

Center for Health + Technology



OUR MISSION

To advance **human therapeutics, health, and knowledge** through exceptional people, skillful research, and partnerships.

OUR STORY

155+
CLINICAL
STUDIES

The **Center for Health + Technology [CHeT]** is an academic research organization within the University of Rochester Medical Center. For more than three decades, CHeT has served as a worldwide leader in the conduct, planning, management, implementation, analysis, and rescuing of large multi-center clinical research studies.

45K+
RESEARCH
PARTICIPANTS

Simultaneously, our innovative and novel technologies and outcome measures have shaped and improved how research is conducted and how therapies are evaluated. Our skilled team of consultants are readily available to provide guidance to academic institutions, pharmaceutical companies, technology firms, not-for-profit foundations, advocacy groups, and the federal government.

12
FDA
APPROVALS

- CHeT Director: Chad Heatwole, MD, MS-CI

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CHeT - CTCC

Clinical Trials Coordination Center

DIRECTOR

Melissa Kostrzebski,
MS, MBA

CTCC specializes in the development, management, and conduct of clinical research studies and provides a full range of research and clinical trial management support services that facilitate the conduct of clinical research from study concept through data analysis, publication, and FDA approval. Over the past 30 years, the CTCC has managed the conduct of more than 155 clinical research studies with 50+ sponsors (government, industry, & private) that enrolled over 45,000 research participants in the US, Canada, Europe, New Zealand and Australia.

Our clinical research expertise includes:

- **Study Start-Up**
Including but not limited to the following: Novel and adaptive trial design; Protocol development and training; Contract facilitation and negotiation; Site selection based on key performance indications and research study datasets
- **Monitoring**
Remote, risk-based quality management, and on-site
- **Data Management**
Clinical Data Management System (21 CFR part 11 compliant); Data sharing, Visualization, and Data standards (CDISC, CDE)
- **Clinical Trial Rescue and Recovery**
Provide services to revamp, refocus, and revitalize your clinical trial
- **Investigational New Drug/Investigational Device Exemption support**
- **Statistical analysis, modeling, and data mining**

CTCC has the infrastructure to conduct worldwide, high quality, regulatory-compliant, and multi-center clinical research:

- + 60+ SOPs and guidelines for audit readiness
- + Direct web-based data entry and ePRO
- + Clinical Trial Management Systems (21 CFR part 11 compliant)
- + Data visualization tools and templates
- + Access to 100+ research study datasets

LEARN MORE



CTCC achieves study start-up, enrollment, database lock and regulatory submission at an accelerated pace due to long-standing-relationships with clinical sites, competitive site start-up, disease specific expertise and many other unique experiences that will benefit your trial.

10 FDA Approvals

Over the last two decades, CHeT CTCC has supported clinical trials that have led to ten FDA-approval treatments. These include first-of-their-kind therapies for Parkinson's disease, Huntington's disease, periodic paralysis, and Friedreich's ataxia.

2023 **Valbenazine**
 SPONSOR: Neurocrine Biosciences
 DISEASE: Chorea for Huntington's disease
 BRAND NAME: Ingrezza

2023 **Omaveloxolone**
 SPONSOR: Reata Pharmaceuticals
 DISEASE: Friedreich's Ataxia
 BRAND NAME: Skyclarys

2017 **Deutetrabenazine**
 SPONSOR: Teva Pharmaceuticals
 DISEASE: Huntington's disease
 BRAND NAME: Austedo

2015 **Dichlorphenamide**
 SPONSOR: Taro Pharma
 DISEASE: Primary Hypokalemic & Primary Hyperkalemic Periodic Paralysis
 BRAND NAME: Keveyis

2008 **Tetrabenazine**
 SPONSOR: Prestwick Pharmaceuticals
 DISEASE: Huntington's disease
 BRAND NAME: Xenazine

2007 **Rotigotine**
 SPONSOR: Schwarz Pharma
 DISEASE: Parkinson's disease
 BRAND NAME: Neupro

2006 **Rasagiline**
 SPONSOR: Teva Pharmaceuticals
 DISEASE: Parkinson's disease
 BRAND NAME: Azilect

2006 **Selegiline**
 SPONSOR: Somerset Pharmaceuticals
 DISEASE: Parkinson's disease
 BRAND NAME: Emsam

2003 **Entacapone**
 SPONSOR: Orion Corporation
 DISEASE: Parkinson's disease
 BRAND NAME: Comtan

1997 **Pramipexole**
 SPONSOR: Pharmacia & Upjohn
 DISEASE: Parkinson's disease
 BRAND NAME: Mirapex

