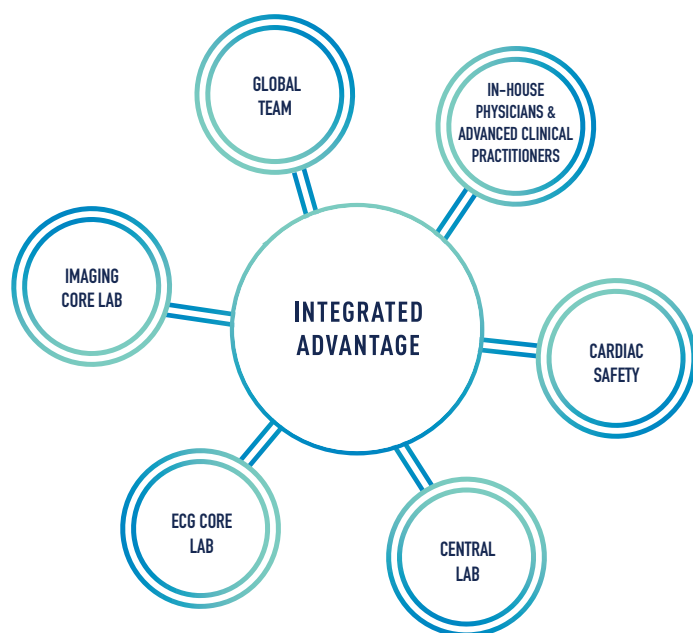


A DEEPER DIVE INTO NEUROMUSCULAR DISEASES



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

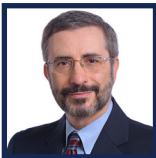
Medpace is unique in its scientifically-driven approach to clinical research. Our model gives Sponsors the advantage of early and ongoing insight and guidance from a team of therapeutic experts throughout trial design and execution. Our highly-experienced team of medical, regulatory, and operational 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.



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.



Rich D. Scheyer, MD
Sr. Vice President,
Medical Department



James Vornov, MD, PhD
Vice President,
Medical Department



Sarah DeRossett, MD, PhD
Sr. Medical Director



Liang Bozarth, MD
Medical Director



Toshihiro Hokonohara, MD
Medical Director



Boban Joksimovic, MD, PhD
Medical Director



Filipe Rodrigues, MD, MSc
Medical Director

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 nurse practitioners 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.



Morgan Ball, MSN, FNP-BC
Advanced Clinical
Practitioner



Danielle Caudell-Stamper, MSN, AGACNP-BC
Advanced Clinical
Practitioner



Danielle Mattingly, FNP-BC, MPH
Advanced Clinical
Practitioner



Daniela Rae, MSc, RGN, IP
Advanced Clinical
Practitioner



IN-HOUSE PHYSICIAN BIOGRAPHIES

Richard D. Scheyer, MD

*Sr. Vice President, Medical Department,
Neurology, Pharmacology*

Dr. Richard Scheyer is a board-certified neurologist with over 30 years of professional medical experience which includes over 20 years dedicated to clinical drug development.

Experience Summary

- Pioneer in translational medicine and Phase I/IIa drug development with special interest in early demonstration of clinical efficacy
- 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
- 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,
Neurology, Analgesia, Psychiatry*

Dr. James Vornov is a board-certified neurologist with over 20 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



Sarah DeRossett, MD, PhD

Senior Medical Director

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

Liang Bozarth, MD

Medical Director

Dr. Bozarth is a board-certified neurologist with special qualifications in child neurology and epilepsy.

Experience Summary

- 13+ years of clinical experience in academic medicine and clinical research
- Prior academic appointment and Principal Investigator (PI) work performed at Seattle Children's Hospital and University of Washington
- Served as a PI for multiple clinical trials, observational studies, and investigator-initiated drug studies
- In-depth knowledge in early onset catastrophic intractable epilepsies
- Strong expertise in pediatric rare genetic disorders from clinical diagnosis/management, basic scientific research, and clinical trials
- Therapeutic expertise in child and adult neurology, including epilepsy, neurogenetic disorders, neuromuscular diseases, and neurodevelopmental disorders
- Multiple publications of original research in peer-reviewed journals

Education Summary

- Doctor of Philosophy in Neuroscience, The University of Hong Kong – Hong Kong, Hong Kong
- Bachelor of Medicine, Bachelor of Surgery, China Medical University – Liaoning, China



Toshihiro Hokonohara, MD

Medical Director

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

Boban Joksimovic, MD, PhD

Medical Director

Dr. Boban Joksimovic is a board-certified neurologist with a PhD in neuroscience and over 20 years of clinical experience in neurology.

