Building New Patient-Centered Research Networks: The T1D Exchange and Registries for All Diseases

A FasterCures Webinar
March 20, 2013
About the FasterCures TRAIN Webinar Series

- We are an action tank that works to improve and speed up the medical research system, so we can reduce the time it takes to get important new medicines from discovery to patients.
How we do what we do

_How do we do this? We..._

- **convene** innovators and create opportunities for collaboration.
- **ensure** our national policy supports accelerated research.
- **develop** programs with a laser focus on what is working.
- **identify problems** standing in the way of progress.
- **turn to disruptive ideas** and best practices to pave the path toward solutions.
New venture philanthropy resources – help us spread the word!
The FasterCures Webinar Series

- Allows **real-time** sharing of ideas, best practices, trends, and lessons learned.
- Disseminates information in an efficient, **low carbon-footprint** way.
- **Amplifies** meaningful solutions and/or encourages action needed to spur medical progress.
Speakers

Dana Ball
CEO
T1D Exchange

Sharon Terry
President and CEO
Genetic Alliance

Moderator:
Margaret Anderson
Executive Director
FasterCures
How Do We Address Time and Cost?

New Estimate of Drug Development Costs Pegs Total at $1.5 Billion

Office of Health Economics suggest more flexibility and better alignment between stakeholders and innovators is needed.

The Burrill Report, December 2012
Daniel S. Levine
T1D Exchange Vision

The T1D Exchange model was conceptualized as a solution to mitigate barriers and inefficiencies for clinical and translational research.
An Integrated Model

Registry

Biorepository

Exchange

Patient Community

T1D Exchange
Improving Care and Accelerating Development

- Understand state of T1D care & management
- Quality of life
- Health care costs
- Patient & caregiver needs
- Clinician needs
- Segment & stratify patients

Patient Care & Clinical Research

- Understand human disease
- Discovery of new pathways & targets
- Access to patients for studies & trials
- Leverage clinicians, network & patients
- End to end discovery–delivery system

Research & Development
Patient Characterization

- Laboratory Tests
- Ocular Health
- Family History T1D & T2D
- Time of Diagnosis
- Blood Sugars
- Payer/Insurance
- Device Use
- Medication Usage
- Pregnancy
- Insulin Use
- Medical Procedures
- Patient Phenotype
- Co-Morbidity
An Integrated Model For Research

National Network of Type 1
- 65+ clinics
- Pediatric and Adult

100,000
- Patients available for participation in studies
- Demographically and socioeconomically diverse

26,000
- Patient registry—longitudinal
- Very well characterized
- Collect 150 different disease parameters

1,000
- Patients in Biorepository
- Linked directly to the patient
T1D Exchange Personnel

- **Clinic Network & Registry**
  - 15 Staff
  - Director
  - Coordinators
  - Project Managers
  - Epidemiologists
  - Statisticians
  - KOLs
  - IT Team

- **Biorepository**
  - 7 Staff
  - Director
  - Project Managers
  - Protocol Expertise
  - Research Assistants
  - Coordinators
  - Biobank Staff

- **T1D First & GLU**
  - 15 Staff
  - Senior Management
  - Strategic Alliance
  - Operations
  - IT Team
  - MarCom
  - Content & Community
T1D Exchange Products & Services

Solutions to Research Obstacles—Faster, Validated Results

☐ MyGlu & Exploration Portal
   ☐ Patient engagement platform
   ☐ Access to T1DX data

☐ Specialty services
   ☐ Patient recruitment & study/trial support
   ☐ Custom study modules

☐ Dynamic biorepository
   ☐ Living biobank strategy
   ☐ Ability to collect targeted samples
Glu: Engagement Platform

Patient & Caregiver Community: A Community with a purpose!

- Community with a common goal: improve outcomes & lifestyle of T1D patients
- Support patients on a daily basis through connecting, education and empowerment
- Integrated with the most comprehensive national T1D patient registry
- Real time access point for community driven research using an innovative mobile platform
Question Of The Day

If you're on a pump, do you change your infusion set at the same time you change your reservoir?

You answered: No Answer

There are 253 responses to this question.
“I change my infusion site every 2 days. I've found that my blood sugars start to go up on the 3rd day if I leave it in any longer. So every other day no matter what. The reservoir I change when it is empty. I keep an eye on it to make sure I don't get caught away from home with no insulin.”

“Depends on if they both run out at the same time. Why waste a good 30 or 40 units of insulin just because the site has gone bad?”