CHeT - CMSU

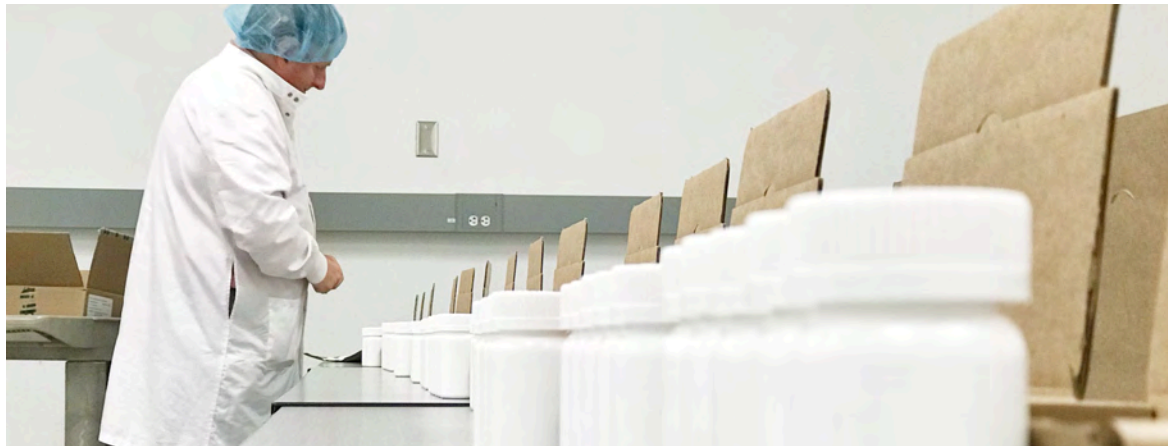
Clinical Materials Services Unit

EXECUTIVE DIRECTOR

Cornelia Kamp,
MBA

SENIOR DIRECTOR

Eileen Fannon



Registered with the New York State Board of Pharmacy as a wholesaler re-packager of drugs and devices, the Clinical Materials Services Unit (CMSU) provides contract pharmaceutical services to the Clinical Trials industry.

The staff of CMSU have over 150 years of collective pharmaceutical experience and have serviced 15-20 multi-center studies concurrently, with an average study size of 200 participants, 25 sites and up to 5 years in duration. CMSU has provided regulatory support for 14 investigator initiated INDs, services to 90 drug and device multi-center clinical trials, and drug/device supplies to over 25,000 participants at more than 2,100 sites.



About Us

CMSU was founded in 2008 when clinical investigators from the University of Rochester determined the need for a dedicated, on-site facility to manage entire supply chains in support of clinical trials conducted at or through the University, a major player in NIH and industry sponsored clinical research globally.

Clinical Trial Services Leading to 3 FDA Approvals

Clinical Trial Packaging and Labeling Services

Clinical packaging services include kit design, label creation, and production, all compliant with cGMP regulations. CMSU uses its validated, 21 CFR Part 11 compliant labeling system (ClinPro LBL™) to produce labels per the Clinical Protocol and Federal Regulations. Packaging and labeling follow customized SOPs, and clinical materials are QA inspected upon receipt, during processing, and at order completion. Material movement is overseen by the Quality Assurance Unit.



LEARN MORE



Storage: Controlled Room Temperature [+2° to 8° C, and -20° C]

Monitored HVAC systems, refrigerators and freezers are backed up by emergency natural gas-powered generators. A dedicated monitoring system alerts CMSU staff in the case of deviation or temperature excursion.

Cold Chain Shipping

CMSU utilizes qualified, cold chain shippers, with Sensitech Temp Tales® programmed to the allowable temperature range, which monitor the in-transit temperature of the clinical materials through to its ultimate destination. The data-logger reports are retrieved from sites, QA reviewed and filed in the regulatory files. Any excursions during transit are vetted to determine if product continues to be fit for use.

Supply Chain Management

We utilize our in-house expertise to assure that the entire supply chain of primary and secondary components, clinical labels, investigational drug distribution and device supply, and concomitant medications are delivered on-time and within budget.

Drug Supply Sourcing

CMSU works with other supply-side vendors and wholesale distributors to ensure the highest quality lowest cost materials are sourced, managed/handled in compliance with Federal and State regulations and all project requirements.

Returns Handling

Return and Disposition of used and unused materials must be carefully coordinated with Sites and Sponsors to assure adequate accountability of supply status. CMSU possesses many years of experience in handling of returns in a regulatory compliant manner from the perspective of both FDA and EPA.



Destruction of Used and Unused Supplies

Once full accountability of the investigational articles is established CMSU will petition the Sponsor to approve its final disposition and ultimate destruction in a regulatory compliant manner.

