

Advancing Human Therapeutics,
Health, and Knowledge

CHET

Center for Health + Technology

2024 Edition



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.

10
FDA
APPROVALS

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

CHeT Units

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CHeT - CTCC

Clinical Trials Coordination Center

DIRECTOR

Melissa Kostrzebski

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 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	<p>Valbenazine</p> <p>SPONSOR: Neurocrine Biosciences DISEASE: Chorea for Huntington's disease BRAND NAME: Ingrezza</p>	2023	<p>Omaveloxolone</p> <p>SPONSOR: Reata Pharmaceuticals DISEASE: Friedreich's Ataxia BRAND NAME: Skyclarys</p>
2017	<p>Deutetrabenazine</p> <p>SPONSOR: Teva Pharmaceuticals DISEASE: Huntington's disease BRAND NAME: Austedo</p>	2015	<p>Dichlorphenamide</p> <p>SPONSOR: Taro Pharma DISEASE: Primary Hypokalemic & Primary Hyperkalemic Periodic Paralysis BRAND NAME: Keveyis</p>
2008	<p>Tetrabenazine</p> <p>SPONSOR: Prestwick Pharmaceuticals DISEASE: Huntington's disease BRAND NAME: Xenazine</p>	2007	<p>Rotigotine</p> <p>SPONSOR: Schwarz Pharma DISEASE: Parkinson's disease BRAND NAME: Neupro</p>
2006	<p>Rasagiline</p> <p>SPONSOR: Teva Pharmaceuticals DISEASE: Parkinson's disease BRAND NAME: Azilect</p>	2006	<p>Selegiline</p> <p>SPONSOR: Somerset Pharmaceuticals DISEASE: Parkinson's disease BRAND NAME: Emsam</p>
2003	<p>Entacapone</p> <p>SPONSOR: Orion Corporation DISEASE: Parkinson's disease BRAND NAME: Comtan</p>	1997	<p>Pramipexole</p> <p>SPONSOR: Pharmacia & Upjohn DISEASE: Parkinson's disease BRAND NAME: Mirapex</p>

CHeT - CMSU

Clinical Materials Services Unit

EXECUTIVE DIRECTOR

Cornelia Kamp

SENIOR DIRECTOR

Eileen Fannon



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.

CMSU offers comprehensive clinical trial supply services including:

- + Secondary packaging and labeling of clinical trial materials (drugs and devices)
- + Package development, integrity, & performance testing
- + Label design and printing using ClinPro LBL™ (21 CFR part 11 compliant)
- + Storage options (room temperature and 2-8°C)
- + Clinical supply chain strategy & management
- + Secure environment
- + Returns management/destruction
- + 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

LEARN MORE



The Comprehensive Resource for Clinical Trial Materials.

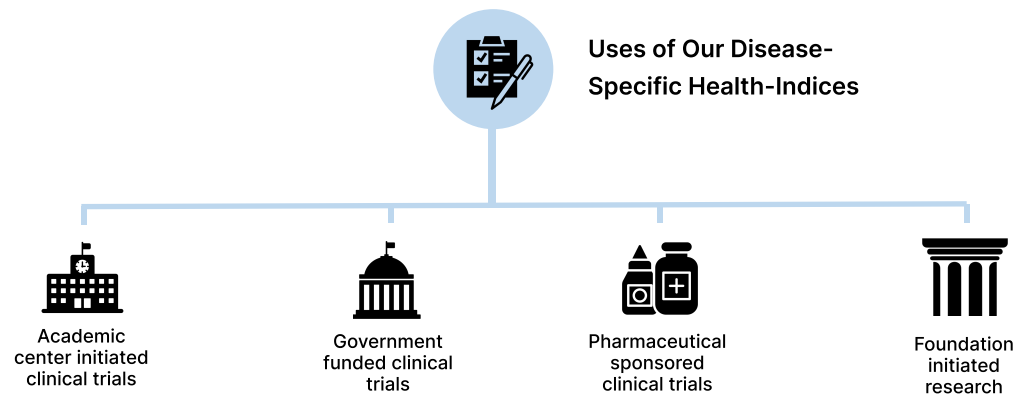
CHeT Outcomes

DIRECTOR

Chad Heatwole

The CHeT Outcomes team specializes in the development and validation of highly sensitive disease-specific, patient-reported and caregiver-reported outcome measures 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 capable of reliably measuring how a patient feels and functions, can reduce sample size requirements, are highly recommended by the NIH's common data elements initiative, and are designed to detect meaningful changes in health prior to traditional and generic outcome measures.

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



Our instruments measure the multifaceted, patient-perceived 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, including instruments for the following diseases:

- + Alzheimer's disease (AD)
- + Adrenomyeloneuropathy (AMN)
- + Amyotrophic lateral sclerosis (ALS)
- + Cerebral cavernous malformation (CCM)
- + Charcot Marie Tooth (CMT)
- + Crohn's disease (CD)
- + Duchenne muscular dystrophy (DMD)
- + Facioscapulohumeral muscular dystrophy (FSHD)
- + Spinal muscular atrophy (SMA)
- + Fibromyalgia (FM)
- + Friedreich's ataxia (FA)
- + Huntington's disease (HD)
- + Inclusion body myositis (IBM)
- + Lung cancer (LC)
- + Myotonic dystrophy Type 1 (DM-1)
- + Myotonic dystrophy Type 2 (DM-2)
- + Parkinson's disease (PD)
- + Spinal-bulbar muscular atrophy (SBMA)

LEARN MORE



CHeT Innovation

DIRECTOR

Jamie Adams

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 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 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 invisible 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

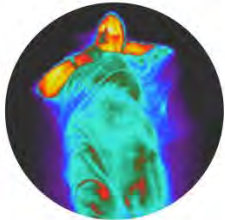
Evaluate the ability of sensors to assess features and progression of symptoms in early, untreated Parkinson's disease. Sensor assessments at home and in the clinic are compared to the traditional in-person assessments.

