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# GENE THERAPY FOR DUCHENNE: THE LONG AND WINDING ROAD

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# DMD

- One of commonest rare diseases
- Severe, progressive, life limiting
- High care burden on family



# Therapies for DMD

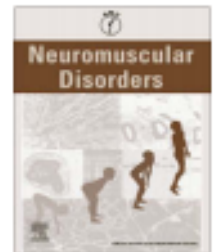
Neuromuscular Disorders 20 (2010) 355–362



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## Neuromuscular Disorders

journal homepage: [www.elsevier.com/locate/nmd](http://www.elsevier.com/locate/nmd)



### Workshop report

## The development of antisense oligonucleotide therapies for Duchenne muscular dystrophy: Report on a TREAT-NMD workshop hosted by the European Medicines Agency (EMA), on September 25th 2009

F. Muntoni \*, on behalf of the meeting steering committee, and of the TREAT-NMD Network

*The Dubowitz Neuromuscular Centre, University College London, Institute of Child Health, 30 Guildford Street, London, WC1N 1EH, UK*

### 1. Introduction

A workshop entitled The Development of Antisense Oligonu-

and safety data can be extrapolated from one AO to another. Since DMD is a relatively rare disease, with only 100 new cases/year in the UK from a range of different genetic mutations in the *DMD*

*‘An imaginary is a collectively embraced actionable future in which technological change will bring about certain positive, culturally intelligible results’ (Weiner et al 2017)*

- Bench to bedside is rarely linear progression with reduction in uncertainty
- Translation into clinical care can introduce complexity and new uncertainties
- Discrepancy – imagined and actual - disruption

# Where are we now?

- Multiple problems, increased complexity
- 2 (restricted) therapies
- Clinical benefit not proven

*“shifting sands and blind alleys that may disorient the modern orphan drug developer as well as the families” (Hoffman & McNally 2014)*



# How we talk about genome editing

- We are talking about genome editing in positive tones
  - ▣ *‘enormous positive potential’*
  - ▣ for DMD, *‘great hope’*
- Disadvantages may become advantages
- “I hope you’re right, I really do”

# Promissory technologies

- *‘what is dangerous is where there are asymmetries of power, information or representation in the public sphere that mean that certain visions and values go unappreciated and others go unchallenged’* (Nuffield Council on Bioethics 2016)
- Better QoL and significant increase in life expectancy for people with Duchenne has come via improved **care**



- Similar uncertainty/complexity 100,000 Genomes
- A decent genetic minimum for rare disease
- Aspiration –versus– actuality
- Under-promise, over-deliver





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