Regulatory and Support Services

CMSU can provide regulatory support services for IND's, CTA's and IMPD's as necessary to complete your applications. CMSU has provided input and "technical writing support for nearly 100 INDs and numerous CTA's and IMPD's.



Quality System

CMSU's Quality System is designed to comply with International Standards for the production of Investigational Product (IP); 21 CFR parts 210 and 211 (current Good Manufacturing Practice (cGMP) regulations for the production of Pharmaceuticals, 21 CFR Part 812 (Investigational Device Exemptions) and Volume 4, Good Manufacturing Practices, Annex 13, Manufacture of Investigational Medicinal Products.

CMSU also provides project management support:

- + Kit design to align with dispensing visits
- + Creation of drug accountability logs and operations/pharmacy manuals
- + Presentation of drug/device supplies at Investigator Meetings
- + Management of expiration/retest dates



**Your Comprehensive
Resource for Clinical
Trial Materials**

CHeT Outcomes



DIRECTOR

Chad Heatwole,
MD, MS-CI

200+

Disease-specific, regulatory-grade, patient and caregiver-reported outcome measures developed and validated using FDA guidance

Measure what matters most to patients in your studies!

The CHeT Outcomes team specializes in developing and validating highly sensitive, disease-specific outcome measures, reported by patients (PRO: patient-reported outcomes) and caregivers (CR: caregiver-reported outcomes, also known as ObsRO: observer-reported outcomes). These measures are designed for use in therapeutic trials and FDA drug-labeling claims. Our group has developed and individually validated more than 200 disease-specific instruments and over 1000 subscales that quantify symptomatic disease burden during clinical trials. These instruments are clinical tools designed to longitudinally measure changes in disease-specific health status in response to therapeutic interventions during clinical trials and FDA drug-labeling claims

In contrast to generic PROs, properly developed and validated disease-specific outcome measures can detect small but highly relevant changes in multiple areas of patient health, improve clinical trial efficiency, reduce sample size requirements, limit floor and ceiling effects, and ensure the relevance of future treatments to patients.

Uses of our Disease-Specific Health Indexes



Academic center initiated clinical trials



Pharmaceutical sponsored clinical trials



Government funded clinical trials



Foundation initiated research & registries

Our instruments are

- ✓ **FDA Compliant**
Designed and validated to fully satisfy regulatory and published FDA guidance for use in drug-labeling claims and measuring changes in how a patient feels and functions
- ✓ **Developed and validated using large-scale patient input**
- ✓ **Preferred by patients over generic instruments and reduce patient burden-of-use**

LEARN MORE



Our Instruments

Our instruments measure the multifaceted disease burden in individual diseases. Our team of epidemiologists, biostatisticians, qualitative researchers, patient advocates, linguists, computer programmers, outcomes researchers, and physicians has developed patient-reported and caregiver-reported outcome measures for adult and pediatric populations for the following diseases:

- + Adrenoleukodystrophy (ALD)
- + Adrenomyeloneuropathy (AMN)
- + Alzheimer's disease (CI)
- + Amyotrophic lateral sclerosis (ALS)
- + Cardiomyopathy
- + Cerebral cavernous malformation (CCM)
- + Charcot Marie Tooth (CMT)
- + Crohn's disease (CD)
- + Dementia (CI)
- + Duchenne muscular dystrophy (DMD)
- + Dystonia
- + Facioscapulohumeral muscular dystrophy (FSHD)
- + Fibromyalgia (FM)
- + Fragile X
- + Friedreich's ataxia (FA)
- + Huntington's disease (HD)
- + Inclusion body myositis (IBM)
- + Lung cancer (LC)
- + Lysosomal acid lipase deficiency (LAL-D)
- + Mitochondrial disease
- + Mild cognitive impairment (CI)
- + Myasthenia gravis (MG)
- + Myotonic dystrophy type 1 (DM, DM1, MDHI)
- + Myotonic dystrophy type 2 (DM2, MD2HI)
- + Non dystrophic myotonia (NDM)
- + Parkinson's disease (PD)
- + Rheumatoid Arthritis
- + SCN2A
- + SHANK3 / Phelan McDermid Syndrome
- + Smith Magenis Syndrome / RAI1
- + Spinocerebellar atrophy (SCA)
- + Spinal-bulbar muscular atrophy (SBMA)
- + Spinal muscular atrophy (SMA)
- + SYNGAP1
- + "Human-HI": Healthy Individual Health Index

Our disease-specific instruments have demonstrated superior responsiveness in detecting clinically relevant changes in patient conditions compared to traditional measures.



