months post transplantation[4]
  - All participants subsequently enrolled in a continued evaluation study (NCT05897957) for assessment through 60 months; trends toward improvement or stability based on clinical evaluation, and a favorable safety profile, were demonstrated through 36 months post transplantation[5]

Objectives

- To describe the design of the Phase 3 exPDite-2 trial (NCT06944522), a multicenter, multisite, randomized, sham surgery-controlled, double-blind trial to evaluate the efficacy and safety of bemdaneprocel in people with PD

Methods

Bemdaneprocel Administration

- Bemdaneprocel will be administered stereotactically into the posterior putamen bilaterally through a single burr hole on each side in a single session **(Figure 1)**
  - A cannula will be used to deliver a total of **9** cell deposits along **3** tracks per hemisphere

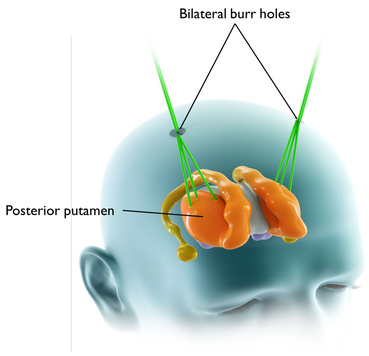
Sham Surgery

- A partial burr hole that does not penetrate the inner table of the skull will be created over each hemisphere

Immunosuppression and Prophylaxis

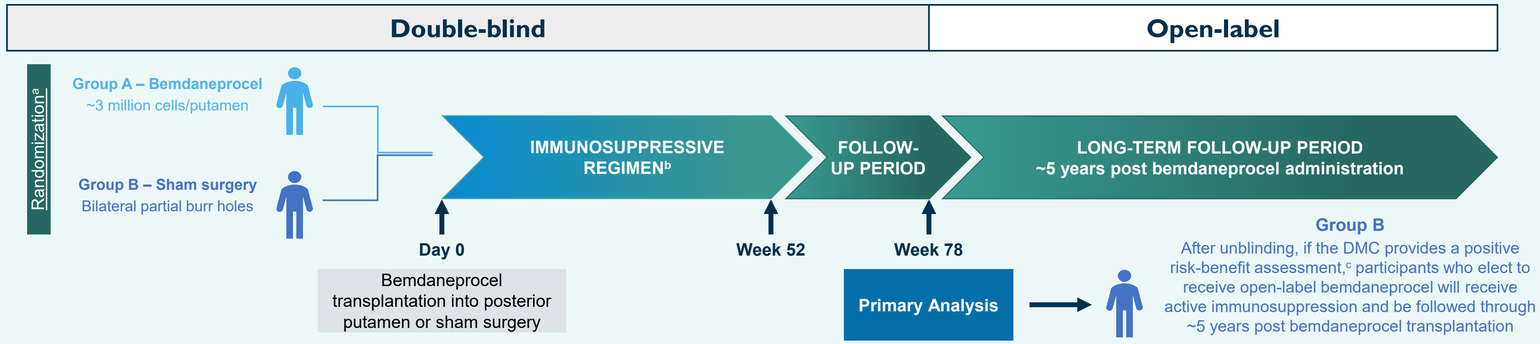
- **Basiliximab** (20 mg, intravenous [IV]) intraoperatively and on postoperative day 4
- **Methylprednisolone** (500 mg, IV) perioperatively followed by **prednisone taper** to 5 mg, oral administration (PO) once daily (QD) and continued for 1 year
- **Tacrolimus** (PO) initiated on postoperative day 1 and adjusted to a target trough blood level of 4 to 7 ng/mL twice daily (BID) for 1 year
- **Lansoprazole** (30 mg, PO) initiated on postoperative day 1 and continued QD for 1 year
- **Sulfamethoxazole/trimethoprim** (400/80 mg, PO) initiated on postoperative day 1 and continued QD for 1 year
  - **Sham group** will receive the same perioperative immunosuppressive regimen of corticosteroids with taper as well as prophylactic lansoprazole. After sham surgery, participants will take placebo versions of the other immunosuppressive agents and prophylactic medications through 1 year

Figure 1. Treatment and Surgical Procedure



Methods

Figure 2. Study Design



- exPDite-2 will enroll participants (US, Canada, and Australia) with PD aged 45 to 75 years with motor symptoms inadequately controlled by standard treatments **(Figure 2)**
  - Eligible participants will have a diagnosis of idiopathic PD[d] for ≥4 to <12 years, a clear and robust response to dopaminergic therapy,[e] ≥2.5 hours of daily OFF time,[f] and a Hoehn and Yahr score of 2 to 3 in the OFF state

