ThermoFisher SCIENTIFIC

Neuroscience research

Accelerate your gene therapy trial

with our expertise in rare neurological disorders

ppd

Gene therapies are changing outcomes for rare neurological diseases. Not only does this momentum benefit the lives of patients, it also creates a sense of urgency for drug developers and CROs alike. At a time when acceleration is essential, the PPD™ clinical research business of Thermo Fisher Scientific is the right partner to advance your therapeutic development. We apply our hands-on global knowledge of the medical and operational needs for programs focused on rare diseases, neurologic and neurodegenerative conditions – including the heterogenous aspects of gene therapy development – to quickly get your trial on the path to success.

In the past five years, our neuroscience team has supported:

- **60,000+** contracted patients
- **450+** neurological disease trials
- **9,000+** contracted sites
- **100+** rare neurological disease trials
- **600+** rare disease studies
- **75+** gene therapy trials

We have strong, longstanding relationships with global sites and can support more efficient study start-up, recruitment, and retention. Leveraging our experience with first-in-human single-ascending dose (SAD) trials and deep knowledge of country-specific timelines for gene therapy studies, we can guide appropriate country and site selection to help accelerate your therapy's speed to market.

End-to-end operational expertise in gene therapy for rare neurological disorders

Since 2016, we have supported seven out of 10 FDA-approved neuroscience therapies. Our neuroscience team leverages the combined expertise of scientists, project managers and clinical managers armed with best practices in neurological genomics research and a deep understanding of the benefits and pitfalls of each approach. We create **streamlined gene therapy study protocols and designs** to help you meet your goals effectively, weaving insights gained from strategic partnerships with leading research sites into the clinical development plan.

We enable our customers to better predict how patients, caregivers, sites, regulators, and payors will react to advanced therapies. Our teams proactively manage all aspects of rare neurological gene therapy development, creating seamless and trackable execution by working closely with sponsors and sites.

Driven by our passion and experience, we understand how to **minimize risks caused by the logistical challenges** faced in gene therapy programs relating to your IP, including gene therapy:

Transportation

Antibody testing

Preparation and administration

Helping sites deliver successful trials

We provide sites with training for healthcare professionals new to clinical research through access to SiteCoach. To ensure that sites are poised to deliver on speed, skill, efficiency, and quality across all rare neuroscience conditions, we also offer ongoing support services for digital and decentralized trials including e-consent, concierge services, and patient reminders.

In addition to our own global network of wholly owned sites, we also provide connection to an extended traditional site network and act as a liaison with all trial vendors. Our project leaders understand the nuances of gene therapy trials and have a keen understanding of how to provide site support for:

- Site infrastructure requirements
- Logistics plans for medicinal products and precious samples
- Patient-centric services to increase participant recruitment, safety, and retention
- Processes required for extensions and long-term follow-ups
- Consent processes for gene therapy trials
- Providing tailored, age-appropriate patient educational materials, including cultural translators for cross-border recruitment
- Reducing site and individual burdens, increasing patient/caregiver comfort and overall trial accessibility

The cell and gene therapy institute and gene therapy operational center of excellence

Our Cell and Gene Therapy Institute (CGTI) brings together our key Centers of Excellence (COE) to best meet your needs, including the Gene Therapy Operational COE. Piloted by operational and medical leadership teams focused on resource and process optimization for IO, adoptive cell therapy, and ex vivo gene therapy studies, this COE aims to enhance delivery to patients, investigators, and sponsors with **connection to our comprehensive knowledge center and gene therapy training academy** for operational tools and the sharing of best practices.

A multi-functional centers of excellence model for improving our clients' gene therapy studies

- **Rare disease and pediatrics COE**
  A cross-functional home for our teams, focused on executing innovative medical and operational solutions
- **Advanced therapies froum**
  Regulatory experts who stay on top of all aspects of current agency perspectives on cell and gene therapy
- **Market access and value demonstration leadership team**
  Strategic, expert-driven market-specific insights on evidence, positioning, and value messaging for payor and health technology assessment stakeholders
- **Gene therapy operational COE**
  Led by experts who build better operationalization and training strategies for gene therapy studies
- **Immuno-oncology and cell therapy COE**
  Operational and medical leadership teams focused on resource and process optimization for IO and adoptive cell therapy studies

Cross-functional expertise at the rare disease and pediatrics center of excellence

We combine niche CRO medical and research expertise with an experienced worldwide site network to provide support for rare and pediatric indications at our Rare Disease and Pediatrics Center of Excellence (RDPCOE).

