

## **Clinical and Translational Science Institute at Children's National (CTSI-CN) v2.0**

The Clinical and Translational Science Institute at Children's National (CTSI-CN) was established in 2010 as a collaboration between Children's National Health System (CNHS) and its academic partner, The George Washington University (GW) with funding from the Clinical and Translational Science Award (CTSA) program of the National Center for Advancing Translational Sciences (NCATS) at the National Institutes of Health (NIH). The CTSI-CN is one of 64 CTSA-funded institutions across the U.S. and has the distinction of being the only child-health focused program. The strategic mission of this program is to promote: 1) high-quality research, 2) efficient translation of discoveries to human applications, 3) effective implementation into clinical practice, and 4) improved quality of life for children and their families locally and across the country.

In July 2016, the CTSI-CN v2.0 was funded through a second, five-year, \$24 million award from NCATS. CTSI-CN v2.0 provides highly integrated, cost-effective and investigator-focused resources in five key strategic areas that have been emphasized by NCATS to support effective clinical and translational research (CTR). These include: workforce development, collaboration and engagement, integration, methods and processes, and informatics.

The CTSI-CN provides highly integrated, cost-effective, investigator-focused resources designed to overcome research barriers, promote collaborative research, and provide research training with a special focus on children's health. With an emphasis on health disparities and childhood antecedents to adult diseases, CTSI-CN builds upon its pediatric research strengths in areas such as rare diseases, asthma, and neuro-developmental disabilities to collaborate with a national network of 1,200 community health centers.

The CTSI-CN provides members with access to: Education opportunities such as career development support, training opportunities, seminars, and symposia; Research resources that include free consultations for CTSI-CN services, reduced rates for NIH-sponsored and pilot research support; Collaboration opportunities to participate in interdisciplinary research and exposure to national opportunities available through CTSA institutions; Funding for pilot award recipients, voucher awards, and KL2 Scholars; Membership in the Association for Clinical and Translational Science; and information about current research, upcoming events, and grant opportunities through frequent e-digests, quarterly newsletters and websites.

CTSI-CN v2.0 is organized operationally into a set of modules (Figure 1) that are designed to: 1) support and empower basic, translational and clinical investigators to collaborate with one another and with community partners; 2) ease the administrative burdens of conducting research; and 3) break down traditional research barriers.