Advantages of Disease-Specific PROs

- **Higher relevance and content validity** for target population
- **Lower burden** to patients and caregivers
- Strong correlation to **functional capabilities**
- Ability to **lower sample size** requirements
- Ability to **emphasize relevant symptomatic themes** of a specific population while excluding non-relevant issues
- **Increased sensitivity** to detect small, but clinically relevant changes in disease-specific health
- **Better suited to measure disease progression** or therapeutic gain over time compared to generic outcome measures

Our group will collaborate with you to develop and fully validate a disease-specific outcome measure for any disease or provide consultation regarding outcome measure selection, use, optimization, and analysis.



If you would like more information about how you can obtain a license to use our health indexes in your study, please contact HealthIndexes@chet.rochester.edu

CHeT Innovation



DIRECTOR

Jamie Adams,
MD

Virtual (or “site-less”) studies use video conferencing to conduct remote assessments, eliminate geographic barriers to participation, and allow for more efficient study conduct. New tools, such as smartphones and wearable sensors, can be incorporated into clinical trials and enable objective and frequent assessments of participants in real-world settings.



Bringing Research to Participants

- Use new technologies to conduct a dozen studies with virtual visits that have reached more than 1500 participants throughout the country
- Recruit and retain a national cohort of clinical trial-ready participants in a longitudinal natural history study
- Amass a local and national registry of highly engaged research participants known as project:brain health. Visit projectbrain.org to learn more



High Frequency Assessments

CHeT has also pioneered studies of smartphones, wearables, video analytics and passive sensors that collect data inside and outside the clinic. Over 20,000 individuals from every state in the country participated in the *mPower* Parkinson’s disease smartphone study that CHeT helped support with colleagues at Sage Bionetworks. This study, along with ten others, have captured how individuals feel and function in their natural environment and provide new insights into the disease and assess the effectiveness of experimental and approved therapies.

2015 *mPower*

1st Apple ResearchKit app for a neurological disorder



2017

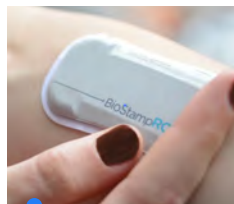


1st mobile app for Huntington’s disease



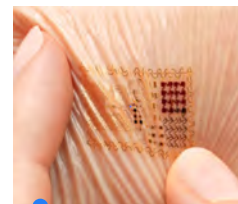
2018

1st longitudinal wearable sensor study for Huntington’s disease



2019

1st use of wearable devices, sensors, polysomnography, and video to detect and quantify scratching in Atopic Dermatitis



LEARN MORE





CHeT has deployed the use of smartphones, wearables, and radio-wave sensors in fully-virtual studies to enable trial participation from anywhere. Soon, we will be taking these tools globally.



WATCH-PD

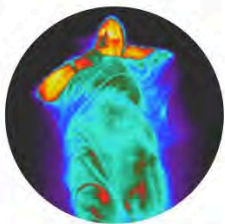
Evaluate the ability of sensors to assess features and progression of symptoms in early, untreated Parkinson's disease. Sensor assessments at home and in the clinic are compared to the traditional in-person assessments.

132 Participants (82 PD, 50 Control)
Collaborators: Biogen, Takeda

AT-HOME PD

Evaluate clinical outcomes using video visits in a virtual national observational study. Capture real-world data using a Parkinson's disease-specific smartphone application.

220 Participants
Collaborators: Massachusetts General Hospital, Northwestern University, Sage Bionetworks, NIH



SQUAD

Assess the use of wearable devices, sensors, polysomnography, and video to detect and quantify scratching. Evaluate the relationship between patient-reported outcomes and scratching and sleep metrics from wearable sensors.

45 Participants
Collaborators: Pfizer

VALOR-PD

Use video visits to evaluate the longitudinal change in individuals at genetic risk (due to mutations in the LRRK2 gene) of Parkinson's disease. Develop a cohort of participants ready for clinical trials of gene-directed therapies.

277 Participants
Collaborators: 23andMe, NIH



2020 WATCH-PD

1st longitudinal multi-site digital technology study in early Parkinson's disease



2021 UR-Udall

1st multi-faceted PD study on disease progression, remote assessments, and digital tools for real world assessments



2022

1st study to identify PD signals at home using artificial intelligence-enabled detection



2023 CELESTE

1st phase III fully remote medical device study in Parkinson's Disease



2024

1st long-term remote digital follow up study of PD clinical trial cohorts



CHeT Analytics

DIRECTOR

Charles Venuto,
Pharm.D.

