NIH Clinical Center Grand Rounds, March 23, 2022

NIH Videocast

Gene Therapy Approaches for Multiple Rare Diseases The Pave-GT and BGTC Initiatives

Philip J. Brooks, PhD

Acting Director, Office of Rare Diseases Research
National Center for Advancing Translational Sciences (NCATS) **Donald C. Lo, PhD**

Director, Division of Preclinical Innovation

Therapeutic Development Branch
National Center for Advancing Translational Sciences

Hopkins Cloud CME Code: 36714

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CME activity code is valid 8 hours after the lecture 1 AMA PRA Category credit is offered for this activity

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Have questions?
Rita Stevens
CME Administrator

Email: rita.stevens@nih.gov

NIH Clinical Center Grand Rounds

The NIH Clinical Center Grand Rounds is a Continuing Medical Education (CME) activity offered by the NIH Clinical Center Office of Clinical Research Training and Medical Education (OCRTME) with the Johns Hopkins School of Medicine as the CME provider.

Accreditation Statement

This activity has been planned and implemented in accordance with the accreditation requirements and policies of the Accreditation Council for Continuing Medical Education (ACCME) through the joint providership of Johns Hopkins University School of Medicine and the National Institutes of Health. The Johns Hopkins University School of Medicine is accredited by the ACCME to provide continuing medical education for physicians.

Credit Designation Statement

The Johns Hopkins University School of Medicine designates this Regularly Scheduled Series (RSS) CME Activity for 1 credit per session for a maximum of 42 AMA PRA Category 1 CreditsTM. Physicians should claim only the credit commensurate with the extent of their participation in the activity.

Policy on Speaker and Provider Disclosure

It is the policy of The Johns Hopkins University School of Medicine and the NIH that the speaker and provider disclose real or apparent conflicts of interest relating to the topics of this educational activity, and also disclose discussions of unlabeled/unapproved uses of drugs or devices during their presentation(s). The Johns Hopkins University School of Medicine Office of Continuing Medical Education has established policies in place that will identify and resolve all conflicts of interest prior to this educational activity. Detailed disclosure will be made in the activity materials.

Speakers Name and Lecture Titles

Philip J. Brooks, PhD

Acting Director, Office of Rare Diseases Research

National Center for Advancing Translational Sciences (NCATS)

Donald C. Lo, PhD

Director, Division of Preclinical Innovation

Therapeutic Development Branch

National Center for Advancing Translational Sciences

Relationship(s): NONE

Off-Label Product Discussion

No off label products will be discussed

Evaluation form NIH Clinical Center Grand Rounds

For CME CREDIT TEXT CODE 36714 TO (443) 541-5052

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Please rate the attai	nment of obi	ectives:
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- 1. Define options and alternatives that will guide clinical practice:
 - **1-**Not at all **2-**Very little **3-**Moderately **4-**Considerably **5-**Completely
- 2. Evaluate practical information about clinical research principles based on state-of-theart information about scientific discovery and clinical advances:
 - **1-**Not at all **2-**Very little **3-**Moderately **4-**Considerably **5-**Completely
- 3. Analyze information and opportunities to increase and improve collaboration between investigators:
 - **1-**Not at all **2-**Very little **3-**Moderately **4-**Considerably **5-**Completely

Please rate the overall effectiveness of the lecture(s):

- 4. The overall quality of the presentation(s) was:
 - 1-Poor 2-Fair 3-Good 4- Very Good 5- Outstanding
- 5. How useful do you think the information that was presented is:
 - **1-**Not very useful **2-**Somewhat useful **3-**Moderately useful **4-**Very useful **5-**Extremely useful
- 6. To what extent did participation in this activity enhance your professional effectiveness:
 - **1-**Not at all **2-**Slightly **3-**Moderately **4-**Greatly **5-**Extremely
- 7. Will you change your practice in any way as a result of attending this activity:
 - **1-**Not at all **2-**Slightly **3-**Moderately **4-**Greatly **5-**Extremely
- What comments or suggestions do you have for the presenter(s):
- What suggestions do you have for future topics or speakers for Clinical Center Grand Rounds:
- Do you have any additional comments or suggestions to enhance the effectiveness of Clinical Center

Grand Rounds:

Please return completed evaluation at the CME Coordinator table at the Lipsett entrance or email to rita.stevens@nih.gov. This form is optional and not required to receive CME credit. We greatly appreciate your feedback.