CTSA Program Webinar

January 24, 2024

Agenda

TIME	TOPIC	PRESENTERS
2:00 PM ET	Welcome	Lauren Fitzharris, MPH PMP CCOS
2:01– 2:10 PM	NCATS/CTSA Updates	Mike Kurilla, MD, PhD NCATS
2:10-2:15 PM	CCOS Updates	Kerry James, MPH, PMP CCOS
2:15-2:30 PM	CTSA Working Group: CTSA Pharmacies and Compounding for Translational Research	Robert MacArthur, PharmD MS, Rockefeller University
2:30-3:00 PM	The Tenant Pilot	Josh Fessel, MD PhD Ken Gersing, MD NCATS
3:00 PM	Adjourn	

National Center for Advancing Translational Sciences

Appropriations: FY 2024

- NCATS is operating under a Continuing Resolution (CR) through March 8, 2024
  - Operating at the Fiscal Year (FY) 2023 enacted level

- House and Senate bills, neither of which have passed the full chambers, propose flat funding for NCATS

$ in millions	FY 2023	FY 2024		
	Enacted	President's Budget Request (Difference vs FY23 Enacted)	House Bill (Difference vs FY23 Enacted)	Senate Bill (Difference vs FY23 Enacted)
NCATS (total)	923.3	923.3 (0%)	923.3 (0%)	923.3 (0%)
- CTSA Programs	629.6	629.6 (0%)	629.6 (0%)	629.6 (0%)
- Other NCATS Programs	293.8	293.8 (0%)	293.8 (0%)	293.8 (0%)

NIH National Center for Advancing Translational Sciences

Incoming CTSA Steering Committee Members

Grace McComsey, MD
Case Western Reserve
University School of Medicine

Mimi Kim, ScD
Albert Einstein College of Medicine

Elizabeth Ofili, MD, MPH, FACC
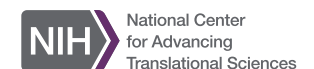Morehouse School of Medicine

Jennifer Kraschnewski, MD, MPH
Penn State College of Medicine

Frederick "Gerry" Moeller, MD
Virginia Commonwealth University

Vesna Garovic, MD, PhD
Mayo Clinic

2024 CTSA Pods

Mount Sinai School of Medicine
Pod Lead: Rosalind Wright

- University of Illinois
- Vanderbilt University
- Rockefeller University
- Northwester University
- Rutgers University

University of Texas- Galveston
Pod Lead: Randy Urban

- University of Michigan
- University of Texas Health Science Center at Houston
- University of Texas Health Science Center
- University of Kansas
- University of Arkansas

University of Cincinnati
Pod Lead: Jessica Kahn

- Indiana University- Purdue at Indianapolis
- University of Minnesota
- University of Wisconsin-Madison
- University of Florida
- University of Iowa

Virginia Commonwealth University
Pod Lead: Frederick "Gerry" Moeller *

- New York University School of Medicine
- University of Massachusetts Medical School Worcester
- University of Virginia
- Georgetown- Howard University
- Yale University

*New Steering Committee Member

2024 CTSA Pods

Einstein-Montefiore
Pod Lead: Mimi Kim *

- Harvard University
- Columbia University
- Washington University – St. Louis
- University of Rochester
- University of Chicago

Pennsylvania State University
Pod Lead: Jennifer Kraschnewski *

- Weill Cornell
- Johns Hopkins University
- University of Pittsburgh
- University of Buffalo
- University of Pennsylvania

Case Western
Pod Lead: Grace McComsey *

- University of Kentucky
- University of Alabama – Birmingham
- Boston University
- University of Texas – Southwestern

Mayo Clinic
Pod Lead: Vesna Garovic *

- Tufts University
- Ohio State University
- University of Southern California
- Medical College of Wisconsin
- University of California Irvine

*New Steering Committee Member

2024 CTSA Pods

University of California – Davis Pod Lead: Theodore Wun
University of New Mexico
University of California – San Francisco
University of Colorado
University of Utah
University of Washington

University of California –Los Angeles Pod Lead: Arleen Brown
Stanford University
Scripps Research Institute
University of California –San Diego
Oregon Health & Science University

Emory University Pod Lead: Elizabeth Ofili *
University of Miami
University of North Carolina – Chapel Hill
Duke University
Medical University of South Carolina
Wake Forest University

