NCATS Improving Health Through Smarter Science

CTSA Program PI Webinar

Wednesday, July 26, 2017
2:00 pm (EDT)

CTSA Clinical & Translational Science Awards Program

NIH National Center for Advancing Translational Sciences

AGENDA

2:00	**Welcome**	**Kathleen Brady** *Medical University of South Carolina*
2:05 – 2:15	**NCATS Update**	**Mary Purucker** *NCATS*
2:15 – 2:45	**Open Science Initiative At The Montreal Neurological Institute**	**Angela Genge** *McGill University*
2:45 – 3:00	**Center for Leading Innovation and Collaboration (CLIC) – the new CTSA Program Coordinating Center**	**Martin Zand** **Deborah Ossip** *University of Rochester*

NCATS Update

Mary Purucker

Montreal Neurological Institute and Hospital
Institut et hôpital neurologiques de Montréal

Tanenbaum Open Science Institute (TOSI)

Dr. Angela Genge – Director, CRU MNI

Ms Annabel Seyller – COO, Tanenbaum Open Science Institute

July 2017

McGill

Centre universitaire de santé McGill

McGill University Health Centre

TOSI Mission

To develop and spread a new model of discovery and innovation based on the usage of Open Science principles as accelerators, for the benefits of patients and community, and the development of best practices globally.

TOSI Goals

1. To expand the impact of MNI research by sharing it with a global community of like-minded scientists;

2. To develop key tools and infrastructures to support data sharing, material sharing and open commercialization;

3. To measure open science impact;

4. To highly encourage the scientific community as a whole to embrace this new way of doing research.

Open Science - an idea whose time has come

- The digital age has brought about an ethos of transparency and information-sharing for the social good
- Complex global problems require unobstructed international collaboration
- Scientists recognize the limitations of a closed, proprietary system
- Powerful computers now allow secure, large-scale data sharing
- Visionary scientists, governments and philanthropists are ready to take the leap

Open science is an increasingly prevalent way of doing biomedical research. Aim is to spur innovation and accelerate knowledge discovery and exchange.

Putting McGill and Canada in the lead

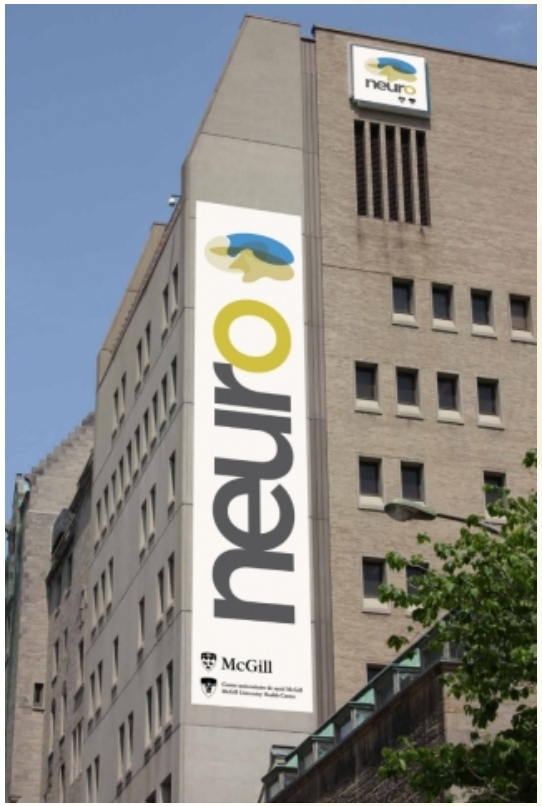
- EU and Japan pursuing open science as national agendas
- The National Institutes of Health (NIH) and Wellcome Trust Open Science Prize to encourage development of new products or services that will advance Open Science
- OECD has identified Open Science as a policy direction with significant social and economic benefits for its members
- The Neuro's Open Science initiative will place Canada at the forefront of a global movement

Why neuroscience?

- Diseases of the brain and nervous system… one of the most compelling unmet medical needs
- Millions of Canadians affected by devastating brain diseases (ALS, Parkinson's, brain tumours)
- At present, limited treatments and no cures for these conditions
- Neurodegenerative diseases = estimated cost of $23B annually (direct health costs and lost productivity)
- According to the World Health Organization, diseases of the brain predicted to surpass cancer as the second leading cause of death in Canada by 2040

➢ Urgency to do something…

Why The Neuro?



