Center for Biologics Evaluation and Research (CBER)
75th Meeting of the Cellular, Tissue, and Gene Therapies
Advisory Committee (CTGTAC)
September 27, 2023
FINAL AGENDA

Meeting Topic: The committee will meet in open session to discuss and make recommendations on biologics license application (BLA) 125782 from BrainStorm Cell Therapeutics, Inc. for debamestrocel (autologous bone marrow-derived mesenchymal stromal cells induced to secrete neurotrophic factors [MSC-NTF], NurOwn). The applicant has requested an indication for the treatment of mild to moderate amyotrophic lateral sclerosis (ALS).

EDT	Presentation/Presenter			
10:00 a.m.	Opening Remarks: Call to Order and Welcome (5 min.)			
	Taby Ahsan, Ph.D. Acting Chair, CTGTAC Vice President, Cell Therapy Operations City of Hope Duarte, CA			
	Administrative Announcements, Roll Call, Introduction of Committee			
	Conflict of Interest Statement (20 min.)			
	Marie DeGregorio Designated Federal Officer Division of Scientific Advisors and Consultants Center for Biologics Evaluation and Research (CBER), FDA			
10:25 a.m.	FDA Introduction (25 min. including Q&A)			
	Opening Remarks (5 min.)			
	Celia Witten, Ph.D., M.D.			
	Deputy Director, CBER, FDA			
	FDA Overview of BLA 125782, debamestrocel (MSC-NTF) for Treatment of Amyotrophic Lateral Sclerosis (ALS) (15 min.)			
	Rosa Sherafat-Kazemzadeh, M.D. Clinical Team Leader			
	Office of Clinical Evaluation (OCE), Division of Clinical Evaluation			
	General Medicine (DCEGM) Office of Therapeutic Products (OTP)/CBER/FDA			
	Q&A (5 min.)			

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10:50 a.m.	Guest Speaker Presentation on CMC (40 min. including Q&A)		
	Some Guiding Principles When Contemplating Stem Cell-Based Approaches to Neurological Disease		
	Evan Snyder M.D., Ph.D. Director Center for Stem Cells and Regenerative Medicine Sanford Children's Health Research Center		
	Q&A (10 min.)		
11:30 a.m.	BREAK (10 min.)		
11:40 a.m.	Applicant Presentation (85 min. including Q&A)		
	Introduction	Stacy Lindborg, Ph.D. Co-Chief Executive Officer BrainStorm Cell Therapeutics	
	Introduction: ALS Landscape and Unmet Need	Anthony J. Windebank, M.D. Judith and Jean Pape Adams Professor and Faculty Development Chair Department of Neurology, Mayo Clinic	
	Efficacy		
	Phase 3 Results	Stacy Lindborg, Ph.D. Co-Chief Executive Officer BrainStorm Cell Therapeutics	
	Totality of Evidence in Prespecified ALSFRS-R ≥ 35 Subgroup	Lee-Jen Wei, Ph.D. Professor of Biostatistics Harvard University	
	Supportive Clinical Evidence	Nathan Staff, M.D., Ph.D. Professor of Neurology Research Chair Department of Neurology, Mayo Clinic	

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	Supportive Biomarker Evidence	Robert Bowser, Ph.D. Chief Scientific Officer, Chair, Department of Translational Neuroscience Barrow Neurological Institute		
	Safety	Kirk Taylor, M.D. Executive Vice President, Chief Medical Officer BrainStorm Cell Therapeutics		
	Benefit/Risk – Clinical Perspective	Anthony J. Windebank, M.D. Judith and Jean Pape Adams Professor and Faculty Development Chair Department of Neurology, Mayo Clinic		
	Q&A (15 min.)			
1:05 p.m.	LUNCH (35 min.)			
1:40 p.m.	Open Public Hearing (60 min.)			
2:40 p.m.	BREAK (10 min.)			
2:50 p.m.	FDA Presentation (85 min. including Q&A)			
	BLA 125782, Application for debamestrocel (MSC-NTF) for Treatmen of Amyotrophic Lateral Sclerosis (ALS)			
	Tom Finn, Ph.D. BLA Chair and Chemistry, Manufacturing, and Controls (CMC) Reviewer Office of Cellular Therapy and Human Tissue CMC, Division of Cell Therapy OTP/CBER/FDA			
	Gumei Liu, M.D., Ph.D. Clinical Reviewer OCE, DCEGM OTP/CBER/FDA			

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	Xue (Mary) Lin, Ph.D. Statistical Reviewer Office of Biostatistics and Pharmacovigilance, Division of Biostatistics CBER/FDA
	Xiaofei Wang, Ph.D. Clinical Pharmacology Reviewer OCE, DCEGM OTP/CBER/FDA
	Q&A (15 min.)
4:15 p.m.	Committee Discussion, Voting, and Vote Explanation (100 min.)
5:55 p.m.	Closing Remarks (5 min.) • Celia Witten, Ph.D., M.D. Deputy Director, CBER, FDA
6:00 p.m.	ADJOURNMENT Marie DeGregorio Designated Federal Officer, CTGTAC