Clinical Trials for Neuropathy: Looking Ahead

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Clinical Trials for Patients with Neuropathy

Clinical trials drive the development and assessment of new, potentially more effective and safer therapies for a wide range of diseases and conditions including various types of neuropathies. These research studies contribute to advancing scientific knowledge, identifying which interventions can improve quality of life and manage symptoms more effectively, while ensuring treatments are validated for safety and efficacy through rigorous, unbiased methods. Participating in clinical trials can also give patients early access to innovative treatments that are not otherwise available, and their involvement helps researchers gather data to refine approaches and understand the nuances of neuropathy care. Ultimately, clinical trials play a critical role in bringing scientific innovations to patients, offering hope and new options to those living with neuropathy. As of October 2025, more than 500,000 clinical trials are registered worldwide, including 160,000+ in the United States. In the U.S. nearly 1,000 address neuropathy including hundreds of studies ongoing or currently recruiting participants. This paper provides an overview of the kinds of neuropathy interventions currently being studied, what innovations are happening in the clinical trial process, the prospects for future research and recommendations for how patients, caregivers and advocates can get involved in advancing clinical trials related to neuropathy.

Clinical Trials: Researching Promising Innovations

Recent neuropathy clinical trials are testing a broad spectrum of interventions, ranging from cutting-edge pharmaceuticals and regenerative medicine, including gene therapy, to devices and non-drug therapies. Neuropathy clinical trials fall into 4 broad categories:

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1. Pharmaceuticals and biologics
2. Advanced therapies/regenerative medicine
3. Neuromodulation therapies
4. Technology-focused

Each of these categories is described in Table 1 and explained in more detail below.

1. Pharmaceuticals and Biologics

There are multitudes of pharmaceutical and biologic interventions for neuropathy undergoing clinical research. Some examples include:

- **Aldose reductase inhibitors:** Drugs like epalrestat and ranirestat that are ARIs are showing promise as therapeutic interventions for diabetic peripheral neuropathy
- **Chemokine receptor inhibitors:** Drugs such as DF2755A are being tested as therapeutic treatments for bladder pain related to peripheral neuropathy.
- **Monoclonal antibodies and biologics:** Mayo Clinic is testing a CGRP monoclonal antibody (eptinezumab) as a treatment for painful diabetic polyneuropathy (DPN), for example. DukeHealth in North Carolina is testing a biologic therapy called Autologous conditioned serum (ACS) for neurological pain.

2. Advanced Therapies/Regenerative Medicine

Advanced therapies for neuropathy focus on regenerative medicine include gene therapy, neurotropic factors and stem cell therapy. These interventions are usually focused on fixing root causes of the various kinds of neuropathies, instead of just managing symptoms.

- **Gene therapy:** A technique to repair or replace faulty genes, gene therapy is being tested in various ways to help patients with neuropathy. A DNA plasmid-based gene therapy called VM202 or Engensis is undergoing trials for nerve system regeneration for patients with diabetic peripheral neuropathy. *Note:* For a deep dive on gene therapy and neuropathy, see NAF's paper from 2024, The Promise of Gene Therapy for Neuropathy and Rare Diseases.

- **Neurotrophic factors and small molecules**: Neurotrophic factors (NTFs) are proteins essential for the growth and function of neurons. Modulating NTFs could be a way to slow or stop the progression of some neuropathic diseases.

- **Stem cell therapy:** Stem cell therapy works by turning special cells in the body called stem cells into specialized cells. In neuropathy, this might mean using the special cells to restore damaged nerve tissues. An example is research funded by the National Institutes of Health that could be the first treatment of its kind in neuropathy.

3. Neuromodulation Therapies

Neuromodulation is a medical treatment that alters the activity of nerves, for example through electric stimulation. These treatments can be invasive, where a device is implanted inside the body, or non-invasive, where the device is outside the body. Below are examples of neuromodulation therapies being tested for different kinds of neuropathy conditions.