- Founded in 1934 by neurosurgeon Dr. Wilder Penfield
- World-leading integrated neuroscience research and clinical centre
- A place where we can make real advances in neuroscience and our understanding and treatment of neurological diseases

Why The Neuro?

- Is a combined research institute and hospital – so close to medical problems and needs
- Unique breadth of expertise, from genes, to cells, to animal models, to patients
- Recognized scientific leadership and credibility
- Access to powerful data infrastructure and specialized human resources
- The MNI Biobank: a one-of-a-kind resource of brain material and data
- A willingness to take measured risks in service of enormous human gains

Open Science as a mission enabler

- From its inception, The Neuro's singular mission has been to deploy scientific research in service of patients, families and society

- We see Open Science as a means to **expand the impact of our research by sharing it with a global community of like-minded scientists**

The result: accelerated discovery, new treatments, and a healthier, more prosperous world

An informed decision

An 18 months consultation process:

A. Definition of Open Science for the MNI

B. Mapping of existing open science activities at the MNI

C. Consultation process with MNI Faculty, staff and students (seminars, polls, town halls, and Q&A sessions)

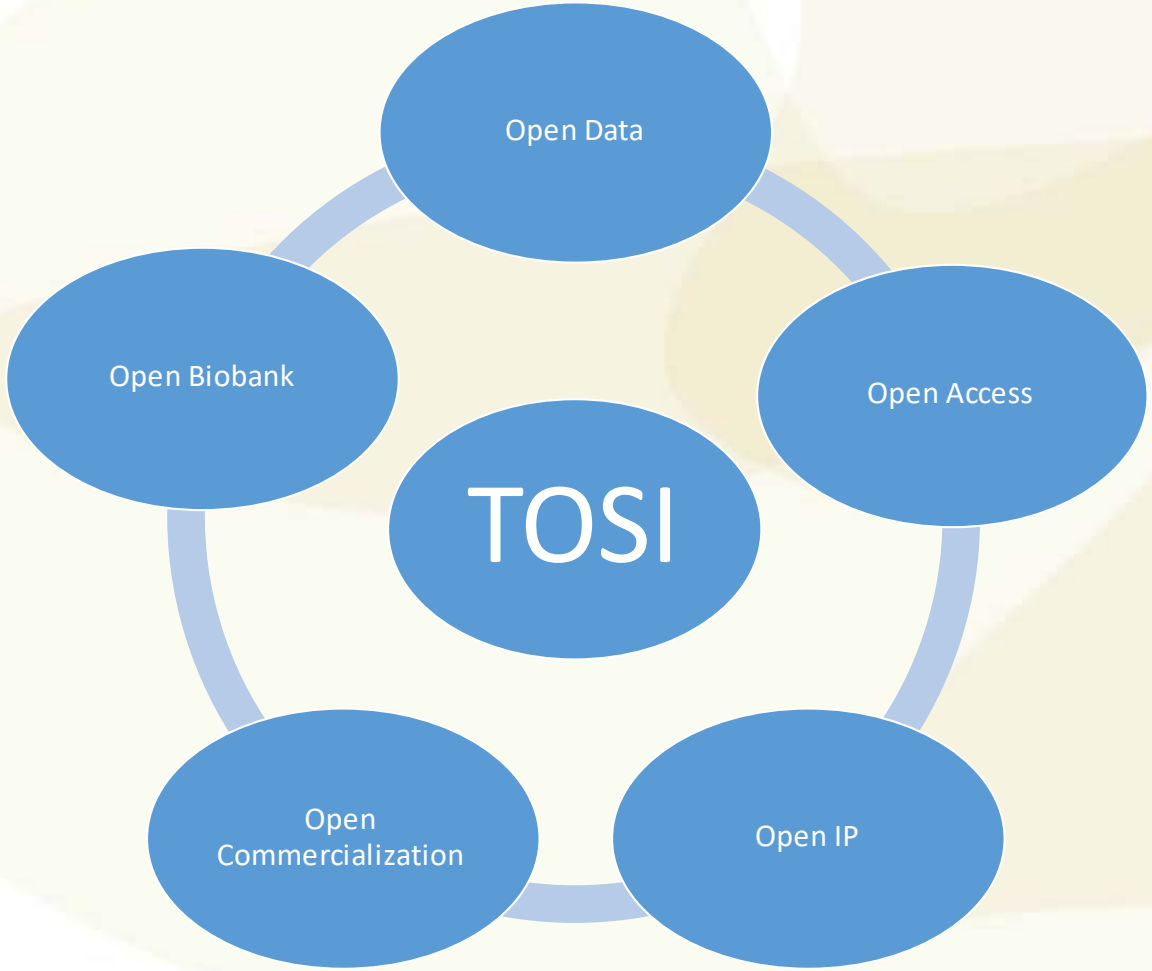
D. Social science study (structured questionnaires) of potential barriers and limitations related to Open Science by an independent research group

E. Definition of Guiding Principles with final buy-in of all MNI members

F. Official support from McGill's Principal

What makes TOSI unique?