“Three years ago I was diagnosed with DKA of unknown origin, and one possible reason was infection at the infusion site, which I had been using for about five days. When I see my endo he checks my sites and I have been told more than once that I need to change every three days. Finances are a problem, so that is why I try to use sets longer than three days. I never thought about reusing the reservoirs, but I may try that now, even though reservoirs are significantly cheaper than infusion sets.”
Education & Study Recruitment
Going from Genotype to Phenotype, with Patient Specificity

- Protocol driven procurement and analysis
  - Access patients under coordinated IRB for studies
    - Can recruit through Registry and Glu
  - Collect biosamples longitudinally
    - Developed Virtual Biobank
  - Re-contact patients for additional studies
  - Access existing biosamples for studies

- Integrated with the patient registry
  - Patient biologic data linked to clinical information
  - Over 6,000 existing biosamples

Locating and procuring the right sample from the right patient at the right time.
Accelerating Timelines

Supporting the research continuum!
T1D Exchange In Action

Ongoing Studies:

- **Observational Studies:**
  - **Sanofi TEENS Quality of Life Study**
    - Goal: 500 subjects
    - Completed within 4 months
    - 25% enrollment each month
    - 0 screening failures
    - No patients discontinued
  - **Joint T1DX-HCT Study: Residual C-Peptide**
    - Goal: 1,000 patients
    - First 800 patients recruited within 3 months
    - approaching completion of enrollment

- **USC–RAND Study: Socioeconomic Determinants of Health Literacy, Diabetes Awareness and Treatment Adherence**
  - Goal: 2,000 patients from various sources
  - T1DX registry recruited 1,663 patients within 60 days (90% enrolled within 10 days)

- **Interventional Studies:**
  - **Large Pharma Partner**
    - Goal: 300 subjects
  - **Biotechnology**
    - Novel glucagon: studies, co-creation and first T1DX development partnership

In Development:

- **Metformin Study: T1DX/HCT Partnership**
- **LADA Study: Partnership with CHOP, Funded by NIDDK**
Publications & Abstracts

Publications

- Severe Hypoglycemia and Diabetic Ketoacidosis among youth with Type 1 Diabetes in the T1D Exchange Clinic Registry. *(Pediatric Diabetes, 3/2013)*

- Evidence of a Strong Association Between Frequency of Self-Monitoring of Blood Glucose & Hemoglobin A1C Levels in T1D Exchange Clinic Registry Participants *(Diabetes Care, 2/2013)*

- A Comparison of Autoantibody Positive & Autoantibody Negative Pediatric Participants Enrolled in the T1D Exchange Clinic Registry *(Journal of Diabetes, 2/2013)*

- Most Youth with Type 1 Diabetes in the T1D Exchange Clinic Registry Do Not Meet American Diabetes Association or International Society for Pediatric and Adolescent Diabetes Clinical Guidelines *(Diabetes Care, 1/2013)*

- The T1D Exchange clinic registry *(Journal of Endocrinology & Metabolism, 12/2012)*

Abstracts

- 32 Abstracts and posters presented (or to be presented in 2013) at 15 meetings around the globe
  - ADA, EASD, ISPAD, ATTD and DT&T
Partnering With T1D Exchange

- We are a Nonprofit with a Different Strategy
  - Develop tools, technologies and systems
  - Accelerate therapies to market
  - All surpluses reinvested into our research mission
  - There is a mission motive, not a profit motive
- Incubated with private charity support by the Helmsley Charitable Trust
- Self-sustaining Through Partnerships
Unitio is a nonprofit organization that unites the greatest minds with the trials, triumphs, and technology for disease exploration.

Our mission is to connect researchers, physicians, and patients to forge dynamic partnerships enabling discoveries and accelerating treatments; providing better answers for the most pressing questions.
Thank You
Registries for All Diseases

TrialsFinder®

Full Launch
Feb 28, 2013!

Full Launch
Feb 5, 2013!

Genetic Alliance

Full Launch
Feb 28, 2013!
Needles in Haystacks
But the haystack is made of needles...

