

FasterCures Webinar Series Presents:

**Collaboration in Action:
The Learning Collaborative™**
Gaining knowledge. Maximizing value.

March 21, 2012

The logo for FasterCures is located at the bottom of the slide. It features a stylized wave graphic with a blue base, an orange middle section, and a grey top section. The text "FasterCures" is written in a blue, sans-serif font across the orange and grey sections of the wave.

FasterCures

MEDICAL PROGRESS

COLLABORATION

PARTNERING FOR CURES

- Pharma
- Investors
- Foundations
- Biotech
- VCs
- Academic
- Gov't
- Patients
- Philanthropists

Research analysis

Investigations

INNOVATION

• TRAIN

TRAIN CENTRAL STATION

briefings

webinars

BEST PRACTICES

CAPITAL

NEW FUNDING MODELS

METRICS

PHILANTHROPY TOOLKIT

ROI

EFFICIENCY

FASTER CURES

Align research priorities with our needs!

PATIENTS

PATIENTS HELPING DOCTORS
EMPOWER

CLINICAL TRIALS
H.I.T.
BIOBANKS
THINK RESEARCH
DATA
INCENTIVIZE PATIENT PARTICIPATION

PROCESS & POLICY

• FDA

• more resources
• regulatory science
• molecule to marketplace

ADVOCACY

DIALOGUE

• NIH

NCATS
DISCOVERY INTO HEALTH

CAN

TRANSLATIONAL RESEARCH

CROSSING THE VALLEY OF DEATH

VALLEY OF DEATH

• LEADERSHIP FORUM

Collaboration in Action

Presenters



Christopher P. Austin, MD
Director, Division of
Preclinical Innovation
National Center for
Advancing Translational
Sciences
National Institutes of Health



Louis DeGennaro, PhD
Chief Mission Officer
Leukemia & Lymphoma
Society



Scott J. Weir, PharmD, PhD
Director, Institute for
Advancing Medical
Innovation
University of Kansas
Cancer Center

