

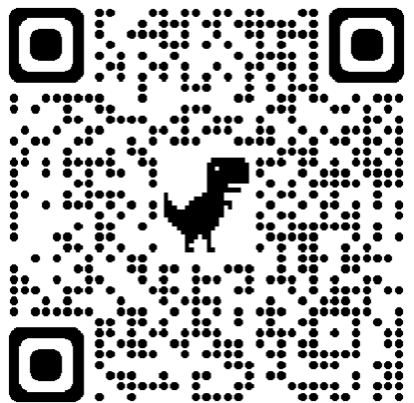
Research at the Intersection of Translational Science and Nursing Research

Joni L. Rutter, PhD

Director

National Center for Advancing Translational Sciences

*National Institute for Nursing Research, Advisory Council
September 12, 2023*



The Public Health Challenge

10,000

Diseases



and only

5%

Have
Treatments
or Cures



Time from early development to the medicine cabinet takes 10-15 years.

9

out of

10

Promising therapeutic candidates that enter clinical trials fail.





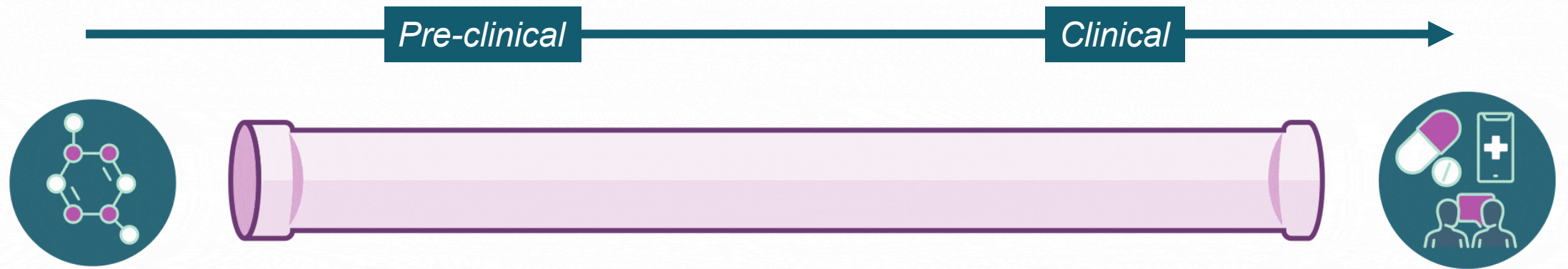
NCATS' MISSION

**Turn research observations
into health solutions through
translational science**



NCATS is Re-engineering the Translational Pipeline

NCATS is advancing translational science by addressing long-standing bottlenecks in the translational pipeline so that new treatments reach people faster.



Examples of bottlenecks

• And solutions

Operational

“One size fits all” approach

- **Adaptive clinical trial design, master protocols**
- **N of small CTs, RDCRN basket/umbrella trials**

Low enrollment and diversity in clinical trials

- **Patient Advisory Groups, Enhanced community engagement efforts (TIN)**

Administrative/Workforce Dev

Administrative burden for study start-up

- **Streamlined business and regulatory processes (SMART IRB)**

Shortage of qualified translational investigators

- **Training and career development best practices (CTSA K, T, R25, R03, DPI)**

Scientific

Insufficient tools and technologies to predict toxicity and efficacy of new drugs

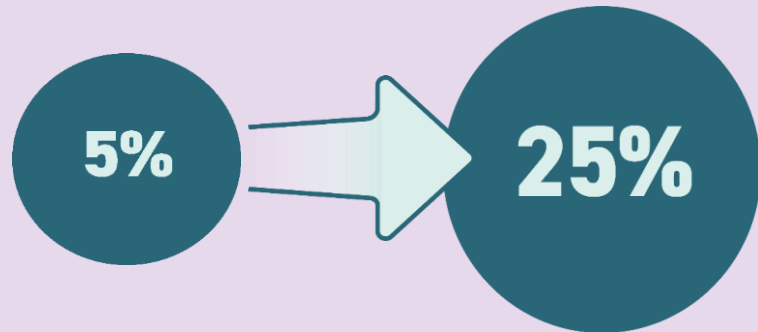
- **Platform-based Tissue/Organ on chips; 3D biofabrication**
- **Gene targeted therapies**
- **AI/ML drug development (ASPIRE)**

Incompatible databases to advance data science

- **Data, interoperability and integration (Translator, N3C, GARD, RARESource)**

NCATS Vision: Three Audacious Goals

More Treatments



**Five-Fold Increase
in Number of Diseases
with Treatments**

All People



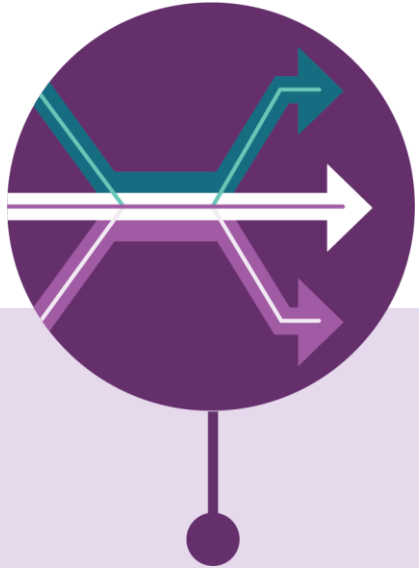
**Dramatically Increase
Inclusivity Across Every
Area We Support**

More Quickly



**Enable Diagnostics and
Therapeutics to Reach
People Twice as Fast**

Key NCATS Approaches



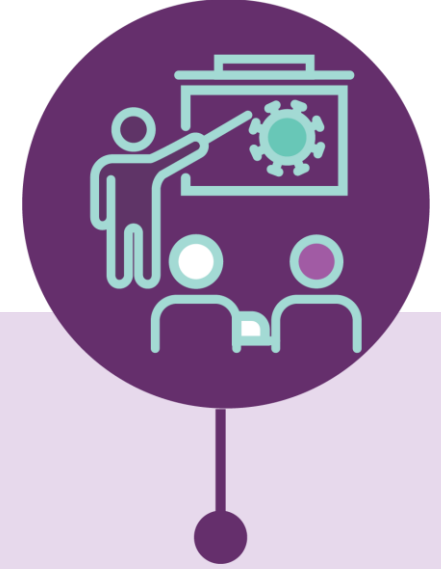
Understanding what's similar across diseases to spur multiple treatments at a time