CHeT Analytics is developing strategies to reduce drug development costs and enhance clinical care for those living with neurological disorders. By leveraging one of the world's largest repositories of clinical trial data for Parkinson's and Huntington's diseases, new insights are being made possible through predictive modeling and simulation.

ASSOCIATE DIRECTOR

Peggy Auinger,
MS



Access

To clinical trial and observational study data from over 100 studies



Extensive Knowledge

Of developing clinical disease progression models of neurological disorders

Existing Study Highlights for Collaborative Research

Parkinson's Disease (75 studies)

55 Interventional, 20 Observational

Huntington's Disease (37 studies)

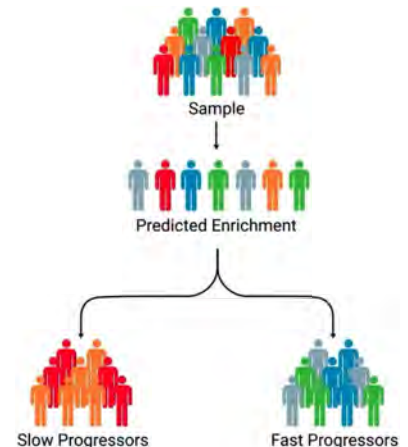
31 Interventional, 6 Observational

Friedreich's Ataxia (6 studies)

HIV/AIDS (8 studies)

Utilize our expertise in statistical methodology and machine learning to provide analytical modeling, consulting and support

- Develop **clinical disease progression models** that predict meaningful clinical outcomes to patients, caregivers, and researchers
- Develop strategies to **reduce drug development costs** through efficient clinical trial designs
- Visualize complex data to **identify patterns and drive strategic analysis**
- Harmonize distinct sources of data to **increase sample size and generate more robust findings**



The Analytics Unit is developing tools for patients, clinicians, and caregivers to interact with the prediction models to help manage their disease in a data-driven manner. We believe creating such tools empowers patients and allows the entire care team to proactively plan for future scenarios that are based on real patients' data of similar profiles.

LEARN MORE



We specialize in drug development rescue re-analysis. Our modeling and simulation methods are able to identify sub-populations of treatment responders and exposure-response relationships, if any such exist, through post-hoc analyses.

Analytical Services - Pharmacology



AIM To provide pre-clinical and clinical pharmacology expertise to guide drug development

- Develop protocols for pre-clinical and clinical testing of small molecule compounds and protein-based therapeutics
- Conduct pharmacokinetics and pharmacodynamics modeling and simulation throughout all phases of drug development
- Analyze pharmacology data to understand patient factors influencing drug exposure and response
- Conduct drug-drug interaction studies and concomitant medication monitoring to ensure optimal safety of experimental therapeutics

CHET Biostatistical Services

Provide Biostatistical Support For New Studies

(Includes clinical trials, other interventional studies, and observational studies)



Knowledgeable in study processes from the planning phase to presentation of results

- Contribute to **protocol development**
- Develop a **statistical analysis plan**
- Provide Data Safety Monitoring Board support (i.e. **unblinded statistician**)
- Perform **statistical analyses**
- Promote standardized data collection based on **CDISC standards**
- Contribute to **preparation of manuscripts**

- Participate in proposal/grant submission
- Participate in protocol development
- Promote standardized data collection
- Create the Statistical Analysis Plan
- Review and provide feedback on study documents
- Perform randomization
- Provide Data Safety Monitoring Board Support
- Review and provide feedback on study reports
- Perform rolling data review
- Perform interim analysis
- Validate data cleanliness
- Sign-off on database lock
- Perform statistical analyses
- Generate tables, listings, and figures
- Create finalized datasets based on CDISC standards
- Provide statistical documents for the Clinical Study Report
- Provide study results to report on ClinicalTrials.gov
- Participate in dissemination of results
- Facilitate data sharing

Pre-Planning

Planning

Enrollment/
Implementation

Closeout

Analysis

Provide Consulting



To assess relevant existing studies to answer research questions

- Develop and evaluate new outcome measures to quantify disease progression (e.g. composite scores)
- Utilize lab data and biospecimens to identify potential biomarkers to quantify disease progression
- Provide replication of analyses in independent studies as well as new exploratory analyses on existing study data

CHeT Health



DIRECTOR

Brett L. Kinsler,
DC

CHeT Health focuses on helping the community benefit from our organization's scientific advancements. We aim to disseminate CHeT's capabilities and discoveries worldwide to increase awareness, empower scientific insights, reduce costs of therapeutic trials, and accelerate the delivery of novel treatments to the people who need them most.