Experience Summary

- 17 years of CRO experience as a Medical Director, Clinical Operations Lead, and Clinical Research Associate in Phase I-IV studies
- Expert in intraoperative neurophysiology and EMNG with neuromuscular disorders in focus
- Previously practiced as a neurophysiologist and introduced intraoperative neurophysiology to Serbia in 2008
- Extensive experience as Sub-investigator and rater in CNS studies
- Rankin Scale, NIHSS, Neurostatus EDSS, MDS-UPDRS, Modified Hoehn and Yahr, MoCA training certificates

Education Summary

- Doctor of Philosophy in Intraoperative Neurophysiology, University of Kragujevac – Kragujevac, Serbia
- Board Certified Neurologist, University of Novi Sad – Novi Sad, Serbia
- Medical Doctor, University of Belgrade – Belgrade, Serbia



Filipe Brogueira Rodrigues, MD, MSc

Medical Director

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

Morgan Ball, MSN, FNP-BC

Advanced Clinical Practitioner

Morgan Ball is a board-certified family nurse practitioner with over 7 years of clinical experience, bringing expertise in interventional pain management and neurology with a focus on movement disorders.

Experience Summary

- Experience providing care both inpatient and outpatient, including pediatric cardiac ICU, general pediatrics, neurology, and interventional pain management
- Assessed, diagnosed, and managed a variety of neurological and chronic pain conditions
- Provided patient and family education about diagnosis and treatment plans
- Served as the lead nurse practitioner and manager
- Active member of the American Academy of Neurology and multiple advanced practice nursing associations

Education Summary

- Post-Master's Family Nurse Practitioner Certificate Diploma, Xavier University – Cincinnati, OH
- Master of Science in Nursing, Xavier University – Cincinnati, OH
- Bachelor of Science in Biology, University of Kentucky – Lexington, KY



Danielle Caudell-Stamper, MSN, AGACNP-BC

Advanced Clinical Practitioner

Mrs. Danielle Caudell Stamper is a board-certified advanced practice registered nurse bringing over 8 years of clinical and preclinical research experience, over 3 years of nursing experience and over 7 years of experience as a nurse practitioner providing direct patient care.

Experience Summary

- Clinical experience in neuro critical care, pulmonary critical care, and transplant medicine.
- Served as a sub-investigator on multiple clinical trials, including investigation of genomic classifiers for idiopathic pulmonary fibrosis, proteomic risk evaluation for lung cancer, the treatment of persistent air leak after pneumothorax, a novel device for the treatment of emphysema, and devices in improving the diagnostic yield of biopsies
- Over eight years of clinical and preclinical neurological research experience as a senior clinical research assistant specializing in hemorrhagic stroke, traumatic brain injury, aneurysm, and ischemic stroke
- Involved in the conduct of over 40 clinical trials as a clinical trial nurse coordinator and regulatory specialist
- Member of the American Academy of Neurology (AAN), American Association of Bronchology and Interventional Pulmonology (AABIP) and American College of Chest Physicians (CHEST)

Education Summary

- Master of Science in Nursing in Adult Gerontology Acute Care Nurse Practitioner, University of Cincinnati – Cincinnati, OH
- Bachelor of Science in Nursing, University of Cincinnati – Cincinnati, OH
- Bachelor of Arts in Biology, Thomas More University – Crestview Hills, KY
- Associate of Arts in Spanish, Thomas More University – Crestview Hills, KY