Developing models that better predict a person's reaction to a treatment

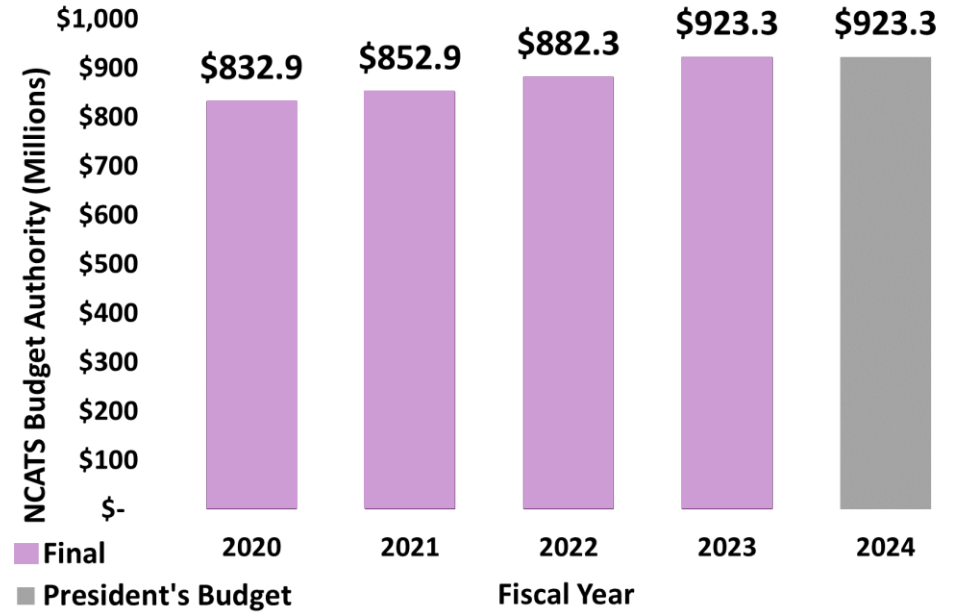
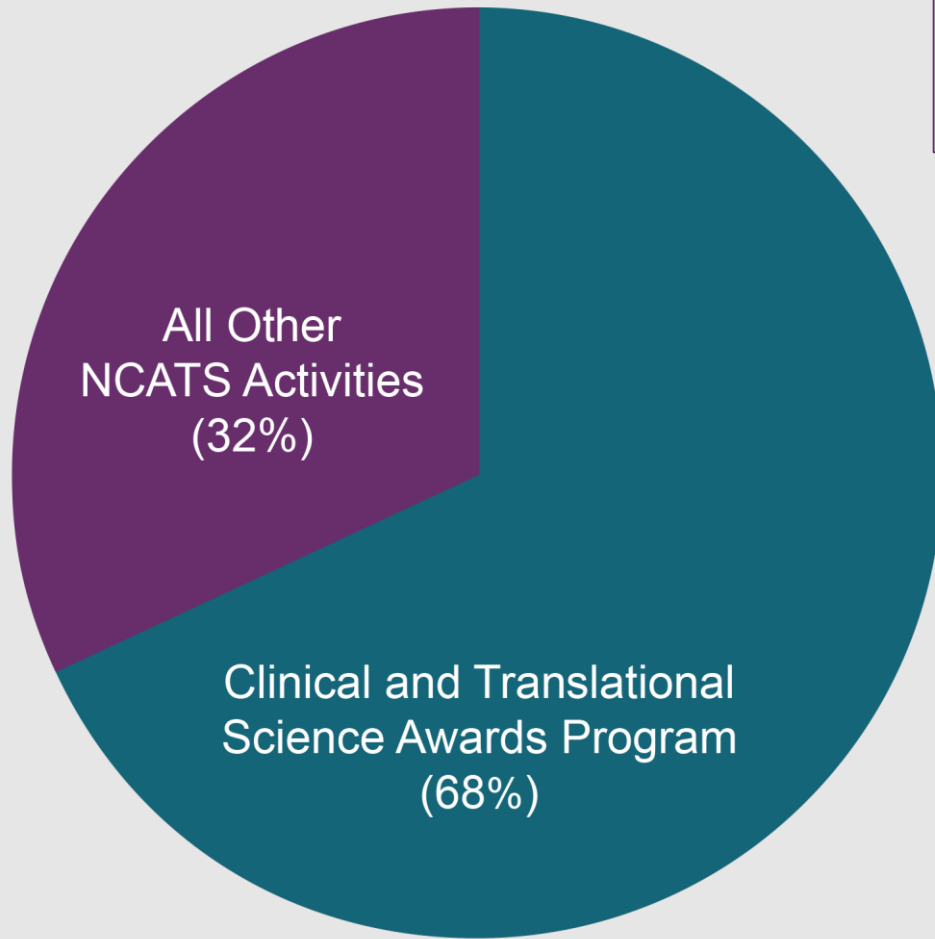


Enhancing clinical trials so the results more accurately reflect the patient population



Leveraging real-world data and data science approaches to address public health needs

NCATS' Budget At-a-Glance



\$ (in millions)	FY 2023	FY 2024	
	Enacted	President's Budget	FY24 PB +/- FY23 Enacted
NCATS			
Total	923.3	923.3	0 (0%)
CTSA	629.6	629.6	0 (0%)
Non-CTSA	293.8	293.8	0 (0%)

