



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

DATE: June 30, 2021

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 (CBER)

Name of Advisory Committee Member: **Kenneth Berns, M.D., Ph.D.**

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

Meeting date: September 2-3, 2021

Description of the Particular Matter to Which the Waiver Applies:

Dr. Kenneth Berns, M.D., Ph.D., a special government employee, has been invited to participate in the September 2-3, 2021, CTGTAC meeting as a standing 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.

On September 2-3, 2021, the CTGTAC will meet in open session on both days to discuss the evaluation of the toxicity risks of adeno-associated virus (AAV) vector-based gene therapy products. The discussion topics include oncogenicity risks due to vector genome integration and/or safety issues identified during preclinical and/or clinical evaluation. The topic of this meeting is a particular matter of general applicability. The discussions will not focus on approval, or ongoing approval, of any specific product. The particular matter will affect entities that make, or are seeking to make, AAV vector-based gene therapy products.

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

Dr. Berns has reported a financial interest in the Bloomberg Health Care Select SPDR fund, an exchange-traded fund (ETF). The objective of the fund is to provide investment results that correspond to an index of companies involved in health care equipment and supplies, health care providers and services, biotechnology, and pharmaceuticals. The value of his holdings in the fund is between (b) (6). As of the writing of this waiver, the fund contains a holding in (b) (4) which is within the class of entities that will be affected by the particular matter under discussion by the advisory committee. (b) (4) represents (b) (6) of the underlying value of the fund.

Under a regulatory exemption issued by the Office of Government Ethics, 5 C.F.R. § 2640.201(b), an employee may participate in any particular matter affecting one or more holdings in a sector mutual fund where the disqualifying financial interest in the matter arises because of ownership of an interest in the fund and the aggregate market value of interests in all funds in which there is a disqualifying financial interest and which concentrate in the same sector does not exceed \$50,000. Most sector ETFs qualify for this regulatory exemption. However, because Dr. Berns' financial interest in the sector fund exceeds \$50,000, he has a disqualifying financial interest based on the fund's holdings of the above-listed company.

Basis for Granting the Waiver:

Based on Dr. Berns' extensive experience in fields of critical importance to the discussion at the upcoming CTGTAC meeting, the need for his participation outweighs any potential for conflict due to the financial interest described above.

Dr. Kenneth Berns has unique qualifications and specialized expertise needed for this particular matter.

Dr. Kenneth Berns received an A.B. degree in Biology, a Ph.D. in Biochemistry, and an M.D., all from Johns Hopkins University. He is a virologist currently serving as a Distinguished Professor Emeritus in the department of Molecular Genetics and Microbiology at the University of Florida College of Medicine. Dr. Berns has more than 40 years of scientific experience and he is primarily known for his work on AAV with more than 100 publications. For his contributions he was elected as a member of the National Academy of Sciences (since 1995). His research group was one of the first which showed the specificity of the integration of the AAV genomes into the cellular genome, and since then, he has made many seminal contributions towards our understanding of AAV replication. His depth and breadth of experience is highly relevant to one of the session topics in the advisory committee meeting (i.e., AAV vector integration and oncogenicity risk) and his contribution to the committee's discussion will be highly valued. Dr. Berns' expertise in this particular matter is necessary in the interest of public health.

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

Among the CTGTAC members, Dr. Berns is the only member with scientific experience that directly relates to the topic of the committee's discussion. It is difficult to locate similarly qualified individuals without a disqualifying financial interest. This is because AAV vectors for gene therapy are showing great promise in clinics and the technology is being rapidly developed for translation with most academic scientists involved in product development.

The particular matter is not sensitive.

The meeting topic for this session is considered not particularly sensitive; however, the meeting may still receive significant public interest or (non-trade) press interest due to the discussions involving various toxicity risks noted in the non-clinical and clinical studies, and the long-term monitoring of human recipients of AAV gene therapies.

Dr. Berns’ expertise in this particular matter is necessary in the interest of public health.

Dr. Berns is well known and has been recognized as an accomplished scientist/researcher with extensive experience in molecular genetics, microbiology, virology, viral vectors and gene therapies. Given his exceptional scientific and public health background, his participation at this meeting will bring the synthesis of his expertise in multiple fields that are critical to the CTGTAC’s deliberations.

Any potential for a conflict of interest is greatly outweighed by the strong need for Dr. Kenneth Berns’ expertise in this matter.

The strong need for Dr. Berns’ 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. Kenneth Berns, a standing voting member of CTGTAC, a waiver from the conflict of interest prohibitions of 18 U.S.C. § 208(a).

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

August 17, 2021
_____ Date