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



Daniela Rae, MSc, RGN, IP

Advanced Clinical Practitioner

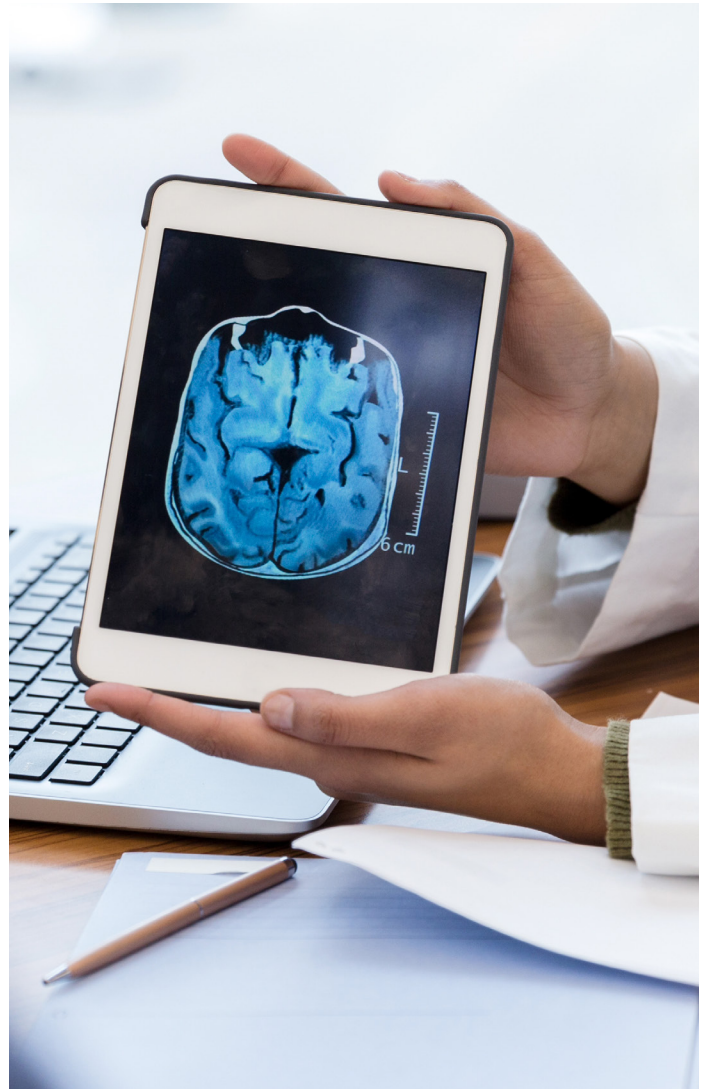
Daniela Rae is a registered Consultant nurse and Independent Prescriber (UK). She brings nearly decades of senior clinical leadership and research experience in neuroscience, with a focus in neurodegeneration, neuropsychiatry and neurogenetics.

Experience Summary

- Extensive experience in Huntington's disease, rare dementia and cognitive disorders – genetic counselling, clinical assessment and management, policy and standards of care development as well as clinical and academic research.
- Former clinical lead providing operational and clinical leadership for a large, multidisciplinary team, including medical consultants, nurses, trainees, and external key stakeholders.
- Managed an autonomous clinical caseload in a world leading tertiary outpatient clinic for patients and families with neurological, neuropsychiatric, neurogenetic, and neurodegenerative disease.
- Former Scottish government research fellow functioning as Chief investigator and Lead researcher for a multi-center organizational case study investigating care delivery systems in Huntington's disease
- Served as lead and key contributor for international working groups developing standards of care guidelines in rare, neurodegenerative disease
- Extensive experience as senior research nurse, trials manager, sub- and principal investigator in clinical trials with neurodegenerative and critical care focus
- Served as lead and key contributor for international working groups/ taskforce developing standards of care guidelines in rare, neurodegenerative disease

Education Summary

- Diploma in Psychological Therapies, Association of Psychological Therapies – London, United Kingdom
- Master of Science in Public Health and Health Services Research, University of Aberdeen – Aberdeen, United Kingdom
- Diploma in Adult Nursing, Berufsfachschule fuer Krankenpflege – Munich, Germany



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.