*New Steering Committee Member

CTSA CCIA NOSIs

- Limited Competition: Clinical and Translational Science Award (CTSA) Program: Collaborative and Innovative Acceleration Award (UG3/UH3 Clinical Trial Optional) (PAR-22-167)

  - Next receipt date: February 15, 2024

  - Technical Assistance Webinar: Slides here / Video here

  - Active NOSIs:

    - Notice of Special Interest (NOSI): Collaborative and Innovative Research to Advance Regulatory Science: Partnership of FDA Centers of Excellence in Regulatory Science and Innovation (CERSI) and NIH Clinical and Translational Science Award (CTSA) Programs (NOT-TR-24-006)

    - Notice of Special Interest (NOSI): Clinical and Translational Science Award (CTSA) Program: Collaborative and Innovative Acceleration Award (UG3/UH3) (Clinical Trial Optional) for Advancing Recruitment through Trial Innovation Network (AR-TIN) (NOT-TR-23-026)

  - Please direct all inquiries about the CCIA Notice of Funding Opportunity Announcement PAR-22-167 to ctsa_collaborativeinnovation@mail.nih.gov. The CCIA TA Webinar mailbox CCIAFOAQuestions@mail.nih.gov will be deactivated.

CTSA RC2 NOFO – Reissued

- Limited Competition: High Impact Specialized Innovation Programs in Clinical and Translational Science for UM1 CTSA Hub Awards (RC2 Clinical Trials Optional) (PAR-24-054)

    - Next receipt date: May 17, 2024

    - Technical Assistance Webinar: Slides here / Video here

- Changes include:

    1. The main change is that the NOFO now allows for Clinical Trials (**CT optional**).

    2. There are now **3 attachments** (instead of just 1):

        a. Attachment #1 (Up to 2 pages in length) called "UM1 and RC2 Coordination and Integration Plan".

        b. Attachment #2 (Up to 1 page in length) called "Program Milestones".

        c. Attachment #3 (Up to 2 pages in length) called "Program Evaluation and Sustainability Plan".

    3. Additional **sentences (in bold) in Section I**; include an **impact statement** in the research strategy; and **updated review criteria**.

- Contact: RC2NOFO@nih.gov

- FAQs: here (under RC2 Companion Application tab)

Surveillance, Epidemiology, and End Results (SEER)

National Cancer Institute's **Surveillance, Epidemiology, and End Results (SEER) data is now also available in N3C.** SEER is NCI's cancer surveillance registry and is the authoritative source of information on cancer incidence and survival in the United States.

What is a Cancer Registry?

About SEER

Program Overview   +

SEER Registries   −

   What is a Cancer Registry?   −

    Cancer Registry

    Data Collection, Storage, & Management

    Dissemination

    Analysis

    Research Application

Learn more about Cancer Registries by clicking on the different steps below!

1. Cancer Registry   2. Data Collection & Storage   3. Dissemination   4. Analysis   5. Research Application

A **cancer registry** is an information system designed for the collection, storage, and management of data on persons with cancer.

https://seer.cancer.gov/registries/cancer_registry/

Upcoming Dates to Remember

Next CTSA Program Webinar

February 28, 2024; 2-3 PM ET. Register here.

NCATS

COLLABORATE. INNOVATE. ACCELERATE.

🌐 ncats.nih.gov   🐦 @ncats_nih_gov   f @ncats.nih.gov   in NIH-NCATS

National Center
for Advancing
Translational Sciences

CCOS Updates

Kerry James, MPH PMP

CCOS Project Manager

SAVE THE DATE: Spring 2024 CTSA Program Groups Meeting

The Informatics EC meeting will be aligned with the AMIA Summit in March 2024 in Boston, MA

DATE: Friday & Saturday, April 5 & 6, 2024 (after ACTS)

LOCATION: Las Vegas, NV

❖ CCOS is working closely with the Association for Clinical and Translational Sciences (ACTS) to coordinate this meeting

See Spring Meeting Website for more details and to access the registration link

CCOS

Spring 2024 CTSA Program Groups Meeting Draft Agenda

Friday April 05, 2024

11:30 AM - 12:30 PM PDT	UL1/UM1 PI In-person Meeting
1:30 PM - 4:30 PM PDT	Communicators Group
1:30 PM - 6:00 PM PDT	CTSA Program Steering Committee Meeting