# CTSI-CN Framework

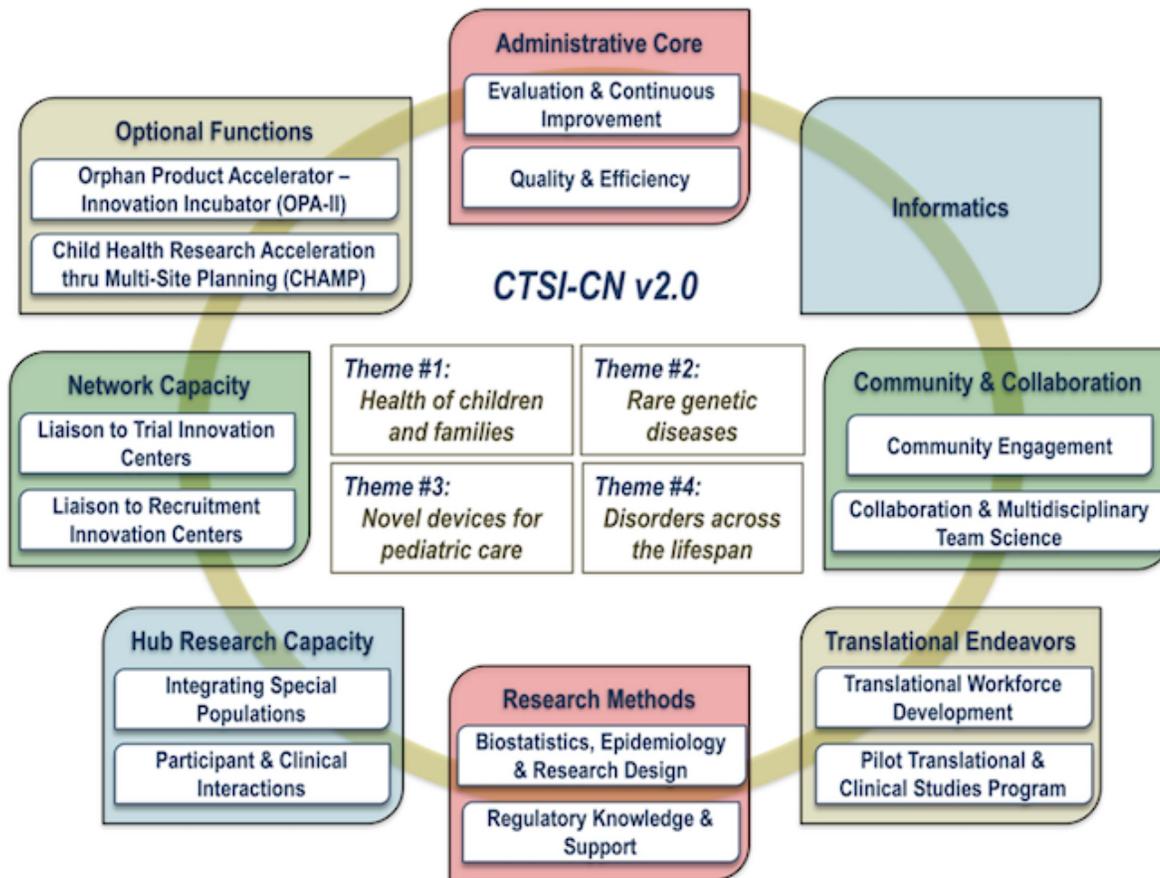


Figure 1

Cores' functions and organizing themes

## CTSI-CN RESOURCES

The mission of the CTSI-CN is to foster broad collaborative investigation that accelerates discovery and drives dialogue across the bench, bedside, and community continuum. Applicants are encouraged to consult with CTSI-CN modules, listed below, to access resources and further develop their proposals. More information can be found and contacts can be found at <http://ctsicn.org>.

**The Biostatistics, Epidemiology and Research Design (BERD)** provides high quality biostatistical and epidemiological expertise for the development and design of pediatric and lifespan CTR. All requestors can be provided with up to four hours of free support. Additional free support can be provided on a case-by-case basis especially for proposal development or for K-awardees. Where appropriate, GW Biostatistics Practicum graduate students provide additional data analysis needs to CTSI-CN investigators, and K awardees receive more support, should they require it. Consultations are coordinated through the CTSI-CN supported Services, Pricing, & Application for Research Centers (SPARC) portal.

The goal of the **Collaboration and Multidisciplinary Team Science (CMTS) module** is to foster collaborative research teams among the varied scientific and clinical disciplines and the broad community (e.g., lay public, patient advocacy groups, foundations, and industry). The strategy involves identifying, training, utilizing, and disseminating best practice in team science as applied to child health CTR. It also includes crediting each member of the team appropriately in recognition of his/her contribution.

The **Community Engagement module** focuses on two communities and their intersection in support of CTR: the lay public in the Washington, DC region and the multidisciplinary academic community of the CTSI-CN. In

a broad sense, community engagement starts at the early stages of a research project's development and continues through its completion and dissemination.

The **Grants Enhancement Program (GEP)** provides critical support for junior faculty in writing successful grant applications. The GEP offers the following services:

- Open Studios for senior faculty input on early project formulation;
- Specific aims and proposal review by senior faculty;
- Monthly meetings for peer review of and by K- and R-focused junior faculty, facilitated by senior faculty members;
- Access to materials such as successful grant applications and guidance documents; and
- By request, external expert review of well-prepared grant applications and mock study sections for multidisciplinary input.

The **Informatics Core** provides a comprehensive, integrated informatics ecosystem to investigators and their study teams by unifying bioinformatics and medical informatics and provides investigators and their teams with easy access to data and analytic tools required for current and future CTR needs. The Informatics Core also provides investigators and their teams with training in informatics methods and tools in order to promote self-sufficiency among researchers in the use of informatics across the enterprise.

The **Integrating Special Populations (ISP) module** provides support, resources, and innovative tools to assist investigators in including special populations in CTR projects. Special populations are defined as:

- Children from underserved populations (i.e., those experiencing health disparities);
- Fetuses and their mothers; and
- Children with rare genetic disorders.

