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AJNR Am J Neuroradiol 2025, 46 (4) 646-647
doi: https://doi.org/10.3174/ajnr.A8745
http://www.ajnr.org/content/46/4/646

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The Gene & Cell Therapy Study Group is a collaborative neuroradiology initiative launched by the American Society of Neuroradiology (ASNR), the American Society of Functional Neuroradiology (ASFNR), and the American Society of Pediatric Neuroradiology (ASPNR). This joint effort aims to establish neuroradiology as a pivotal player in the rapidly advancing fields of genetic and cellular therapies for neurologic disorders.

The Rise of Gene and Cell Therapy

Genetic and cellular therapies have transitioned from experimental laboratory treatments and small-scale clinical trials into clinical practice.[1-3] These innovative tools hold immense promise as a vehicle for precision medicine. By advancing education, research, leadership, and collaboration, the Gene & Cell Therapy Study Group seeks to enable neuroradiologists to assume central roles in the development and delivery of these transformative treatments.

Key areas of focus include integrating neuroradiology expertise into disease phenotyping, optimizing patient selection, monitoring therapeutic responses, identifying adverse reactions, delivering these treatments precisely, and applying advanced imaging techniques including PET for these purposes.

The Growing Role of the Neuroradiologist

Gene therapies typically involve the ex vivo or in vivo introduction of genetic material into a patient's cells using viral vectors, nanoparticles, or other delivery vehicles to integrate the information into the host genome or episome. Cellular therapies leverage transplanted or modified cells to replace damaged tissues or restore function, such as using genetically-engineered stem cells to address neurologic injuries, neurodegenerative diseases, or cancers. These therapies aim to address the root causes of diseases at the genetic or cellular level, offering hope for previously intractable conditions.

Approved therapies include those for cerebral adrenoleukodystrophy (CALD),[4] amyotrophic lateral sclerosis (ALS),[5] metachromatic leukodystrophy (MLD),[6] spinal muscular atrophy (SMA),[7,8] aromatic L-amino acid decarboxylase deficiency (AADC),[9,10] and sickle cell disease (SCD).[11] Currently, >4000 human clinical trials are underway in the field of genetic and cellular therapies, with approximately 540 targeting neurologic disorders of all types, including inherited metabolic diseases, epilepsies, neoplasia, neuropsychiatric disorders, traumatic brain or spinal cord injury, and multiple neurodegenerative diseases.[12] Fetal treatments for neural tube defects are being piloted, with many treatments for inherited disorders in development.[13] Neuroimaging is integral at every stage of these therapies, from diagnosis and patient selection to delivery and outcome monitoring.

Imaging-Guided Delivery and Monitoring

Modern neuroimaging plays a critical role in the precise planning and monitoring of genetic and cell therapies. Delivery methods in current use include IV, intra-arterial, intrathecal, intracisternal, intraventricular, and intraparenchymal approaches.[14,15] Advanced molecular imaging techniques and image-guided procedures such as MRI-guided focused ultrasound[16,17] are being investigated to facilitate precise delivery within the CNS.

Beyond delivery, neuroimaging ensures appropriate engagement of therapeutic targets, assesses biodistribution, and monitors therapeutic efficacy and safety.[18,19] The unbiased, quantitative imaging insights provided by neuroradiology experts enhance clinical trial designs and therapeutic applications, fostering progress in this rapidly evolving field.

Goals of the Gene & Cell Therapy Study Group

The Gene & Cell Therapy Study Group has outlined the following objectives:

1) Inform and educate: provide the diagnostic and interventional neuroradiology community with knowledge of advancements in genetic and cellular therapies for neurologic diseases
2) Cultivate thought leadership: advance the neuroradiologist's role in imaging and image-guided delivery of these therapies
3) Foster collaboration: strengthen partnerships with clinical and industry stakeholders to contribute to clinical trials, set new standards of care, and guide the development of therapeutic imaging technologies
4) Establish clinical imaging standards: advocate for and publish evidence-based guidelines to standardize clinical imaging practices for gene and cell therapy.

An Invitation to Join

The Gene & Cell Therapy Study Group of the ASNR, ASFNR, and ASPNR welcomes diagnostic and interventional neuroradiologists, from seasoned experts to early-career professionals, to contribute to this exciting new initiative. Whether through active participation in study group meetings, contributing to collaborative research, or disseminating knowledge within their networks, neuroradiologists in the study group will have the opportunity to contribute to shaping the future of cell and gene therapy for neurologic disease. By building expertise, fostering collaboration, and standardizing practices, the Gene & Cell Therapy Study Group will empower neuroradiologists to elevate the role of the specialty within therapeutic medicine and improve outcomes for patients undergoing these transformative treatments.

Disclosure forms provided by the authors are available with the full text and PDF of this article at www.ajnr.org.

http://dx.doi.org/10.3174/ajnr.A8745

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