CHeT Health is



Disseminating CHeT's Capabilities and Discoveries to Improve Clinical Studies

From CHeT's research breakthroughs, our clinical trials support, and our design and consulting services, we foster collaboration, and accelerate the integration of innovations into studies and practice around the world.



Facilitating Access to Research and Improving Engagement

We ensure widespread access to CHeT's research and promote engagement and participation in studies. This encourages an environment of research involvement, increasing diversity and strengthening the connections between science and society.



Improving the Health of our Communities

More comprehensive and applicable research findings allow greater numbers of people to benefit from our scientific advancements and ultimately leads to better health outcomes for all.

LEARN MORE



Leadership & Faculty

CHeT leverages the expertise of our faculty and leading experts in the fields of neurology, biostatistics, pharmacology, clinical trial operations, health equity, among others. Representing departments from across the University of Rochester Medical Center as well as external institutions and agencies, our faculty bring decades of experience to CHeT's cutting edge research.

Chad Heatwole, MD, MS-CI
Director, Center for Health + Technology
Professor, Department of Neurology

Jamie Adams, MD

Associate Director, Center for Health + Technology
Associate Professor, Department of Neurology

Peggy Auinger, MS

Statistician, Department of Neurology

Christopher A. Beck, PhD

Associate Professor of Biostatistics and Orthopedics

Gretchen Birbeck, MD, MPH

Professor, Department of Neurology, Epilepsy

Cynthia Casaceli, MBA

Research Associate Professor, Department of Neurology

Ray Dorsey, MD, MBA

David M. Levy Professor, Department of Neurology

Robert Dworkin, PhD

Professor of Anesthesiology, Neurology, Oncology, and Psychiatry

Robert G. Holloway, MD, MPH

Chair, Department of Neurology
Professor, Department of Medicine, Palliative Care

Cornelia Kamp, MBA

Research Associate Professor, Department of Neurology

Karl Kieburtz, MD, MPH

Professor, Department of Neurology

Brett L. Kinsler, DC

Research Assistant Professor of Neurology

Benzi M. Kluger, MD, MS

Director, Center for Palliative Care
Chief, Division of Neuropalliative Care
Professor of Neurology

Melissa Kostrzebski, MS, MBA

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Michael P. McDermott, PhD

Professor, Department of Biostatistics and Computational Biology

Ruth B. Schneider, MD

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Sandhya Seshadri, MA, MS, PhD

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Robert L. Strawderman, ScD

Chair, Department of Biostatistics and Computational Biology

Christopher G. Tarolli, MD

Assistant Professor, Department of Neurology, Movement Disorders

Charles A. Thornton, MD

Professor, Department of Neurology, Neuroscience

Charles Venuto, PharmD

Associate Professor, Department of Neurology

Scan this QR code to view our featured publications on our website:



Key Clinical Research

Sample out of 200+ studies

HUNTINGTON'S DISEASE

KINECT-HD

120 participants

FDA Approval August 2023

A randomized, double-blind, placebo-controlled phase 3 multi-center clinical trial using UHDRS Total Maximal Chorea (TMC) score to evaluate the safety and tolerability of valbenazine for participants with Huntington's disease.

FIRST-HD

90 participants

FDA Approval April 2017

A randomized, double-blind, placebo-controlled study of SD-809 extended release for the treatment of chorea associated with Huntington's disease.

TETRA-HD

72 participants

FDA Approval August 2008

A randomized, double-blind, placebo-controlled, study of Tetrabenazine for the treatment of Huntington's chorea.

SIGNAL

301 participants

A study in individuals with late prodromal and early manifest Huntington's disease to assess the safety, tolerability, pharmacokinetics, and efficacy of Pepinemab. (VX15/2503)

PARKINSON'S DISEASE

PPMI

1,700 participants

The Parkinson's Progression Markers Initiative is a global, longitudinal observational study seeking markers of progression in Parkinson's disease.

DATATOP

800 participants

FDA Approval February 2006

A 2 × 2 factorial, double-blind, placebo-controlled, phase 3 multi-center clinical trial in participants with early Parkinson's disease to assess the efficacy of Tocopherol and Deprenyl.

NILO-PD

76 participants

A randomized, double-blind, placebo-controlled, phase 2 study to define the safety, tolerability, clinical and exploratory biological activity of the chronic administration of Nilotinib in participants with Parkinson's disease.

STEADY-PD3

336 participants

A phase 3, double-blind, placebo-controlled parallel group study of Isradipine as a disease modifying agent in participants with early Parkinson's disease.

NET-PD LS1

1,720 participants

Multi-center, double-blind, parallel group, placebo-controlled study of creatine in subjects with stably treated Parkinson's disease.

