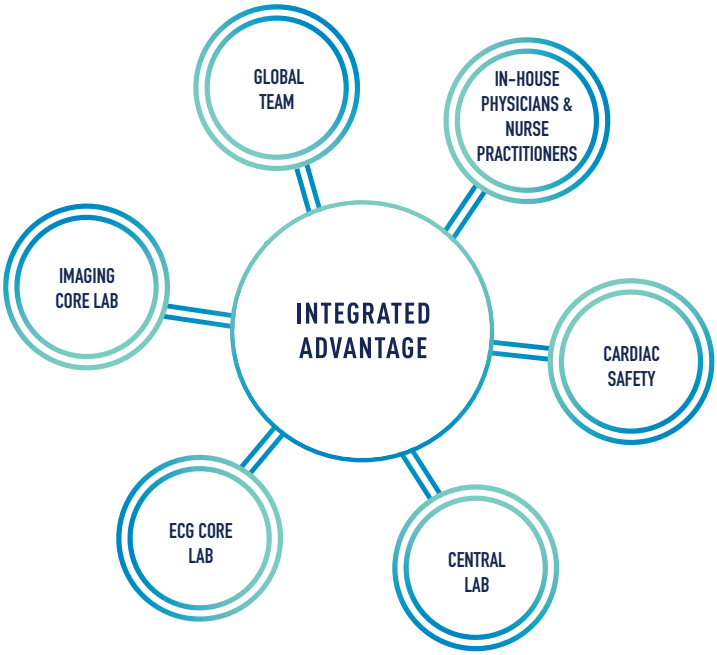
A DEEPER DIVE INTO NEUROMUSCULAR DISEASES

AN INTEGRATED ADVANTAGE

Diseases characterized by neuromuscular degeneration are particularly challenging when it comes to study design and endpoint selection. As a full-service and therapeutically-focused CRO, Medpace specializes in the design and conduct of global trials in neurology and psychiatry, including neuromuscular disorders. We bring a global footprint, strategic medical, regulatory and operational leadership as well as fully integrated central labs and core imaging services to enhance and expedite development.



THERAPEUTICALLY-FOCUSED TEAM

Medpace is unique in its scientifically-driven approach to clinical research. Our model gives Sponsors the advantage of early and ongoing insight and guidance from a team of therapeutic experts throughout trial design and execution. Our highly-experienced team of medical, regulatory, and operational experts work collaboratively to conduct clinical trials. We understand issues from the perspective of Sponsors, clinical investigators, scientific leaders, and reviewers at regulatory agencies. With this insight, we successfully define and execute clear development plans from beginning to end.

The complexities of mental health research require a heightened level of integration and efficiency. We combine strategic medical, regulatory and operational leadership to execute at the highest level.

EXPERTS

EXPERT INSIGHT

Serving as therapeutic team leaders, our in-house medical doctors provide strategic direction for study design and planning, train operational staff, work with Investigators, provide medical monitoring, and meet with regulatory agencies as well as our global regulatory affairs experts to provide strategic guidance into the best pathways to accelerate approvals. Our MDs are embedded throughout every study, providing greater depth of expertise and the ability to tackle complex and challenging diseases.

In-House Physicians

Sarah DeRossett, MD, PhD
Senior Medical Director, Medical Department

Toshihiro Hokonohara, MD
Medical Director, Medical Department

Filipe Brogueira Rodrigues, MD, MSc
Medical Director, Medical Department

Richard D. Scheyer, MD
Vice President, Medical Department

James Vornov, MD, PhD
Vice President, Medical Department

ADVANCED CLINICAL PRACTITIONERS ADD A PATIENT'S PERSPECTIVE

Our medical directors are supported by experienced ACPs whose unique perspective brings added value to the clinical development team. These highly-trained experts with advanced degrees and hands-on clinical experience provide an additional layer of knowledge and insights that can streamline your studies and address potential challenges early in the study planning. ACPs are embedded in the teams to ensure your study is feasible and operationalized for efficient execution by looking at the protocol through the lens of the patient and site staff. ACPs also help with the review of safety events, ongoing team education, recruitment strategies, and study document review. Their understanding of what works in a clinical setting makes them a valuable part of our embedded medical expertise model.

