GRANDRx

Gateway to Rare and Neglected Disorders Therapeutics

Introduction and Overview

International Congress on Rare Disorders
19 March 2010

Sharon F. Terry, MA
President & CEO
Genetic Alliance
Transforming Health Through Genetics

Openness is our product and process

Shared infrastructure to transform health is our goal

1200 disease groups, 5000 diseases
**Drug Development Process**

- 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

**Versus**

- 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
Assay Bootcamp

Therapeutic Discovery

- HTS Assay
- HTS System
- Hits
- Lead Compounds
- Compound for Pre-clinical and Clinical Studies
- cGMP Manufacturing

Non-Clinical Research and Development

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

Assay Bootcamp
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, Ophtherion, Parent Project Muscular Dystrophy, Pfizer, PTC Therapeutics, PXE International, Therapeutics for Rare and Neglected Disease, Third Rock Ventures, Vertex Pharmaceuticals

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

Treatment Advances

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

Treatment Advances

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, Ophtherion, Parent Project Muscular Dystrophy, Pfizer, PTC Therapeutics, PXE International, Therapeutics for Rare and Neglected Disease, Third Rock Ventures, Vertex Pharmaceuticals
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.

From Power and Love, by Adam Kahane
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.