Advancing Human Therapeutics,
Health, and Knowledge

CHeT

Center for Health + Technology

2023 Edition

WHAT DRIVES US

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

OUR STORY

135+
CLINICAL STUDIES

40,000+
RESEARCH PARTICIPANTS

7
FDA APPROVALS

The Center for Health + Technology 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.

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.

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

CHeT Units

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 135 clinical research studies with 50 sponsors (government, industry & private) that enrolled over 40,000 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, STDM, CDASH, 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 multi-center clinical research:

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

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.

7 FDA Approvals

Over the last two decades, CHeT has supported clinical trials that have led to seven FDA-approved treatments. These include first of their kind therapies for Parkinson's disease, Huntington's disease, and other neuromuscular disease.

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

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

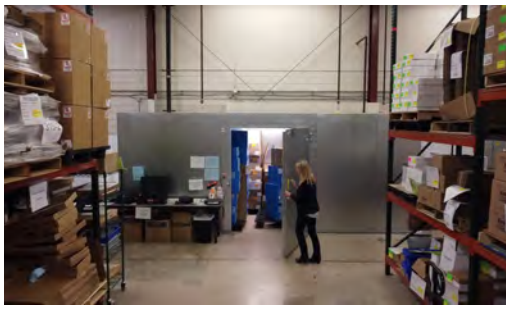
1997 **Pramipexole**
SPONSOR: Pharmacia & Upjohn
DISEASE: Parkinson's disease
BRAND NAME: Mirapex

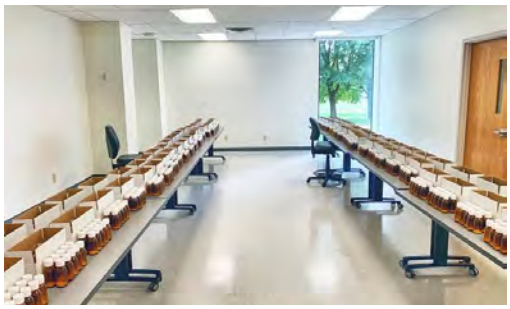
CHeT - CMSU

Clinical Materials Services Unit

EXECUTIVE DIRECTOR

Cornelia Kamp, MBA





The staff of CMSU have over 150 years of collective pharmaceutical experience and have serviced 15-20 multi-center studies concurrently, with 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 the drug/device supplies at Investigator Meetings
- Management of expiration/restest dates

The Comprehensive Resource for Clinical Trial Materials.

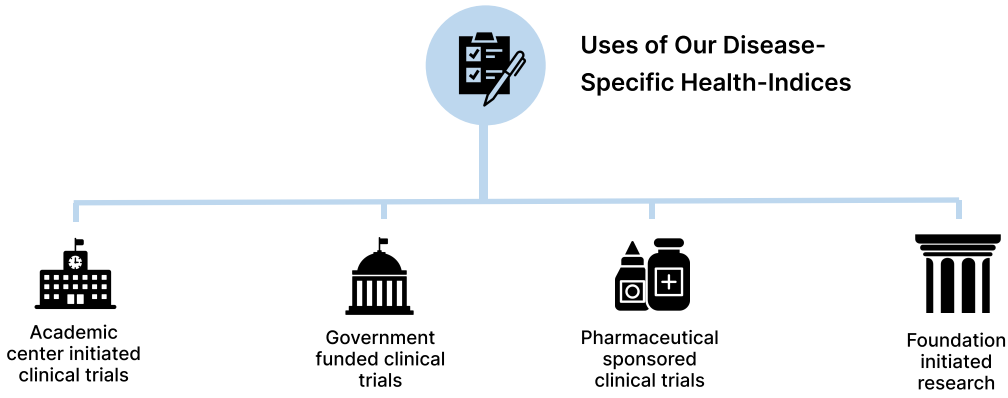
LEARN MORE

CHeT Outcomes

DIRECTOR

Chad Heatwole, MD, MS-CI

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 100 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

Ray Dorsey, MD, MBA

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

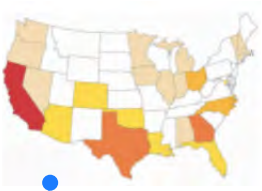
CHeT has pioneered the use of these new technologies for over a decade. CHeT has conducted a dozen studies with virtual visits that have reached more than 1500 participants throughout the country. We have recruited and retained a national cohort of clinical-trial ready participants in a longitudinal natural history study. In addition, CHeT has amassed a local and national registry of highly engaged participants known as project:brain health. You can visit projectbrain.org to learn more.

High Frequency Assessments

CHeT has also pioneered studies of smartphones, wearable, video analytics and invisible sensors that collect data in the clinic, the home, and the real world. 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.

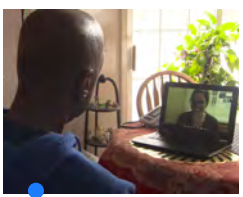
2012

1st virtual research study for Parkinson's disease



2014

1st virtual research study for Huntington's disease



mPower

2015

1st Apple ResearchKit app for a neurological disorder

2017 George

1st mobile app for Huntington's disease

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

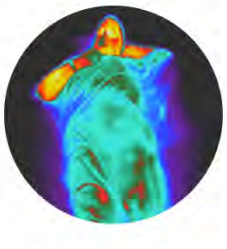
Evaluate the ability of sensors to assess features and progression of symptoms in early, untreated Parkinson's disease. Sensor assessments at home and in the clinic are compared to the traditional in-person assessments.

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

2018

1st longitudinal wearable sensor study for Huntington's disease

2019

1st bio-sensor used in Phase II drug trial for Huntington's disease

WATCH-PD

2020

1st longitudinal multi-site digital technology study in early PD

UR-Udall

2021

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

CHeT Analytics

DIRECTOR

Charles Venuto, PharmD

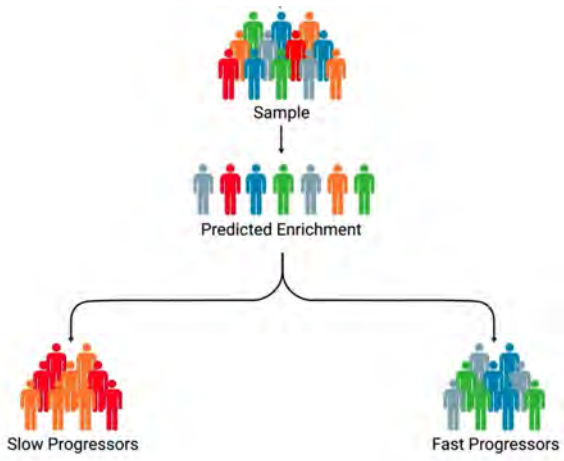
CHeT Analytics is developing strategies to reduce drug development costs and enhance clinical care for those living with neurodegenerative diseases. By leveraging one of the world's largest repositories of clinical trial data for Parkinson's and Huntington's diseases, new insights are being made possible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

Modeling capabilities for simulation of novel clincical trial design enrichment strategies in order to:

- Reduce trial sample size
- Truncate trial follow-up times
- Decrease drug development costs



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 exists, through post-hoc analyses.

Let us help you make the most of the data you have collected.

CHeT Health

DIRECTOR

Christine Zizzi, MPA

CHeT Health aims to advance evidence-based health policies to help all individuals, reduce health disparities, inform government regulatory agencies, and augment the involvement of diverse populations during therapeutic trials.

Generating Evidence

CHeT Health is exploring the role of health policy in shaping health outcomes and reducing health disparities through primary and secondary data.

Improving Diversity in Clinical Trials

CHeT Health is working to increase understanding of barriers to participation in clinical trials in underrepresented communities and to improve the representation of participants across CHeT clinical research studies.

Informing Action

CHeT Health is leveraging knowledge within and outside of CHeT in the areas of health policy, equity, and government regulation and regulatory science to inform meaningful change and advance health equity.

Key Clinical Trials

Sample out of 200+ studies

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 access the safety, tolerability, pharmacokinetics, and efficacy of Pepinemab. (VX15/2503)

PARKINSON'S DISEASE

DATATOP
800 participants

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.

PPMI
1,700 participants

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

NEUROMUSCULAR AND 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

1000+ participants

Friedreich's Ataxia Clinical Outcome Measure Study.

CCM LONGITUDINAL

400+ participants

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

PRISM FM

1044 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.

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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Professor, Department of Neurology

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Notes

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