Reflects Fiscal Year 2023 Enacted Appropriations
 NCATS Budget: \$923,323,000

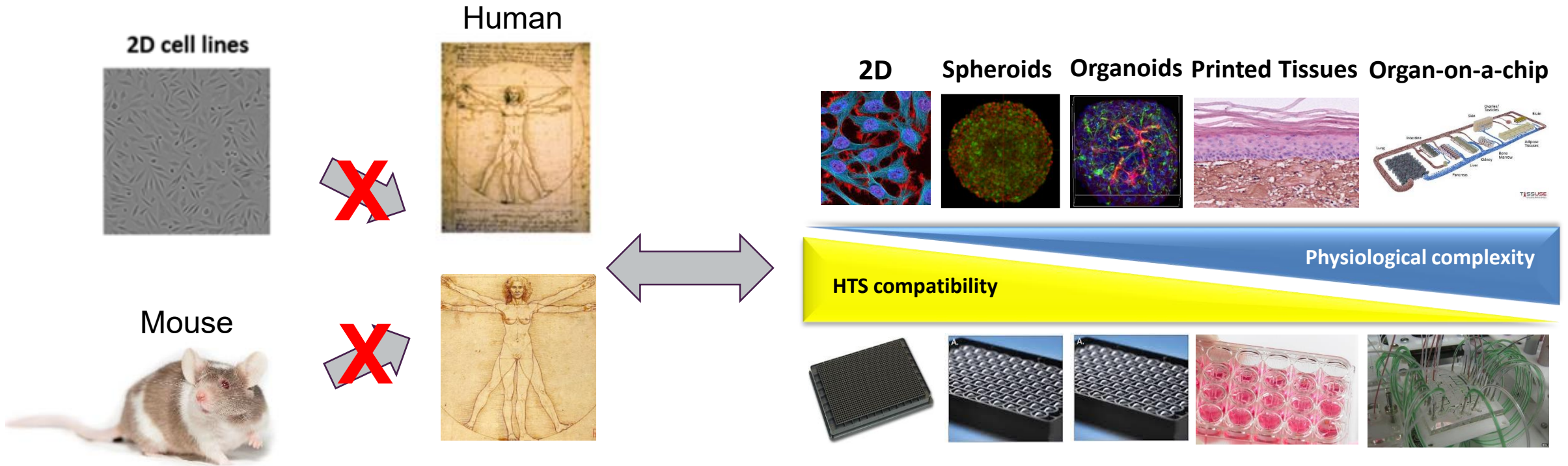


Specific Efforts in Translational Science for Nursing Research

Pre-clinical --- Clinical --- Data Science

Revolutionizing Drug Development Approaches

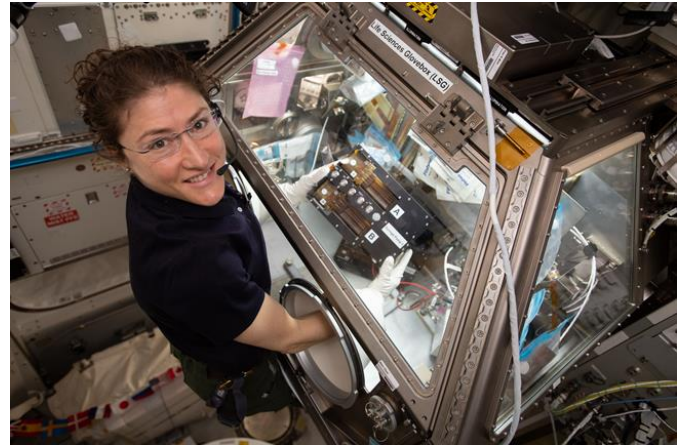
(PhRMA, Biopharmaceutical Research Industry Profile, 2016)



Need for new technologies and better predictive tools across the translational pipeline



Physiological Changes under Prolonged Microgravity: Chips in Space



Resupply Mission
March 14, 2023

TISSUE CHIPS IN SPACE

NCATS Study Suggests People with Rare Diseases Face Significantly Higher Health Care Costs

Individual medical costs for people with a rare disease are

3-5 TIMES

greater than for those who do not have a rare disease.

The medical costs of rare diseases have been underestimated.



Yearly direct medical costs estimated at around

\$400 BILLION

are similar to those of cancer, heart failure and Alzheimer's disease.

Rare diseases are collectively common, affecting an estimated

**25-30
MILLION**

people in the United States.



Source: The IDeaS Initiative: Pilot Study to Assess the Impact of Rare Diseases on Patients and Healthcare Systems



National Center
for Advancing
Translational Sciences

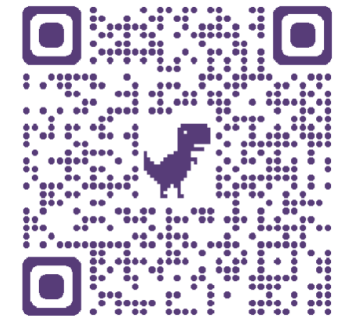
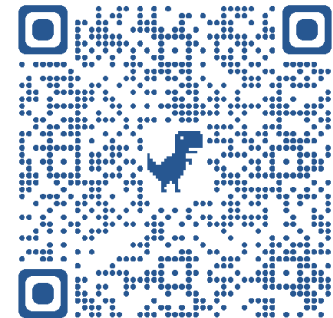
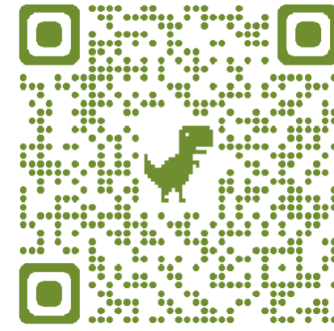
Developing and Streamlining Gene Targeted Delivery Approaches

Development



Clinical Trials

- 1) Somatic Cell Gene Editing – (SCGE)
 - 1) NIH Common Fund Program
 - 2) Moving to clinical studies for second phase
 - 3) Toolkit – data on performance of delivery technologies
- 2) Accelerated Medicines Program[®] – Bespoke Gene Therapy Consortium (BGTC)
 - 1) Enhancing vector manufacturing
 - 2) Enhancing gene expression
 - 3) Regulatory playbook
- 3) Platform Vector Gene Therapy – (PaVe-GT)
 - 1) Single AAV vector as a platform for multiple therapeutic genes
 - 2) Testing ability to increase efficiency to clinical trial start-up



CTSA Program: Premier National Network Speeds Health Solutions



Develop, demonstrate, and disseminate innovations that turn science into health faster



