VISION-DMD

Project website: www.vision-dmd.info

Venture Philanthropy in Orphan Drug Development

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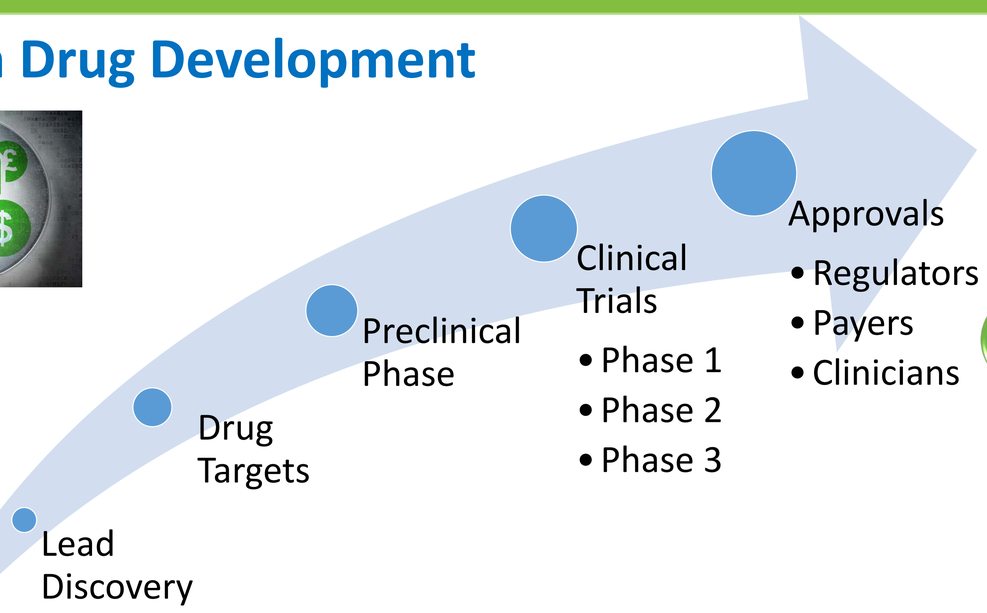
VISION-DMD is a US-EU collaborative project undertaking Phase 2 Clinical Trials of Vamorolone an Innovative Steroid-like Intervention for Duchenne Muscular Dystrophy.

Orphan Drug Development



TRADITIONAL VENTURE CAPITAL	HIGH RISK	HIGH COST	LESS INNOVATION
VENTURE PHILANTHROPHY	DE-RISK	REDUCED COST	MORE INNOVATION

Implementation

- **Duty to stake holders - Not stock holders**
- Run by academic medicine + stake holders
- Investments by stake holders
  - Forgivable funding/loans
  - Patient-led foundations as co-funders
- Faster development
- Extensive **de-risking**
- Focus on **innovations** in the drug development pathway (***risk tolerant***)
- Extensive peer review
  - Grant reviewers (government, foundation)
- Milestone and deliverable driven
- Sharing risk, sharing benefits with Stakeholders.

Benefits of Venture Philanthropy for orphan drug development

- Integrating patients/parents into the development process
- Focus on what matters to the patient/family
- **Investors bring high level knowledge**
  - **Expertise and focus on Disease**
  - **Patient's perspective**
  - **Knowledge base for trial design**
- Stake holders more risk tolerant; fosters innovation
- Improved patient recruitment and retention in clinical trials
- Return on Investment for funders (stock; royalties; % of sales; interest; cash-back).
- EMA increasingly includes trained, expert stake holders into committees – leverage this expertise
- Drug development transparent
- High degree of independent validation

Maximising financial return on investment is **NOT A CORE DRIVER**

Expected outcomes

- Reduced development timescale
- Potential to develop more affordable drugs
- Improved Standards of Care
- Enhanced **shared** knowledge base
- New clinical and biochemical outcomes
- Sustainability of funders allowing new investments
- Accelerated pipeline of products with new targets

Good practice guidelines for the rare disease community

Case study: VISION-DMD, Vamorolone clinical development for DMD

PRECLINICAL 2009-2015
Proof of Concept CMC, API , Pre-clinical, De-risk, Biomarkers

2009 GRANTS $490K	Foundation to Eradicate Duchenne Muscular Dystrophy Association, US Department of Defense, Children's National Medical Center
2010 $244K	US Internal Revenue Service (QDTP)
LOAN $360K	Muscular Dystrophy Association Venture Philanthropy
2011 $3m	US National Institutes of Health (TRND) (in kind)
LOAN $681K	Cure Duchenne, Children's National Medical center, Eric Hoffman.
2012 $50K	Foundation to Eradicate Duchenne
Venture P $1.55M	Muscular Dystrophy Association Venture Philanthropy
2013 $70K	Parent Project Muscular Dystrophy Foundation to Eradicate Duchenne
LOAN $360K	Save our Sons

$ 1.38M US NIH grants for New Indications: Asthma – Sickle cell – IBD-Arthritis – Multiple sclerosis

PHASE I 2015--16

2014 $275K	Foundation to Eradicate Duchenne, Action Duchenne
Venture P $1.55M	Duchenne Children's Trust, Joining Jack, Duchenne Research Fund, Muscular Dystrophy Association Venture Philanthropy

PHASE IIa 2016-17

2015 $3.3M	National Institute of Health NIAMS and NINDS

$6.17M Patient funding for Duchenne

PHASE IIb 2018-2020

2016 $7M	EC Horizon 2020 Programme
LOAN $360K	Private individuals, Children's National medial Center
Venture P $1.55M	Duchenne Alliance [Save Our Sons (Aus), Michael's Cause (USA), Pietro's Fight (USA), Alex's Wish (UK), Ryan's Quest (USA)] Parent Project Muscular Dystrophy
PHARMA $10M	Actelion option agreement – milestone payment

$22.35 M Venture Philanthropy Model

Pharma Option Payment agreed

VISION-DMD International Consortium

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