

NIH Clinical Center Grand Rounds, February 27, 2019

**Severe Combined Immune Deficiency in the Newborn Screening Era
Genetic Landscape and Current Challenges**

Luigi Notarangelo, MD
Head, Immune Deficiency Genetics Section and Chief
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Update on Clinical Outcome of Gene Therapy for X-linked Severe Combined Immune Deficiency

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*One Continuing Medical Education (CME) credit will be offered for attending this lecture.

The Activity Code for this lecture is: 16601

In order to receive CME credit for attending this lecture, please text this code to the Johns Hopkins CME phone number (443) 541-5052.

***Important Note: If you have not already done so, please be sure you have set up your profile with Johns Hopkins CME before you text the code. Please see below for detailed instructions.**

*** To receive credit for attending an NIH CME activity, each attendee must:**

1. Register with the Johns Hopkins School of Medicine Office of CME at <https://hopkinscme.cloud-cme.com/aph.aspx>. You will provide your name, email address, and cell phone number. If you have attended a NIH CME activity in the past, and requested CME for your attendance, then your email address should be registered with Hopkins CME. ***Note:** The Hopkins CloudCME website is a third party website which is not managed by the NIH. The Johns Hopkins School of Medicine privacy policies will apply, and they have been reviewed and approved by the NIH Clinical Center Privacy Office. The Johns Hopkins Privacy Policy may be viewed at <http://www.hopkinscme.edu/migration/privacypolicy.html>.
2. Next text your email address to the Hopkins CME phone number, **(443) 541-5052**, which will pair your mobile phone with your Hopkins CME account that had been created/ used on past CME Self-Report Credit Forms.
3. After you have attended, you will text the assigned "activity code" to the Hopkins CME phone number at (443) 541-5052 to receive CME credit. Once the text message is sent, your account is updated to reflect the CME credit earned.

***Important Note:** The activity code is only valid 15 minutes prior to the start of the lecture until 8 hours after the lecture. Afterwards, the code becomes inactive and you will not be able to submit your attendance for CME credit for that activity.

If you have any questions on how to use the new CloudCME record keeping, please contact the Clinical Center's CME Coordinator Rita Stevens, by email at rita.stevens@nih.gov or by phone at 301-435-6188.

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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 Credits™*. 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.

Speaker Names, Lecture Title, Relationship(s)

Severe Combined Immune Deficiency in the Newborn Screening Era: Genetic Landscape and Current Challenges

Luigi Notarangelo, MD

Update on Clinical Outcome of Gene Therapy for X-linked Severe Combined Immune Deficiency

Harry Malech, MD

NONE

RELATIONSHIPS:

No other planners have indicated that they have any financial interests or relationships with a commercial entity.

Off-Label Product Discussion

- Ex vivo lentivector transduced autologous CD34+ hematopoietic stem cells

For CME CREDIT TEXT CODE 16601 TO (443) 541-5052

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

Speaker: Luigi Notarangelo, MD, Harry Malech, MD

Objectives:

1. To describe the genetic and phenotypic heterogeneity of severe combined immune deficiency (SCID)
2. To illustrate how newborn screening has changed the genetic landscape of SCID, and helped improve outcome
3. Define the genetic and immunological abnormalities associated with X-linked Severe Combined Immune deficiency (XSCID), and in particular the clinical problems occurring in older children and young adults who received lymphocyte-depleted haploidentical transplants without conditioning as infants.

Please rate the attainment of objectives:

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-the-art 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:
