

DO THE UNPRECEDENTED

Center for Health + Technology



UNIVERSITY *of* ROCHESTER

CHET

CENTER FOR HEALTH + TECHNOLOGY



WHAT DRIVES US

We want to enable **anyone, anywhere** to receive care and participate in research.

OUR STORY

130+

CLINICAL
STUDIES

40,000+

RESEARCH
PARTICIPANTS

7

FDA
APPROVALS

The Center for Health + Technology is a research organization within the University of Rochester Medical Center that designs and conducts multi-center clinical research studies and uses technology to make unprecedented advances. Over the last 2 decades, CHeT has re-shaped the conduct of clinical research and advanced knowledge to improve health for thousands, if not millions, of individuals. CHeT has supported over 130 clinical studies, enrolled over 40,000 research participants, and conducted pivotal trials leading to 7 FDA-approved treatments. CHeT collaborators span the globe and include leading academic institutions, pharmaceutical companies, technology firms, not-for-profit foundations, advocacy groups, and the federal government.

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 rare disorders.

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

Achievements

Over the past 10 years, CHeT has been involved in over 20 studies utilizing novel technologies including wearable sensors, smartphone technology, and telemedicine including:

2011

1st national randomized controlled trial of telemedicine for Parkinson's disease



2012

1st virtual research study for Parkinson's disease



2014

1st virtual research study for Huntington's disease



mPower

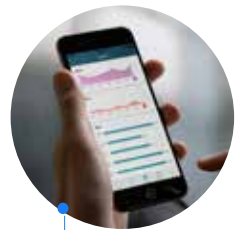
2015

1st Apple ResearchKit app for a neurological disorder



2016

1st to incorporate smartphone into a phase 3 clinical trial



COLLABORATORS



2017

1st mobile app for Huntington's disease



2018

1st longitudinal wearable sensor study for Huntington's disease



2019

1st bio-sensor use in Phase III drug trial for Huntington's disease



2020

1st fully remote multi-site clinical trial for PD using an interventional device



CHeT Units

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CHeT - CTCC

Clinical Trials Coordination Center

DIRECTOR

Cindy Casaceli,
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 130 clinical research studies with 45 sponsors (government, industry & private) that enrolled over 40,000 research participants in US, Canada, Europe, New Zealand and Australia.

Our clinical research expertise includes:

Novel and adaptive trial design

Study protocol development and training

Project, data, and site management

Contract facilitation and negotiation

Site selection based on key performance indicators

Remote, risk-based quality management, and on-site monitoring

Statistical analysis, modeling, and data mining

Data sharing and visualization

Investigational New Drug/Investigational Device Exemption support (over 25 submissions)

Remote sensor technology

Study communication via blogs, videos, and other social media

Accelerated enrollment and closeout

Neurologic and cognitive outcome measures

Data standards (CDISC, STDM, CDASH, CDE)

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

Over 200 credentialed investigators and coordinators

Direct web-based data entry and ePRO

Access to over 100 research study datasets

Data visualization tools and templates

Clinical Trial Management Systems (21 CFR part 11 compliant)

Over 60 SOPs and guidelines for audit readiness

CTCC study teams include:

Clinical project manager

Medical and site monitor

Database manager and programmer

Clinical data manager and information analyst

Biostatistician, data scientist, data visualization analyst

Administrative support

CTCC works to achieve study start-up, enrollment, database lock and regulatory submission at an accelerated pace.

Long-standing relationships with clinical sites

Direct to participant trials

Competitive site startup

Customized disease template documents

Experienced study teams with disease specific expertise

Central IRB experience

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 IND s, services to 56 drug and device multi-center clinical trials, and drug/device supplies to over 14,500 participants at more than 1,100 sites.

CMSU offers comprehensive clinical trial supply services including:

Secondary packaging and labeling of clinical trial materials (drugs and devices)

Distribution services/strategies (including cold chain)

Package development, integrity & performance testing

Label design and printing (21 CFR part 11 compliant)

Storage options (room temperature and 2-8°C)

Clinical supply chain strategy & management

Secure environment

Returns management/destruction

CMSU also provides project management support:

Kit design to align with dispensing visits

Creation of drug accountability logs and operations manual

Selection/auditing GMP-compliant contract manufacturing organizations

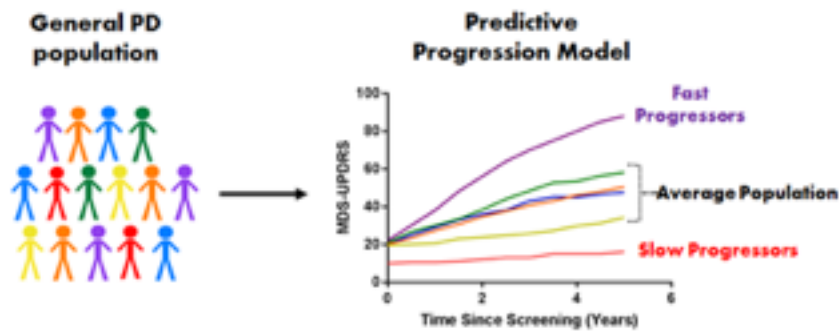
Management of expiration dates

CHeT Analytics

DIRECTOR

Charles Venuto, Pharm.D.

CHeT Analytics leverages one of the world’s largest repositories of clinical trial data for Parkinson’s and Huntington’s diseases to improve the efficiency and conduct of clinical trials. One early effort is to identify a targeted sub-population to delineate the effects of the drug versus the underlying condition.



In addition, CHeT Analytics is developing predictive models that forecast the rate of clinical progression in Parkinson’s disease using artificial intelligence. This information can be used to differentiate participants in clinical trials who are likely to be slow or fast progressors.



CHeT Analytics is working toward applying these disease models to clinical trial simulations. The hope is that investigators and sponsors will soon be able to predict the likely outcomes of clinical trials before they occur.

CHeT Innovation

DIRECTOR

Ray Dorsey,
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 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 of virtual visits that have reached more than 1500 participants throughout the country. In addition, we now use video visits to care for over 400 individuals with Parkinson’s disease throughout New York state who receive expert care without ever leaving their homes.

High frequency assessments

CHeT has also pioneered studies of smartphones, wearable, and invisible sensors. 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. These and ten other studies capture 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.

CHeT Outcomes

DIRECTOR

Chad Heatwole,
MD, MS-CI

CHeT Outcomes focuses on developing and validating disease-specific patient-reported outcome measures (PROMs) for use in clinical trials and FDA drug-labeling claims. Our group has developed 62 instruments, including short forms, and translated versions in 10 languages.

Over the last ten years, our PROMs have been extensively used in therapeutic trials worldwide, including those sponsored by academia, industry and government. As therapies are developed, outcome measures that can reliably and sensitively detect meaningful changes in disease burden over time are critical. The FDA has identified disease-specific PROMs as a valid, responsive, and potentially ideal mechanism to measure therapeutic change during clinical trials. Our lab develops these instruments from the ground up to fully satisfy all FDA criteria.

Our instruments

Our instruments measure the multifaceted, patient-perceived disease burden in individual diseases. Our team of epidemiologists, biostatisticians, qualitative researchers, patient advocates, linguists, computer programmers, and neurologists has developed disease-specific PROMs including instruments for the following diseases:

- Charcot-Marie-Tooth disease
- Congenital myotonic dystrophy
- Facioscapulohumeral muscular dystrophy
- Huntington's disease
- Myotonic dystrophy type-1
- Myotonic dystrophy type-2
- Spinal muscular atrophy

Key Clinical Trials

HUNTINGTON'S DISEASE

FIRST-HD

90 Participants

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

FDA Approval April 2017

TETRA-HD

72 Participants

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

FDA Approval August 2008

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

DATATOP

800 Participants

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

336 Participants

A Phase III 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.

OTHER RARE NEUROLOGICAL DISORDERS

HYPHOP

42 Participants

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

FDA Approval August 2015

FACOMS

1000+ Participants

Friedreich's Ataxia Clinical Outcome Measure Study.

STEADFAST

86 Participants

Randomized, placebo-controlled clinical trial evaluating the safety, pharmacokinetics, and efficacy of Actimmune in children and young adults with Friedreich's ataxia.

We have conducted additional clinical trials for other conditions, including dental caries, epilepsy, HIV, influenza, intracranial hypertension, stroke, and testicular cancer.



Novel Digital Studies

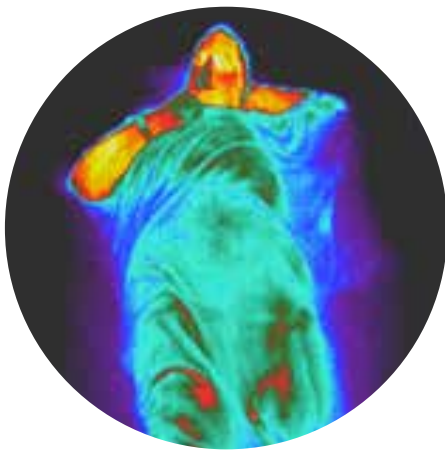


WATCH-PD

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 traditional in-person assessments.

132 Participants (82 PD, 50 Control)

Collaborators : Biogen, Takeda



Scratch and Sleep Quantification in Atopic Dermatitis (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 devices and sensors.

45 Participants

Collaborator : Pfizer



AT-HOME PD

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

220 Participants

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



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

Select Publications

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The Parkinson Study Group. Effects of tocopherol and deprenyl on the progression of disability in early Parkinson's disease. *New England Journal of Medicine* 1993; 328:176-183.

Let's do the
unprecedented
together.

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