DMC, data monitoring committee; PD, Parkinson's disease. MDS-UPDRS, Movement Disorder Society-Unified Parkinson's Disease Rating Scale.
[a]Randomization will be stratified by age at informed consent (aged ≤60 and >60 years). [b]Group B will receive perioperative immunosuppression followed by a placebo regimen. [c]After the last actively enrolled participant completes the Week 78 visit. Participants and blinded personnel at the clinical site will be unblinded when the decision to move forward with the transition of participants in Group B to active treatment in the open-label period is made. [d]Based on the Movement Disorder Society Clinical Diagnostic Criteria. [e]Defined as ≥30% improvement on MDS-UPDRS Part III. [f]Daily OFF time was determined by the average of PD diaries collected over 3 consecutive days, adjusted for a 16-hour waking day and confirmed by another set of diaries performed over 3 consecutive days ≥14 days later.

Table 1. Assessments

Objectives	Endpoints	
Primary		
To evaluate the efficacy of bemdaneprocel on motor symptoms in participants with PD compared with participants who undergo sham surgery	Change from baseline to Week 78 in PD diary measure of ON time without troublesome dyskinesia[a-c]	
Secondary		
To evaluate the effects of bemdaneprocel on motor function, quality of life, non-motor symptoms, disease severity, and use of PD medications or therapies	*Key Secondary:* Change from baseline to Week 78 • PD diary OFF time[a,c] • MDS-UPDRS Part III OFF score • MDS-UPDRS Part II score • PDQ-39 summary index	*Other Secondary:* Change from baseline over time • PD diary measures[a,c] • MDS-UPDRS scores • PDQ-39 summary index • PDSS-2 score • MDS-NMS scores • CGI-S score • LEDD
Safety		
To evaluate the safety of bemdaneprocel	Incidence and severity of TEAEs • AEs related to cellular drug product • AEs related to surgical procedure • AEs related to immunosuppression regimen	Change from baseline over time • UDysRS score • C-SSRS score

Power calculation and target enrollment

A total sample size of **N=102** randomized participants will provide **90% power** (2-sided α=0.05) to detect a difference of **1.85 hours** between bemdaneprocel and sham surgery on the primary endpoint of ON time without troublesome dyskinesia

AE, adverse event; CGI-S, Clinical Global Impression-Severity; C-SSRS, Columbia-Suicide Severity Rating Scale; LEDD, levodopa equivalent daily dose; MDS, International Parkinson and Movement Disorder Society; MDS-NMS, International Parkinson and Movement Disorder Society-Non-Motor Rating Scale; MDS-UPDRS, International Parkinson and Movement Disorder Society-Unified Parkinson's Disease Rating Scale; PD, Parkinson's disease; PDQ-39, 39-Item Parkinson's Disease Questionnaire; PDSS-2, Parkinson's Disease Sleep Scale, Version 2; TEAE, treatment-emergent adverse event; UDysRS, Unified Dyskinesia Rating Scale.
[a]The PD diary will be given to participants to be completed at home prior to the scheduled study visit. Participants are to complete the diary over the course of 3 consecutive days in the 1 week prior to the scheduled study visit. [b]ON time without troublesome dyskinesia is the sum of ON time without dyskinesia and ON time with non-troublesome dyskinesia. [c]Adjusted for a 16-hour waking day.

Conclusions

- Bemdaneprocel demonstrated a favorable safety profile and maintained improvements or stability across clinical assessments through 3 years, supporting the ongoing development of bemdaneprocel for the potential treatment of PD[5]
- The exPDite-2 trial is a Phase 3, randomized, sham surgery-controlled, double-blind trial that will evaluate the efficacy and safety of bemdaneprocel on motor symptoms and safety in participants with Parkinson's disease
- exPDite-2 will be the first large-scale, potentially registrational trial of stem cell-derived midbrain dopaminergic neuron progenitors in people with Parkinson's disease
- The first participant was randomized and underwent surgery in August 2025

References: **1**. Kim TW, et al. *Cell Stem Cell*. 2021;28(2):343-55.e5. **2**. Piao J, et al. *Cell Stem Cell*. 2021;28(2):217-29.e7. **3**. Kriks S, et al. *Nature*. 2011;480(7378):547-551. **4**. Henchcliffe C, et al. Presented at: International Congress of Parkinson's Disease and Movement Disorders; August 27-31, 2023; Copenhagen, Denmark. **5**. Sarva H, et al. Presented at: Annual Meeting of the International Congress of Parkinson's Disease and Movement Disorders; October 5-9, 2025; Honolulu, HI. **Disclosures:** **N Abid**, **M Adera**, **M Louie-Gao**, and **G Belfort** are full-time employees of BlueRock Therapeutics, LP.

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

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