

KD Research in London UK

Prof Linda Greensmith

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UK KD Team: a coherent research program from the lab to the clinic

- **Queen Square UCL Institute of Neurology:**

Linda Greensmith, Pietro Fratta, Michael Hanna, Bilal Malik, Charlotte Spicer & Helen Devine

- **Oxford University:**

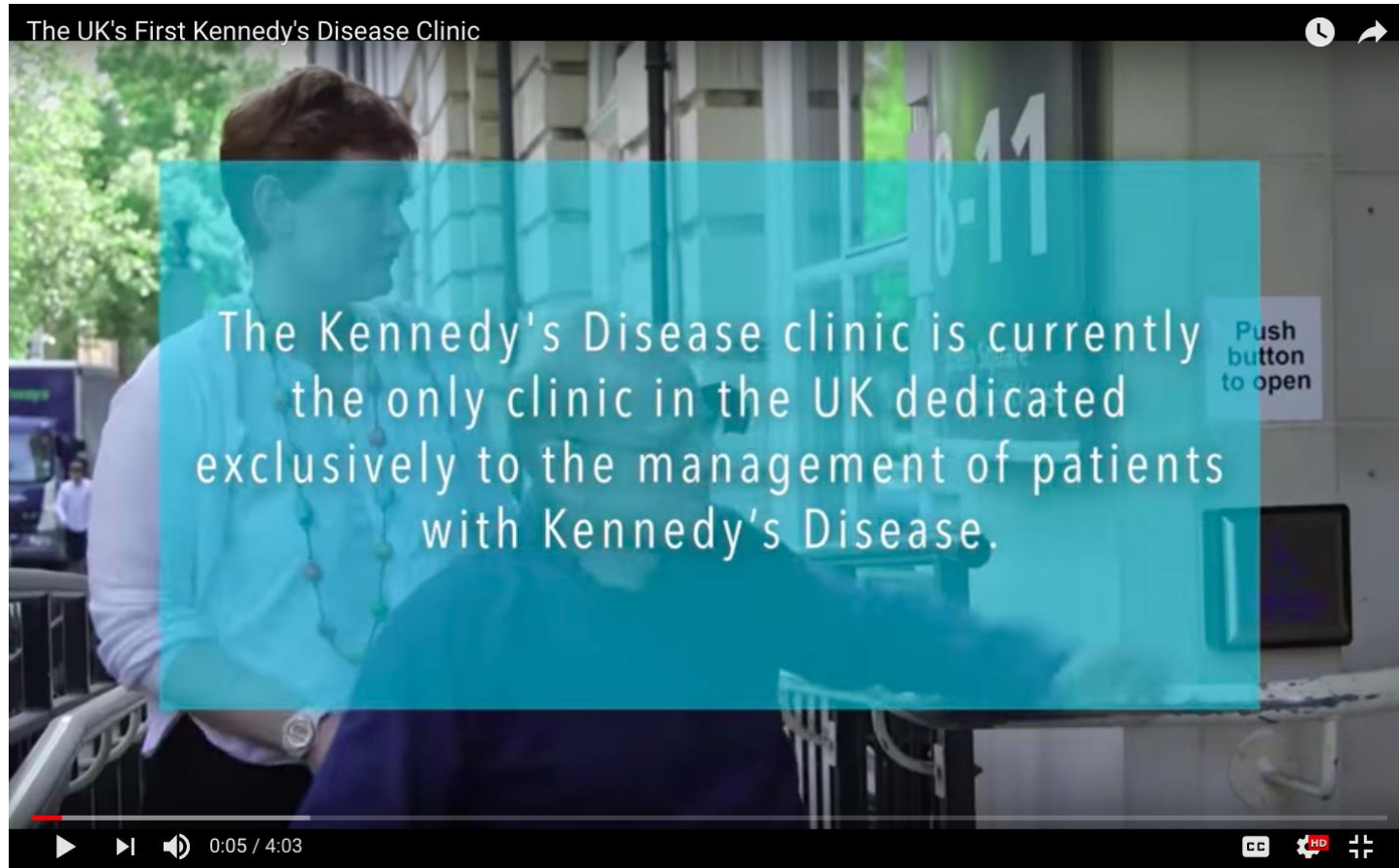
Carlo Rinaldi, Catheryn Wooi Lim, Mitra Forouhan, Ruth Ellerington & Subhashis Banerjee

A collegiate & collaborative research group

UK National KD Clinic at Queen Square Institute of Neurology

- National Registry established of all KD patients in UK in 2005
- KD Clinic established by Pietro Fratta at Queen Square in 2014
- Clinic joined by Carlo Rinaldi (Oxford) in 2018
- Team includes a specialist research nurse (Luca Zampedri) and a MND nurse consultant (Jan Clarke), a research physiotherapist (Gita Ramdharry)
- Dedicated National Clinic enables
 - KD Patient cohorts
 - KD natural history studies
 - KD patient biobank of samples
 - KD biomarker studies: “wet”; “imaging” eg muscle MRI: QUANTITATIVE

“TRIAL READY”



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Eric Mager - UK

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2012/13 KD-UK Charity established by Kate Hoppps: The UK's first and only charity for KD



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UCL KD Research Newsletter July 18

Hi and welcome to the inaugural research newsletter from the Kennedy's Disease Clinic at UCL! We always enjoy letting you know about



The Grand Tour of Wessex 16th & 17th June 2018

The Devon Austin Seven Club is organising a Grand Tour Of Wessex to take place on 16th & 17th June 2018. The aim of the tour is to give owners...

