This research has received funding from the European Community’s Seventh Framework Programme (FP7/2007-2013) under grant agreement n° 305697

**OPTIMISTIC**

Observational Prolonged Trial In Myotonic dystrophy type 1 to Improve quality of life Standards, a Target Identification Collaboration

[University of Glasgow] [Optimistic]
This research has received funding from the European Community’s Seventh Framework Programme (FP7/2007-2013) under grant agreement n° 305697

- the first international clinical trial in myotonic dystrophy type 1
- collaboration between the UK, France, Germany and the Netherlands
- coordinated by Baziel Van Engelen
- funded by the European Commission for 4 years
  €3,000,000

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the hypothesis

• non-drug based interventions can significantly impact on the disease course and quality of life in the management of myotonic dystrophy type 1

• specifically combined cognitive behavioural and exercise therapy
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the plan

- recruit ~280 patients
- baseline testing – blood samples, urine, physical activity and quality of life questionnaire
- randomise to receive CBT and exercise therapy or standard care
- intervention over 10 months, with 6 month follow up
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progress

• project started in October 2012
• first patient recruited April 2014
• 94 patients recruited as of end of August 2014
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primary objective

• test the effectiveness of a personalised programme of cognitive behavioural therapy and exercise in mediating an increase in physical activity and improved quality of life

secondary objectives

• determine the genetic factors mediating symptomatic variation
• define biomarkers of disease progression and treatment response
• determine the utility of clinical outcome measures
• establish the framework and experience to implement international clinical trials in myotonic dystrophy
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more info at:

www.optimistic-dm.eu