

TRAIN Workshop

Boston, MA

06 June 2012

The Learning Collaborative™

What Have We Learned?

What Will Be Important?

FasterCures



Background

Capitalizing on Strengths

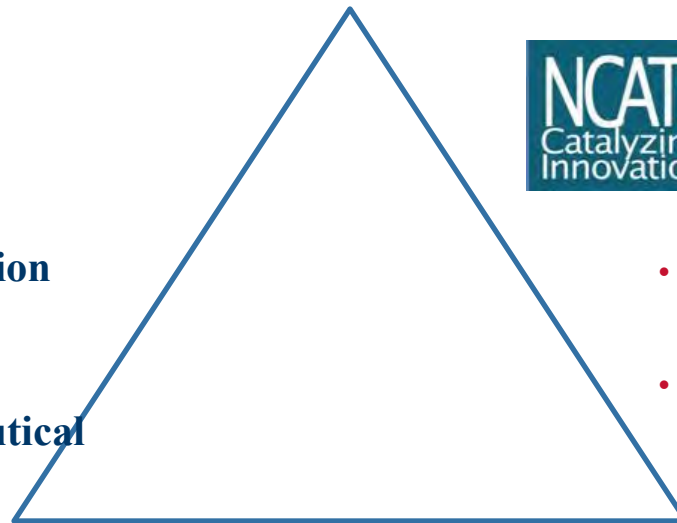
Discovering and developing drugs for the treatment of rare hematological malignancies



National Institutes of Health
NATIONAL CENTER FOR ADVANCING
TRANSLATIONAL SCIENCES

- **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**



LEUKEMIA &
LYMPHOMA
SOCIETY
fighting blood cancers

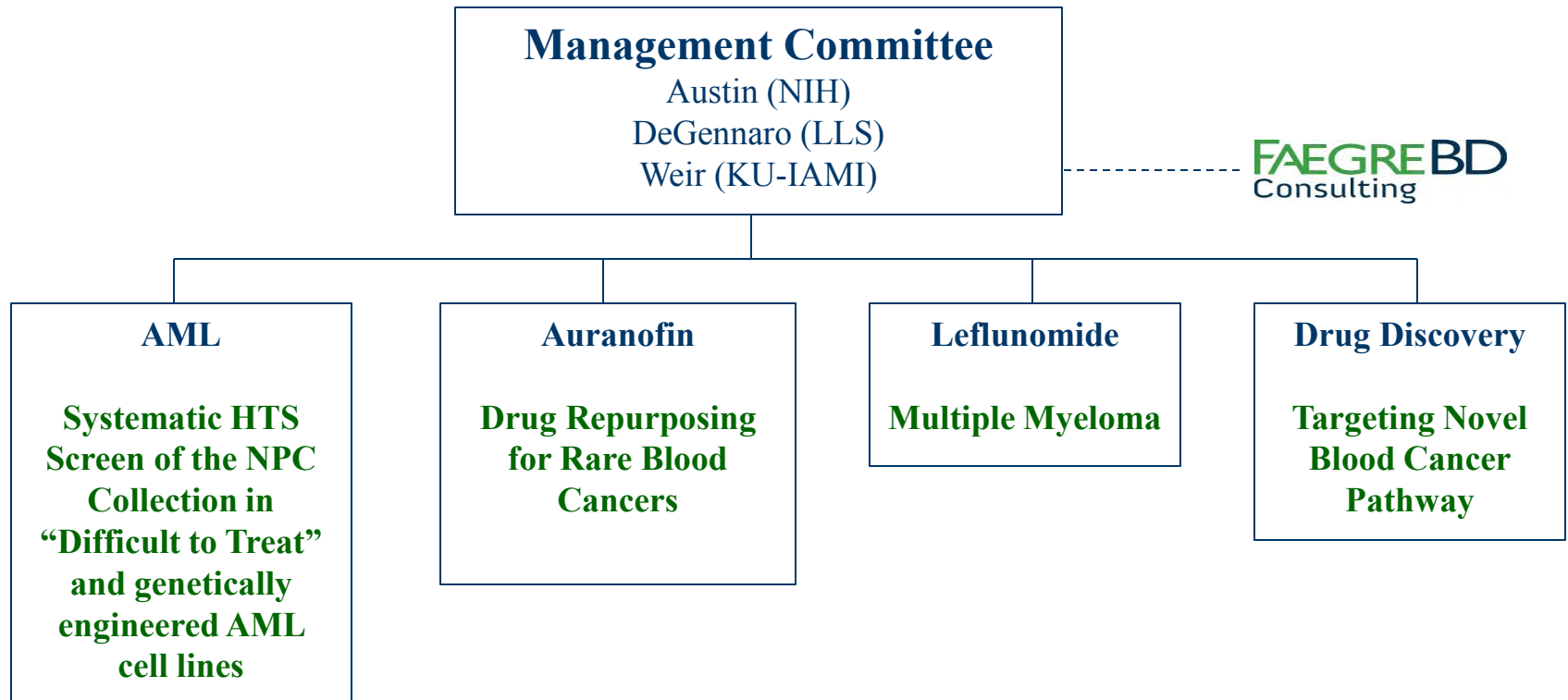
- **~ 400 active research projects**
- **World-wide network of blood cancer experts**
- **Track record of commercial partnerships**
- **Pharma experience**

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





What Have We Learned?

Defining the Collaboration is Important

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

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 is critical

Industry Best Practices

- Industry veterans within each of the partnering organizations
- Empowered project teams led by industry-experienced project managers
- Manage ALL activities 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”
- Clearly defining and managing TLC™ expectations to teams and collaborators

Gaining Knowledge and Maximizing Value

- Projects 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 approved and/or abandoned drugs
- It’s never too early to develop reimbursement strategies



What Will Be Important?

Replicating the Model

- 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
- Note that the best interests of The Learning Collaborative™ are always put before the interests of the three individual partners

What if there is no exclusivity path?

- Hypothetical Example – Approved, off patent drug product (in current marketed form) is suitable for administration in new indication
- Define
 - Minimum required study or studies to support off-label prescribing
 - Minimum data required to create reimbursement opportunity
- How do we fund these studies?
- How can we incentivize generic and/or innovator firms to support?