Saturday April 06, 2024

9:00 AM - 12:00 PM PDT	Integration Across the Lifespan Enterprise Committee
9:00 AM - 12:00 PM PDT	Workforce Development Enterprise Committee / KL2 Directors / TL1 Directors Group
9:00 AM - 12:00 PM PDT	Collaboration and Engagement Enterprise Committee
1:00 PM - 4:00 PM PDT	Diversity, Equity, Inclusion and Accessibility Enterprise Committee

Spring 2024 CTSA UL1/UM1 PI Meeting

- **Purpose of UL1 / UM1 PI meeting (1 hour)** is for PIs to engage in conversation with NCATS leadership about:
  - High level perspectives on translational science and challenges
  - Overarching themes that the CTSAs as a community / consortium would like to address over the next several years
- CCOS to reach out to UL1 / UM1 PIs to solicit input on agenda topics and planning needs in coming weeks – stay tuned
  - Please email steeringcmte@ccos.ctsa.io if you are interested in planning this 1-hour meeting

- *NOTE: This meeting will be in person only (no virtual option)*

Registration is Open

Learning Health Systems Collaborative Workshop

Co-sponsored by:
- **The Coordination, Communication, and Operations Support (CCOS) Center**
- **Wake Forest CTSI and Wake Forest University School of Medicine**

What's Our Focus?

Bringing together:

- CTSA Program representatives
- Researchers
- Clinicians
- Policymakers
- Healthcare innovators
- Opinion leaders
- Other healthcare professionals

Wednesday, May 22, 2024
8:00 am – 4:30 pm ET
In-Person Meeting

Sanger Heart & Vascular Institute
Kenilworth Medical Office Building 1
1237 Harding Place
Charlotte, NC 28204

Stay Updated on Collaborative Workshops!
Visit ccos-cc.ctsa.io/groups/collaborative-workshops
Email collaborative_workshop@ccos.ctsa.io

SCAN ME TO REGISTER

Why Attend?

This is an opportunity for CTSAs to:

- Form teams around LHS
- Learn about the ways the CTSAs have and can support LHS
- Research implementation science and operations to address translational science barriers

TEAM SCIENCE

National Center for Advancing Translational Sciences (NIH)

Wake Forest University School of Medicine

CTSI Your Partner In Research

CCOS Website Updates

- Recent Updates include:

  - Meeting materials and recordings from the **Fall 2023 CTSA Program Annual Meeting**

  - **Pod Submission Form** for Steering Committee Pod Leads and Pod **Submission archive**

  - **Collaborative Workshop** landing page and other links to the LHS Workshop in May

  - **CTSA Program Webinar** landing page with link to archived meetings and materials/recordings (live today!)

  - **Spring 2024 CTSA Program Group Meeting** details and registration (live today!)

  - **CTSA Program Group Guidance document** (live today!)

  - **Survey Strategy and Design Plan and Guidance** (live today!)

COORDINATION

Next Cycle for Working Group Proposals

Working Groups Proposal Submission portal for Cycle XII open on CCOS website during the month of **March 2024**

- The proposal submission period will open on **March 1st**
- All proposals should be submitted no later than **April 1st at 3pm ET**

NOTE: March 31st is a Sunday

| System Live March 1st | → | Proposals Due April 1st | → | SC Votes June | → | Earliest WG Start July 1st |
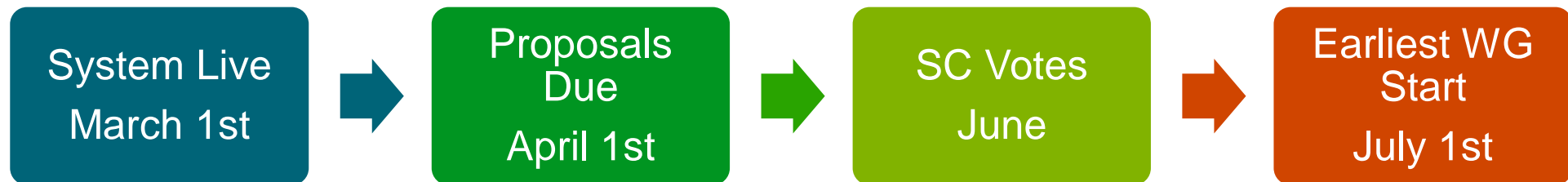
Working Group Proposals – Key Topic Areas

The CTSA Steering Committee is looking for Working Group proposals that align within the following key areas:

- Learning health and research systems
- Best practices for navigating the Science of Translation
- Causes of rising midlife mortality in America (Case & Deaton, PNAS, 2015)
- Climate change and health
- National training curricula in CTS
- Diversity, equity, inclusion, and accessibility
- Enhancing the impact of clinical trials
- Artificial Intelligence

Please visit the Working Group section of CCOS website for detailed outline of submission process, sample proposal form, and other guidance.