Danielle Mattingly, FNP-BC, MPH
Advanced Clinical Practitioner

Sarah DeRossett, MD, PhD

Senior Medical Director, Medical Department

Dr. Sarah DeRossett is a board-certified neurologist with 15 years of experience in clinical and academic neurology, plus more than 10 years of drug development experience.

Experience Summary

- Drug development expertise in neuropsychiatric disorders, dementia, opioid use disorder, sleep, and other CNS disorders
- Clinical and drug development experience in pain and analgesia
- Extensive research experience in psychopharmacology with special focus on opioids
- Leadership of clinical development programs in Parkinson's disease, Alzheimer's disease, Migraine, Restless Leg Syndrome (RLS), Neuropathic pain, Epilepsy, and Opioid Use Disorder
- Broad experience in pharmaceutical R&D, including single point of accountability for clinical development plans, medical governance, and support of regulatory submissions
- Well-published in the peer-reviewed medical and scientific literature

Education Summary

- Doctor of Medicine, cum laude, Emory University School of Medicine
- Postdoctoral Fellowship, Pharmacology, Emory University School of Medicine
- Residency in Neurology, The Johns Hopkins Hospital

Toshihiro Hokonohara, MD

Medical Director, Medical Department, Neurology, Psychiatry

Dr. Toshihiro Hokonohara is a board-certified neurologist with over 15 years of clinical practice and academic neurology including experience in leadership roles at pharmaceutical and global contract research organizations.

Experience Summary

- Drug development expertise in neuropsychiatric disorders, dementia, autoimmune disease, oncology and other CNS and Internal Medicine disease
- Clinical and drug development experience in neuro-immunological disease
- Extensive research experience in some neurological hereditary disease
- Pharmacovigilance Leader of clinical development programs in Epilepsy
- Broad experience in pharmacovigilance medical governance and support of regulatory submissions

Education Summary

- Doctor of Medicine, Kyushu University – Fukuoka, Japan
- Residency in Neurology, Kyushu University Hospital – Fukuoka, Japan

Filipe Brogueira Rodrigues, MD, MSc

Medical Director, Medical Department

Dr. Filipe Rodrigues is an academic neurologist with over 6 years of drug development experience in neurology and psychiatry.

Experience Summary

- Served as academic neurologist at University College London Queen Square Institute of Neurology and as clinician at the National Hospital for Neurology & Neurosurgery in London
- Hands-on clinical trial experience in neurodegeneration, movement disorders and neurogenetics, including antisense technology and intrathecal delivery
- Member of working groups for Movement Disorders Society, Research Ethic Committee, Critical Path Institute, and Cochrane Collaboration
- Academic research in biofluid biomarkers and clinical pharmacology
- 50+ peer-reviewed medical and scientific publications

Education Summary

- Master's Degree in Medicine, Faculty of Medicine, University of Lisbon – Lisbon, Portugal
- Bachelor of Science Degree in Medicine, Faculty of Medicine, University of Lisbon – Lisbon, Portugal

Richard D. Scheyer, MD

Vice President, Medical Department

Dr. Richard Scheyer is a board-certified neurologist with over 30 years of professional medical experience which includes 18 years dedicated to clinical drug development.

Experience Summary

- Led a team that designed and executed an early development program for next-generation alpha-2 delta ligand, including capsaicin challenge, and crafting Proof of Concept strategy for novel ion channel blockers
- Pioneer in translational medicine and Phase I/IIa drug development with special interest in early demonstration of clinical efficacy
- Highly-regarded author with over 60 manuscripts and abstracts, with a focus on clinical pharmacology and therapeutic activity in areas ranging from diabetes to oncology
- Launched first controlled trial natural product-derived protein kinase C activator in Alzheimer's at previous company

Education Summary

- Bachelor of Science, Physics, Stanford University
- Doctor of Medicine, The State University of New York, Upstate Medical University
- Residency in Neurology, Yale University
- Fellowship Training in Epilepsy, Yale University

James Vornov, MD, PhD

Vice President, Medical Department

Dr. James Vornov is a board-certified neurologist with over 18 years of director level clinical development experience.