Promote impactful partnerships and collaborations



Address health disparities



Provide a national resource for the rapid response to urgent public health needs



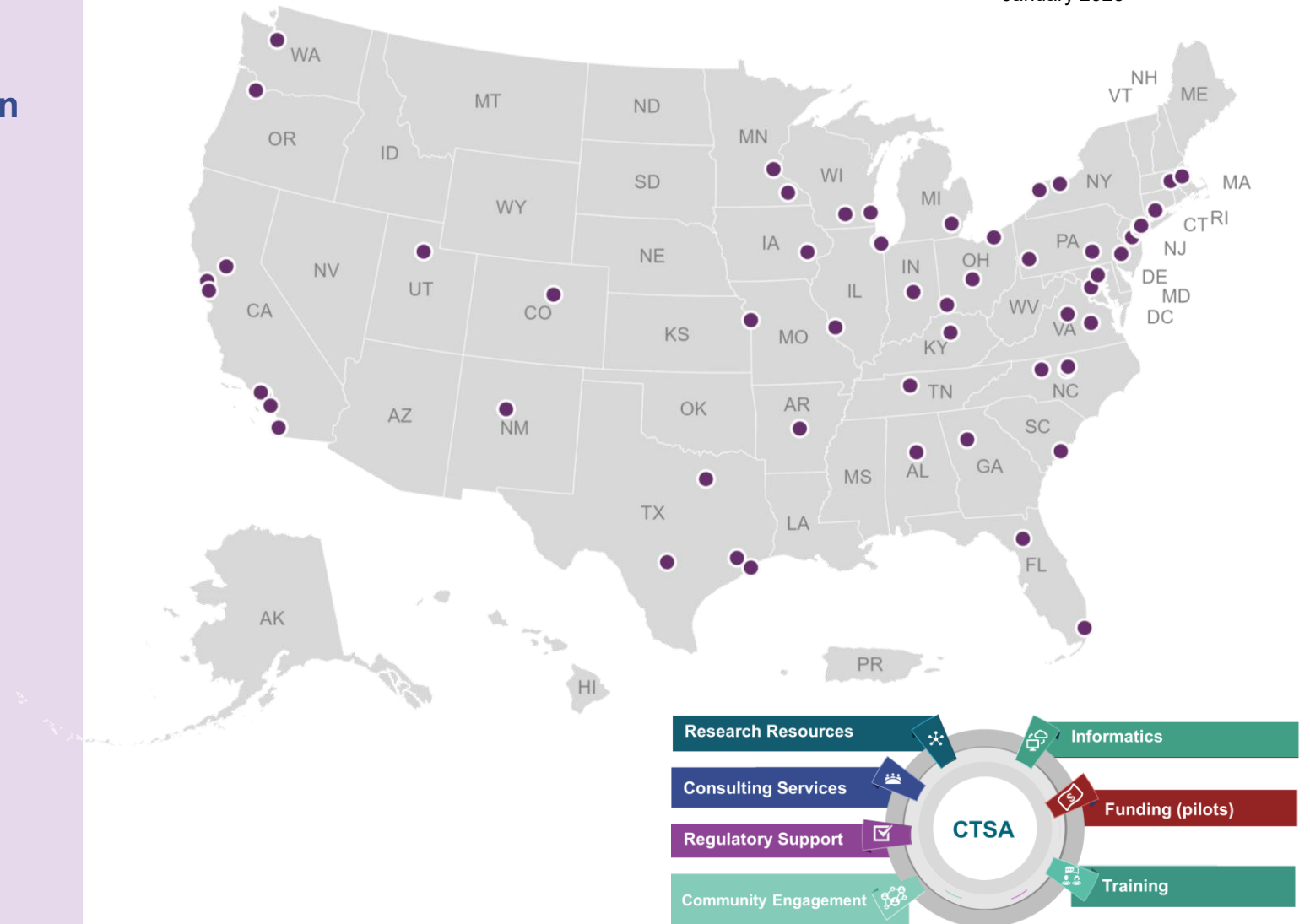
Promote training and career support



Nurture the field of translational science

Clinical and Translational Science Awards Primary Institutions

January 2023



Nursing by the Numbers within the NCATS CTSA program

CTSA Hubs and PIs

- **4 PIs** with nursing degrees
- **43 CTSA** institutions have a nursing school

Scholars and Trainees

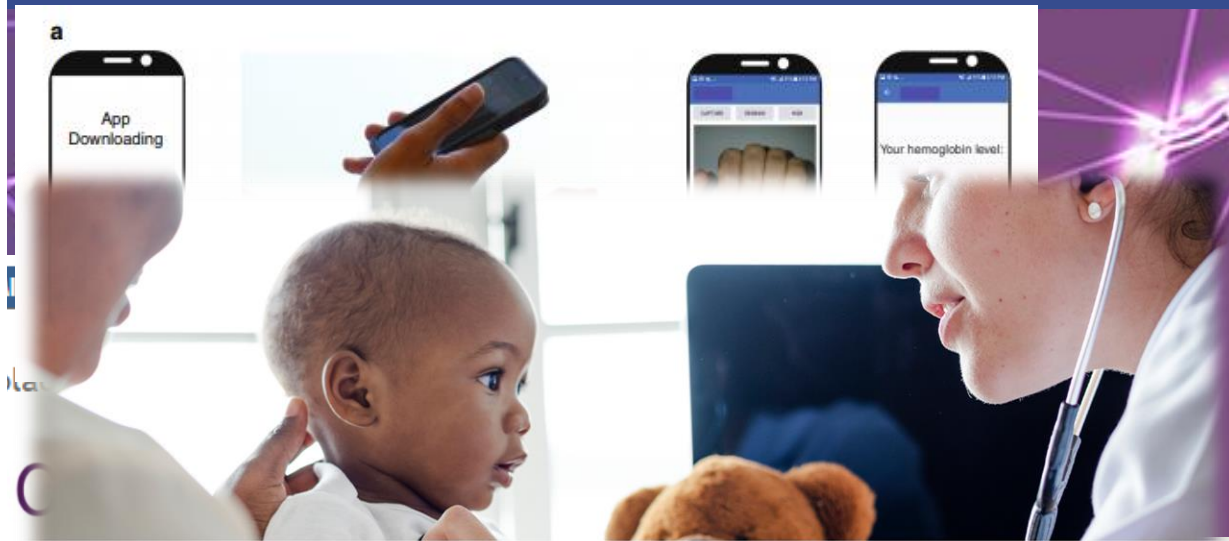
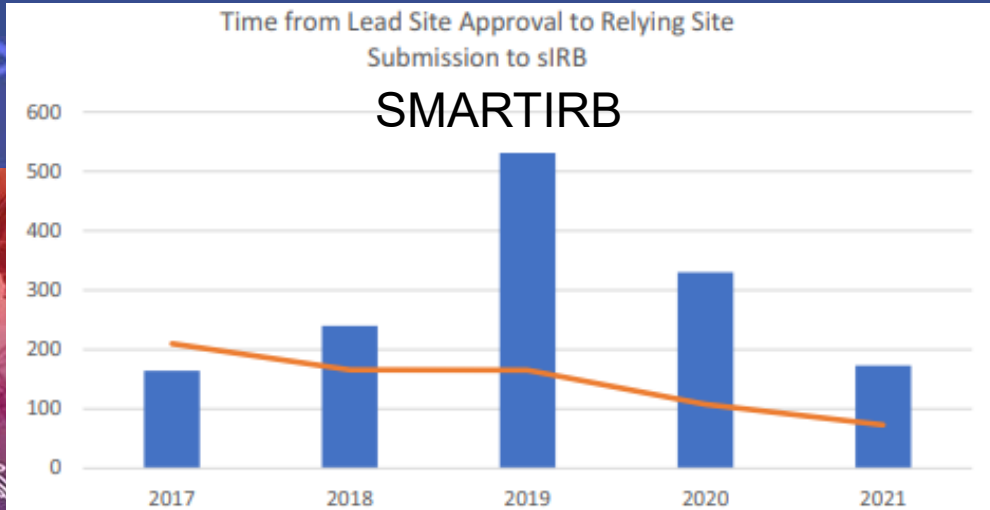
- **24** with nursing degrees