Moderator



Kristin Schneeman
Program Director
FasterCures

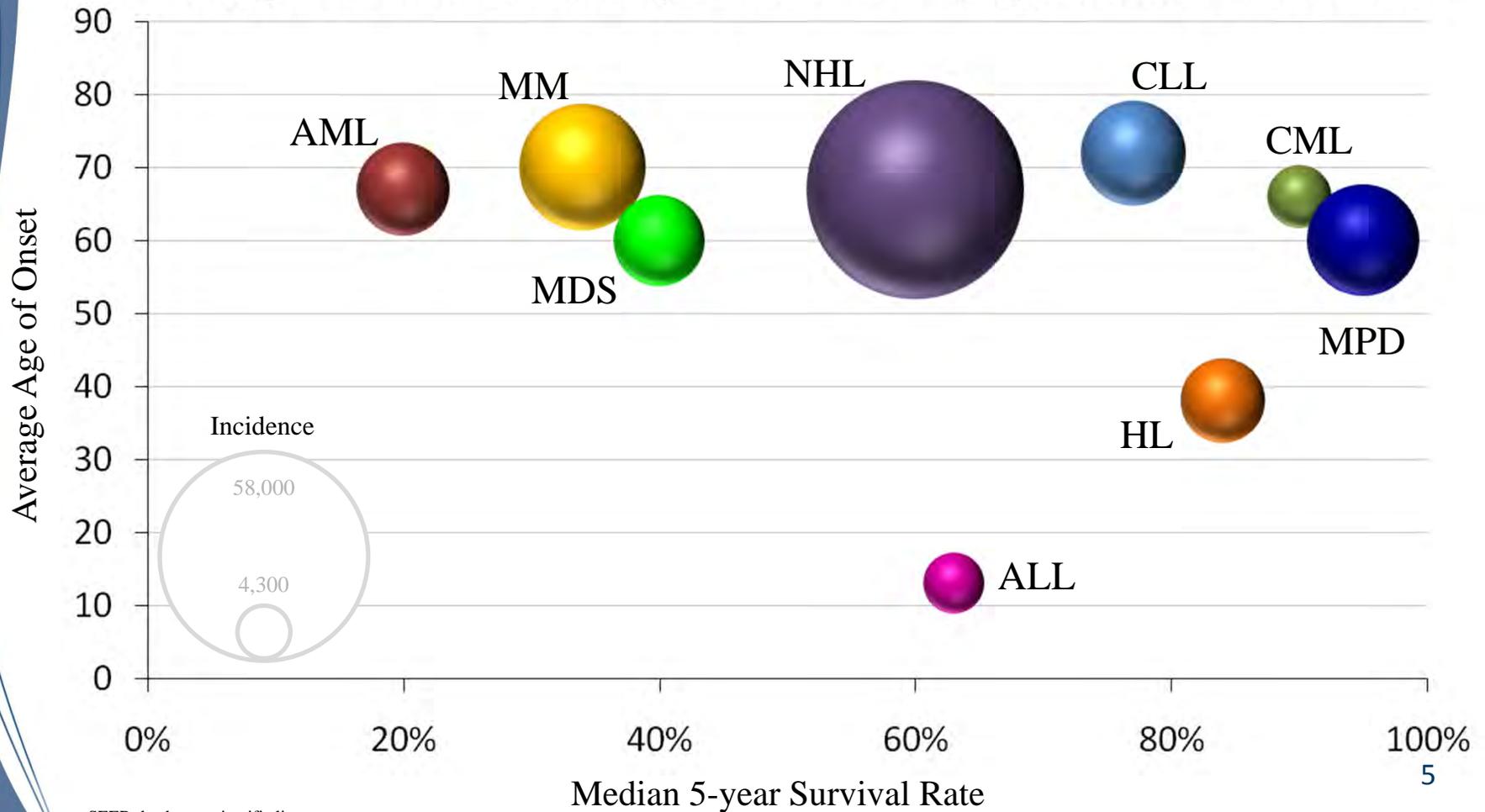


Presentation Outline

- Complexity of the Disease Set
- Patient Mission
- The Learning Collaborative™
 - Capitalizing on Strengths
 - Therapeutic Strategy
 - Organization
 - Auranofin for the Treatment of CLL
- What Have We Learned?
- What Will Be Important?
- Take Home Points

Confronting a Broad Spectrum of Diseases With Diverse Outcomes

Comparison of Diseases by Survival Rate, Age of Onset & Incidence



SEER database, scientific literature



Delivering the LLS Mission

- Comprehensive Patient Services
 - Local, National & International footprint
 - Education, support groups, financial aid
- Proven Research Strategy
 - 400 cutting-edge research projects funded world-wide
- State & Federal Public Policy Initiatives
- **A sense of urgency**

Capitalizing on Strengths

Discovering and developing drugs for the treatment of rare hematological malignancies



- **Bench to bedside translation in drug repurposing**
- **National leadership in medicinal and pharmaceutical chemistry**
- **Pharma experience**



- **Focus on rare and neglected diseases**
- **Industrial scale HTS, medicinal chemistry, and bioinformatics capabilities**
- **Pharma experience**