The Neuro is the only institution pursuing Open Science along 5 key axes:



A step further in the global OS movement

Open Access

Open Data

Open Access & Open Data

Open Access
Open Data
Open IP
Open Biobank
Open Commercialization

Three core platforms

- **The Neuro-Informatics Platform**
  - Using informatics platforms and innovative mathematical and statistical approaches developed by MNI scientists, to integrate clinical, psychological or neuroimaging phenotypes with genotypic information, we can take advantage of powerful computer networks that can now secure large-scale data sharing capabilities and multifaceted analyses of data.

- **The Clinical Biological Imaging and Genetic Repository (C-BIGR)**
  - Set to become the world's largest library of brain imaging, clinical, demographic, genetic (DNA), and cellular data and samples from patients with neurological disorders, with data opened up to the world.

- **The Open Drug Discovery Platform**
  - Dedicated to bringing new drugs to market, unique public-private partnership jointly managed by the Structural Genomic Consortium (SGC), the Centre for Drug Research and Development (CDRD) and the MNI
  - Three diseases targeted initially: brain tumours, ALS, and Parkinson's

Main objectives of the Open Drug Discovery platform (ODDP)

- Leverage breakthroughs in stem cell (iPSC) technology towards building a suite of industry-standard assays.
- Work with pharma and other partners to build “in house” expertise, with the goal of building new technologies and discovering new patient-centric therapies.
- Build a sustainable model, capable over time to work with freedom and autonomy with different institutes and companies in a global context.

Brief overview of ODDP

- One of MNI key strategic and top-priority initiatives
- Open Drug Discovery Platform sprung up through initial investment by Brain Canada to develop a provincial stem cell platform for academic users located at the MNI and Laval University.
- Over the past 2 years, a team has been assembled and SOPs have been implemented at the MNI iPSC/CRISPR platform
- Agreements have been reached with SGC and CDRD to develop indutry-standard assays and implement automation for screening.
- This initial investment has led to interest from pharma, with Merck as first partner, and Takeda a second partner to be finalized in the summer of 2017, bringing both funds and industry expertise.

Update on current situation

- Initial disease focus has been ALS and Parkinson's disease with funding secured until 2020 ($8M).
- Funding also secured from Alain and Sandra Bouchard Foundation ($4M) to focus on intellectual disabilities (ID).
- Ongoing efforts are now focused on generating MNI's own iPSC lines as an open resource (in collaboration with C-BIG-R).
- We are currently developing assays with input from SGC, CDRD and pharma partners (first assays due by year end and drug screen for 2018).

Next milestones 2017-2018

- Large effort underway to explore other disease areas to expand the portfolio of the platform including autism, epilepsies, ataxias, MS, neuro-oncology and rare genetic CNS disorders ($1.5M proposal under review for autism).

- Attract interest into the platform from foundations as source of new technologies and resources (discussions with Michael J. Fox Foundation).

- Identify 1-2 new pharma partners/start-ups with new projects or portfolios that can be brought into the Open Drug Discovery Platform.

- Identification of one lead compound to demonstrate the merits of open drug discovery.

An Open Publishing Portal

- Partnership under development with Faculty1000 in London
- Objective to build a unique platform for rapid author-led publication and open peer review of research
- Tailored to MNI needs
- Targeted delivery of the portal: Fall 2017

A robust Evaluation Process

The Neuro Open Science Initiative is a world-first social science experiment in how we collectively govern, conduct research and develop innovations.

It seeks to:

- Determine if and in which contexts openness is a more effective research and innovation strategy than are other models
- Measure results and encourage deeper understandings of the process by which we create, share and use knowledge
- Develop international standard measures so that institutions and governments can assess different models of innovation
- Provide data to researchers around the globe studying innovation systems and innovation models

A robust Evaluation Process

- Independent Open Science Evaluation Committee, chaired by Richard Gold and composed of international social scientists from Biomedical Ethics, Economics and Innovation policy

- Will measure the impact of open science at the MNI

- The committee will work with organizations such as the OECD to define appropriate metrics and evaluate the open science model in order to identify academic, innovation, and research policies that promote global economic and social well-being.