Pilot Projects

- **12** Pilot project PIs with nursing degrees
- **8** serving on projects related to nursing interests



...Local strengths enable nimble, rapid, and robust responses to national public health challenges



Support
Pediat
Resear
Outco
Utilizat
Telehe

Telehealth and Virtual Care Challenges (pre-COVID)

- Variation in services provided via telehealth and virtual care
- Lack of adequate research and reliable, generalizable data to support practices, reimbursement, and provider adoption
- Different focus for different stakeholders: clinicians, payers, patient urgent cares, primary care

SPROUT



Last updated: 03-23-2022

NCATS Funds Network to Improve the Use of Telehealth in Children's Health Care

Home > News > Researchers Shed Light on a Rare Genetic Disease in Children

Researchers Shed Light on a Rare Genetic Disease in Children

Findings could lead to better treatments for primary ciliary dyskinesia

partners to better

19 clinical questions.



Mobile health vehicles offer health resources, vaccine education and outreach opportunities through the Our Community, Our Health programs.

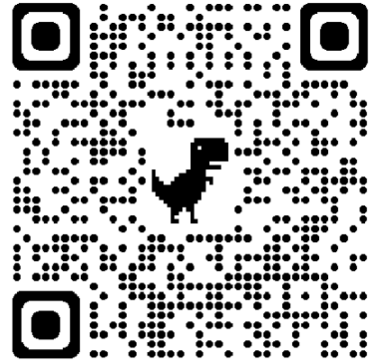
trial to test immune modulators for treatment of COVID-19

COVID-19 can trigger an immune response that causes inflammation that can lead to life-threatening conditions. ACTIV-1 will determine if regulating the immune response with immune modulators can reduce the need for ventilators and shorten hospital stays.



TRIAL INNOVATION NETWORK – Partnering Resources

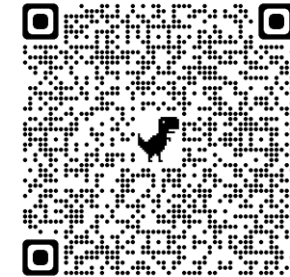
How We Can Help



The Walkability Project



Watch Debra Oto-Kent, HEC Founder and Executive Director, describe the Walkability Project and its sustained community impact.



National Academy of Medicine's Assessing Meaningful Community Engagement

“
We did kitchen table discussions, we met in people's backyards. We engaged with parents after school. We cast a very wide net when talking to people...and based on that input, we pivoted and changed our approach.

*Debra Oto-Kent
HEC Founder and Executive Director*

KEY ENGAGEMENT ACTIVITIES

To align the project with community priorities, HEC pursued a series of engagement strategies to incorporate community members into all aspects of the work.

Built a mechanism for broad community inclusion throughout the project. A five-member, cross-sector team and advisory committee of community-based organizations and residents were assembled to confirm project goals with the community. This was important because other neighborhoods in the city had neighborhood associations that could advocate for their needs; this community did not.

Engaged as many stakeholders as possible, and met them where they lived and worked. The advisory committee sought input from diverse stakeholders with everyday experience of the community. These included residents in core neighborhoods, neighborhood associations, city officials, hospitals, health and social service agencies, faith organizations, and law enforcement. To do so, the advisory committee held key informant interviews, backyard chats, after-school engagement with parents, and focus groups. Notably, they reached out to people who had not been involved and did not know who to turn to in order to make their voices heard.



Partnership between ComPASS and NCATS conducted by VICTR Meharry-Vanderbilt Community Engaged Research Core (CERC)

C3E Aims and Dissemination Outcomes

- Identify resources, tools, and best practices for community-driven and community-engaged research in health equity research.
- Capture the voices of historically marginalized and excluded communities to understand additional needs and strategies for engaging these groups.
- Conduct hybrid meetings focused on identified priority areas derived from NIH needs assessment and community expert panel.
- Create a publicly available resource for dissemination on the web.

