

# ***OPTIMISTIC***

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



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of Glasgow



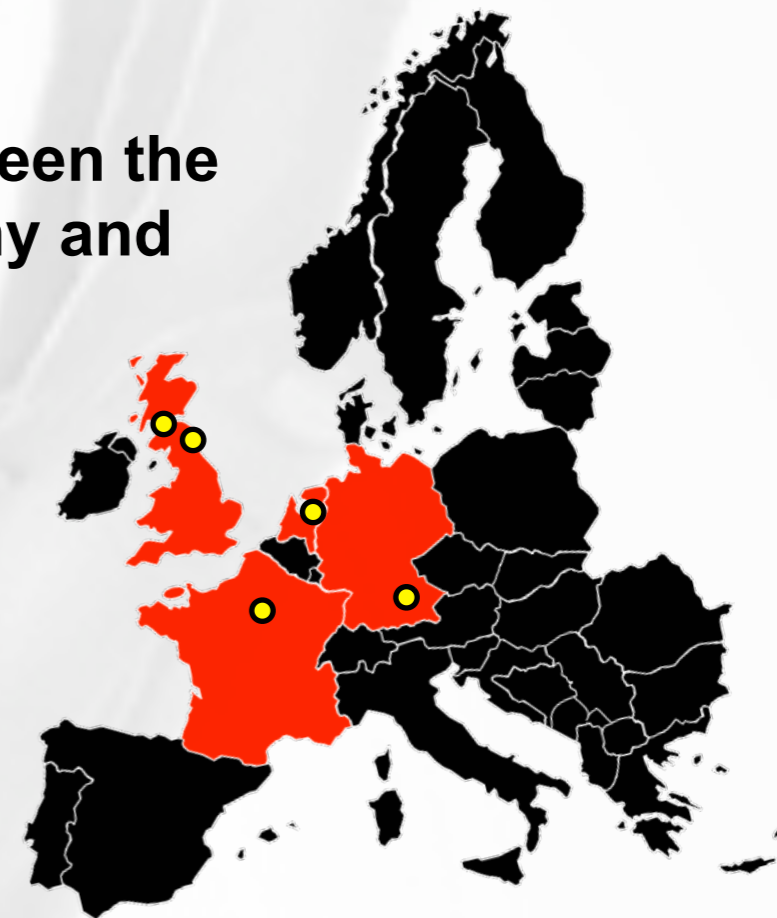
This research has received funding from the European Community's Seventh Framework Programme (FP7/2007-2013) under grant agreement n° 305697

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- 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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- Nijmegen
- Baziel Van Engelen



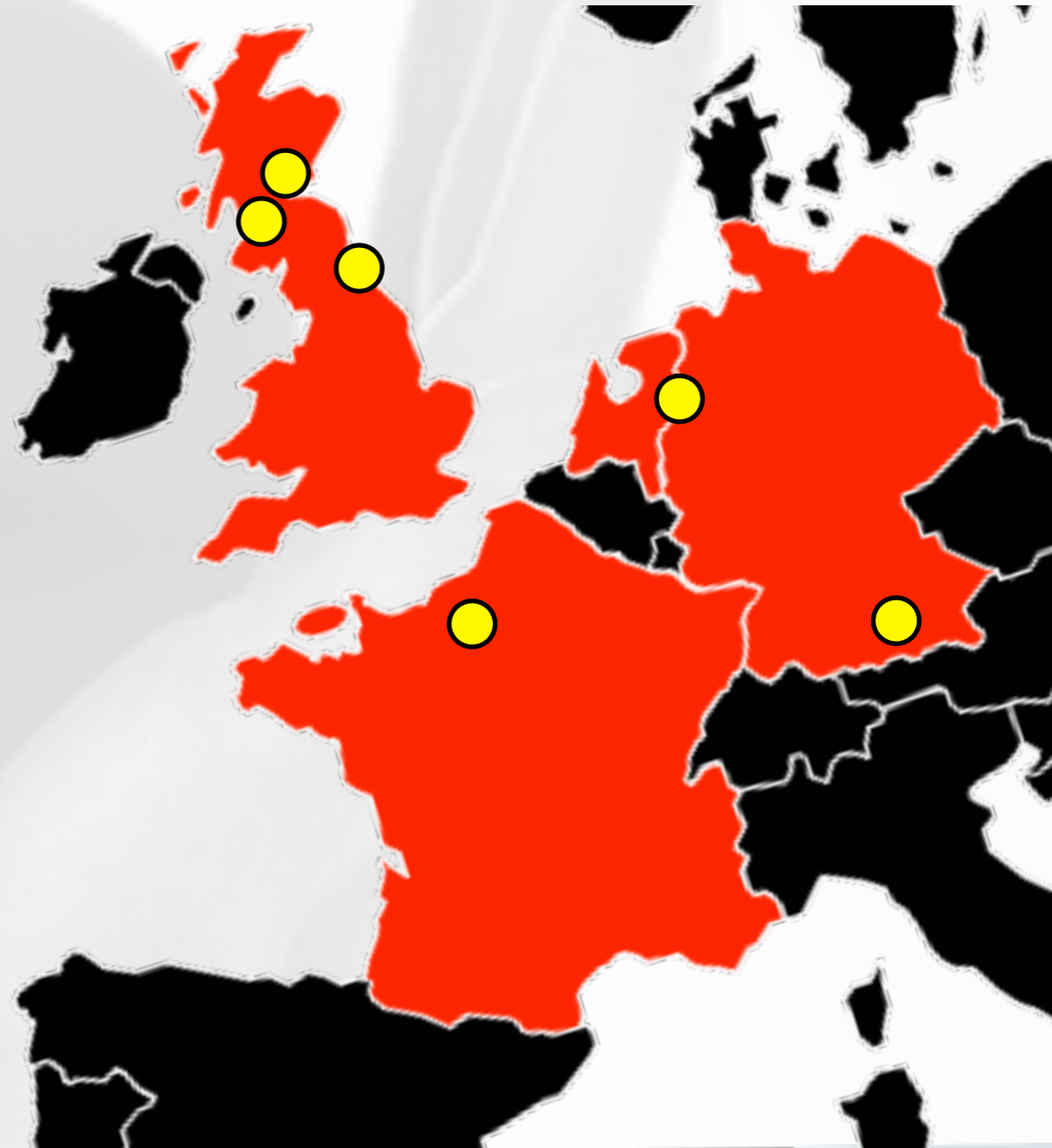
- Munich
- Benedikt Schoser



- Newcastle
- Grainne Gorman



- Paris
- Guillaume Bassez



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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](http://www.optimistic-dm.eu)**



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