The Learning Collaborative™

What Have We Learned?
What Will Be Important?
Background
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

Management Committee
Austin (NIH)
DeGennaro (LLS)
Weir (KU-IAMI)

- AML
  Systematic HTS Screen of the NPC Collection in “Difficult to Treat” and genetically engineered AML cell lines
- Auranofin
  Drug Repurposing for Rare Blood Cancers
- Leflunomide
  Multiple Myeloma
- Drug Discovery
  Targeting Novel Blood Cancer Pathway
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?