The goal of the **Liaison to Trial Innovation Centers (LTIC) module** is to provide trial readiness and an efficient and effective environment to participate in multicenter studies through the CTSA TIC streamlined procedures for the implementation of multicenter research projects. The primary objective of LTIC is to facilitate the initiation and implementation of clinical studies in CTSI-CN, functioning as a liaison between CTR investigators and the planned CTSA TICs.

The **Liaison to Recruitment Innovation Centers (LRIC)** assists with maximizing the recruitment to pediatric and rare genetic diseases studies by using a variety of existing and developing informatics tools, educating users on their use, and reaching out to the community to maximize the buy-in of community stakeholders and their encouragement of their constituents about participation in CTR.

The **Orphan Product Accelerator – Innovations Incubator (OPA-II)** provides the infrastructure, assistance, and training for CTR investigators in the development of orphan products, specifically those aimed at the diagnosis and treatment of rare diseases, many of which are particularly relevant to children. The overarching goal of the OPA-II is to develop innovative methods for reducing both the cost and time required to bring orphan products to market.

The **Participant and Clinical Interactions (PCI) module** is a dedicated clinical research unit. The mission of the PCI is to provide a high-quality, safe, and welcoming environment for pediatric study participants and investigators. PCI encompasses a variety of resources and services divided into various components. Each component provides specialized services for investigators/research staff and their clinical research protocols. Services can be performed in the PCI or other hospital areas, both inpatient and outpatient. All PCI personnel have comprehensive training, including GCP, to perform each service/task with the highest level of efficiency, quality, and ethical standards. In addition, the PCI leadership and management team has many years of combined experience in clinical research in pediatrics and other vulnerable populations.

- ClinicalTrials.gov Support: In partnership with the IRB, PCI sends reminders to PIs to register and provide study results on ClinicalTrials.gov and in collaboration with the Informatics Core, ensures that the ClinicalTrials.gov NCT# is entered in our EHR for patients enrolled in research studies.
- Scientific Review Committee: The new Scientific Review Committee (SRC) pre-screens human research IRB submissions (i.e., pilot projects; clinical trials by K, T, or other trainees; and projects funded by foundation, small pharmaceutical, or biotech grants). The SRC exempts proposals with prior rigorous peer-review (e.g., NIH R, other federal awards) unless the associated human research protocol has not been reviewed

- **Bionutrition Services:** This service provides:
  - Caloric intake assessment;
  - Special meal design;
  - Nutrient analysis;
  - Anthropometric measurements using stadiometers, length board, knee height and Lange calipers, infant and standing weight scales;
  - Body composition assessments using air displacement plethysmography and/or bioelectrical impedance analysis; and
  - Energy expenditure and fitness studies utilizing a metabolic cart.

Up to 4-hours of free services are provided for preliminary data gathering with structured cost recovery built into subsequent grant budgets.

- **Neurobehavioral and Psychosocial Evaluation Core:** This Core function provides assessments of infants, children, adolescents, and adults, including cognition, behavior, social/environmental and family dynamics assessments, as well as consultation on behavioral phenotyping, selection of optimal assessment tools, and functional MRI applications for research on neurodevelopmental disorders.
- **Biorepository:** The CTSI-CN Biorepository provides expert assistance with:
  - Bio-specimen collection, processing (e.g., DNA and/or protein extraction), and storage for IRB-approved protocols; and
  - Data and sample management including the FreezerPro laboratory management system of over 10,000 samples.

In addition to maintaining this resource, the CTSI-CN Biorepository leverages existing expertise and resources to include a state-of-the-art HIV-related biorepository.

The **Pilot Translational and Clinical Studies Program (PTC) program** is an essential underpinning of a strong CTR program. Without the support to develop methods, test concepts, or establish feasibility, the successful conduct of definitive evaluative research is virtually impossible.

The primary goal of the **Regulatory Knowledge and Support (RKS) module** is to assist CTR investigators and their teams by providing proactive, innovative regulatory and research ethics education and support services to assure that child health CTR research meets the highest standards of ethical conduct and regulatory compliance.

The overall objective of the **Translational Workforce Development (TWD) module** is to provide workforce members with a flexible and continuous learning environment that will lead to high quality, efficient, and effective CTR. The major TWD initiatives focus on:

- Expanding the portfolio of on-demand training opportunities targeted at faculty and trainees, as well as staff and community members;
- Integrating a team science curriculum into our training and educational initiatives; and
- Focusing on team and leadership development within translational research teams.