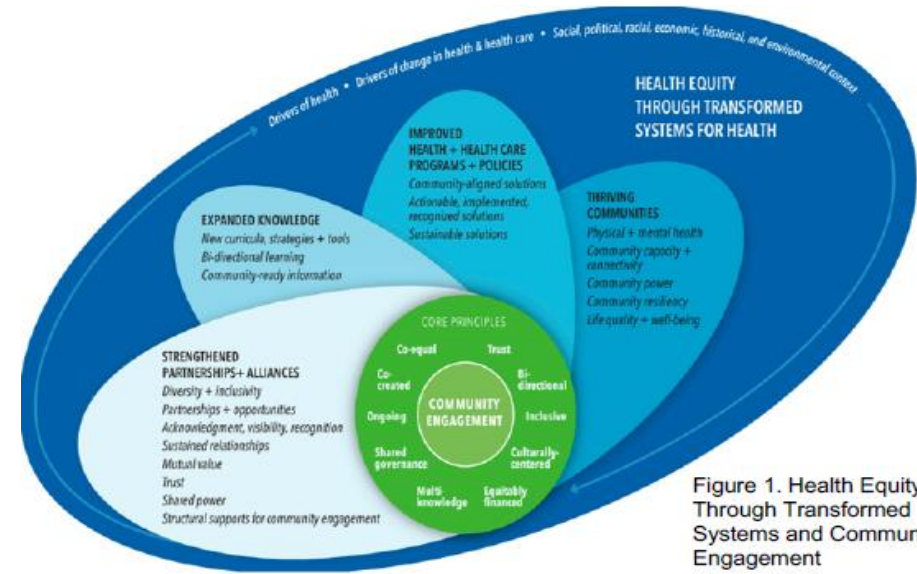


Figure 1. Health Equity Through Transformed Systems and Community Engagement

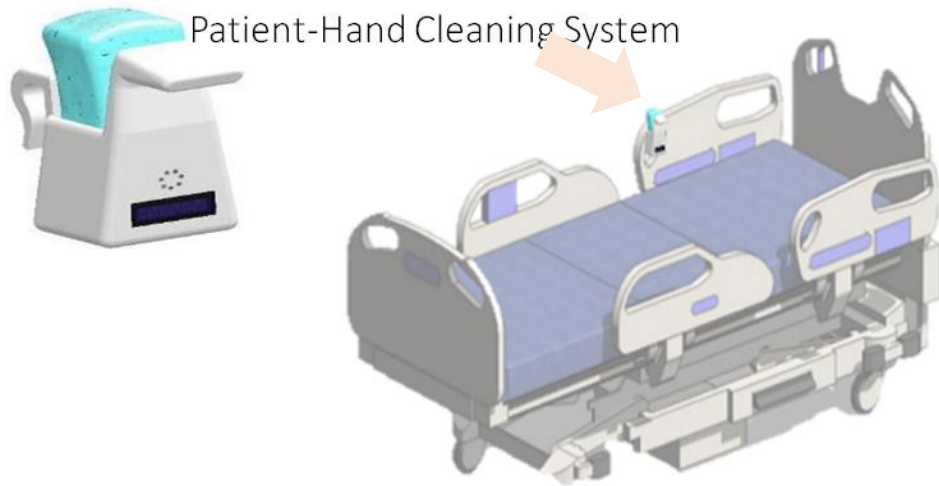


Shanina C. Knighton, PhD, RN, CIC

Instructor, Frances Payne Bolton School of Nursing
Case Western Reserve University
KL2 Scholar, Pilot Award Recipient

Project Aims:

1. Develop expertise in translational health technology
2. Explore the feasibility, and acceptability of technology-based patient hand hygiene self-management interventions among older adults in an acute care setting and retirement community



“It is not about reinventing the wheel but improving the way it turns.”
— Shanina C. Knighton, PhD, RN, CIC

Source:
<https://ownquotes.com/quote/98274>

Alexis Dunn-Amore, CNM, PhD

Assistant Professor, Emory University, Nell Hodgson Woodruff School of Nursing
Diversity Supplement Awardee 2020-21



The Convergence of COVID-19 and Systemic Racism: An Evaluation of Current Evidence, Health System Changes, and Solutions Grounded in Reproductive Justice

DOI: <https://doi.org/10.1111/jmwh.13250>

- Certified Nurse-Midwife (CNM) at the Atlanta Birth Center
- Nurse scientist trying to figure out why so many women in the U.S. — especially Black women — are dying of complications from pregnancy or childbirth
- Mentor and advisor



“Making sure certain populations birth safely is justice – social justice.”
— Alexis Dunn-Amore, assistant professor, Nell Hodgson Woodruff School of Nursing at Emory University

Two Heilbrunn Nurse Scholars (Rockefeller CTS) received prestigious awards including a Fulbright Award and the National Academy of Medicine fellowship.

2016 Heilbrunn Nurse Scholar, Dr. Paule Joseph, Receives 2022 Brilliant New Investigator Award and Inaugural American Academy of Nursing/National Academy of Medicine Fellowship

By Bernadette 'Candy' Capili, PhD, NP-C



Dr. Paule Joseph received two awards reflecting her outstanding achievements. First, she was awarded the 2022 Brilliant New Investigator Award from the Council of

the Advancement of Nursing Science based on her accomplishments since receiving her PhD. The second was her selection as a Fellow at the National Academy of Medicine (NAM). The American Academy of Nursing (AAN) partnered with NAM on this fellowship to advance interprofessional collaboration and to recommend sound policy on pressing issues related to public health.

The purpose of the AAN NAM Fellowship program is to provide talented nurse scholars with an opportunity to experience and participate in evidence-based public health studies that improve the access of care to patients in domestic and global health care systems.

Dr. Joseph is a Lasker Clinical Investigator at the National Institutes of Health (NIH)/National Institute on Alcohol Abuse and Alcoholism (NIAAA) and the National Institute of Nursing Research (NINR). She is also an alumna of the 2016 Heilbrunn Nurse Scholar program. Dr. Joseph is an internationally known chemosensory-trained nurse scientist whose research on taste and smell disorders has proven highly relevant during the

COVID-19 pandemic when many patients reported suffering loss of taste and smell. Her work also explores the intersection between chemosensation, environment, culture, dietary norms and behaviors, and the food industry as they relate to substance use disorders and obesity. Dr. Joseph also develops outreach and training initiatives that promote diversity, equity, inclusion, and access in biomedical research both within and outside the NIH.

The Heilbrunn Family Center for Research Nursing congratulates Dr. Joseph on these well-deserved recognitions for advancing nursing research.

Heilbrunn Nurse Scholar – Cassandra Godzik – Fulbright Award

By Bernadette 'Candy' Capili, PhD, NP-C



Heilbrunn Nurse Scholar Awardee (2021) Cassandra Godzik, PhD, APRN, PMHNP-BC, CNE, a Dartmouth-

Hitchcock Medical Center & Dartmouth College T32 Research Fellow, was recently selected to participate in the United States Post-doctoral Fellows Fulbright

Israel program. Dr. Godzik's research and clinical practice as a psychiatric nurse practitioner has focused on the home sleep environment of adults in the community in non-assisted living, nursing homes, or other residential facilities.

The Heilbrunn award supports her research focusing on older adults in rural communities in the Northeastern U.S. through survey development and virtual health technologies. She will continue this research at the University of Haifa alongside sleep researcher and psychologist Dr. Tamar Shochat.

Godzik's planned research project is entitled "Investigation of the underlying mechanisms that contribute to older adults' poor gait and motor functioning in the context of insomnia." For more about her upcoming studies and travel, please visit: <https://fulbright.org.il/node/459>. We are very proud that Dr. Godzik is receiving this recognition and will be able to expand her research internationally.



National Center
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Translational Sciences

Georgia CTSA Joins Forces with Children's Healthcare of Atlanta to Develop Nurse-Patient Communicator App



- Dr. Christina Calamaro and team joined forces with Children's and the Emory University Nell Hodgson Woodruff School of Nursing & AppHatchery
- Awarded \$100K from J&J for a proposal for the Johnson & Johnson to turn the 'Nurse Communication Board' into a mobile application
- Nurses' innovation for helping limited English proficiency (LEP) patients.

