

GRANDRx

*Gateway to **R**are and **N**eglected **D**isorders **T**herapeutics*

Introduction and Overview

International Congress on Rare Disorders
19 March 2010

Sharon F. Terry, MA
President & CEO
Genetic Alliance

Transforming Health Through Genetics



Network of 10,000 organizations, universities and companies

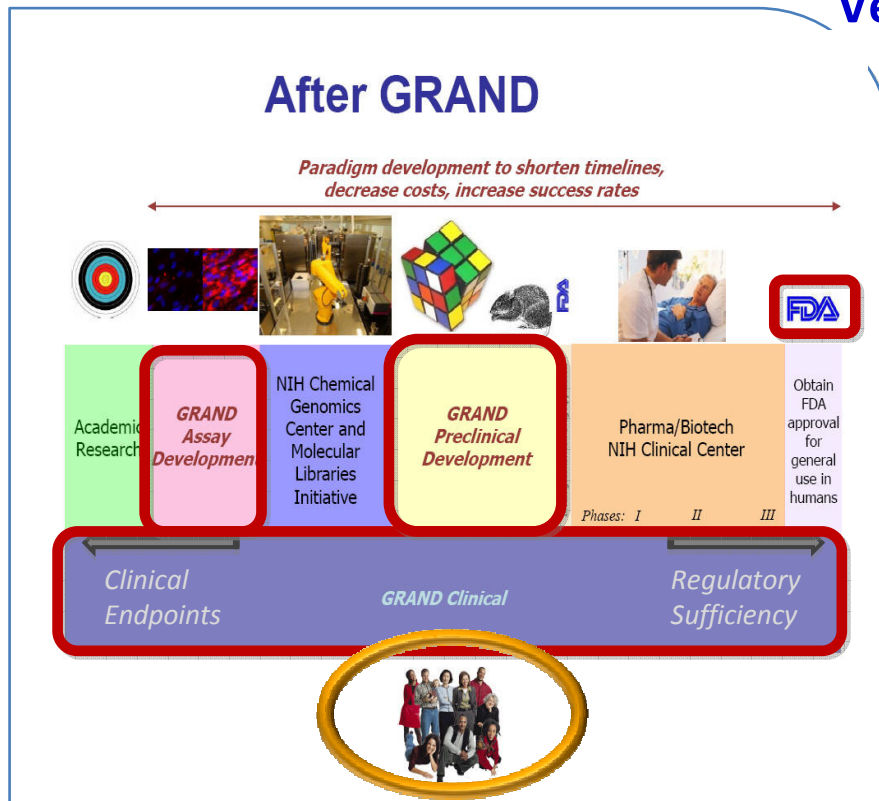
Openness is our product and process