- **Direct Current Stimulation:** Direct Current (DC) Stimulation is a way to use a device to provide targeted electrical neuromuscular stimulation. such as The Neubie device, aka the Neuro-Bio-Electric-Stimulator. DC stimulation is often used for physical therapy but is now being tested specifically for neuropathy.

- **MC5-A scrambler therapy:** Scrambler therapy is another method of using electrical stimulation of nerves to reduce pain. The therapy is currently being tested in head-to-head clinical trials for neuropathic plan, for example by Johns Hopkins Medicine.

- **Spinal Cord Stimulator (SCS):** Spinal cord stimulation had positive results in patients participating in a clinical trial called Inspire focused on painful diabetic peripheral neuropathy.

- **Transcutaneous Electrical Nerve Stimulator (TENS):** Nerve stimulation, such as that achieved by TENS devices, can be used to ameliorate some of the symptoms of neuropathic disorders, such as numbness, tingling and pain. Recent clinical trials are focusing on using wireless TENS units paired with a smart phone application that tracks symptom status and adherence to the therapy for Chemotherapy Induced Peripheral Neuropathy (CIPN).

4. Technology-Focused Interventions

Technology-focused interventions use telehealth, computers, smart phones and other technological devices, such as immersive virtual reality or virtual reality headsets in the treatment of symptoms, or to support adherence to interventions. The Burke Neurological Institute at Weill Cornell Medical School, for example, is currently conducting a clinical trial testing immersive virtual reality (IVR) protocols for people with neuropathic pain. The study is investigating two different IVR protocols: 1) Somatic IVR that helps people disassociate pain by visualizing the movement of affected limbs and 2) Distractive IVR where the virtual reality headset helps distract people from their pain by showing engaging landscapes.

Table 1: Examples of Neuropathy Clinical Trials by Intervention Type

Intervention Type	Example	Condition Focus
Pharmaceuticals and biologics	• Aldose Reductase Inhibitors • Chemokine receptor inhibitors • Monoclonal antibodies and biologics	Multiple neuropathies and chronic neuropathic pain
Precision medicine/ Regenerative medicine	• Gene therapy • Neurotrophic factors • Stem cell therapy	• Common peroneal nerve (CPN) • Diabetic • Inherited
Neuromodulation devices	• Direct Current (e.g., NEUBIE) • MC5-A scrambler therapy • Spinal Cord Stimulator (SCS) • Transcutaneous Electrical Nerve Stimulator (TENS)	Multiple neuropathies
Technology focused	• Virtual Reality Therapy including Distractive IVR and Somatic IVR	Multiple neuropathies

Innovations in Clinical Trial Processes

Research and development of interventions to address disease aims to discover new ways to use existing drugs, biologics or devices, or to find completely novel compounds or approaches. At the same time, the clinical trial process itself is undergoing innovation, and new ways to conduct research are being implemented by scientists.

Precision medicine

One of the most significant and hopeful clinical trial innovations is the use of what is called personalized medicine. The use of biomarkers and precision medicine to personalize medicine is transforming the way clinical trials are designed and conducted, especially for complex conditions like neuropathy. Traditionally, clinical trials have relied on large, heterogeneous patient populations and a "one-size-fits-all" approach, which often obscured meaningful treatment effects for subgroups of patients. Now, with advances in genomics, proteomics, and digital health technologies, biomarkers can identify biological signatures that predict how individual patients will respond to a therapy. This enables more targeted trial enrollment, faster identification of effective compounds, and reduced trial costs and durations. In neuropathy, where symptoms and underlying causes vary widely, precision medicine allows researchers to classify patients by molecular or genetic subtype, increasing the likelihood of finding effective, disease-modifying treatments rather than simply managing pain. Ultimately, this approach accelerates the discovery of new therapies and brings more personalized, effective options to people living with neuropathic conditions.