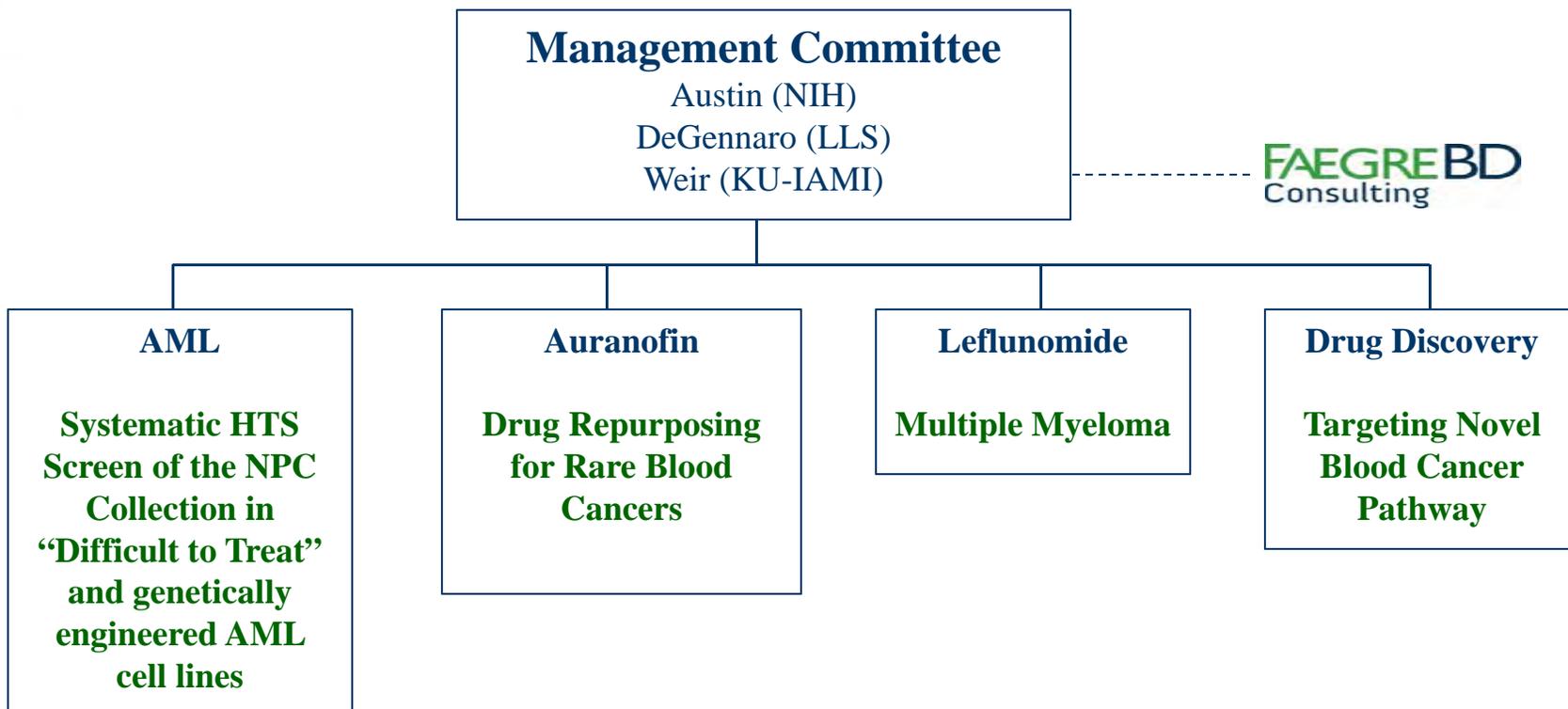
- **~ 400 active research projects**
- **World-wide network of blood cancer experts**
- **Track record of commercial partnerships**
- **Pharma experience**