Shared infrastructure to transform health is
our goal

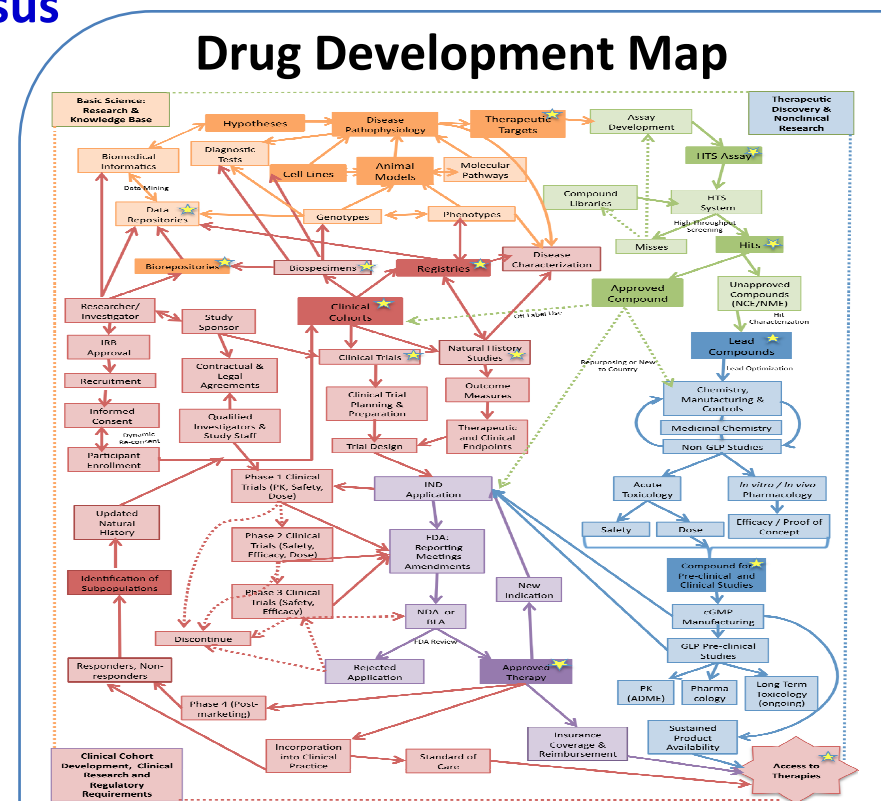
1200 disease groups, 5000 diseases

Drug Development Process

Versus

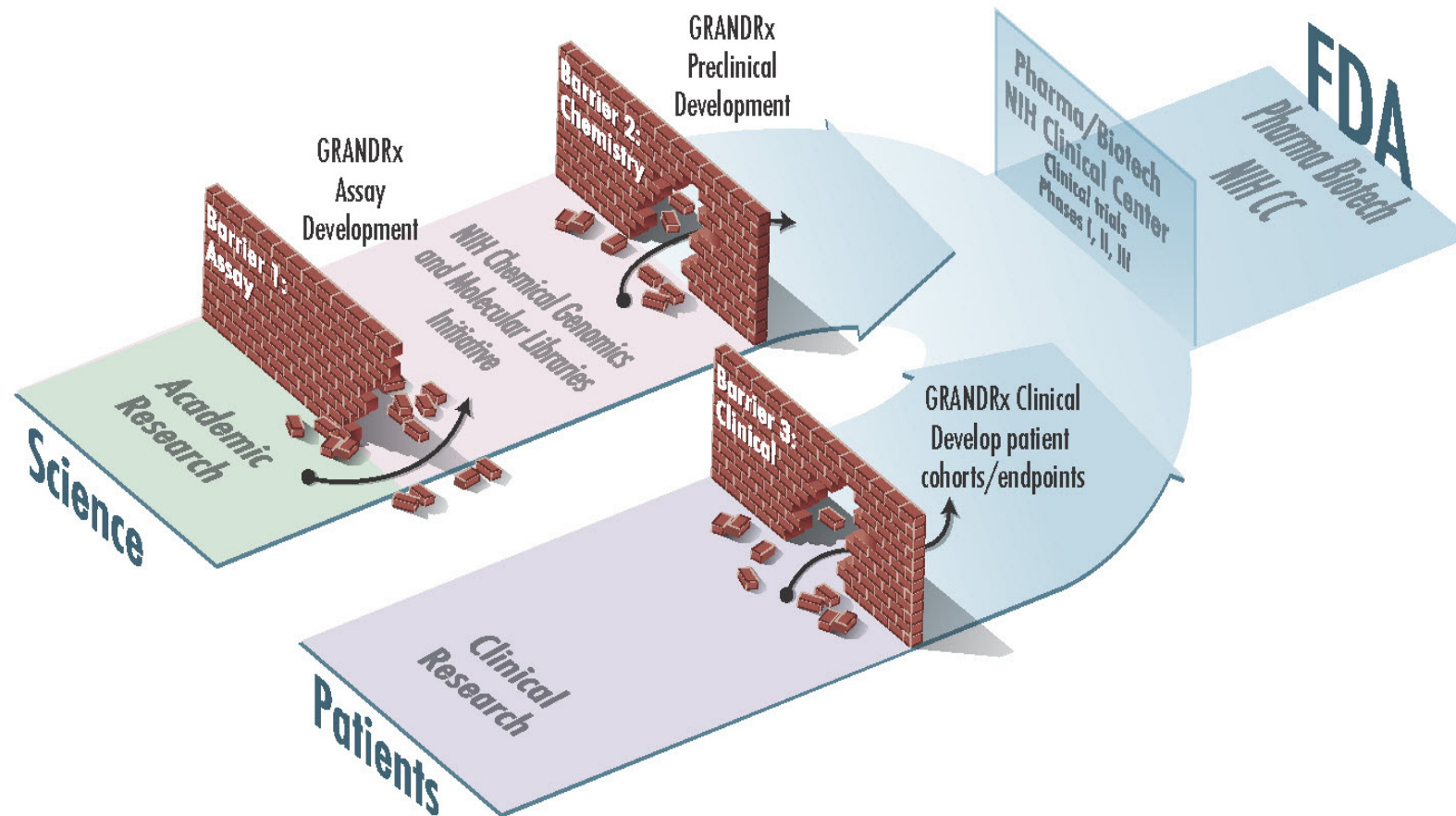


- Usually represented as a pipeline
- Barriers in this “pipeline” model are represented with Brick Walls
- These barriers are the cause of the current “Valley of Death” associated with Drug Development