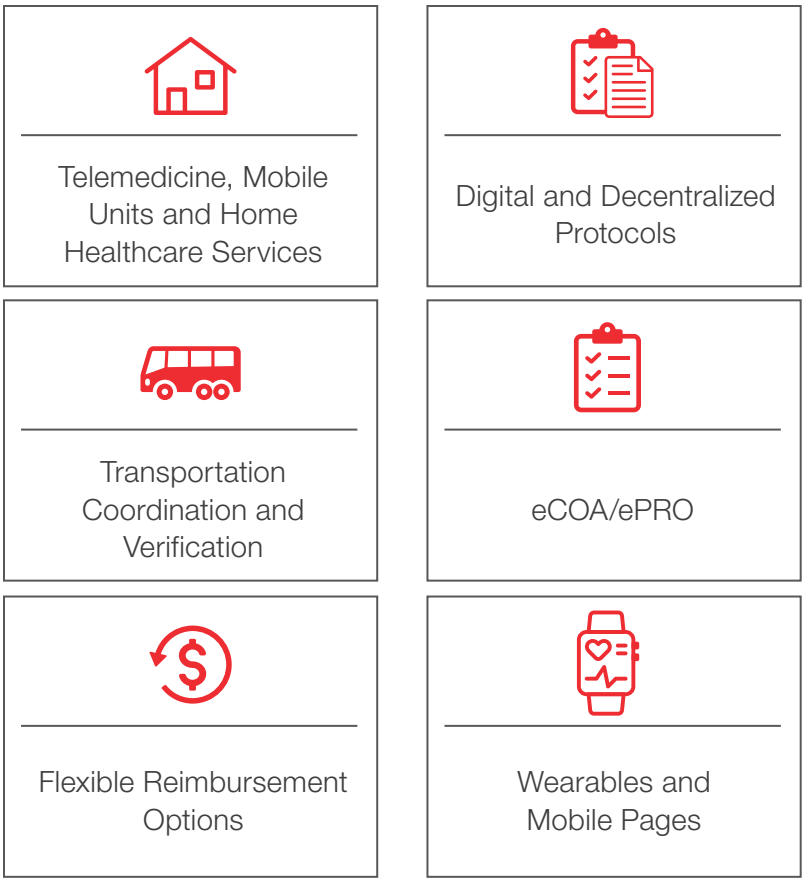
We provide consistent input and proactive solutions for our clients by **integrating into one executional engine our expertise in**:

- Support Services
- Medical Services
- Regulatory Services
- Operational Services

Our groups at the RDPCOE contain therapeutic indication and subject matter experts, including medical experts in neurology, genetics, and neonatology/pediatrics who can support the development of custom solutions.

Easing enrollment and increasing retention with patient-centric services

We recognize how challenging managing clinical trial burdens can be for patients with rare neurological disorders and their caregivers. We provide concierge services to make it easier for patients to participate in gene therapy trials by offering:



These services help produce timely and high-quality data for our clients while saving patients time and cost. Our patient-centric approach has led to over 90% patient retention over five years for a recent long-term follow-up trial.

To simplify recruitment, we offer accelerated enrollment solutions (AES) that give access to a robust and ever-expanding patient database including patients with rare conditions.

Leveraging technological insights for proactive study design

Our **clinical trial simulator** can help you identify potential gaps in your rare neurological program ahead of time. We help you use its insights to guide the selection of design parameters for clinical trials.

Regulatory strategies to support successful filings

Our global regulatory experts have expertise in early and frequent collaboration with regulators to ensure successful submissions for gene therapies in rare neurological indications.

We can support you with protocols for:

- Early planning
- Real World Evidence (RWE) generation
- Natural history, registry, and endpoint development and analysis

Learn more at **ppd.com/therapeutic-expertise/neuroscience/**