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First UK KD Patients Day March 2017



>140 patients, carers and healthcare workers - UK, Canada, Denmark and Australia!

European collaborations in KD: European Neuromuscular Centre

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Introduction

ENMC: a worldwide network

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ANBI

INTRODUCTION

Most neuromuscular disorders are rare (orphan) diseases, meaning that fewer than 1 in 2,000 people are affected. They all cause a weakening of muscles, which could lead to tremendous disability. Because of this, international collaboration is extremely important to accelerate our knowledge and discovery of new medicines in the field of neuromuscular diseases.

Historical background

The European Neuromuscular Centre (ENMC) was founded in 1992 by a group of European patient associations that dedicated itself to bring leading researchers and clinicians from all over the world together. The ENMC is unique within the scientific world to achieve this by financing and organising workshops. Topics of the workshops vary from outcome measures, clinical trial readiness and preclinical studies, to diagnosis and care for all neuromuscular diseases for instance spinal muscular atrophy, Duchenne muscular dystrophy, limb girdle dystrophies and congenital muscular dystrophies.

ENMC Mission statement

"The mission of ENMC is to encourage and facilitate communication and collaboration in the field of neuromuscular research with the aim of improving diagnosis and prognosis, finding effective treatments and optimizing standards of care to improve the quality of life of people affected by neuromuscular disorders".

Achievements by ENMC

Introduction

ENMC: a worldwide network

Member Organisations

Committees and staff

Annual Report

ANBI

ENMC
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The Netherlands

Route

in

New submission deadline of workshop applications :
15 March 2019

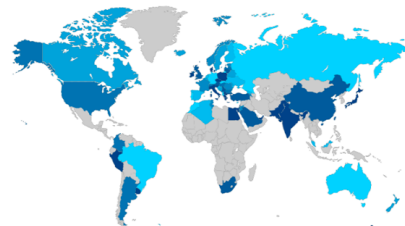
Workshops



GLOBALIZATION OF THE ENMC

Since 1990, the ENMC was started as a European initiative but gradually the workshops included researchers and clinicians from all over the world. This empowers the ENMC's mission : to establish international consortia, a cooperation of experts in the world to discover therapeutic interventions for patients with a neuromuscular condition at a much faster speed than before. In the map below all countries which had at least 1 representative in an ENMC workshop in the last 20 years, are colored a shade of blue.

Over 65 countries are represented at ENMC workshops



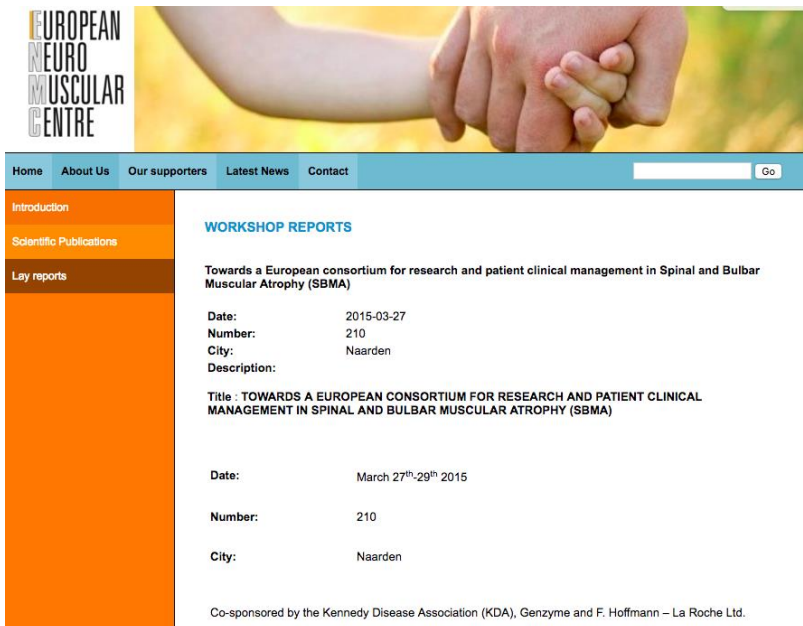
Internationally, researchers and clinicians within the ENMC network collaborate together :

- in clinical trials
- in research validating the diagnostic markers for a specific neuromuscular condition
- in uniforming treatment guidelines
- in scientific publications

A worldwide network

1st ENMC Workshop in KD: March 2015

TOWARDS A EUROPEAN CONSORTIUM FOR RESEARCH AND PATIENT CLINICAL MANAGEMENT IN KD



15 - 17 February 2019

Second ENMC meeting on KD

Organisers: Dr Pennuto (Italy), Dr Soraru (Italy), Prof Greensmith (UK), Dr Pradat (France)

Key Outcomes: 1st International Conference in KD 2019/20

Investigating Disease Pathways in Kennedy's disease

Department of Neuromuscular Diseases
UCL Queen Square Institute of Neurology
University College London

Our lab research in KD is focused on two main areas

1. to fully understand the reasons why motor neurons and muscle degenerate in KD - to *pinpoint specific targets for drug development*
2. Test treatment strategies in mouse model and human cells