

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



Lead Discovery

Drug Targets

Preclinical Phase

Clinical Trials
• Phase 1
• Phase 2
• Phase 3

Approvals

• Regulators
• Payers
• Clinicians



TRADITIONAL VENTURE CAPITAL

HIGH RISK

HIGH COST

LESS INNOVATION

VENTURE PHILANTHROPY

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

2016 \$7M
EC Horizon 2020 Programme

\$6.17M
Patient funding for Duchenne

PHASE IIb 2018-2020

\$22.35 M
Venture Philanthropy Model

Parent Project Muscular Dystrophy
LEADING THE FIGHT TO END DUCHENNE

Foundation to Eradicate Duchenne

ACTION DUCHENNE

Joining Jack

SAVE OUR SONS
HELP. CURE. DMD.

MDA
Fighting Muscle Disease

Ryan's Quest
To Fight Duchenne Muscular Dystrophy

DUCHENNE UK

Duchenne Research Fund

DUCHENNE ALLIANCE

THE TRANSFIGHT DMD
BY WICKED BYWIND

LOAN \$360K
Private individuals, Children's National medical 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

Pharma Option Payment agreed

VISION-DMD International Consortium

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