Five guiding principles

Principle 1: Public release of scientific data and resources

The MNI and its researchers will render all positive and negative numerical data, models used, data sources, reagents, algorithms, software and other scientific resources publicly available no later than the publication date of the first article that relies on this data or resource.

Principle 2: External research partnerships

All data and scientific resources generated through research partnerships – whether with commercial, philanthropic, or public sector actors – are to be released on the same basis as set out in Principle 1.

Five guiding principles

Principle 3: Access to the MNI Clinical Biological Imaging and Genetic Repository (C-BIGr)

The MNI C-BIGr supports knowledge creation and innovation by maximizing the long-term value of the contributions made by research participants and the scientific resources created by MNI researchers and their collaborators. The C-BIGr will generate revenues, while continuing to enrich and strengthen its informational content and the knowledge it provides. In the conduct of MNI C-BIGr, the MNI recognizes the primacy of safeguarding the dignity and privacy of patient-participants, and respecting the rights and duties owed them through the informed consent process.

Five guiding principles

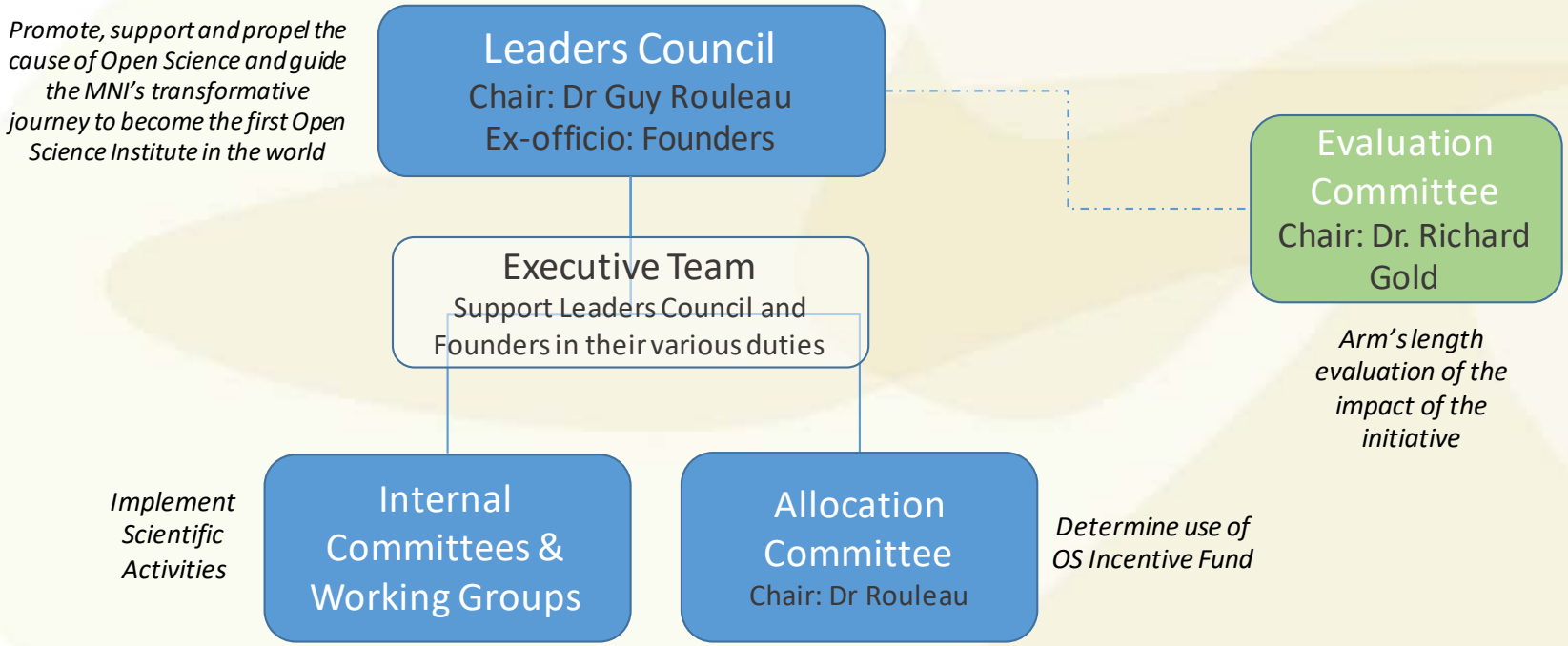
Principle 4: Intellectual Property

Subject to patient confidentiality and informed consent given, neither the MNI nor its researchers in their capacity as employees or consultants of McGill – MNI unit will obtain patent protection or assert data protection rights in respect of any of their research.

