Therapeutics for Rare and Neglected Diseases (TRND) & Bridging Interventional Development Gaps (BrIDGs)

Nora Yang, Ph.D.
Senior Scientist, TRND & BrIDGs
Director, Portfolio Management and Strategic Operations
Division of Pre-Clinical Innovation
TRND & BrIDGs

Translational Science Issue:
- There is the “Valley of Death” when developing a therapy
- Rare Diseases are “orphans” when it comes to developing therapies
TRND & BrIDGs

Translational Science Issue:

• There is the “Valley of Death” when developing a therapy
• Rare Diseases are “orphans” when it comes to developing therapies

TRND & BrIDGs Programs: goals and objectives

• Collaborate with external scientists and physicians to fast-track promising therapies that are stuck in the “Valley of Death”
• Provide in-kind funding ($30MM annually) and scientific know-how (team of experienced ex-industry therapeutic developers) to advance promising therapies
TRND & BrIDGs:
Engagement with patient partners

One of many examples of patient partner engagement with TRND

Disease: Duchenne muscular dystrophy (DMD)

TRND Collaborator

Therapy: VBP15 (vamorolone)
TRND & BrIDGs: Engagement with patient partners

One of many examples of patient partner engagement with TRND

How did Muscular Dystrophy Association (MDA) help?
• Awarded ReveraGen grants to conduct a key study in mice
• Raised clinical trial funding along with a consortium of DMD advocacy groups
• Helped recruit DMD boys for phase 2 clinical trial

Results:
• Accelerated the timeline to pass the “valley of death”
• Enabled clinical trial so there is no delay in development

Benefits for TRND & BrIDGs

Patients participation:
• Enhances our understanding of the disease – particularly for rare diseases
• Enables clinical trials for rare diseases – patient recruitment & clinical endpoint definition
• Provides additional resources and expertise to accelerate developments
• Motivates scientists

Stop by our poster!