The Learning Collaborative

A Unique Government-Disease Philanthropy-Academia Collaboration Model

Clinical Trials Transformation Initiative
Patient Group and Clinical Trials Expert Meeting
January 21-22, 2015
Fairmont Hotel
2401 M Street Northwest
Washington DC

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National Center for Advancing Translational Sciences at NIH
The Learning Collaborative

- National leadership in medicinal and pharmaceutical chemistry
- Product development-focused translational research
- Pharma experience
- Public-private partnerships

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

- ~350 active research projects
- ~$70M annual investment in blood cancer research
- World-wide network of blood cancer experts
- Track record of commercial partnerships
- Pharma experience
- Public-private partnerships
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**Product Development-Focused Translational Research Strategy**

- Basic Research
- Drug Discovery
- Early Stage Development
- Late Stage Development
- FDA Approval

"The Valley of Death"

The Learning Collaborative | Industry Partner

"The Bridge of Life"

Building the Bridge of Life:
- Multi-disciplinary, multi-organizational teams
- Translational research best practices
- Seek and engage industry partners
- Advance new therapies to patients
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Repurposing Auranofin for Blood Cancers

TLC MOU Signed

CLL Project Named TRND Pilot Project

2010 2011 2012 2013 2014 2015

Auranofin Activity in CLL Discovered by NIH

KUCC and University of Rochester Discover Auranofin Activity in Mantle Cell Lymphoma (MCL)

NCATS Confirms Activity in MCL

First CLL Patient Receives Auranofin at KUCC

LLS Kansas City Chapter Raises $1.1M

CRO Engaged to Establish Multicenter Trial in MCL

Screen to Patient in 11 Months
The Learning Collaborative “Learnings”

- TLC is the sponsor!
  - IND holder
  - IP
  - Patient Focus

- TLC partners versus “Collaborators”
  - Defining terms and conditions for “Collaborators”

- Clinical Proof of Concept versus Investigator-Initiated Trials
  - Input from disease expert KOL’s (secured through LLS), pharma expert consultants, and partnering CRO
  - Data package that supports partnering beyond early phase clinical trials
  - Requires recruitment of many trial sites (“Collaborators”)

- Partner with qualified CRO to manage clinical proof of concept trials from site selection through clinical study report. Qualified CRO’s may act as TLC’s regulatory agent but one of the full TLC partners (e.g., KUCC) will serve as IND holder. Costs for securing CRO support will be incurred by TLC.