Want to stay up to date on the CTSA Program?

Follow this link or scan the QR code to join the CCOS All Communications Email List to receive CTSA Program communications and updates:

http://eepurl.com/iw9nZA

Scan to Receive CTSA-wide Communications

Remember to add communications@ccos.ctsa.io to your contacts list to prevent important CCOS emails from ending up in your spam folder!

CTSA Working Group: CTSA Pharmacies and Compounding for Translational Research

Robert MacArthur, PharmD, MS, BCSCP

Rockefeller University Hospital

Presentation Goals

- Overview of the CTSA Research Pharmacy Working Group
- Present initial data findings
  - Connect to FDA approved drugs to CTSA sites
  - Connect to FDA approved orphan drugs to CTSA sites
  - Describe drug products compounded for Ph1 and 2 studies
  - Update on survey
  - Update on SOPs
- Summarize next steps and deliverables

Background

- CTSA hospital pharmacies have long provided compounding services as part of routine patient care.

- Compounding is also used to support clinical trials, especially early phase studies.

- Research pharmacists and compounded products are occasionally cited in clinical trial publications and in clinicaltrials.gov

- This project brings together Research Pharmacists from multiple CTSAs, to document their contributions to institutional research

Project Goals

- Identify FDA approved products, including orphan drug products, that utilized CTSA research pharmacy compounding services.

- Provide examples of compounded research products that have supported clinical and translational studies.

- Develop and administer a survey to CTSA Research Pharmacists to capture scope of practice.

- Develop a unified Research Pharmacy SOP list for sharing among sites, training, and publication,

Phase 1 or 2 Studies at the 10 CTSA Sites

Study Site	Study Phase		Totals
	1	2	
Columbia	61	81	142
Duke Univ	181	124	305
Hopkins	207	181	388
Mayo	322	223	545
RW Johnson	2	3	5
Rockefeller	23	3	26
Univ Alabama	51	50	101
Univ Cal Davis	11	7	18
Univ Penn	192	141	333
Total	1050	813	1863

Among the 10 CTSA sites there are 1863 phase 1 or 2 clinical trials listed in clinicaltrials.gov

Some sites perform more phase 1 and 2 studies than others, so the project may expand to include phase 3 and 4 research pharmacy services to capture those efforts

*Clinicaltrials.gov

January 25, 2024

Example: CTSA Ph 1/2 Studies That Preceded FDA Product Approval

NCT Number	Study Phase	FDA Approved Drug	Study Start Date	FDA Approval Date	Years Ph1 to Approval
NCT00000189	2	GEPIRONE	01JAN1990	22SEP2023	33.75
NCT00002239	2	EFAVIRENZ	01MAY1999	17FEB2016	16.81
NCT01236638	2	MOMELOTINIB	01NOV2010	15SEP2023	12.88
NCT00002239	2	ENFUVIRTIDE	01MAY1999	13MAR2003	3.87
NCT02052791	1	NUSINERSEN	01JAN2014	23DEC2016	2.98

Example: Orphan Products Approved After CTSA Phase 1/2 Studies

Study Phase	Drug	CTSA Location(s)	Approved Orphan Indication
2	MOMELOTINIB	Mayo and Multisite	Treatment of myelofibrosis
1	NUSINERSEN	Columbia and Multisite	Treatment of spinal muscular atrophy

Example: Approved High Profile Product that followed CTSA Phase 1/2 Studies

A Study of Tirzepatide in Overweight and Very Overweight Participants

Study Phase	Drug	CTSA Location(s)	Approved Indication
1	TIRZEPATIDE (Mounjaro)	Hopkins and Multisite	GLP-1 receptor agonist indicated as an adjunct to diet and exercise to improve glycemic control in adults with type 2 diabetes mellitus.