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



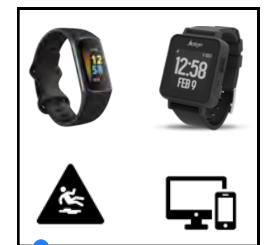
2023 CELESTE

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

ASSOCIATE DIRECTOR

Peggy Auinger

CHeT Analytics is developing strategies to reduce drug development costs and enhance clinical care for those living with neurodegenerative diseases. 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.



Access

To clinical trial and observational study data from over 100 studies



Extensive Knowledge

Of developing clinical disease progression models of neurodegenerative diseases

Existing Study Highlights for Collaborative Research

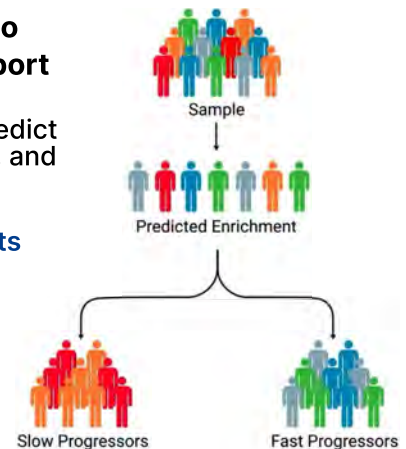
Parkinson's Disease (75 studies)
55 Interventional, 20 Observational

Huntington's Disease (37 studies)
31 Interventional, 6 Observational

Friedreich's Ataxia (6 studies)
HIV/AIDS (8 studies)

Utilize our expertise in statistical methodology 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.

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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](https://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 Kinsler

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.



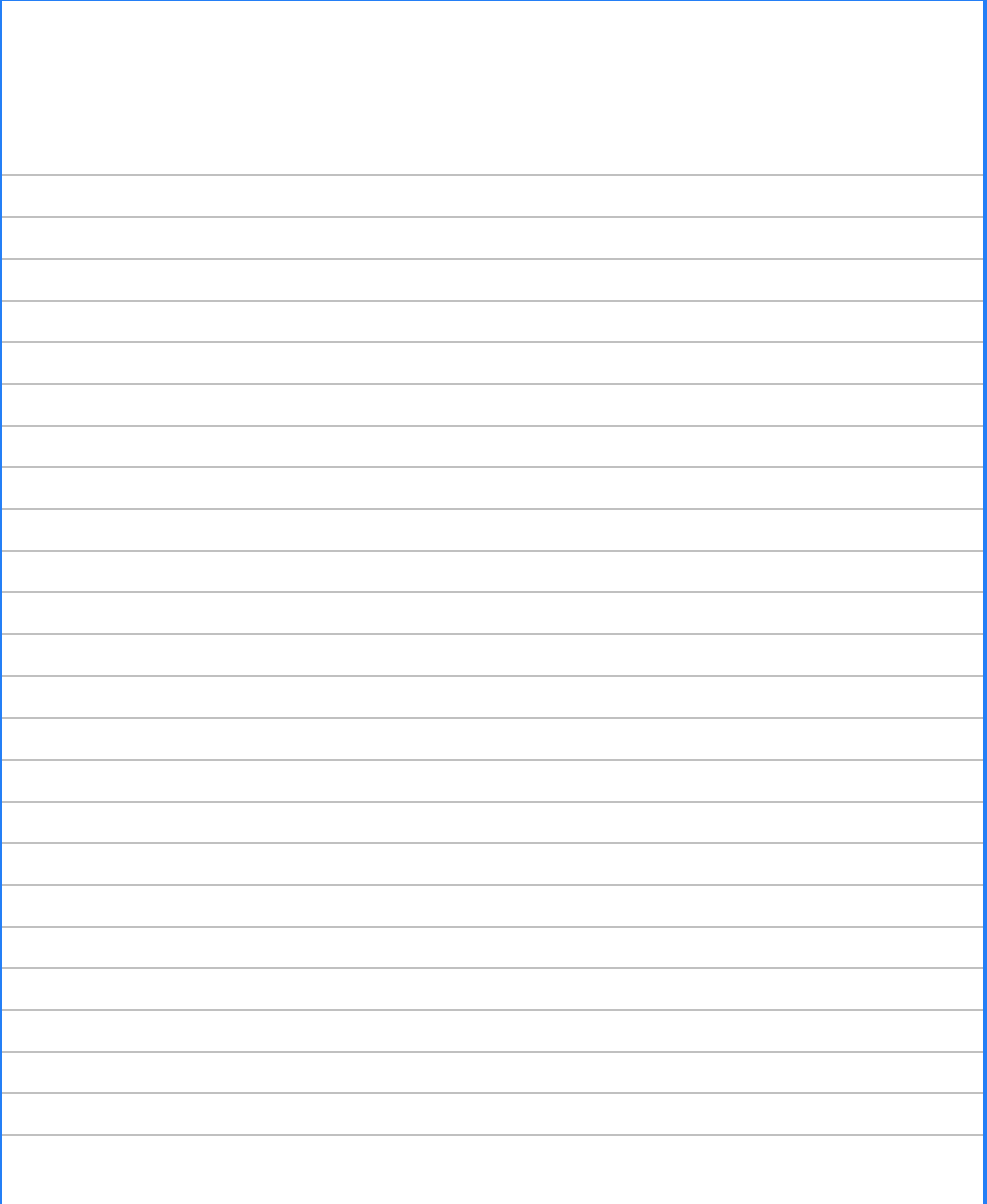
Improving the Health of our Communities

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



Notes

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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:



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.

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