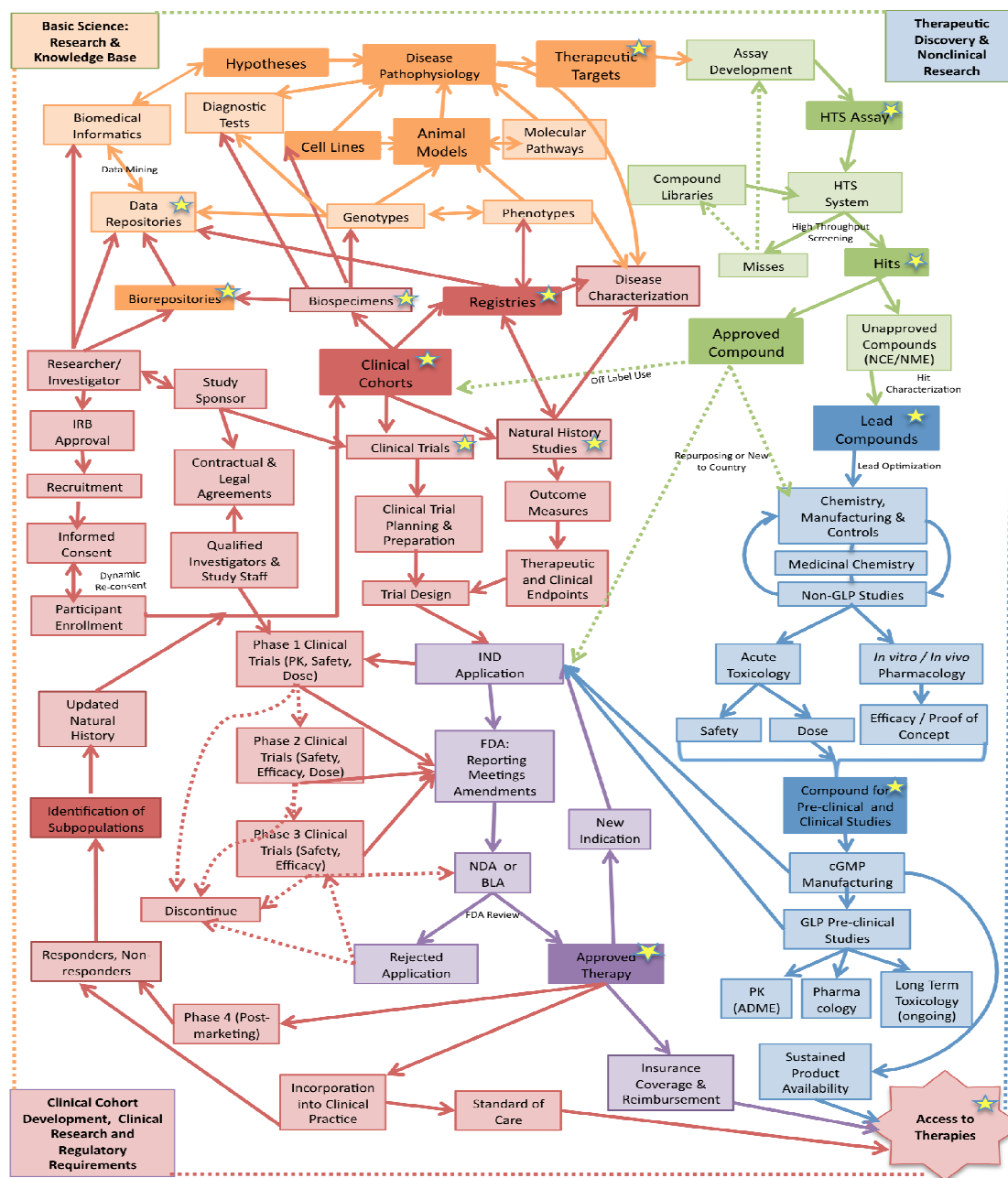


- Takes into consideration many entry points
- Shows the complicated connections between development areas
- Shows each separate area cannot exist in its own vacuum and be successful