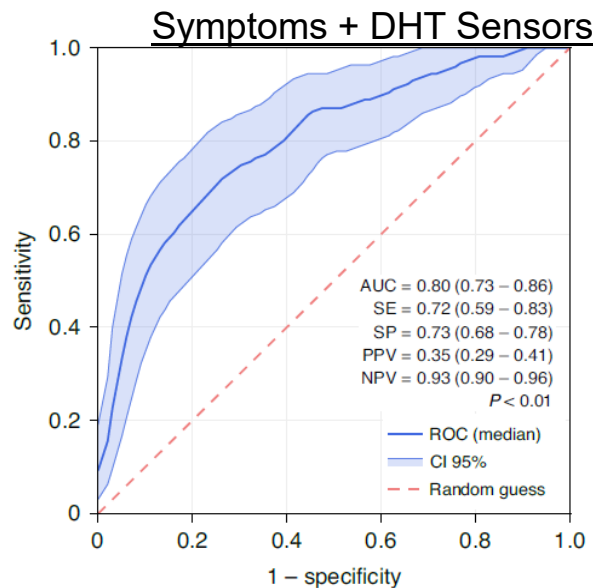
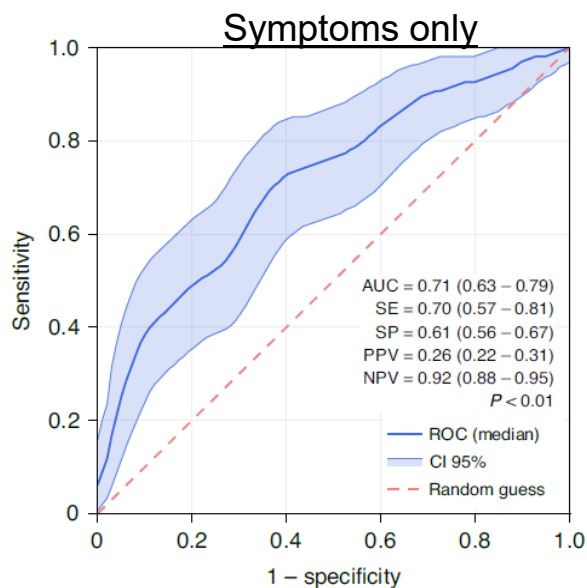
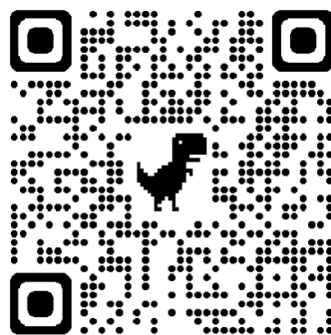
"We want to give the best care to patients that we possibly can. Nothing feels worse than when you leave for the night knowing that you might have missed something with a patient because you weren't able to fully understand them, because of language barriers."

– Christina Calamaro, PhD, Director of Nursing Research and Evidence, Children's Healthcare of Atlanta

Wearable sensor data and self-reported symptoms for COVID-19 detection

Giorgio Quer^{1,3}✉, Jennifer M. Radin^{1,3}, Matteo Gadaleta^{1,3}, Katie Baca-Motes¹, Lauren Ariniello¹, Edward Ramos^{1,2}, Vik Kheternal², Eric J. Topol¹ and Steven R. Steinhubl¹

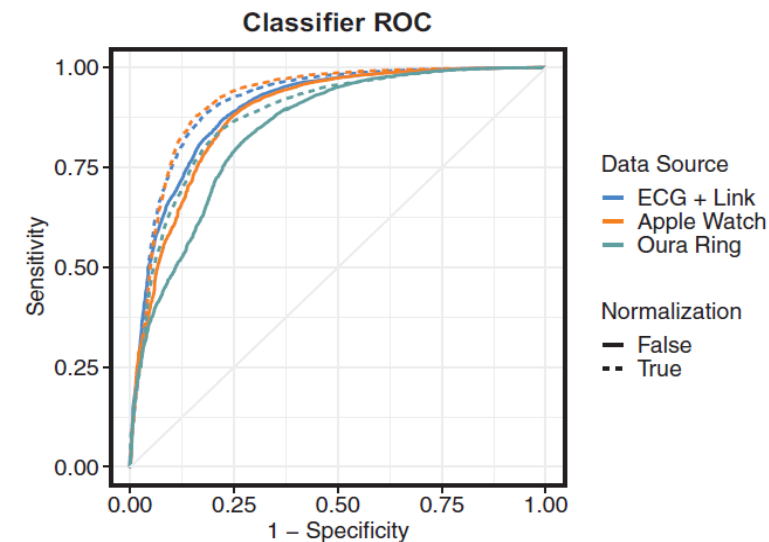
- Scripps CTSA developed a smartphone app that collects smartwatch and activity tracker data, as well as self-reported symptoms and diagnostic testing results
- DHT sensor and symptom data predicts COVID infection with AUC = 0.8, > symptoms alone = 0.71



Detecting sleep using heart rate and motion data from multisensor consumer-grade wearables, relative to wrist actigraphy and polysomnography