Early Phase Dosage Forms

Type	Dosage Form
Oral, Non-Sterile	API powder in capsule or bottle API liquid in capsule Aqueous solutions and suspensions Formulated capsules
Injection or Other, Sterile	Prefilled syringes Vials + syringes Small volume infusions
Inhaled, Sterile	Vial + nebulizer (Mesh or Jet) Sterile powder + powder inhaler

Dosage forms commonly compounded for early phase studies, a time when the final marketed dosage form is not yet available

Via survey and literature review, pharmacists will describe research drug compounding performed at their site to support clinical trials

THE ROCKEFELLER UNIVERSITY HOSPITAL

CENTER FOR CLINICAL AND TRANSLATIONAL SCIENCE

No. of Journal Articles Connected to CTSA Phase 1 and 2 Studies

Site	No. Full Text References
Columbia	73
Duke	77
Emory	44
Howard	24
Mayo	84
Penn	52
Rockefeller	53
RW Johnson	3
Univ Alabama	13
Total	423

423 publications identified which are connected to the 10 CTSA sites and their combined 1863 phase 1 and 2 studies

Where possible, articles will be used to document the pharmacy compounded products administered during the study

If pharmacy not mentioned that will also be noted

Survey and SOP Lists

Survey was reviewed and edited by all collaborating pharmacists and has been submitted to CCOS for finalization

Survey will be used to describe Research Pharmacy services provided by each CTSA site, in detail

CCOS has developed submission portal for SOP list upload from each collaborating pharmacist and site

Deliverables

A publication that includes the survey results and documented connections between compounded products, early phase CTSA site studies, and, where possible, FDA approved and orphan drug products

A publication that combines the SOP lists from all CTSA Research Pharmacies

A webinar, "Investigational Drug Compounding for Translational Research"

Please enter questions for Dr. MacArthur in the Q&A box in Zoom

Robert MacArthur, PharmD, MS, BCSCP

Rockefeller University Hospital

rmacarthur@rockefeller.edu

Quick Overview for Today

- First 2 phases of the Tenant Pilot

- What we're hearing and some shorter term priorities

- A closer look at the Tenant Pilot model

- Focusing on the education pieces

But first...addressing some misconceptions...

- Data in the National COVID Cohort Collaborative (N3C) **are not** being used for any non-COVID related research, cannot be used for any purposes not authorized by fully executed agreements, and have not/cannot be moved to any other enclave.

- If you have any questions: please contact NCATS. If you hear something regarding N3C or any other NCATS-supported program from another source, please confirm or clarify with NCATS before taking action.

  - Contact Ken Gersing – kenneth.gersing@nih.gov

What *Is* Happening with Infrastructure?

No N3C COVID data were used in the tenant pilots

Clinical Tenant Pilots with New Data and Functionality: *Can the N3C infrastructure be used and built upon to study diseases other than COVID? What would that look like?*

Stage 1: Assess efficient, scalable governance and data contribution processes supporting multiple research areas

- 12 organizations volunteered to **sign new agreements** to participate in 3 phenotype-focused tenants (dementia, renal disease, COPD)

- Goals achieved: Development and execution of non-COVID data transfer and data use agreements, data acquisition and harmonization, demonstrate ability to analyze data

What *Is* Happening with Infrastructure? (cont.)

Stage 2: Extend assessment to other data structures and additional potential uses

- Create a shared collaborative data science educational resource for training.

  - Educational Tenant: Partnership between NCATS & AIM AHEAD (NIH ODSS-supported)

- Test feasibility of linking data from multiple sources to generate a more comprehensive data environment and assess more complex funding arrangements

  - Cancer Tenant: Partnership between NCATS & NCI – Supported by NCI, ASPE, FDA

  - Renal Tenant: PENDING FUNDING FROM NON-NCATS PARTNERS

    - Partnership between NCATS & NIDDK, SRTR (Scientific Registry of Transplant Recipients), HRSA (Health Resources and Services Administration), AIM AHEAD – Supported by ASPE, HRSA, NIDDK, NIH ODSS

What We've Heard

- Ongoing discussions with CTSAs are needed - we are partners!
  - ✓ Formation of RWD Task Force
  - ✓ Fall Program Meeting
  - • ECs/Pods/WGs/etc.

- Crowdsourcing a Data Science Glossary and a set of practically-minded FAQs

- Training/retraining in data science is a high priority
  - • Data science education tenant pilot is a direct response, with more to do

- Wider access to the data science expertise within the CTSAs and community engagement are high priorities

Next Steps

- Immediate next steps
  - Completing tenant pilot stage 2
  - RWD Task Force will be working with the Pods to get Hub-level examples of successes and challenges encountered
  - NCATS will be working with CCOS to create a data science "one stop shop" on the CTSA website
- Immediate opportunity
  - Extend access to existing COVID data in N3C?