The Brick Wall Barriers

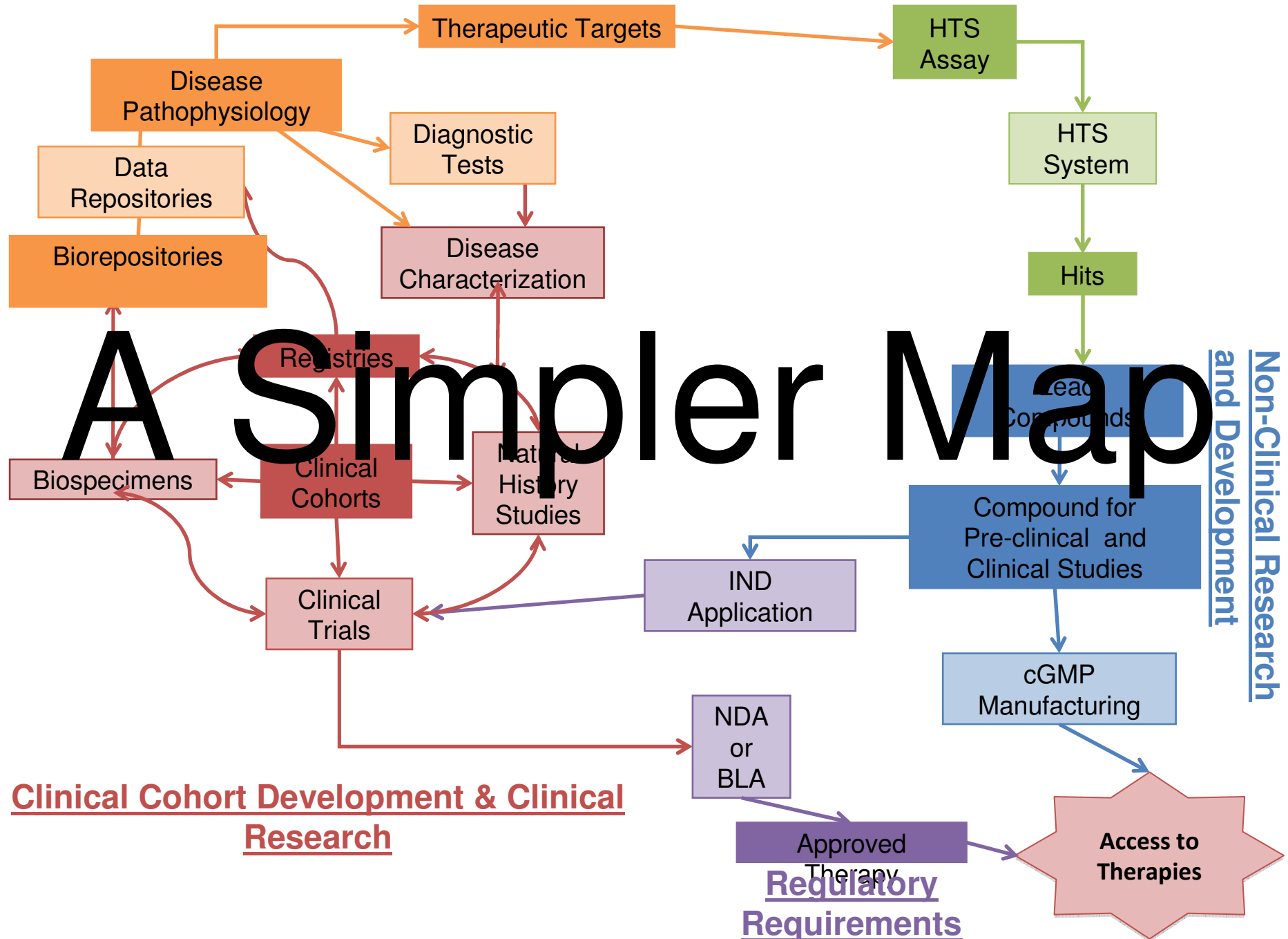


Drug Development Map – A Closer Look



Basic Science: Research & Knowledge Base

Therapeutic Discovery





Genetic Alliance BioBank

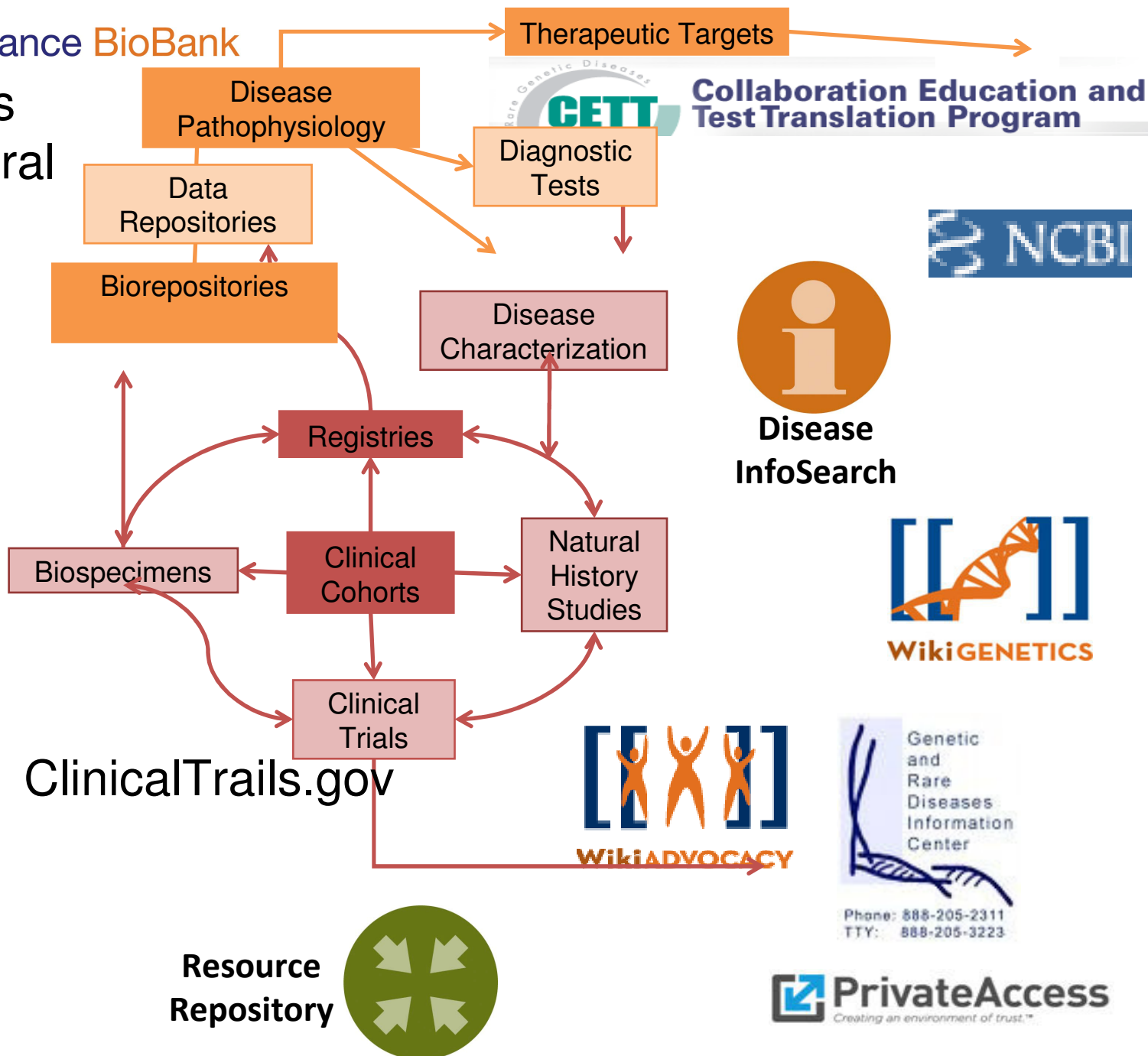
FasterCures
Biobank Central



Family Health
History



Access to
Credible
Genetics
Resources
Network

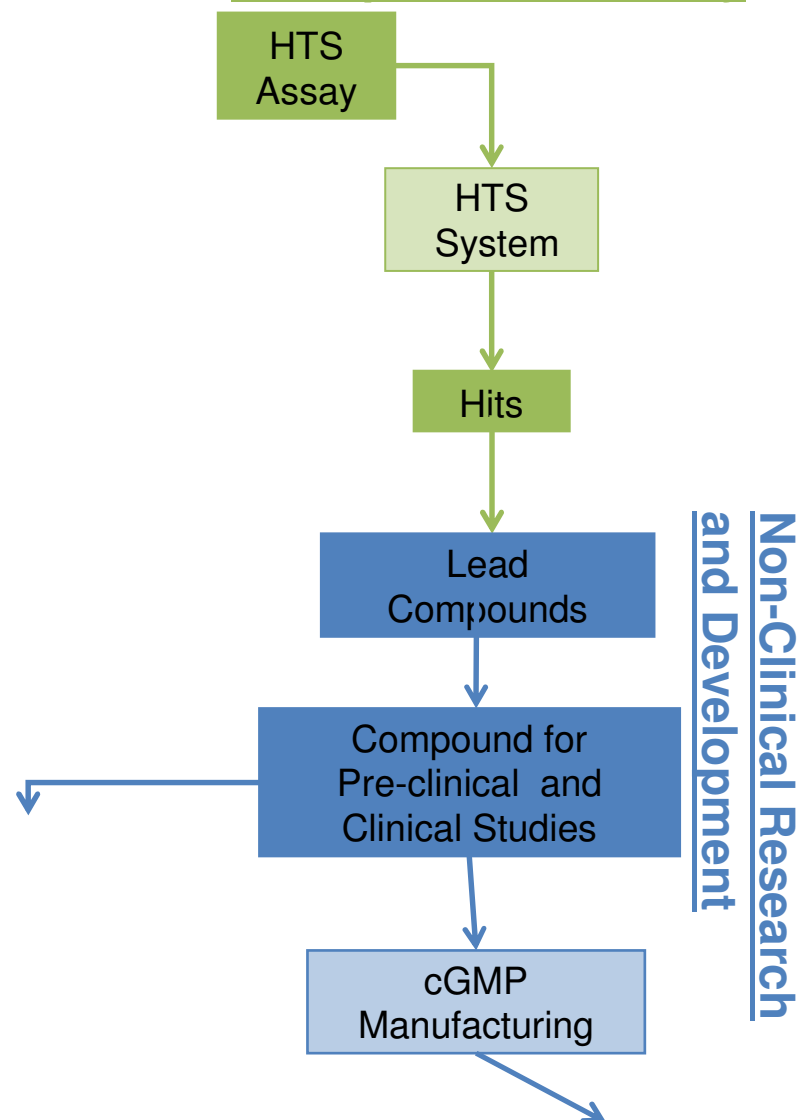


Therapeutics for
Rare and
Neglected
Disorders
(TRND)
ORDR/NIH

Assay
Bootcamp



Therapeutic Discovery



GRANDRx

Gateway to Rare and Neglected Disease Therapeutics

Steering Committee: FasterCures, Foundation Fighting Blindness, Genetic Alliance NORD, Project R.A.R.E.

Membership: Rare and Neglected Disease Stakeholders From Academia, Advocacy, Industry, Federal Health Agencies, and Others

Workgroups: Assay, Preclinical, Clinical, Regulatory and Policy