Every one of us will participate when given the tools!
<table>
<thead>
<tr>
<th>Industrial Age (old)</th>
<th>Information Age (new)</th>
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<tbody>
<tr>
<td>Control</td>
<td>Open</td>
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<tr>
<td>Based on scarcity</td>
<td>Based on abundance</td>
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<tr>
<td>Hierarchical/Command</td>
<td>Network/Collaboration</td>
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<td>Linear/Sequential</td>
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<td>Win/Lose</td>
<td>Win/Win</td>
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<td>Materials</td>
<td>Information</td>
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...but biomedical research and healthcare lag
In this **new age**: we have the **tools** to address the problem of **fragmented systems**, and still we **lose opportunities** every day.

Data can accelerate **research and services**, help us to get **better patient outcomes**, and save money...
Getting Individuals onto the Information Highway

Disease Specific Data Elements
Common Data Elements
Gamified Survey

Privacy Assured
Trust
Education
Engagement

Disease InfoSearch
Powered by Genetic Alliance

Reg4ALL.org
Join Reg4ALL

13,000 disease pages
Hundreds of thousands of visitors
High SEO

Pharma and Biotech analyzing data to choose diseases

DiseaseInfoSearch.org
Liver cancer
To find out how your experience compares with others about this condition Click Here.

Common Name(s)
Liver cancer, Cancer, Liver

Hepatocellular carcinoma is the major histologic type of malignant primary liver neoplasm. It is the fifth most common cancer and the third most common cause of death from cancer worldwide. The major risk factors for HCC are chronic hepatitis B virus (HBV) infection, chronic hepatitis C virus (HCV) infection, prolonged dietary aflatoxin exposure, alcoholic cirrhosis, and cirrhosis due to other causes. Hepatoblastomas comprise 1-2% of all malignant neoplasms of childhood, most often occurring in children under 3 years of age. Hepatoblastomas are thought to be derived from undifferentiated hepatocytes (Tanguchi et al., 2002).

Source: Online Mendelian Inheritance in Man

Advocacy and Support Organizations
Condition Specific Organizations
**Articles from the PubMed Database**

Research articles describe the outcome of a single study. They are the published results of original research. The terms "Liver cancer" returned 841 free, full-text research articles on human participants. First 3 results:

**High cytidine deaminase expression in the liver provides sanctuary for cancer cells from decitabine treatment effects.**

Author(s): Quteba Ebrahim, Rada Z Mahfouz, Kwok Peng Ng, Yogesh Saunthararajah

We document for the first time that sanctuary in an organ which expresses high levels of the enzyme cytidine deaminase (CDA) is a mechanism of cancer cell resistance to cytidine analogues. This mechanism could explain why historically, cytidine analogues have not been successful chemotherapeutics...

**Radiofrequency ablation versus resection for colorectal cancer liver metastases: a meta-analysis.**

Author(s): Mingzhe Wang, Yong Zhang, Di Zhou; Yang Yang, Zhaohui Tang, Mingning Zhao, Zhiwei Quan; Wei Gong

No randomized controlled trial (RCT) has yet been performed to provide the evidence to clarify the therapeutic debate on liver resection (LR) and radiofrequency ablation (RFA) in treating colorectal liver metastases (CLM). The meta-analysis was performed to summarize the evidence...
Symptoms, Diagnosis, and Treatment

This information is provided by Genetics Home Reference.


Full Results at Genetics Home Reference: 89 matches

There are currently no related results available in GeneReviews.

This information is provided by Genetic Testing Registry.


Clinical Trial Information

This information is provided by ClinicalTrials.gov

**Sorafenib Tosylate With or Without Doxorubicin Hydrochloride in Treating Patients With Locally Advanced or Metastatic Liver Cancer**

Status: Recruiting
Condition Summary: Adult Primary Hepatocellular Carcinoma; Advanced Adult Primary Liver Cancer; Localized Unresectable Adult Primary Liver Cancer; Recurrent Adult Primary Liver Cancer

Last Updated: 25 Feb 2013

**MK2206 in Treating Patients With Advanced Liver Cancer That Did Not Respond to Previous Therapy**

Status: Recruiting
Condition Summary: Adult Primary Hepatocellular Carcinoma; Advanced Adult Primary Liver Cancer; Localized Unresectable Adult Primary Liver Cancer; Recurrent Adult Primary Liver Cancer

Last Updated: 4 Mar 2013
Liver cancer

To find out how your experience compares with others about this condition Click Here

