



Waiver to Allow Participation in a Food and Drug Administration Advisory Committee

DATE: August 31, 2023

TO: Russell Fortney
Director, Advisory Committee Oversight and Management Staff
Office of the Chief Scientist

FROM: Prabhakara Atreya, Ph.D.
Director
Division of Scientific Advisors and Consultants
Center for Biologics Evaluation and Research

Name of Advisory Committee Meeting Temporary Voting Member: **Jun Li, M.D., Ph.D.**

Committee: Cellular, Tissue, and Gene Therapies Advisory Committee (CTGTAC)

Meeting date: September 27, 2023

Description of the Particular Matter to Which the Waiver Applies:

Dr. Jun Li, a Special Government Employee, has been invited to participate in the September 27, 2023, CTGTAC meeting as a temporary voting member. The committee reviews and evaluates available data relating to the safety, effectiveness, and appropriate use of human cells, human tissues, gene transfer therapies and xenotransplantation products which are intended for transplantation, implantation, infusion and transfer in the prevention and treatment of a broad spectrum of human diseases and in the reconstruction, repair, or replacement of tissues for various conditions. The Committee also considers the quality and relevance of FDA's research program which provides scientific support for the regulation of these products and makes appropriate recommendations to the Commissioner of Food and Drugs.

On September 27, 2023, the committee will 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). The applicant has requested an indication for the treatment of amyotrophic lateral sclerosis (ALS). The meeting topic is a particular matter involving specific parties.

Type, Nature, and Magnitude of the Financial Interest(s):

Dr. Li is Chairman of the Department of Neurology at Houston Methodist Hospital in Houston,

Texas. Dr. Li has disclosed imputed financial interests of his employer, Houston Methodist Hospital. Specifically, Dr. Li reported that ALS research is being conducted in his department related to the matter before the committee, but he has never been personally involved in this research. Dr. Li disclosed that one of the faculty members in his department, (b) (4) is involved in conducting the ALS research, but his research or the related clinical trial are not funded by either BrainStorm Cell Therapeutics, Inc. or by other affected firms. The research and the related clinical trial being conducted in the department are primarily supported by the non-profit-foundations (b) (4) (b) (4)

Basis for Granting the Waiver:

Dr. Jun Li has unique qualifications and specialized expertise needed for this particular matter.

Dr. Jun Li currently serves as Chairman of the Stanley H. Appel Department of Neurology at the Houston Methodist Neurological Institute, effective June 1, 2022. Additionally, he also serves as a Professor of Neurology at Weill Cornell Medical College. Prior to this appointment, Dr. Li served as the Chairman of the Department of Neurology, the Scientific Director of Translational Neuroscience Initiatives at Wayne State University, and the Specialist-in-Chief of Neurology at the Detroit Medical Center.

Dr. Li received his medical degree from Anhui Medical University in the People's Republic of China in 1985. He received his doctoral degree in Neurosciences in 1995 from the Drexel University College of Medicine (formerly Hahnemann University). He completed a Neurology residency in 1999 at the Ohio State University, and an EMG/Neuromuscular Fellowship in 2000 in the Department of Neurology at the University of Utah. Dr. Li currently serves as an attending Neurologist at Houston Methodist Hospital in Houston, Texas. His research focuses on neurological diseases, neuropathies, and neuromuscular diseases.

ALS, or amyotrophic lateral sclerosis, is a progressively neurodegenerative disease that affects nerve cells in the brain and spinal cord. More and more people are being diagnosed with this disease these days. For about 90% of all cases, there is no known family history of the disease or presence of a genetic mutation linked to ALS.

Dr. Li is uniquely qualified to serve on the committee as a temporary voting member because he is an accomplished medical scientist/researcher with extensive experience in neurology and neuroscience.

There is limited expertise available, and it is difficult to locate similarly qualified individuals without a disqualifying financial interest.

Dr. Li's strong scientific background in neurology, neuromuscular diseases, and neuroscience will be critical to the September 27, 2023, CTGTAC discussions. The FDA division responsible for conducting this meeting has struggled to find a qualified expert in clinical neurology and neurodegenerative diseases, who does not have disqualifying financial interests.

In order to ensure a productive committee discussion, we need experts with not only clinical experience but also extensive research experience in progressive neurodegenerative diseases. Several candidates have been removed from consideration due to either scheduling conflicts or disqualifying conflicts of interest. At this stage, it would be extremely difficult to find a replacement for Dr. Li, given his unique qualifications in neuroscience, neurology, and neuromuscular diseases. Dr. Li's conflicts are also less critical as he is not directly involved in the research related to the product or matter before the committee. His imputed interests are due to the fact that one of the faculty members in his department, (b) (4) is conducting ALS research and a clinical trial related to the particular matter before the committee, with funding not by the applicant before the committee or affected firms, but from non-profit foundations. Excluding Dr. Li from participation in the meeting will have a deleterious impact on the committee's available expertise and deliberations and may potentially affect public confidence in those deliberations.

The particular matter is sensitive.

The meeting topic for this session is considered sensitive and the FDA Review Division with responsibility for these products does expect that the meeting is likely to receive significant public interest and (non-trade) press interest, and the matter is considered highly controversial.

Dr. Li's expertise in this particular matter is necessary in the interest of public health.

As a physician-scientist, Dr. Li subspecializes in peripheral nerve diseases and myelin biology. He has published more than 90 articles in peer-reviewed journals and book chapters. These contributions earned him a Wolfe Research Prize from the American Neurological Association (ANA) in 2014. He is a current member of the ANA Board of Directors and has also served as a member of the Scientific Advisory Board for the Muscular Dystrophy Association, Charcot-Marie-Tooth Association, and the scientific committee of the Peripheral Nerve Society. He has been a member of NIH study sections for more than a decade.

Dr. Jun Li is well known and has been recognized as an accomplished clinician and scientist/researcher with extensive experience in neurology, neuroscience, and neuro-muscular diseases. Given his exceptional scientific and public health background, his participation at this meeting will bring the unique combination of his expertise in multiple fields that are critical to the CTGTAC committee deliberations.

Any potential for a conflict of interest is greatly outweighed by the strong need for Dr. Jun Li's expertise in this matter.

We believe the strong need for Dr. Jun Li's demonstrated expertise and diverse perspective that he can bring to this matter before the committee greatly outweighs any potential for a conflict of interest.

Accordingly, I recommend that you grant Dr. Jun Li, a temporary voting member of the Cellular, Tissue, and Gene Therapies Advisory Committee (CTGTAC), a waiver from the conflict-of-interest prohibitions of 18 U.S.C. § 208 (b)(3).

Certification:

The individual may participate, pursuant to 18 U.S.C. 208(b)(3) – The need for the individual’s services outweighs the potential for a conflict of interest created by the financial interest involved.

Limitations on the Regular Government Employee’s or Special Government Employee’s Ability to Act:

Non-voting

Other (specify):

Denied – The individual may not participate.

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Russell Fortney
Director, Advisory Committee Oversight and Management Staff
Office of the Chief Scientist

September 11, 2023
Date