Partnerships

Programs

Initiatives

Projects

**Systems
Change**

Federal Agencies

National Institutes of Health (LOS)

National Human Genome Research Institute

Office of Rare Disease Research

NIH Chemical Genomics Center and
Therapeutics for Rare and Neglected
Disorders

Food and Drug Administration

**Centers for Disease Control and
Prevention - NOPHG**

Academia, Advocacy, Industry, Others: meetings

Amgen, AMRI, BioFocus, DPI, BioMarin
Pharmaceutical, Celgene Corporation, ChanTest
Corporation, Chordoma Foundation, CollabRX,
DNDi, FasterCures, FoldRx Pharmaceuticals,
Genzyme Corporation, Michael J. Fox Foundation
for Parkinson's Research, Muscular Dystrophy
Association, National Marfan Foundation, One
World Health, Optherion, Parent Project Muscular
Dystrophy, Pfizer, PTC Therapeutics, PXE
International, Therapeutics for Rare and Neglected
Disease, Third Rock Ventures, Vertex
Pharmaceuticals

**Treatment
Advances**

The system is perfectly designed to produce the results it is now producing.

Leadership is about shaping an unknown future.

And a challenge is *generatively complex* when its future is fundamentally unfamiliar and undetermined: such challenges cannot successfully be addressed by applying “best practice” solutions from the past, but only by growing new, “next practice “ solutions.

Until we break down the boundaries between our organizations we will not change the paradigm. We must function as one, focused on what matters most: health.

To create this profound change, we must transform ourselves personally to be without ego and to celebrate each other's successes. We must share everything.