Transition to Ken

N3C Announcements:
Clinical Tenants Pilot Phase II, Education, New Resources

N3C Extending Access

- N3C, per existing agreements, will stop accepting new Data Use Requests on **4/30/2024**
- Per the existing agreements, N3C data will be archived **05/01/2025**

New Resources and Linked Data

- SEER Cancer Registry Data,
- CMS expanded to all linked patients

New Resources

- LLM/N3C Assist, Jupyter, R Studio, LLM/N3C Assist, Protocol Pad

In the news

- N3C team Center for Linkage and Acquisition of Data (CLAD)
- Charisse Madlock-Brown, PhD Iowa U.S. Senate Committee on Health, Education, Labor and Pensions Long COVID
- President Biden signed Executive Order 14110 NSF and partners kick off the National Artificial Intelligence Research Resource Pilot Program
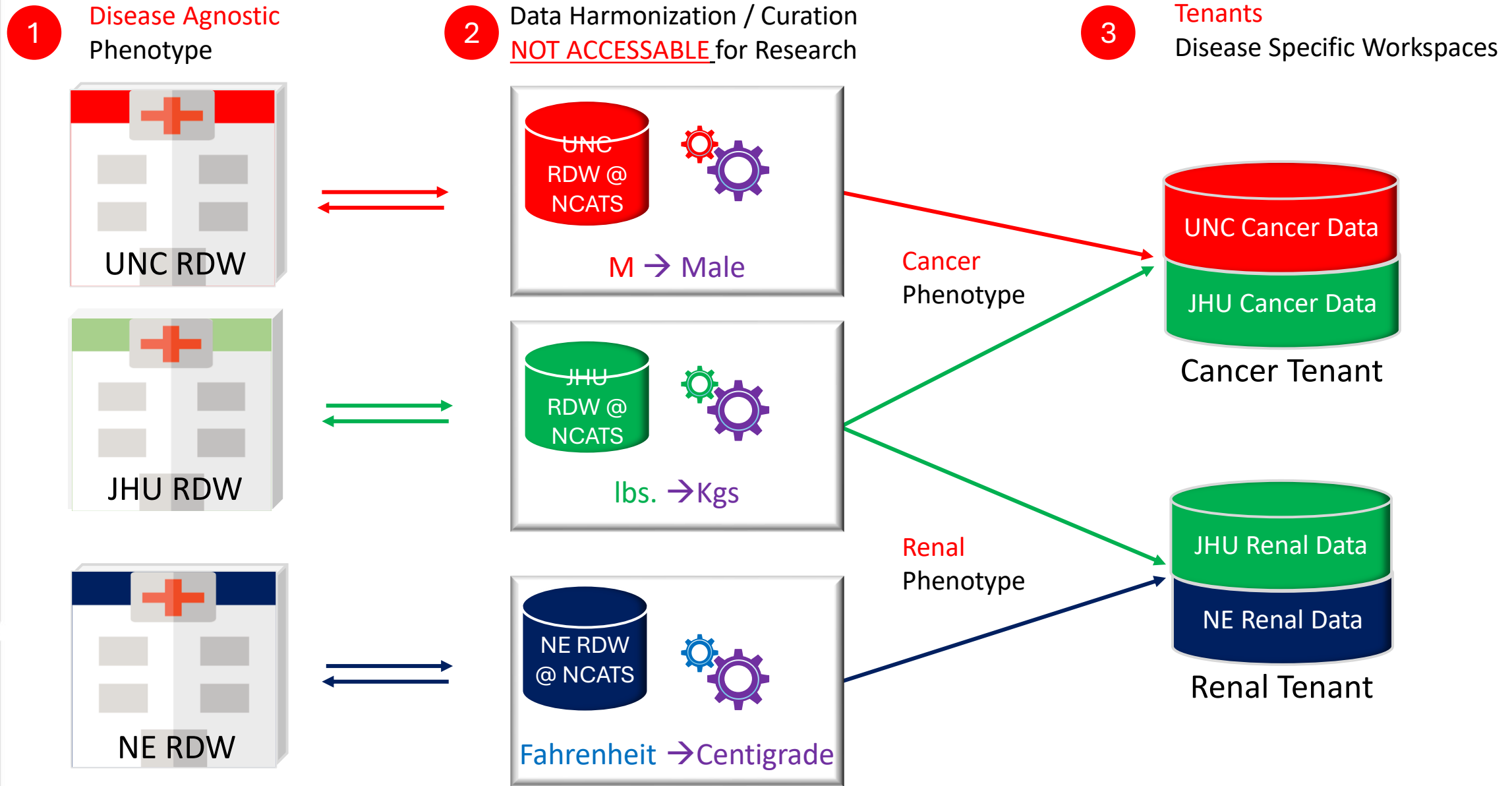
N3C Clinical Tenant Pilot Phase II

