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Neuropathy Action Foundation Launches White Paper on Future of Gene Therapy & Urges Innovative Policy Approaches to Ensure Patient Access

NAF recommends policy changes to address cost, risk, and outcomes of innovative new treatments

SANTA ANA, CA (March 4, 2024) – The Neuropathy Action Foundation (NAF), a non-profit organization dedicated to ensuring neuropathy patients have access to the treatments necessary to improve their quality of life, today announced the release of a new white paper, [*“The Promise of Gene Therapy for Neuropathy and Rare Diseases.”*](#) The white paper explores the transformative potential of gene therapy for neuropathy and other patients and details policy considerations to ensure equitable patient access to these revolutionary treatments.

“The promise of gene therapy is immense, particularly for patients with neuropathy and other conditions with limited or no treatment options. Neuropathy patients, researchers, and clinicians are all hopeful that gene therapy products can create new options for treatment for certain kinds of neuropathy, including inherited peripheral neuropathy (IPN), which is caused by generic mutations in more than 100 genes,” said Dominick Spatafora, NAF board president. “For patients to access these innovative therapies, however, innovative policies will also be required.”

Neuropathy Action Foundation lays out **three foundational adaptations** that the healthcare system will have to make to ensure patient access to gene therapy, including:

Centering Patient and Family Input: The white paper highlights the critical need for policies that prioritize patient-driven preferences and values when it comes to coverage decisions. Shared decision-making at its best is patient-centered and focuses on a patient’s (and sometimes a family’s) preferences about whether to use a medical intervention and which one might work best considering the patient’s unique medical and non-medical circumstances.

Assessing Value Appropriately: Traditional methods of evaluating the value of medical interventions must evolve to incorporate patient-centered outcomes and preferences. Incorporating a broader range of outcomes and patient-driven value elements will become even more critical as interventions continue to be made available to patients.

Creating New Payment Models: Innovative payment models should offset the current exorbitant costs and uncertainties associated with gene therapy. These models should mitigate financial risks, ensure equitable access, and accommodate the unique challenges posed by gene therapy's upfront costs and uncertain outcomes.

Gene therapy is a cutting-edge approach that leverages genes to prevent, treat, or cure medical conditions by inactivating, repairing, or replacing faulty genes. Despite its decades-long scientific foundation, gene therapy represents a shift in healthcare and the translation of gene therapy from laboratory research to clinical application remains in its infancy. The first gene therapy product — for young patients with a form of leukemia — was only [approved](#) for use in the U.S. in 2017. According to the [American Society of Gene + Cell Therapy](#), more than 2,000 gene therapies are in development worldwide. As of December 2023, more than 30 gene therapy products have been [approved](#) by the U.S. Food and Drug Administration's (FDA) Center for Biologics Evaluation and Research (CBER). If this rate of development continues, in the next ten years, an [estimated](#) 750,000 to 1 million patients will have access to more than 60 gene therapy products in the U.S.

To access the full white paper and delve deeper into its findings, please visit www.neuropathyaction.org or click [here](#).

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About Neuropathy Action Foundation

The Neuropathy Action Foundation (NAF), a 501(c)(3) non-profit, is dedicated to ensuring neuropathy patients obtain the necessary resources to access individualized treatment to improve their quality of life. The NAF increases awareness among physicians, the general public and public policy officials that neuropathy can potentially be a serious, widespread and disabling condition, which may be treatable when appropriate medical care is provided. <http://www.neuropathyaction.org/>

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