Principle 5: Autonomy

The MNI supports the autonomy of its stakeholders, including but not limited to researchers, staff, trainees and patients, through recognizing their right to decline to participate in research and associated activities under an OS framework. However, the MNI will not support activities that compromise the previously outlined OS principles.

Governance


Promote, support and propel the cause of Open Science and guide the MNI's transformative journey to become the first Open Science Institute in the world
Leaders Council
Chair: Dr Guy Rouleau
Ex-officio: Founders
Evaluation Committee
Chair: Dr. Richard Gold
Arm's length evaluation of the impact of the initiative
Executive Team
Support Leaders Council and Founders in their various duties
Implement Scientific Activities
Internal Committees & Working Groups
Allocation Committee
Chair: Dr Rouleau
Determine use of OS Incentive Fund

Leaders Council Mandate

- Broker, foster and nurture relationships to guide the MNI's transformative journey to become the first Open Science institute in the world.
- Promote, support and propel the cause of open science to accelerate discovery and enhance the consolidation of a robust framework for strategic partnerships in Open Science.
- Enable the acquisition of required resources to support MNI's Open Science vision and full implementation.
- Provide insight and oversight to the management, policy setting, and funding for institution-wide open science at the MNI.
- Advise on issues and opportunities in the fields of science, governance, finance, and human resources in a complex transformational environment.
- Evaluate the advancement of this innovative research model, upon monitoring by the Evaluation Committee.

Official Launch in December 2016

- December 16, 2016 - A Transformative $20 Million Donation to the Montreal Neurological Institute and Hospital

- Tanenbaum Open Science Institute - opening new horizons and accelerating discovery in neuroscience

- http://www.mcgill.ca/neuro/open-science-0

CTSA Clinical & Translational Science Awards Program

The Center for Leading Innovation and Collaboration

Martin S. Zand MD PhD
Deborah Ossip PhD

University of Rochester Medical Center
Rochester, NY

National Center for Advancing Translational Sciences

CLIC Center for Leading Innovation and Collaboration

University of Rochester

CLIC Team Leadership

"We are eager to serve the CTSA Program and its community of outstanding investigators and to work with NCATS to continue to create innovative approaches that make translational science vibrantly visible."

Deborah J. Ossip, Ph.D.
Co-Director, Center for Leading Innovation and Collaboration

"We are honored to have been chosen for this critical role. We know that we are standing on the shoulders of giants and offer our deepest gratitude to Vanderbilt and Tufts for their amazing work. We look forward to carrying on your mission."

Martin S. Zand, MD, PhD
Co-Director, Center for Leading Innovation and Collaboration
Co-Director, University of Rochester Clinical and Translational Science Institute

CLIC Mission and Stewardship

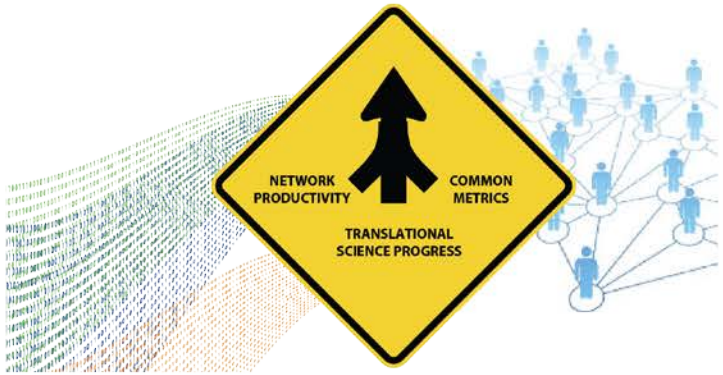
Our mission is to serve the CTSA Program through coordination, transparent communication, actionable metrics, network analytics, and developing innovative collaboration tools.

Our goal is to make the work and accomplishments of the CTSA Program Network vibrantly visible to all stakeholders.

We are stewards of the network, and will actively partner with CTSA Program our colleagues and external stakeholders.

