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

Sitta Sittampalam, PhD

Senior Scientific Officer

National Center for Advancing Translational Sciences at NIH

The Learning Collaborative

THE UNIVERSITY OF KANSAS
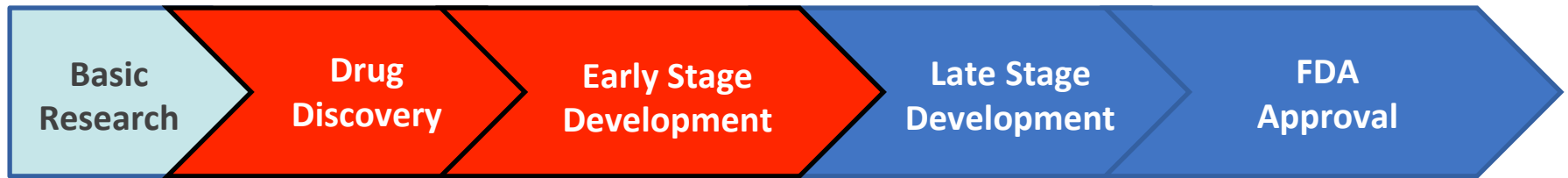The Institute for Advancing Medical Innovation

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

The Learning Collaborative™

NIH
National Center for Advancing Translational Sciences

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

LEUKEMIA & LYMPHOMA SOCIETY®
fighting blood cancers

- ❑ ~ 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"

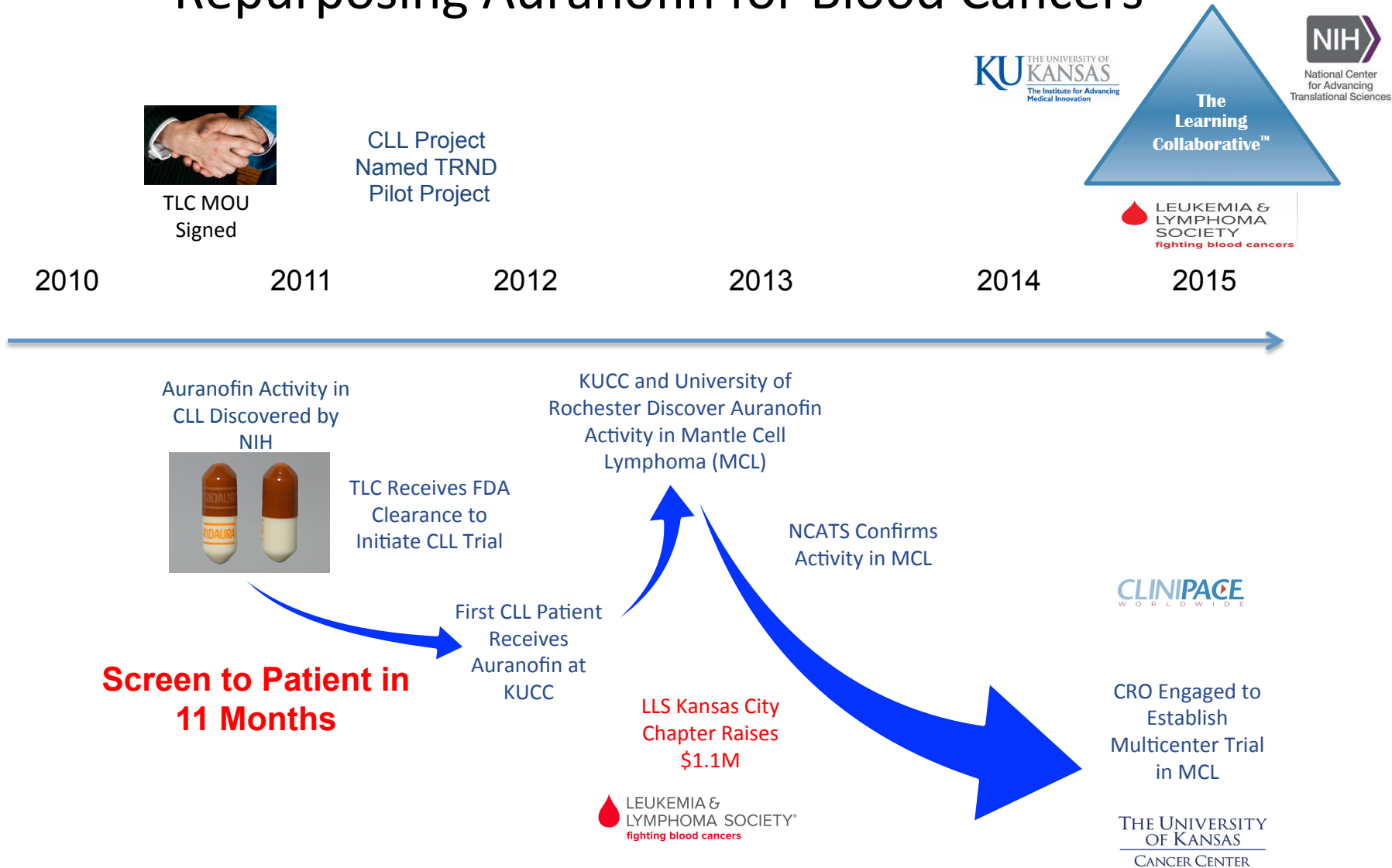| The Learning Collaborative	Industry Partner

"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

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2010   2011   2012   2013   2014   2015

Auranofin Activity in CLL Discovered by NIH

TLC Receives FDA Clearance to Initiate CLL Trial

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

Screen to Patient in 11 Months

LLS Kansas City Chapter Raises $1.1M

CRO Engaged to Establish Multicenter Trial in MCL

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.