OTHER NEUROLOGICAL DISORDERS

HYPHOP

42 participants

FDA Approval August 2015

A randomized, controlled study of Acetazolamide vs. Dichlorphenamide vs. placebo in individuals with hyperkalemic and hypokalemic periodic paralysis.

FACOMS

1,300+ participants

FDA Approval February 2023

A multi-center natural history and clinical outcome measures study in Friedreich's Ataxia.

CCM

LONGITUDINAL

400+ participants

A 12-month large scale, international, online longitudinal natural history study in Cerebral Cavernous Malformations.

PRISM FM

1,044 participants

An international cross-sectional study to ascertain the symptoms and symptomatic themes most important to individuals with fibromyalgia.

PRISM ALS

497 participants

A cross-sectional study to ascertain the symptoms and symptomatic themes most important to adults with amyotrophic lateral sclerosis.

PRISM DMD

113 caregiver participants

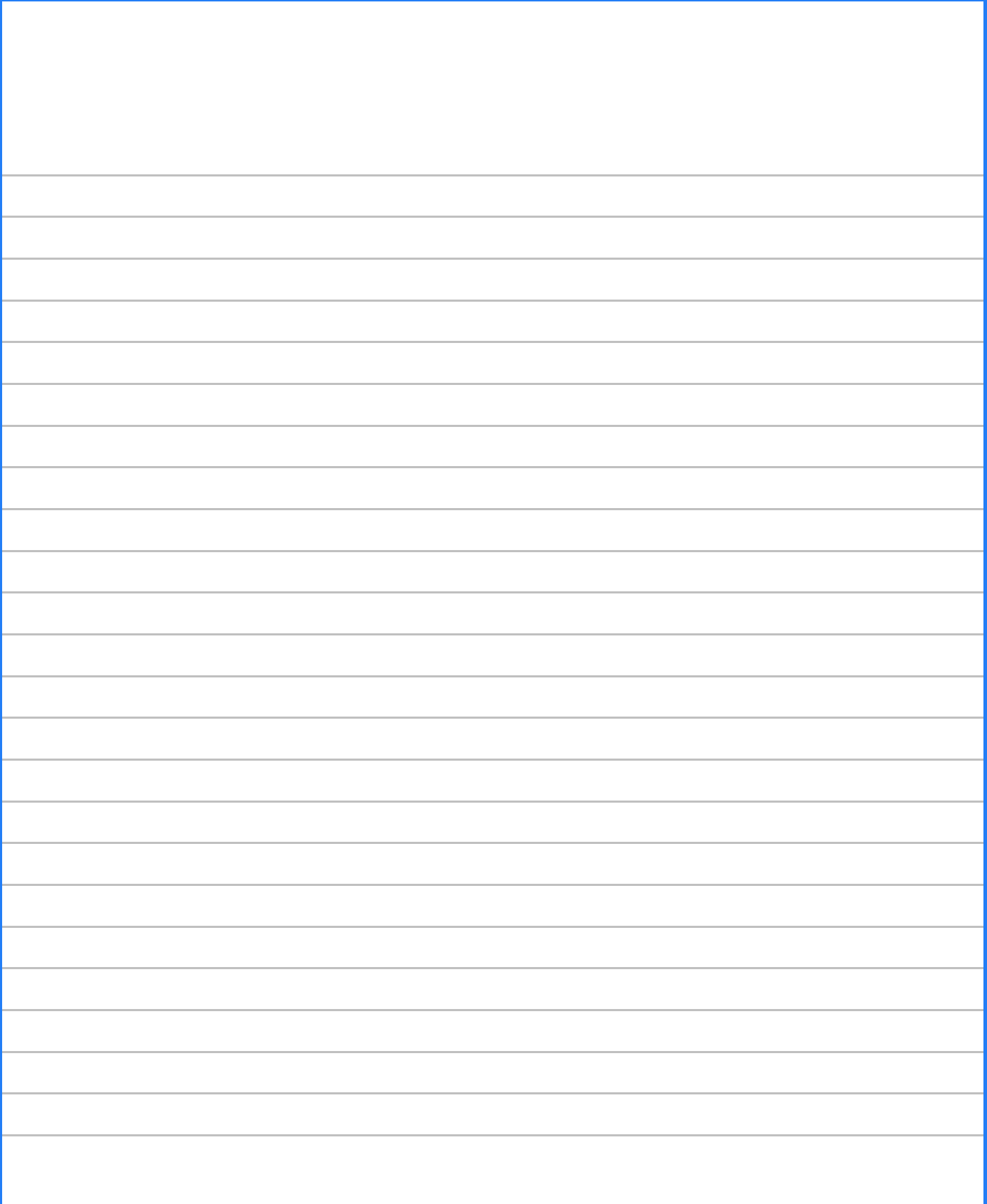
87 adult & minor participants

A cross-sectional study to ascertain the symptoms and symptomatic themes most important to adults and minors with duchenne muscular dystrophy and caregivers of individuals with duchenne muscular dystrophy.