CLIC Functions

Aim 1 - Data:
Integration, Analysis, Action

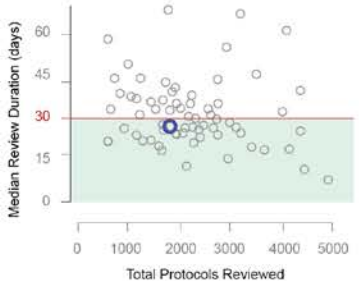


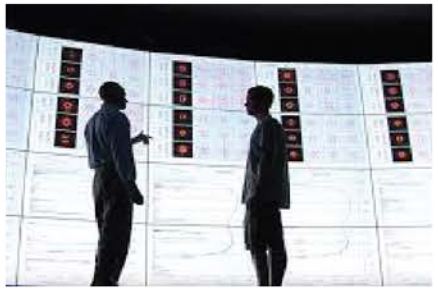
Aim 2 - Collaboration:
Team Science & Synergy

Aim 3 - Engagement:
Meetings, Communication, Stakeholders

Created from data at Grants.gov, all CTSA Program sites that have ever had an active award 2006 - 2011.

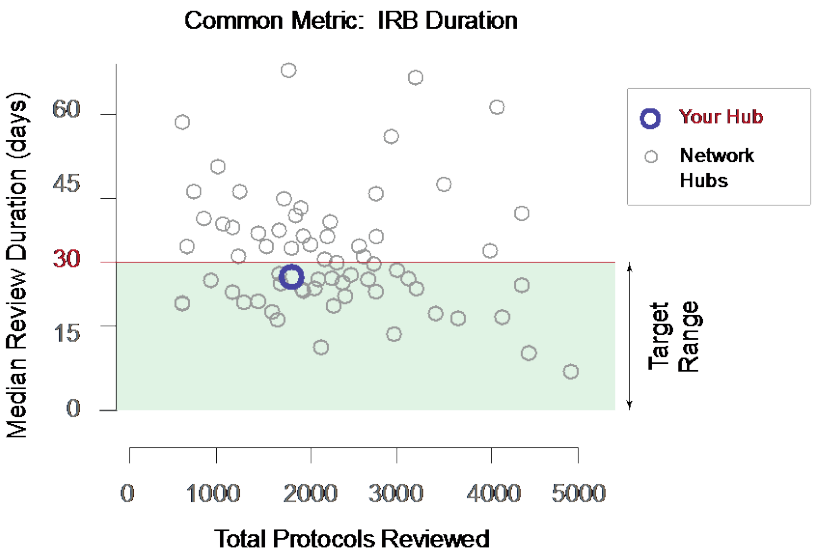
Infrastructure & Technology
Analytics, Visualization, Coordination, Team Support, Media

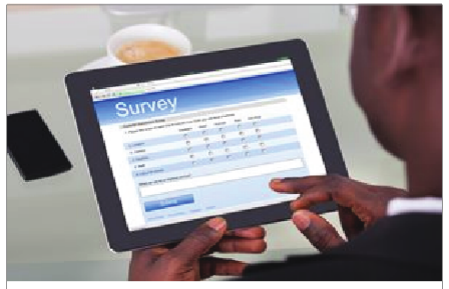




CLIC Innovations

● Coordination ● Communications ● Common Metrics Visualization ●
● *Connect Board* (Job – Fellowship – Internship – Exchange Postings) ●
● Synergy Papers ● Un-meetings ● Stakeholder Collaboration ●





Can eScreening Improve Enrollment of At-Risk Populations in Clinical Research

Young men of color who have sex with men and transgender individuals are at "ultra-high" risk for contracting HIV, but have historically made up a very small portion of volunteers for HIV clinical trials. This pilot is a trial to test the hypothesis that eScreening will improve study enrollment in this high risk group.

Medicine | Recruitment | Clinical Trial

John Cullen
University of Rochester CTSA

18%	45%	27%	14
complete	enrolled	data collected	months left

> Learn more about this research
> Read the Project Blog
> See publications
> Link to downloadable data
> Projects from the University of Rochester CTSA

Translational Research Stories

CLIC Timeline

C4 Transition

- Web site: September 2017
- DTF Coordination: October 2017
- Rocket Workspace: October 2017
- Spring Meeting: Now

Common Metrics Transition

- Accrual Metric: December 2017
- Informatics Metric: October 2017 (Pilot)
- Confidential Visualizations: Early 2018

Innovation

- *Connect Board*: Early 2018
- Synergy Papers: October 2017
- Subscription e-Calendars: October 2017
- Un-meetings: Spring-Summer 2018
- Network data repository: Winter 2017