	Education & Training	*Renal (pending)	Cancer
Entities	NIH ODSS AIM-AHEAD NCATS, CTSA, CTR, N3C	NIDDK, ODSS, ASPE, SRTR/HRSA, CTR, CTSA, Rob Starr @ NIDDK	NCI, ASPE, CTSA, FDA, CTR Gurvaneet Randhawa @ NCI
Objective	Comprehensive Virtual Research Environment provides open access for collaborative analytics environment for educators across the CTSA to teach, develop, and share educational material on data science.	Research • Chronic Kidney Disease; • End Stage Renal Disease & • Transplant outcomes and bias Test feasibility of "Freeport" an Inter HHS Agency data sharing enclave.	Research • ID Pre-Cancer Biomarkers • Long-term Cancer Outcome Test feasibility of linking data from multiple sources to generate a comprehensive data enclave.
Data	Synthetic data sets (Level I)	De-identified Level II EHR data from CTSAs; SRTR Transplant registry; *CMS	De-identified Level II EHR data from CTSAs; SEER; *CMS.
Status	1st Quarter	*ASPE in negotiations (2nd/3rd Quarter)	2nd Quarter
Access	Open Access (CTSA, CTR, AIM AHEAD (HBCU)	Limited Access based on resources available	Limited Access based on resources available

*In negotiations

Educational Tenant for Data Science
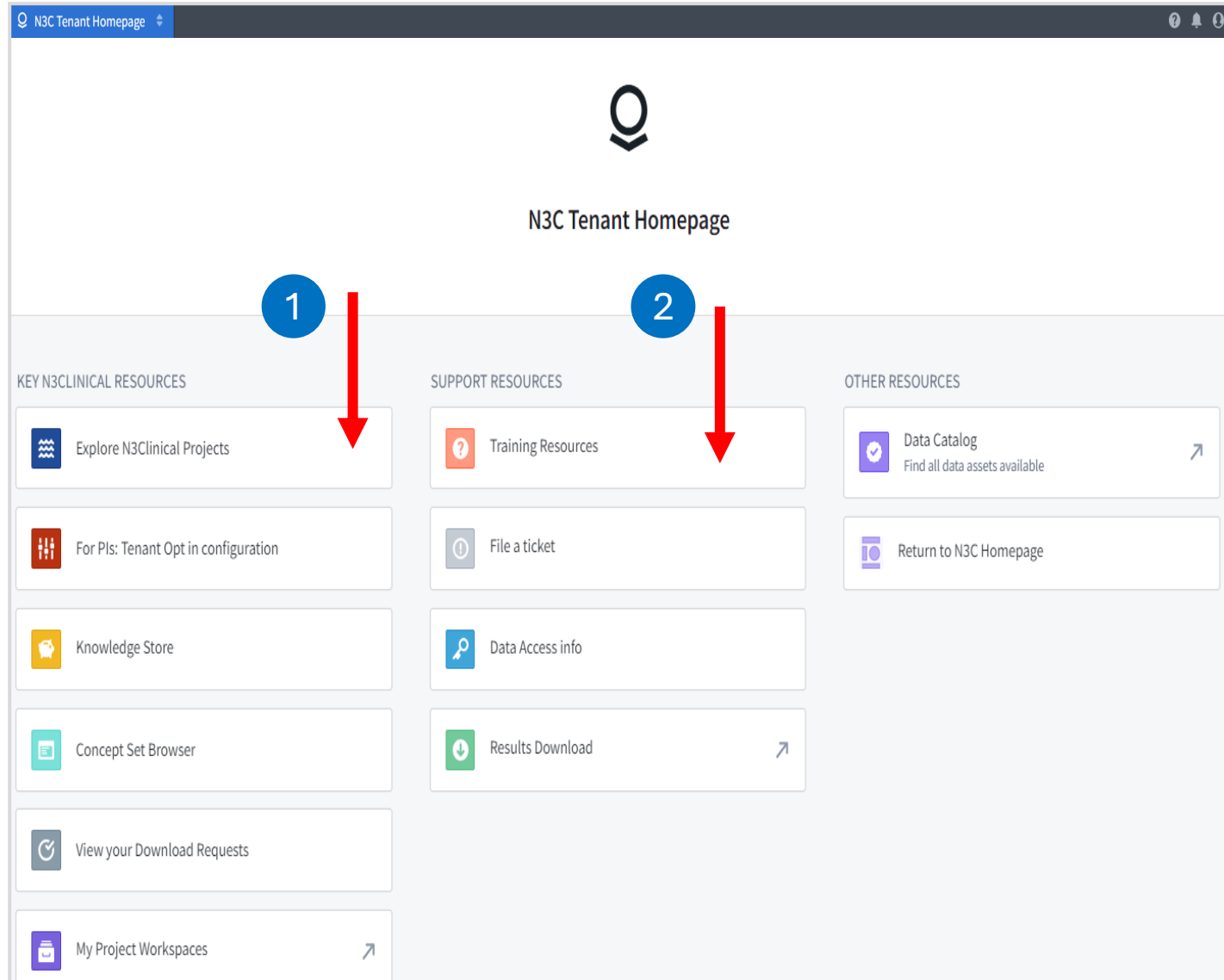
Partnership with AIM AHEAD