Common Name(s)
Liver cancer, Cancer, Liver

Hepatocellular carcinoma is the major histologic type of malignant primary liver neoplasm. It is the fifth most common cancer and the third most common cause of death from cancer worldwide. The major risk factors for HCC are chronic hepatitis B virus (HBV) infection, chronic hepatitis C virus (HCV) infection, prolonged dietary aflatoxin exposure, alcoholic cirrhosis, and cirrhosis due to other causes. Hepatoblastomas comprise 1-2% of all malignant neoplasms of childhood, most often occurring in children under 3 years of age. Hepatoblastomas arise from undifferentiated hepatocytes ((31;Taniguchi et al.;)

Source: Online Mendelian Inheritance in Man

Advocacy and Support Organizations

Condition Specific Organizations
Reg4ALL

Information Highway

Disease Specific Data Elements
Common Data Elements
Gamified Survey

Trust
Education
Engagement

Disease InfoSearch
Powered by Genetic Alliance

Reg4ALL.org
Engagement

Education

Trust

Information Highway

Disease Specific Data Elements

Common Data Elements

Gamified Survey

Reg4ALL.org
Common Data Elements

Measure the same thing
The same way
Across all diseases
Disease Specific Elements

Detailed disease information

• Signs
• Symptoms
• Progression
• Adverse reactions
The Problem

The Internet makes it so easy for us to share parts of our lives with the people we care about and to keep track of the information we use everyday to help our lives run smoothly. From social networks to online banking, our lives have slowly made their way online.

Why isn't there a safe place to keep track of our health information online?

And what if we could use that place to share our information with the medical community, the people who can make a difference for the health of our friends, families, communities, and, not to mention, us?
The Solution

Reg4ALL is a powerful tool designed to help you find better treatments for your health conditions, to inspire you to take control over your health in a new way, and to make it simpler to participate in relevant research.

This means sharing some of your health information. Reg4ALL understands that you might have a unique opinion when it comes to sharing that information, and so it respects and celebrates that.
Reg4ALL lets everybody decide how much they want to share and with whom they want to share it.

The potential of this technology is endless, but the first goal of Reg4ALL is to revitalize clinical trials. Only 5% of cancer patients in the United States participate in clinical trials - imagine how much progress could be made if more of us work together in a way that makes it easy and safe to participate.

Reg4ALL is that simple and safe path to participation.
The People Behind Reg4ALL

Reg4ALL is the brainchild of Genetic Alliance, a non-profit leader in health and patient advocacy, and Private Access, a pioneer of modern privacy technology. This partnership arose from years of discussion on how to best serve the public need for research and sharing of health data, while still protecting people’s right to privacy.

Genetic Alliance is dedicated to providing the best possible solutions to improve public health. A partner of thousands of disease advocacy organizations, GA works hard to support both the needs of those struggling with poor health, and those hoping to maintain good health for themselves and their loved ones.

Private Access is the incarnation of its founder’s vision for a healthcare community that supports every individual’s unique needs for privacy and care. PA has a vested interest in ensuring that individuals have access to their health information and can choose who can see it and who can use it.
How it works

Reg4ALL contains simple and engaging surveys that ask general questions about your health and medical conditions. Then it lets you set specific privacy settings for the information you report.
Within those privacy settings, you can choose to share everything with everyone, share some things with some people, or share nothing with no one, and all variations in between. The technology is designed to replicate how you feel about your privacy, as simple or as complicated as that may be.
Based on your privacy settings, Reg4ALL can help connect you with medical researchers, disease advocacy organizations, and other helpful resources.

All of these connections will work for and with you to create the best possible healthcare solutions for you and for your community.
TrialsFinder.com

Relief is when the right researcher and you find each other TrialsFinder®

Finding the right clinical trial for your diagnosis can be painstaking. With TrialsFinder, enter your data privately once and have researchers find you instead.

Start Here

We want to hear from you! Your feedback and suggestions are very important. Please email us with your comments.

Help speed up cures for thousands of diseases by sharing TrialsFinder® with everyone you know:
Coming Soon

- Full featured data access choices (Q1)
- Prior data transfer – electronic and paper (Q2)
- EMR connections (Q3)
Onward to the WE

N = They

N = Me

N = WE
Acknowledgements

- Health Resources Services Administration/Maternal and Child Health/Genetic Services Branch
- Sanofi US
- Genzyme
- Private Access
- Traitwise
- The Leona M. And Harry B. Helmsley Charitable Trust
Q&A

Dana Ball
CEO
T1D Exchange

Sharon Terry
President and CEO
Genetic Alliance

Moderator:
Margaret Anderson
Executive Director
FasterCures
View an archive of this Webinar
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connect