Drug Discovery and Development Strategy

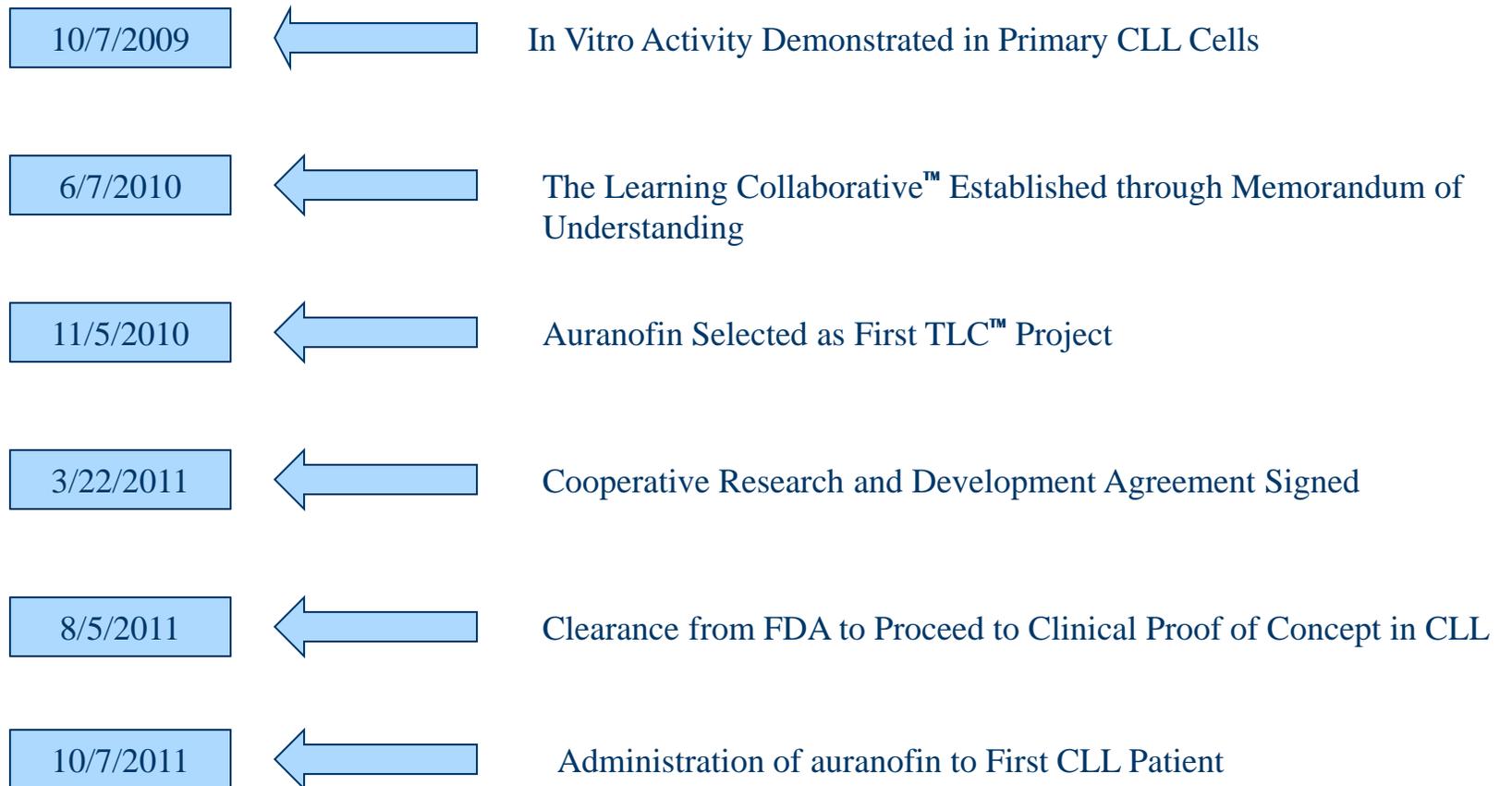


- Translate basic discoveries into blood cancer therapies from multiple sources
- Formal project selection process
- Empowered multi-disciplinary, multi-organizational teams
- TLC™ Management Committee obtains project funding from multiple sources
- Project teams define and execute approved “de-risking” project plans
- Proactively and prospectively define exclusivity and reimbursement strategies
- Goal is to advance projects to clinical proof of concept
- Seek and engage industry partners along the way
- LLS leads licensing efforts targeting its network of commercial partners

The Learning Collaborative™ Organization



Auranofin Project Timeline





Auranofin Project Plan

Distinguishing Elements

- In vitro proof of concept directly into patients
- Parallel activities to accelerate bench-to-bedside translation
- Strong industry-like project management function
- Multi-center clinical proof of concept trials: University of Kansas Cancer Center, NHLBI, and The Ohio State University to accelerate patient recruitment
- Correlative studies conducted at three clinic sites
- Centralized clinical pharmacology support
- Supportive drug development activities
- Cataloging “Learnings”

“What have we learned?”

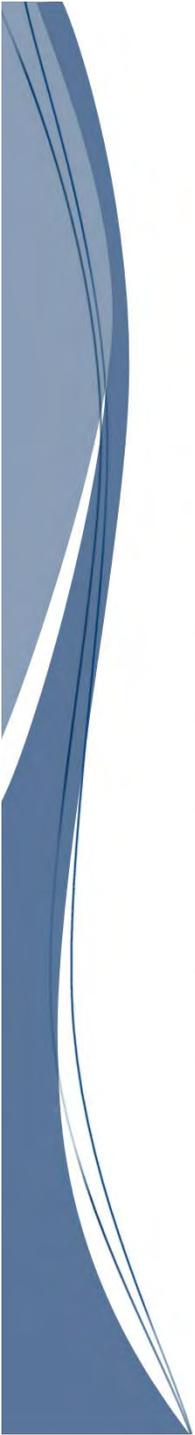
It's About Patients!

- *Fast into patients*
- Determine clinical proof of concept within 14 months
- National visibility has resulted in patients contacting us directly expressing interest in participating
- Patients extremely knowledgeable of their disease
- Evaluating auranofin in other blood cancers

“What have we learned?”