These patient-centered trials customize therapeutic interventions by developing biomarker-guided therapies in clinical trials. An example of this new approach to clinical trials is a project at Memorial Sloan-Kettering Cancer Center (MSKCC) in New York. The Center conducted genomic sequencing on 10,000 patients with metastatic cancer and found more than a third of those patients had potentially treatable genetic changes. Their discovery has led to innovations in drug development and clinical trials that investigate biomarker-guided therapies to target gene alterations that drive disease. This precision approach is now being used in diseases other than cancer, including neuropathic conditions.

Artificial Intelligence (AI)

It seems that AI is everywhere and that is definitely true for clinical trials. The two most prominent uses of AI application are in the field of drug discovery for neuropathy treatments and for the optimization of clinical trials processes.

- **Direct Current AI in Drug Development:**
  - AI is accelerating the development of new drugs and devices for treating neuropathy by predicting which drug compounds are most likely to be effective in treating nerve damage. Machine learning algorithms can analyze vast amounts of data on drug interactions, side effects, and molecular mechanisms to identify potential candidates for neuropathy-related conditions.
  - AI models can also help identify biomarkers for neuropathy, leading to more targeted therapies and more efficient drug development pipelines.

- **Optimizing Clinical Trials:**
  - AI is being used to improve the design and recruitment processes of clinical trials for neuropathy treatments. By analyzing patient data, AI can help identify individuals who are most likely to benefit from a specific drug or intervention, improving the efficiency of clinical trials and reducing costs.
  - Additionally, AI can help monitor patient responses during trials, allowing for real-time adjustments and ensuring that the treatments are working as expected.

Prospects for Future Research

For patients with neuropathy, currently available treatments are limited and tend to be focused on relieving symptoms, not on halting the process of nerve degeneration. Research and development on interventions to prevent and treat neuropathic diseases and conditions is essential for people living with neuropathy, but discovering and developing these interventions is an uncertain and risky endeavor that requires significant investment. According to the Congressional Budget Office (CBO), the R&D costs for a new drug average between "less than $1 billion to more than $2 billion per drug," and "only about 12 percent of drugs entering clinical trials are ultimately approved for introduction by the U.S. Food and Drug Administration (FDA)."

Prospects for Research and Development Funding

Private spending is the primary source of investment for clinical trials. In 2024, the largest pharmaceutical companies invested nearly $200 billion on research and development (R&D), according to a March 2025 IQVIA Institute report, Global Trends in R&D 2025: Progress in recapturing momentum in biopharma innovation. This amount is up 73% since 2019, when industry spent just $80 billion. The 2024 R&D spending exceeded 25% of sales, setting a new record for expenditure intensity.

Additional biopharma investment from private sources, such as initial public offerings, and public sources such as federal and state investments, totaled more than $100 billion in 2024. Most of the investment came from private investment, but public funding did account for 25% of the biopharma investment that was outside companies' investment according to IQVIA. As in previous years, biopharmaceutical companies headquartered in the U.S. accounted for the majority of clinical trial starts in 2024, and "the U.S. was the most common first-launch country."

While the prospects for innovation and private funding of clinical trials for neuropathy interventions are high, the prospects for public funding that support the foundational science drug development is often built on is at risk. Research shows that public funding for the basic science underpinning biopharma development usually stimulates private R&D. As such, federal cuts announced in 2025 to reduce the annual National Institutes of Health (NIH) budget by 40% are being called a direct assault on the backbone of biomedical discovery. Federal budget cuts would also increase FDA times for new drug applications. Analysis of these cuts estimate the combined effects would "ultimately decrease the number of new drugs coming to market."

The private sector has been increasing its investment in discovering new treatments over the past decade and has been implementing clinical trials productivity enablers, such as AI and patient-centric models to improve efficiency. These efforts have proven successful as "the number of novel drugs produced per year has increased over the last decade, with an average of 44 compared to the average annual productivity of the last 30 years, which was 34." Balancing this increased investment and productivity improvement from the private sector with the radical cuts to early-stage biomedical research will be essential to ensuring novel interventions reach patients living with neuropathy in the coming decade.