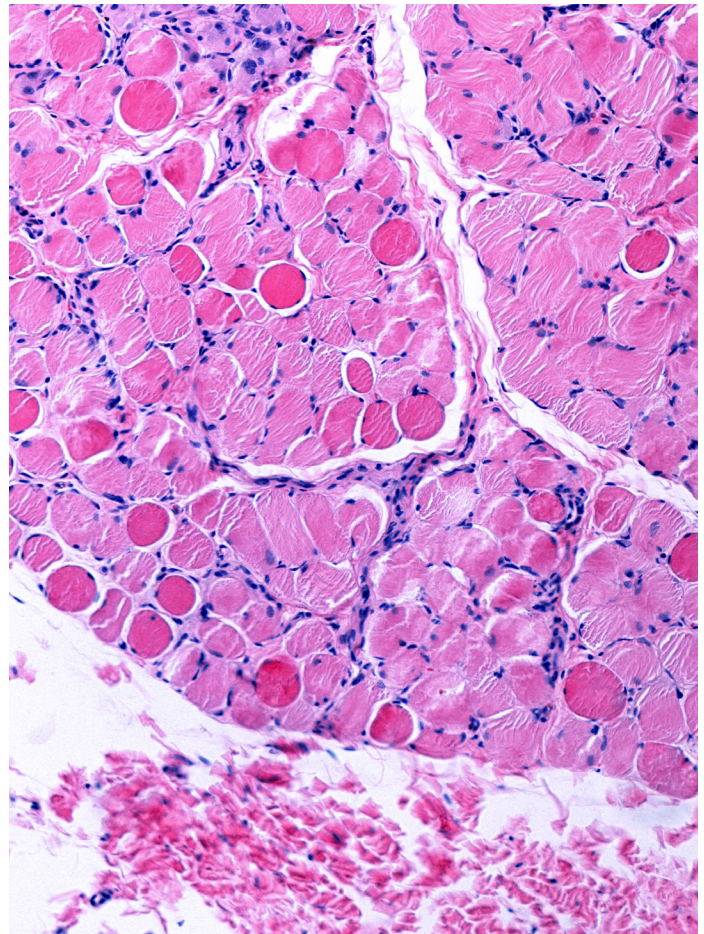
We Have Amassed Experience in Various Rare Neuromuscular Indications Such As:

- 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 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.

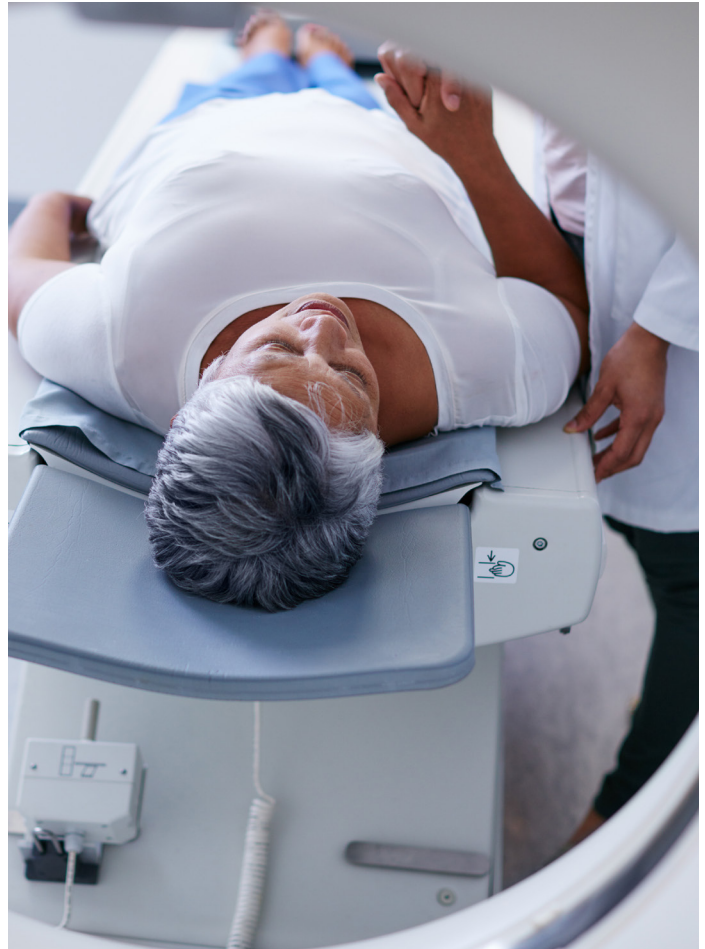
CORE IMAGING

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.

MAKING THE COMPLEX
SEAMLESS®