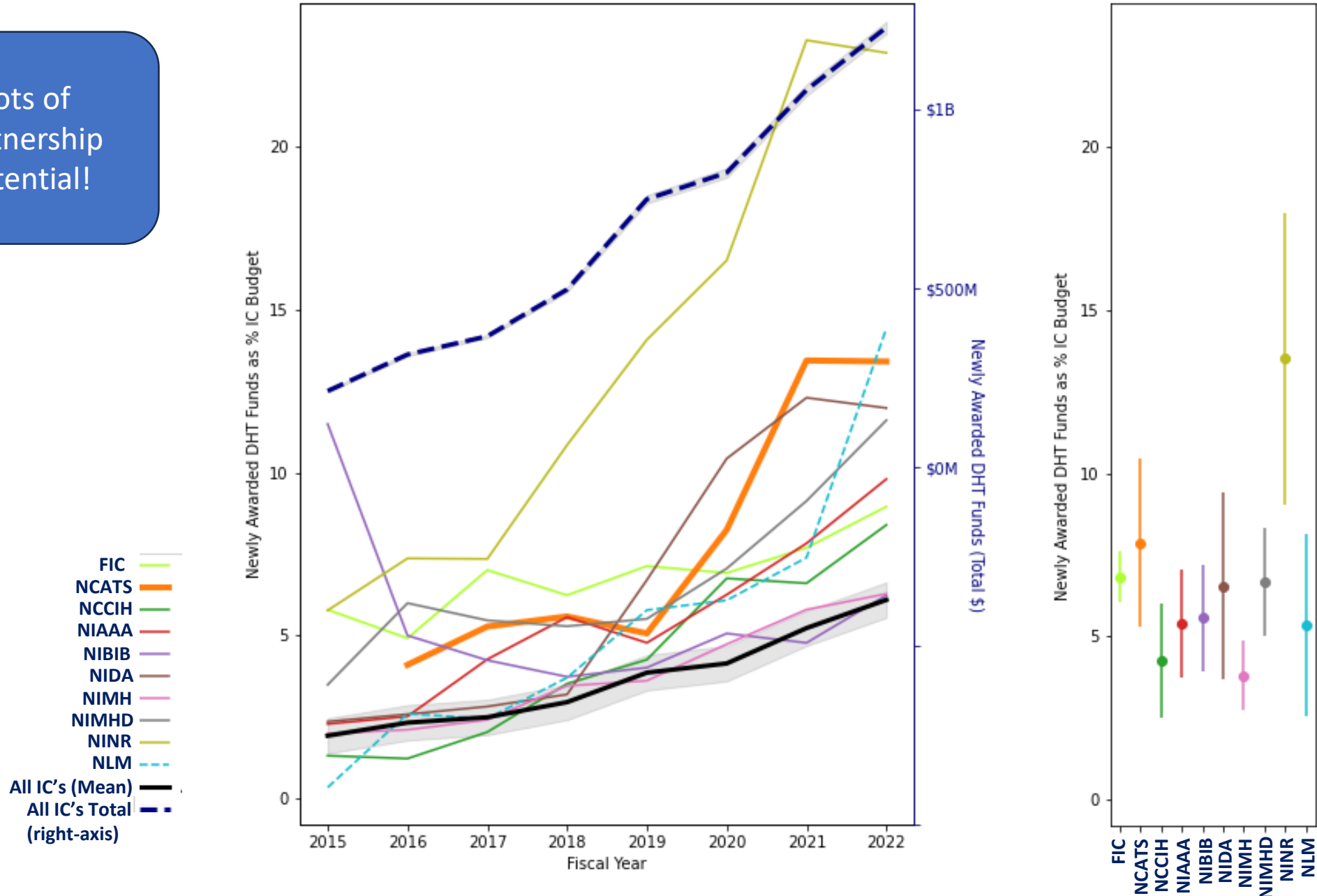
Daniel M. Roberts^{1,*}, Margeaux M. Schade², Gina M. Mathew², Daniel Gartenberg¹ and Orfeu M. Buxton²✉

- Led by Hershey (U. Penn) CTSA
- Sleep epoch classification for multi-sensor wearables (Apple Watch and Oura Ring) comparable to research-grade actigraphy (ECG + Actigraph Link device)

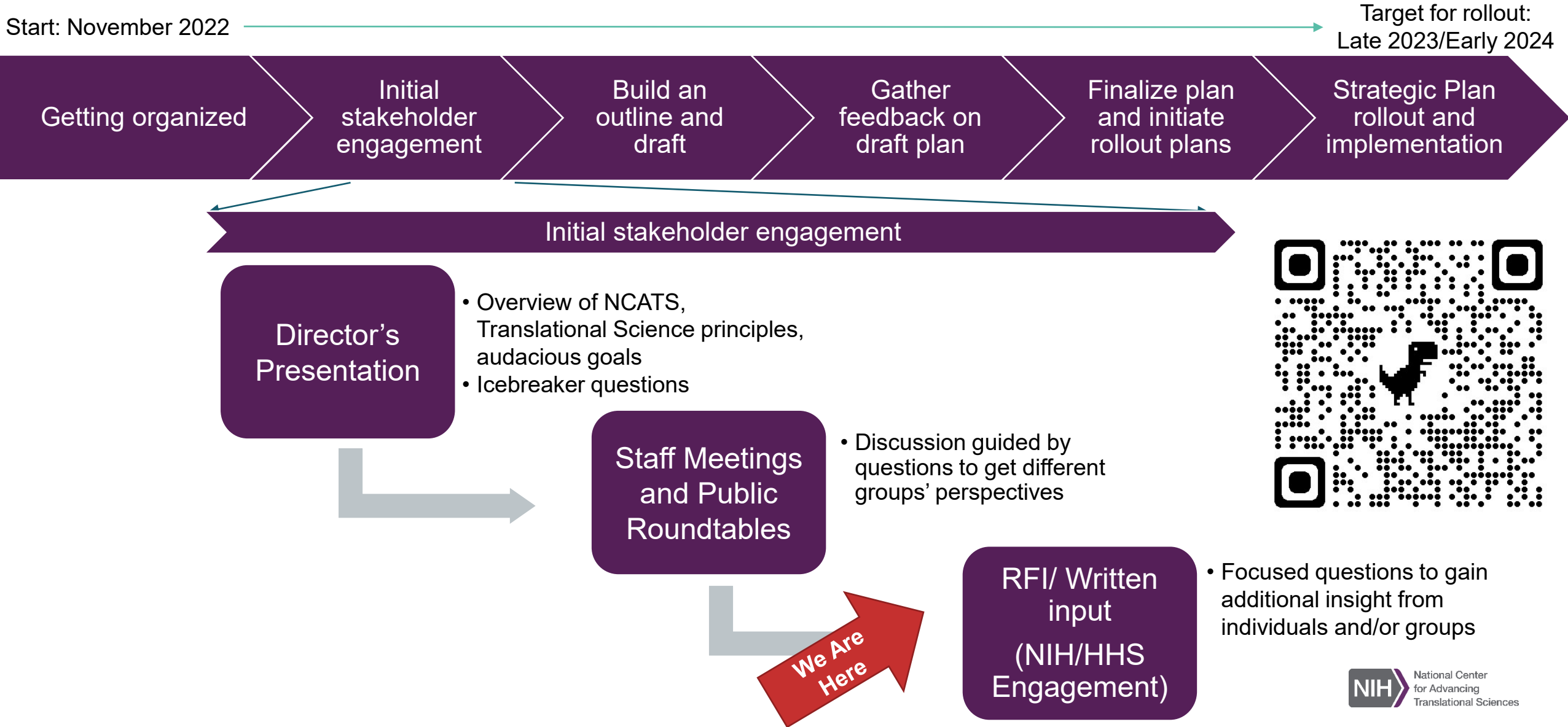


% NIH Funding by IC Budget in Digital Health (2015-2022)

Lots of partnership potential!



NCATS Strategic Planning Process 2024-2029: Stakeholder Engagement





Thank You!

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