

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

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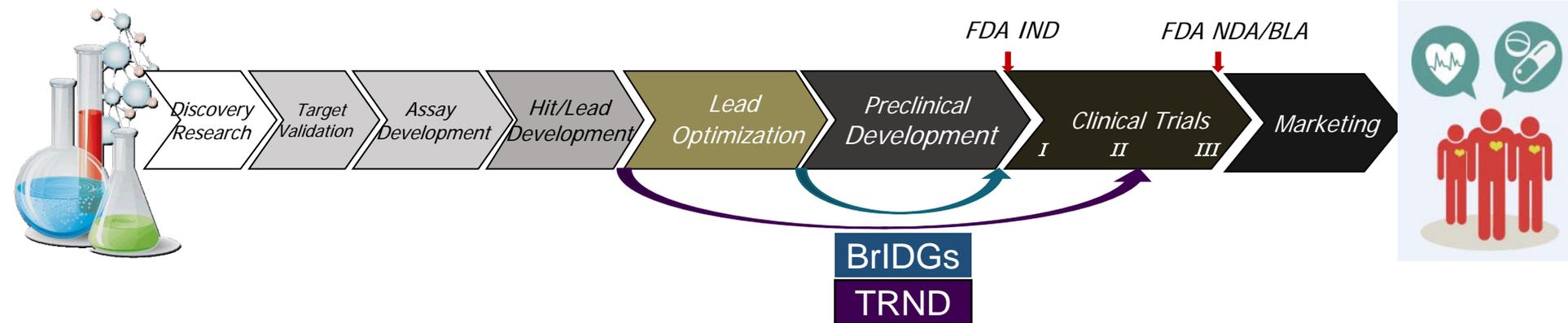
Division of Pre-Clinical Innovation



TRND & BrIDGs

Translational Science Issue:

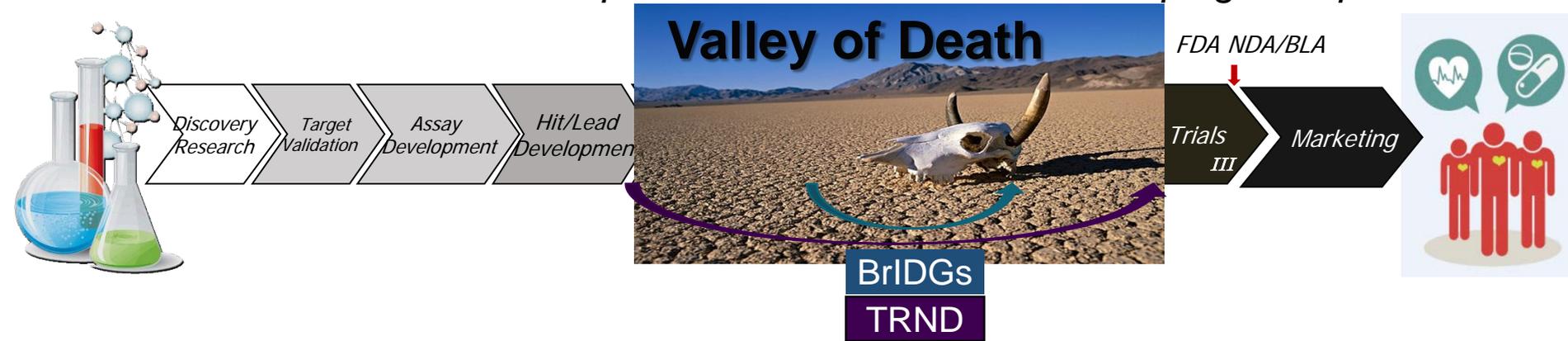
- There is the “Valley of Death” when developing a therapy
- Rare Diseases are “orphans” when it comes to developing therapies



TRND & BrIDGs

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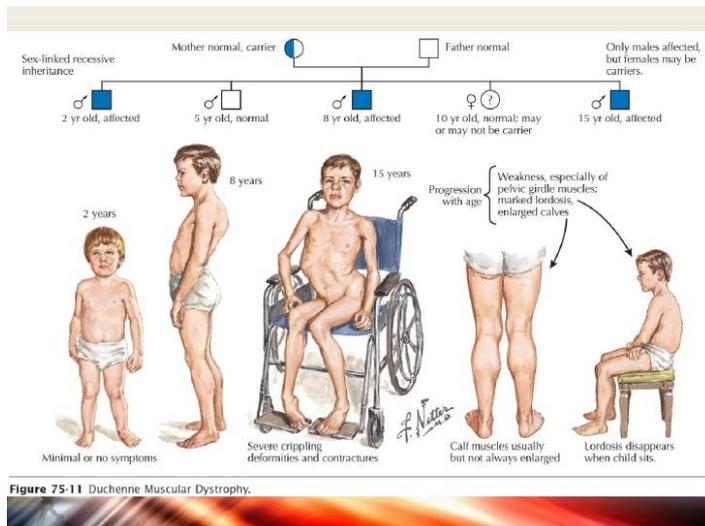
TRND & BrIDGs Programs: goals and objectives

- 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



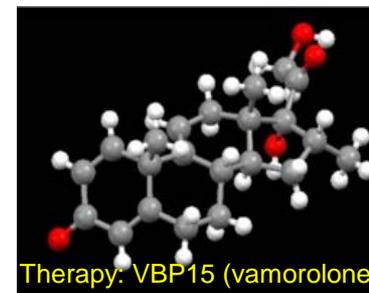
TRND & BrIDGs: Engagement with patient partners

One of many examples of patient partner engagement with TRND



Disease: Duchenne muscular dystrophy (DMD)

ReveraGen
BioPharma
TRND Collaborator



NIH National Center
for Advancing
Translational Sciences

TRND & BrIDGs: Engagement with patient partners

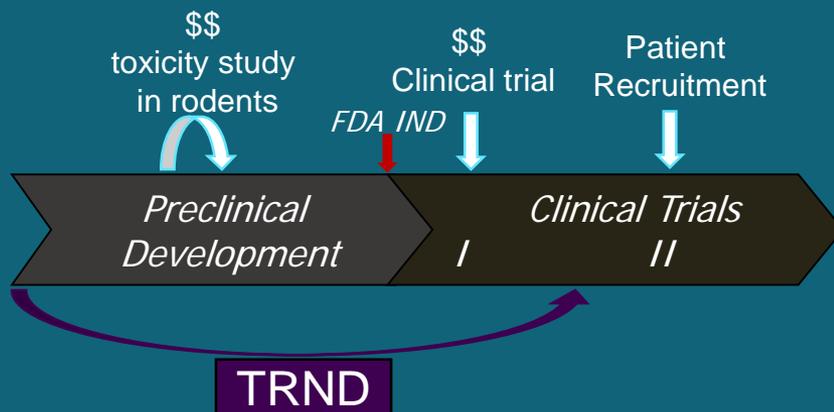
One of many examples of patient partner engagement with TRND

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!