Defining the Collaboration is Important

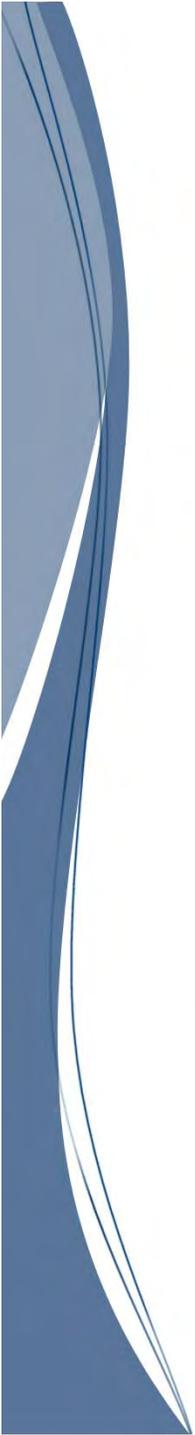
- Memorandum of Understanding sets collective objectives and manages expectations
 - Roles and responsibilities
 - TLC™ Management Committee
 - Project selection process
 - Funding strategies
 - Formation of project teams
 - Data sharing
 - Intellectual property management



“What have we learned?”

Cooperative Research and Development Agreement (CRADA)

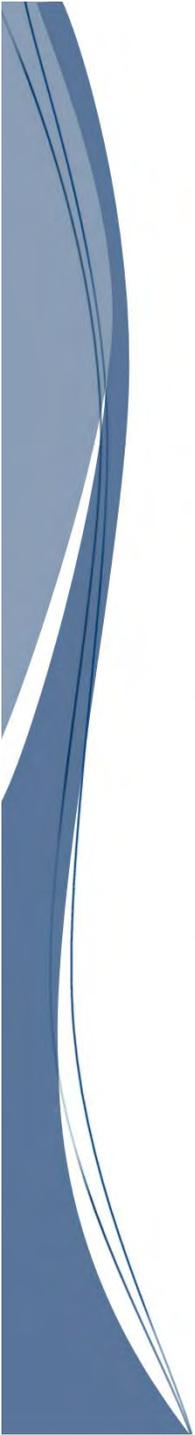
- First step in establishing first project
- Unique agreement with non-profit partner
- Defines resources and expertise each collaborator brings to the TLC™
- Demonstrates the capacity of LLS to commercialize
- Leverage



“What have we learned?”

Project Management is Critical

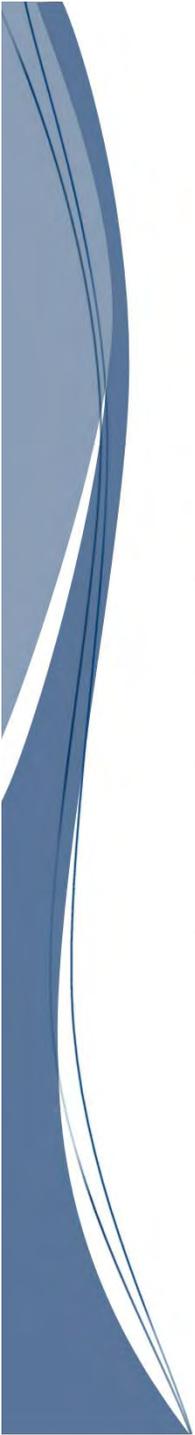
- Industry quality project management at NIH, LLS and KU
- Manage all activities and across collaborating organizations
- Lead teams to define project plans, go/no go decision points, pre-defined go/no go decision criteria
- Escalate issues to TLC™ Management Committee
- Capture “Learnings”



“What will be important?”

Gaining Knowledge. Maximizing Value.

- Auranofin project supported by NIH, LLS, philanthropic and economic development funding sources
- Rapid results lead to philanthropic funding opportunities
- “Marrying” funding sources (and restrictions) to support specific project activities
- Integrate technology transfer into teams
- Defining, capturing and maximizing exclusivity path(s) to interest for-profit partners
- Address regulatory science issues that impact the repurposing of patent and/or abandoned drugs



“What will be important?”

Integration of Technology Transfer

- Technology transfer experts must be integrated into teams
- Critical to development and execution of CRADA's
- Becomes more complex as more academic institutions get involved; new culture, new shared objectives
- Optimizing the leverage of therapeutic use data



“What will be important?”

Regulatory Science

- Each project is unique, but in general, projects employ one or two “common” strategies
 - Therapeutic indication
 - ◆ Related indication (e.g., blood cancer indication leads to study in solid tumors)
 - ◆ Unrelated indication (e.g., auranofin, FDA approved arthritis agent, currently in use, for the treatment of CLL)
 - Improved delivery
 - ◆ Elimination of excipients associated with safety issues
 - ◆ Different route of administration
 - ◆ Combination products
 - ◆ Overcome pharmacokinetics issues
- 505(b)2 path
- Accessing data generated by innovator firms



“What will be important?”

