Open Translational Science in Schizophrenia

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Fostering Use of the Janssen CT Data

• Goal: Foster collaborations around Janssen’s CT data & advance the science around its use
  – Using CT with other publicly available data about schizophrenia

• Open translational science holds promise in the study of complex problems including psychiatric illnesses
  – No one group has all the answers
  – This pilot project will help determine the benefits of open-science collaborations for schizophrenia
Schizophrenia

• Schizophrenia is a chronic, severe, disabling brain disorder that affects approximately 1% of the population worldwide
  – Men/women equally

• Even with treatment, most patients remain at least partially disabled

• Current therapies target the dopaminergic system
  – A strategy that hasn’t changed since the 1950s
Schizophrenia

- Despite best efforts, there are no new ground-breaking therapies on the horizon
- No clear taxonomy of subtypes has yet been developed
- Predictors of the course of illness not well understood
Unmet Medical Need

• In order to address the unmet medical need we must:
  
  – Better understand differential response to treatment – both safety and efficacy
  
  – Design treatment strategies to deliver the best therapy for individual patients at the opportune moment in the disease course
Advancing Treatment of Schizophrenia

• No one group can accomplish this goal
  – Innovative analytic approaches
  – Creative ways of combining disparate data sets

Innovation and collaboration are necessary for making breakthroughs in identifying causal pathways and ultimately new therapies & therapeutic strategies.
The OPTICS Project Mission

Improve the understanding and treatment of schizophrenia using an open-science approach to foster collaborations leading to innovative solutions regarding:

1. Therapeutic efficacy and safety
2. Disease etiology, subtypes, and course
3. Novel analytic/design methods

Ultimately leading to accelerated amelioration of disease burden and increased collaboration among researchers

Intellectual property generated from this project will be dedicated to the public and free for everyone to use
Disciplines and Data: Beyond the Usual

Schizophrenia: natural history, subtypes, etiology

Design/Analysis Methods

Rx Efficacy

Rx Safety
How It Works

Investigators applying to participate in OPTICS:

• Agree to conduct research toward at least 1 project goal:
  – Therapeutic safety, efficacy
  – Disease understanding (e.g. subtypes, course)
  – Analytic and design methods for disparate data types

• Apply for access to OPTICS Project data resources:
  – YODA & dbGaP/NIMH
  – Must meet data holder Data Use Agreements

• Goal: Integration of CT and other data about schizophrenia
The real voyage of discovery consists not in seeking new landscapes, but in having new eyes.

*Marcel Proust*
17 Paliperidone Trials

**Disorders**
- Schizophrenia
- Schizoaffective Disorder
- Bipolar Disorder

**Therapies**
- **Primary:**
  - Paliperidone ER
  - Pali palmitate
  - Placebo
- **Additional:**
  - Risperidone
  - Quetiapine
  - Olanzapine
  - Lithium
  - Valproate

**Outcomes**
- **Symptoms:**
  - PANSS
  - HAM-D, CGI-S, YMRS
- **Functional:**
  - PSP
  - Time-to-Relapse
- **Adverse events**

**Design Elements**
- Most: Acute, randomized, double-blind, placebo-controlled
- Others: Relapse prevention, active comparator, and open designs
NIH OPTICS Project Data: NCBI - dbGaP

Preview Site
dbGaP Collection: Open Translational Science in Schizophrenia (OPTICS)
dbGaP Study Accession: phs000887.v1.p1

Jump to: Authorized Access | Attribution | Authorized Requests

Schizophrenia is a chronic, severe, disabling brain disorder that affects approximately 1% of the population worldwide. Epidemiologic studies have clearly demonstrated that genetics play a strong role in etiology, but the inheritance is very complex. Innovative analytic approaches and creative ways of combining disparate data sets will be necessary for making breakthroughs in identifying causal pathways and ultimately new drug targets.

This dbGaP collection consists of all genetic studies of schizophrenia available in dbGaP that have been consented for general research use. The goal is to facilitate identification of datasets with related scientific content in order to expedite the application process and ascertainment of datasets of interest for increased scientific discovery.

