Gene Replacement Therapy in Practice: Considerations & Case Studies in Process Release Date: November 20, 2023 End Date November 20, 2024

Activity Overview

This Grand Rounds webinar with Bo Hoon Lee, MD and Erin Collins, Medication Access Manager, of the University of Rochester and Natalie Goedeker, CPNP, of Washington University in St. Louis will discuss practicalities, infrastructural needs, and their firsthand learnings from experience administering Gene Replacement Therapy (GRT).

Target Audience

This activity is designed for an audience of pediatricians, pediatric nurses, nurse practitioners, pharmacists, and physician assistants.

Learning Objectives

Upon completion of the educational activity, participants should be able to:

- Recognize and anticipate unique infrastructural needs for administering gene replacement therapies (GRT) for neuromuscular disease
- Identify specific opportunities for you and your care team to support patients receiving GRT
- Discuss how to locate resources to help integrate GRT into practice and/or support patients who receive GRT

Criteria for Success

To obtain a certificate of completion, a score of 70% or better on the post-test is required. Please proceed with the activity until you have successfully completed this program, answered all test questions, completed the post-test and evaluation, and have received a digital copy of your certificate. You must participate in the entire activity to receive credit. There is no fee to participate in this activity. If you have questions about this activity, please contact AKH Inc. at weendi@akhcme.com.



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In support of improving patient care, this activity has been planned and implemented by AKH Inc., Advancing Knowledge in Healthcare and MDA. AKH Inc., Advancing Knowledge in Healthcare is jointly accredited by the Accreditation Council for Continuing Medical Education (ACCME), the Accreditation Council for Pharmacy Education (ACPE), and the American Nurses Credentialing Center (ANCC), to provide continuing education for the healthcare team.



This activity was planned by and for the healthcare team, and learners will receive 1 Interprofessional Continuing Education (IPCE) credit for learning and change.

Physicians

AKH Inc., Advancing Knowledge in Healthcare designates this enduring material for a maximum of 1 AMA PRA Category 1 $Credit(s)^{TM}$. Physicians should claim only the credit commensurate with the extent of their participation in the activity.

Nurses

Credit being awarded: 1 ANCC contact hour.

Pharmacists

AKH Inc., Advancing Knowledge in Healthcare designates this continuing education activity for 1 contact hour.

Physician Assistant

AKH Inc., Advancing Knowledge in Healthcare has been authorized by the American Academy of PAs (AAPA) to award AAPA Category 1 CME credit for activities planned in accordance with AAPA CME Criteria. This activity is designated for 1 AAPA



Category 1 CME credits. Approval is valid until 11/14/2024. PAs should only claim credit commensurate with the extent of their participation

Nurse Practitioners



This activity has been planned and implemented in accordance with the Accreditation Standards of the American Association of Nurse Practitioners (AANP) through the joint providership of AKH Inc., Advancing Knowledge in Healthcare and MDA. AKH Inc., Advancing Knowledge in Healthcare is accredited by the American

Association of Nurse Practitioners as an approved provider of nurse practitioner continuing education. Provider number: 030803. This activity is approved for 1 contact hour(s) (which includes .10 hour(s) of pharmacology).

Commercial Support

There is no commercial support for this activity.

Disclosures

| Name | Relationship | Commercial Interest |
|---|--------------|-------------------------------------|
| Erin Collins | N/A | Nothing to disclose |
| Natalie Goedeker, DNP | Adviser | Sarepta |
| Bo Hoon Lee, MD | Researcher | AMO Pharma, Sarepta, Sanofi Genzyme |
| Bernadette Makar, MSN, APRN-BC, NP-C | N/A | Nothing to disclose |
| (planner/reviewer) | | |
| Dorothy Duffy, RPh (planner/reviewer) | N/A | Nothing to disclose |
| AKH Inc Staff and Planners | N/A | Nothing to disclose |
| MDA Staff and Planners | N/A | Nothing to disclose |
| All of the relevant financial relationships listed for these individuals have been mitigated. | | |

Disclosures

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Disclosure of Unlabeled Use and Investigational Product

This educational activity may include discussion of uses of agents that are investigational and/or unapproved by the FDA. Please refer to the official prescribing information for each product for discussion of approved indications, contraindications, and warnings.

Disclaimer

This course is designed solely to provide the healthcare professional with information to assist in his/her practice and professional development and is not to be considered a diagnostic tool to replace professional advice or treatment. The course serves as a general guide to the healthcare professional, and therefore, cannot be considered as giving legal, nursing, medical, or other professional advice in specific cases. AKH Inc. specifically disclaim responsibility for any adverse consequences resulting directly or indirectly from information in the course, for undetected error, or through participants misunderstanding of the content.

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