Experience Summary

- Brought multiple compounds into first-in-man to proof-of-concept and successful NDA submission. Provided broad clinical trial design expertise, clinical pharmacology experience, operational excellence and global regulatory strategy development across a broad range of CNS diagnostics and therapeutics
- Expertise in the rapid transition of compounds from the laboratory to clinical proof of concept using Critical Path technologies such as biomarkers, PK/PD modeling and clinical trial simulation
- Worked in multiple CNS therapeutic areas having directed programs in Depression, Suicidal Ideation, Parkinson's disease treatment and diagnosis, stroke, neuropathic pain, diabetic and chemotherapy-induced peripheral neuropathies, anesthesia and brain tumors

Education Summary

- Bachelor of Arts, Biology & Psychology, Columbia University
- Doctor of Medicine, Emory University, School of Medicine
- Doctor of Philosophy, Anatomy, Emory University, School of Medicine

Danielle Mattingly, FNP-BC, MPH

Advanced Clinical Practitioner

Ms. Mattingly is a certified Family Nurse Practitioner and Neuroscience Registered Nurse. She brings experience in clinical research, public health, and health policy.

Experience Summary

- Extensive experience as a Neurology Nurse Practitioner at multiple university medical centers
- Experience providing both inpatient and outpatient care of patients, including patients with epilepsy and sleep disorders
- Served as sub-investigator on active clinical trials for novel treatments of localization related epilepsy, narcolepsy, and idiopathic hypersomnia
- Actively worked to create an education lecture series for neurology advanced practice providers
- Active member of multiple neurology and advanced practice nursing associations

Education Summary

- Master of Science in Nursing, Family Nurse Practitioner, Columbia University – New York, NY
- Master of Public Health in Health Policy and Management, Columbia University – New York, NY
- Bachelor in Nursing Science, Columbia University – New York, NY
- Bachelor of Arts in Public Health, Vanderbilt University – Nashville, TN

EXPERIENCE

Medpace combines our team of neurologists with our cross-functional team of rare disease experts to support Sponsors developing therapies for rare neurological disorders.

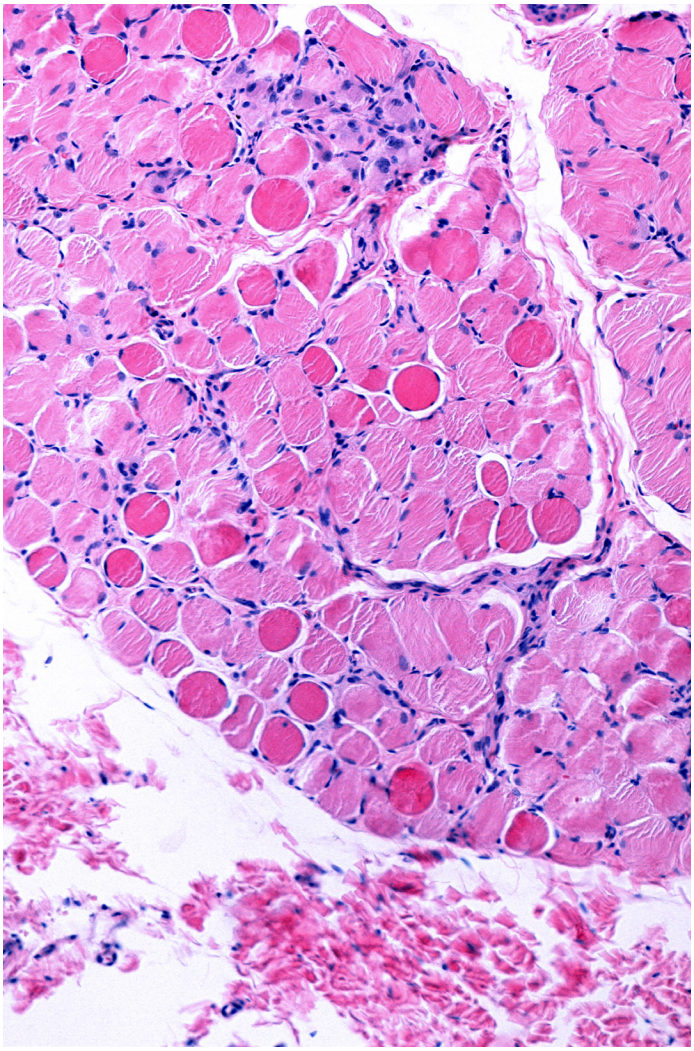
We have amassed experience in various rare neuromuscular indications such as:

- Charcot Marie Tooth Disease
- Duchenne Muscular Dystrophy
- Familial Amyloidosis Polyneuropathy
- Mucopolysaccharidosis
- Multiple Sclerosis
- Muscular Dystrophies
- Myasthenia Gravis
- Myotonic Dystrophy
- Pompe disease
- Spinal Muscular Atrophy

RECRUITMENT AND SITE RELATIONSHIPS

Due to the unique challenges associated with rare disease studies, partnering with a CRO with expertise in enrolling rare disease patients and maximizing site selection to create realistic study feasibility is crucial. Medpace understands this challenge. Our multi-dimensional recruitment model enables us to implement innovative, comprehensive, and customized recruitment and retention strategies that identify, recruit, and retain members of specific patient populations.

Medpace has also established a productive network of key clinical sites and large academic institutions who specialize in CNS rare disease disorders ensuring study timelines and key milestones are achieved. Our physicians and professional staff have in-depth knowledge of screening tools and rater scales and will provide oversight for rater services including subjective assessments, inter-rater reliability, and scale validation.



RELATIONSHIPS WITH PATIENT ADVOCACY GROUPS

Medpace recognizes the importance of collaborating with advocacy groups. Although advocacy groups vary greatly in size, scope, and purpose, they regularly offer insight into who is treating patients, what is important to patients, and lessons learned from past clinical trials. Advocacy group collaboration raises awareness for your clinical trial and can be an invaluable partnership for the life of your drug.

Medpace is well versed in working with sponsors and advocacy groups of all different sizes globally in order to ensure clinical trials are designed and operated with the patients in mind. Medpace can help sponsors forge new relationships with advocacy groups or support existing relationships seamlessly.

EXECUTION

The complexities of mental health research require a heightened level of integration and efficiency. We combine strategic medical, regulatory and operational leadership as well as well as fully integrated Central Labs and Core Imaging Services to execute at the highest level.

CENTRAL LABS

Medpace Central Labs provide consistency in methods and instrumentation across wholly-owned and purpose-built laboratories located in the US, Europe, China, and Singapore. We offer a wide range of relevant biomarker assays for stroke and have the ability to rapidly establish and validate novel assays as needed. Over the past several years, Medpace has validated hundreds of new biomarker assays based on guidelines from the Clinical and Laboratory Standards Institute (CLSI) and in accordance with CAP and CLIA regulations.

CORE IMAGING

High-quality image acquisition and interpretation is crucial for the success of trials relying on imaging, including neuroimaging for patient selection or as a primary endpoint. Medpace provides centralized neuroimaging expertise, led by radiologists, neurologists and neuroscientists with decades of clinical and trial experience.

Medpace Imaging Core Lab provides a suite of imaging services to enhance and expedite biopharmaceutical and imaging contrast agent development, including a broad spectrum of imaging biomarkers for rare neuromuscular disease trials.

Capabilities include advanced quantitative magnetic resonance imaging measures for:

- Muscle Fat Fraction (MRI-PDFF, MRS)
- Elastography
- Muscle cross-sectional area or volume
- Anatomic and functional brain mapping

FULL-SERVICE CLINICAL DEVELOPMENT

Medpace is a scientifically-driven, global, full-service clinical contract research organization (CRO) providing Phase I-IV clinical development services to the biotechnology, pharmaceutical and medical device industries. Medpace's mission is to accelerate the global development of safe and effective medical therapeutics through its high-science and disciplined operating approach that leverages local regulatory and deep therapeutic expertise across all major areas including oncology, cardiology, metabolic disease, endocrinology, central nervous system and anti-viral and anti-infective.