How Can Clinical Trials Be Improved?

Clinical trials are a key building block of bringing new treatments to people living with neuropathy, but the process for participating could be improved. One of the biggest areas of improvement is to increase representation in clinical trials so that people involved in research more closely match the types of patients with the condition or disease. Historically, many trials have not reflected the diversity of the broader patient population—they have often underrepresented women, older adults, racial and ethnic minorities, and people from rural or lower-income communities. This lack of diversity can lead to treatments that work well for some groups but are less effective or have unexpected side effects in others. Because genetic, environmental, and social factors all influence how diseases develop and how patients respond to therapies, inclusive participation is necessary for accurate and equitable science.

Expanding representation in clinical trials requires removing barriers that prevent people from joining. Common challenges include limited access to research centers, lack of awareness about available studies, language and cultural differences, and mistrust of the medical research system—particularly among communities that have experienced historical abuses. Addressing these issues means meeting people where they are: partnering with community clinics, providing materials in multiple languages, covering travel costs, and engaging trusted community leaders to share information about trial opportunities. Decentralized or "virtual" trials—where participants can take part from home using digital tools—are also helping to reach more diverse participants by reducing logistical and geographic burdens. For example, a clinical trial of the TENS device for relief of cancer-induced peripheral neuropathy (CIPN) sought to address gaps in care for people living in remote areas or who are too frail to travel. Because these populations faced access disparities to conventional rehabilitation that was conducted in person, the Duncan Cancer Center at Baylor College of Medicine is testing the wireless TENS managed through a smart phone application to help CIPN patients use the device and track their symptoms.

Increasing representation in clinical trials to match U.S. Census representativeness, as well as disease-specific epidemiology are also ways clinical trials could be improved. In 2024, "U.S. Census levels were met or exceeded for less than half of U.S. studies," according to the March 2025 report from the IQVIA Institute for Human Data Science, Global Trends in R&D 2025: Progress in recapturing momentum in biopharma innovation. The share of participation in U.S. clinical trials by patients who are Black/African American is at or below the U.S. Census level, but Hispanic inclusiveness, in comparison, has never read U.S. Census level and decreased from 19% to 16% in 2024, even though people of Hispanic or Latino ethnicity represented 19.5% of the U.S. population in 2023.

Expanding representation in clinical trials requires removing barriers that prevent people from joining. Common challenges include limited access to research centers, lack of awareness about available studies, language and cultural differences, and mistrust of the medical research system—particularly among communities that have experienced historical abuses. For example, most people are not able to participate in a clinical trial at the same location where they are receiving care for their disease or condition according to a recent report by McKinsey & Company called, Achieving best-in-class clinical trial delivery: The road to 2035.

Addressing these issues means meeting people where they are: partnering with community clinics, providing materials in multiple languages, covering travel costs, and engaging trusted community leaders to share information about trial opportunities. Decentralized or "virtual" trials—where participants can take part from home using digital tools—are also helping to reach more diverse participants by reducing logistical and geographic burdens. Greater representation in research and development strengthens both science and public health. When clinical trial populations mirror the real-world diversity of those affected by disease, researchers gain more reliable insights into safety, efficacy, and optimal dosing across different groups. This leads to treatments that are more effective for more people and builds public trust in medical research. Inclusivity in trials is not only a scientific necessity but also an ethical commitment—to ensure that all communities share equally in the benefits of innovation and progress in healthcare.

Clinical Trials Participation: Patients, Caregivers and Advocates

Clinical trials are foundational for uncovering novel interventions to help patients living with neuropathy. Participating in a study can be a powerful way for people with neuropathy to contribute to medical progress while also gaining access to potential new treatments. The benefits of participating in a clinical trial go beyond access to experimental therapies. Participants receive close medical monitoring, often from leading specialists, and may experience symptom improvement from promising treatments not yet widely available. Even if the investigational treatment does not directly benefit them, trial participants contribute valuable data that can shape future therapies. Participation also provides a sense of empowerment—helping individuals play an active role in advancing the understanding and management of neuropathy.