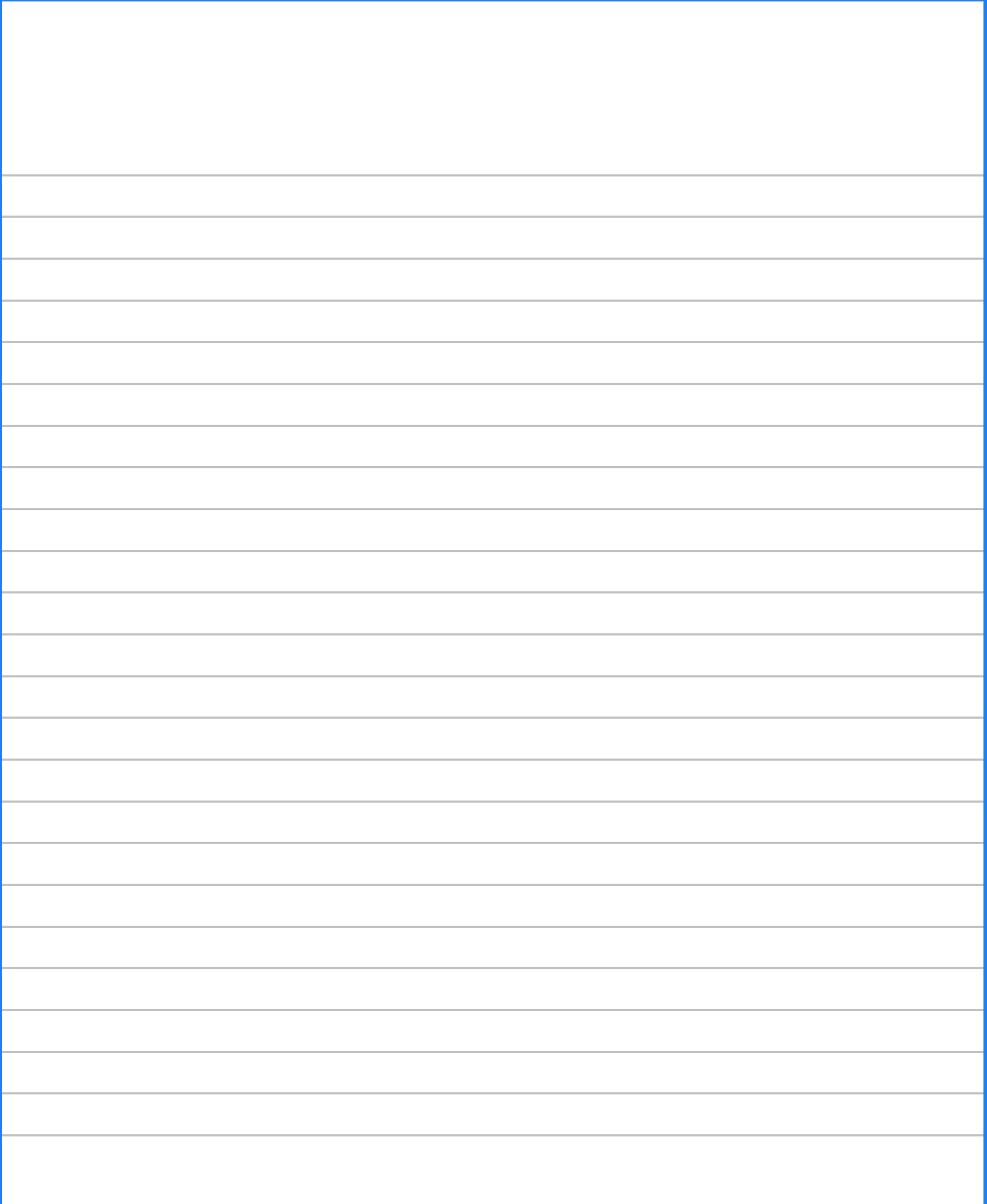
We have conducted additional clinical trials for other conditions, including dental caries, epilepsy, HIV, influenza, intracranial hypertension, stroke, and testicular cancer. Scan the QR code to view our Key Clinical Research on our website:



Notes

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Notes

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Advancing human
therapeutics, health,
and knowledge through
exceptional people,
skillful research, and
partnerships.

ADDRESS

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KEEP UP WITH US

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