The Open Translational Science in Schizophrenia (OPTICS) Project, was launched by Janssen Research & Development, LLC, part of the Janssen Pharmaceutical Companies of Johnson & Johnson, to create a new forum for collaborative analysis of Janssen’s schizophrenia clinical trial data and other publicly available data about schizophrenia with the goal of creating new models for conducting research. The OPTICS project is one part of a larger effort at Janssen and other Johnson & Johnson research companies to share clinical trials data to enhance public health and advance science and medicine. Qualified investigators and physicians may apply for access to anonymized clinical trials data from Janssen, for more information please visit https://sites.google.com/site/opticschizophrenia/.

- Study Type: Collection
- Number of study subjects that have individual level data available through Authorized Access: 0

Important Links and Information
- Request access via Authorized Access
  - Instructions for requestors
  - Data Use Certification (DUC) Agreement

Publicly Available Data (Public ftp)

Note to previewer: A web link to an ftp site will appear on this page when this study is released to the public.

Selected publications

There are no selected publications related to this study.

Diseases/Traits Related to Study (MESH terms)
Participant Investigators

• Goal: Encourage collaborations among industry, academia, and others
  – Including those outside traditional disciplines
    • Cross industry; cross academic
    • Industry-academic
    • Outside the usual silos
      – What if...

• Web-based workspace in which collaboration can occur
  – Groups with similar methods and research topics encouraged to work together
The Process

- Limited **analytic period**
  - 9 months from launch

- **Abstracts**: Extended abstracts due following the analytic period

- Merit adjudicated by the **Scientific Advisory Board**
  - Advisory Board: scientists from academia, industry, other sectors
  - Abstracts must have results

- **Meeting**: Results presented and discussed
  - No spectators at this meeting
  - Invitations issued to those who have done a qualifying analysis of the data
The Process

• Groups with similar strategies will be encouraged to integrate efforts in combined manuscript(s)

• Publication:
  – All manuscripts passing peer-review will be published
  – Open-access online journal (BMC)

• Follow-on activities:
  – Evaluation of this pilot
  – Planning next steps
Measures of Success

Cross-sector collaborations that result in:

- Substantive contributions to the schizophrenia disease area
- On-going collaborations involving CT data
- Development of new methods for use across data types/designs
- Peer-reviewed publications relevant to the objectives of the collaboration
Benefits

- Researchers contribute to at least 1 of 3 broad areas of work
- Potential to collaborate with others working in the same area
  - Potentially outside the usual silos
- Limited, but sufficient, period of time to conduct analyses
- Opportunity to meet with other investigators
- All manuscripts passing peer review will be published
  - Preliminary work for larger applications
  - NIH RDOC
- Help shape the future of open science collaborations around clinical trial data
How is this different from other efforts?

• The proposed effort is substantively different from other open science efforts:
  - Time-limited proof of concept for an analytic collaboration based on diverse data sources including CTs
  - No requirement for participation other than being a qualified investigator
  - IP is dedicated to the public

• This is *not* the establishment of a data repository with a common data model for use in perpetuity; instead, it is an open-science collaboration.
Summary

• Open science holds promise in the study of complex problems including psychiatric illnesses
  – No one group has all the answers
  – This pilot project will help us determine the benefits of open-science collaborations for schizophrenia

• Despite current medications and scientific advances, schizophrenia continues to be a debilitating, costly illness
  – Collaborative projects such as this have the potential to examine schizophrenia in a manner that may spur the development of novel interventions

• This project will allow us to evaluate a model to establish collaborations involving CT and advancing the science around its use
Invitation

Please join:

Adam Savitz, MD PhD
Director, Neuroscience
Janssen

Michelle Williams, ScD
Stephen B. Kay Family Professor of Public Health
Chair, Department of Epidemiology
Department of Global Health and Population
HSPH

...and me, for a discussion of the OPTICS Project tomorrow at
the Catalyst Workshop

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Thank You!