Reliable sources of information on participating in clinical trials include:

- ClinicalTrials.gov maintained by the U.S. National Institutes of Health
- Centerwatch.com a privately run website with a centralized clinical trial database
- ICH GCP the International Council for Harmonization Good Clinical Practice Network offers a worldwide clinical trials registry that can be searched
- Mayo Clinic based in Minnesota offers a searchable clinical trials database

Patients, caregivers and advocates can get involved in advancing clinical trials related to neuropathy in a variety of ways.

Patients

To find clinical trials to participate in, patients can start by asking their health care provider about open studies. Talking to a doctor about joining a clinical trial is an important step. Patients should ask whether their specific type of neuropathy qualifies for any ongoing studies and whether participation would interfere with current treatments. It can be helpful to bring printed trial information or links to discuss with the doctor. Physicians can help interpret the inclusion and exclusion criteria, explain potential risks and benefits, and ensure the study aligns with the patient's overall care plan. Many trials require referrals or baseline evaluations that can be facilitated by a patient's regular healthcare provider.

Caregivers

One of the most powerful supports caregivers can provide is helping patients navigate the logistics and emotional challenges of joining a trial. Caregivers often assist with scheduling appointments, managing medications, and providing transportation, the practical supports that can make or break participation, especially for those with mobility or financial limitations. Emotional encouragement from caregivers—emphasizing that participation contributes to both personal care and broader scientific progress—can also make a significant difference in a patient's decision to join or stay in a study.

Advocates

Advocacy groups can improve the clinical trial process, enhance access and expand participation by sharing credible information about what trials are, how they work, and where to find them, using trusted channels like support groups, social media, and patient networks. By normalizing discussions about research participation and clarifying misconceptions, advocates can help people feel more confident exploring these opportunities. Advocates can also push for policies that reduce barriers to participation, such as the use of decentralized trial models that allow for remote participation. Encouraging increased funding for basic research and policies that support the R&D process are also important pursuits for advocates.

Conclusion

Clinical trials are vital to scientific progress. Every new medication, from pain relievers to disease-modifying drugs, depends on the willingness of volunteers to take part in carefully designed studies. For neuropathy in particular, where causes are diverse and treatment options remain limited, robust participation ensures researchers can collect enough data to understand what works, for whom, and why. By joining clinical trials, people with neuropathy not only help accelerate the search for better treatments but also contribute to a broader mission: improving quality of life for millions affected by nerve disorders worldwide. Caregivers and advocates also have important roles to play in sharing information and promoting policies that support research and development efforts aimed at identifying novel targets to treat neuropathic pain.

Future clinical trials have the potential to transform the diagnosis, amelioration and treatment of a multitude of neuropathic conditions. Through the testing of innovative therapies and the refinement of existing approaches, these studies may lead to more personalized and effective treatment options for patients. As researchers explore cutting-edge therapies and improve current clinical trial methods, patients can look forward to more individualized, effective and targeted therapeutic strategies. Clinical trials have paved the way for remarkable progress in neuropathic research. As innovation continues to accelerate, there is growing hope that future treatments will bring meaningful relief and improved quality of life for those affected.

The Neuropathy Action Foundation (NAF) is a 501(c)(3) nonprofit dedicated to ensuring neuropathy patients obtain the necessary resources to access individualized treatment to improve their quality of life. The NAF increases awareness among providers, the general public and public policy officials that neuropathy can be a serious, widespread and disabling condition, which may be treatable when appropriate medical care is provided. The NAF's goals include:

Patient Empowerment
Educates and assists neuropathy patients on how to become informed advocates.

Public and Physician Awareness
Supports programs that create public and physician awareness of neuropathy, the use of IVIG and other treatments to improve patient care.