Defining Exclusivity Path(s) and Reimbursement Strategies

- Difficulties in establishing exclusivity for approved drugs has deterred industry from drug repurposing
- Requires multiple, innovative approaches integrated into one comprehensive strategy
- Regulatory science plays a critical role in defining
- It's never too early to develop reimbursement strategies
- May require public policy initiatives to encourage drug repurposing for rare and neglected diseases
- ValueMaP™ (“Value Maximization Path”) under development



Take Home Points

The Value of Collaboration

- Accelerating new treatments to patients suffering from rare blood cancers
- Power of partnership between government, disease philanthropy and academic organizations
- Sustainable model supporting multiple projects
- The model is:
 - Scalable to support a portfolio of projects
 - Applicable across disease areas
 - Replicable by organizations with a commitment to collaboration, shared vision and mission

Q&A

Presenters



Christopher P. Austin, MD
Director, Division of
Preclinical Innovation
National Center for
Advancing Translational
Sciences
National Institutes of Health



Louis DeGennaro, PhD
Chief Mission Officer
Leukemia & Lymphoma
Society



Scott J. Weir, PharmD, PhD
Director, Institute for
Advancing Medical
Innovation
University of Kansas
Cancer Center

Moderator



Kristin Schneeman
Program Director
FasterCures

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CENTRAL STATION
A platform for venture philanthropy in medical research

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TRANSLATIONAL RESEARCH

Download “**Crossing Over the Valley of Death**” on the importance of translational research, and “**Trends in Translation**,” which highlights models of collaboration in early-stage R&D.

Crossing Over the Valley of Death

Trends in Translation

TRAIN Central Station Features

TRAIN E-News	Blog	Discussion Board
Epilepsy foundations collaborate on new therapy commercialization grant program	01/06/12: Top 10 Medical Research Trends to Watch in 2012	Send us your questions for NIH Director Francis Collins!
NIH debuts site about clinical trial participation	12/20/11: Value of Establishing the NIH Center on Advancing Translational Sciences to Patients	What Metrics Matter Most in Nonprofit Medical Research?

WHAT'S NEW AT TRAIN

Events

- Apr. 18 Webinar: Engaging with FDA
- March 21 Webinar: Collaboration in Action
- Feb. 27 Webinar: View archive of the Virtual Town Hall with NCATS Leadership

Case Studies

- Michael J. Fox Foundation for Parkinson's Research
- Cystic Fibrosis Foundation

Tools and Resources »

Featured Publication: Back to Basics: HIV/AIDS Advocacy as a Model for Catalyzing Change

Resource: 2011 Partnering for Cures Innovator Presentations

Tool: IP & Data Sharing Principles

Current News

NIH offers in-kind resources for preclinical therapy development

INNOVATOR SPOTLIGHT

Q&A with Monica Coenraads, Executive Director, Rett Syndrome Research Trust

www.fastercures.org/index.cfm/Publications/Translational_Research_Crossing_Over_the_Valley_of_Death

Register now for the next FasterCures Webinar:

Engaging with FDA:

A Guide for Foundation Funders of Research

Apr

18

1-2 pm
(Eastern)

Patient-driven foundations that fund medical research are increasingly seeking to productively engage with the FDA, to ensure that regulators understand the needs of patients and have the knowledge they need to review and approve treatments that are important to patients. What can be achieved by building relationships with and working more closely with the FDA? What unique assets can patient groups bring to the table? How should you prepare, and what challenges can you expect?

Learn from the experiences of senior leaders of two foundations that are pioneers in engaging in product development and in working with the FDA:

- Mary B. Dwight, Vice President of Government Affairs, Cystic Fibrosis Foundation
- Cynthia Rice, Vice President of Government Relations, Juvenile Diabetes Research Foundation

REGISTER:

www.fastercures.org/train

FasterCures

PARTNERING FOR CURES



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- **PARTICIPATE** in outcomes-oriented dialogue on solving the challenges that slow medical progress
- **DISCOVER** new and scalable models for improving research efficacy and efficiency
- **PARTNER** with others who share your goals, and could advance your programs.
- **CONSULT** with strategy and regulatory experts onsite, for FREE, on tailored advice on your initiatives
- **PRESENT** your transformative multi-sector partnership/project to potential collaborators and supporters

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