- ADVANCED DATA ANALYSIS USING NCATS DATA AND THE N3C DATA ENCLAVE
  - Shared education material
  - Jointly development with NCATS, NIGMS, AIM AHEAD
  - New DATA and DUA

Resources Available

- Collaborate Analytics, Tools (Code Workbooks), Educational, Tools Code Workbook, R, JIRA, LLM, AI/ML Resources

Data Available - Synthetic

Chao Pang (Columbia) Synthetic Data Open-Source Generation

Andrew Williams Tufts – Help with bringing OHDSI tool stack to N3C and Synthetic Data for Education

Educational Resource for Data Science (AIM-AHEAD, NCATS, NIGM...

Lectures

Support - Office Hours

Training Datasets Interactive

Educational Material

Student Evaluation

Course Schedule

Course Website	See AIM-AHEAD Connect > Courses
Class Sessions	Tuesdays 6:30 – 8:00 PM Eastern Time See AIM-AHEAD Connect > Calendar for videoconference (Zoom) link
Office Hours	Thursdays 1:00 - 2:00 PM Eastern Time See N3C Data Enclave for videoconference (Zoom) link

Modules	Topics
Module 1	**Overview of translational research & the National COVID Cohort Collaboration (N3C)**
Module 2	**Translational research using the N3C**: understanding the real-world data life cycle and project life cycle
Module 3	**Ethics & equity** when using artificial intelligence, machine learning, and real-world data to improve health and health care
Module 4	**Understanding the vocabulary** of electronic health records and real-world evidence: OMOP, OHDSI, & Concept sets
Module 5	**Governance, ethics, and use**: an example from the N3C
Module 6	**Getting started in the N3C Data Enclave**
Module 7	**Data access & management**
Module 8	**Analysis tools I**: Contour & Code Workbooks
Module 9	**Analysis tools II**: OMOP, Concept Sets, Filtering
Module 10	**Analysis tools III**: Knowledge Store, Logic Liaison templates, and beyond OMOP
Module 11	**Best practices for effective research**: a practical demonstration
Module 12	**Developing testable hypotheses & research questions**

Team Science Domain Teams

Getting Access to the Educational Tenant

First Time Users

Register at N3C

HSP / Security Training

Sign DUA

Returning Users
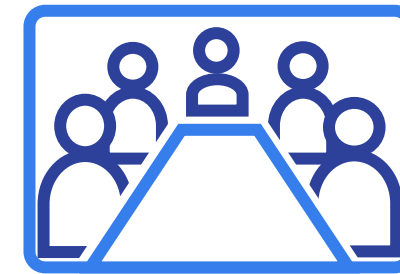
Submit Proposal

Reviewed

Investigator Notified

Please enter questions for Dr. Fessel and Dr. Gersing in the Q&A box in Zoom

Thank you!

Next webinar is **February 28, 2024;** 2-3 PM ET.