Rare Disease Day
Research brings hope to people living with a rare disease
Friday 24 February 2017
This special day aims to draw attention to rare diseases and the millions of people who are affected by them, highlighting the need for more research and funding to help sufferers and their families. The theme for 2017 is ‘Research brings hope to people living with a rare disease.’

In Europe, a rare disease is defined as one with an incidence of less than 1 in 2,000 people. While each of them is rare, there are 6–8,000 rare diseases, which together affect 3 million people in the UK, taking 20% of all health care costs. Rare diseases are a serious public health concern and an international priority, but unfortunately often very little is known about them by health professionals and the public alike.

People who are affected by a rare disease will mostly receive symptomatic and palliative care because there are hardly any curative treatments. And we have not started talking about the relatives who will have to stop working and become full-time carers.

Rare Disease Day encourages us to continue finding ways to work together to provide the different kinds of care that people living with a rare disease need.

**Rare disease research at Royal Holloway**

Royal Holloway is a leading institution in the development of novel therapies for rare diseases, including Spinal muscular atrophy, Ataxia telangiectasia, Duchenne muscular dystrophy, Motor neuron disease and Severe Combined Immunodeficiencies (referred to as ‘child in a bubble’ disease). Our scientists are at the forefront in developing innovative techniques in gene based therapy.

**Today’s Organisers**

Dr Rafael J. Yáñez-Muñoz, Reader in Advanced Therapy in the Centre for Biomedical Sciences, is the Editor-in-Chief of Gene Therapy, the Treasurer of The British Society for Gene and Cell Therapy and a trustee of the Genetic Alliance UK. Professor George Dickson (co-organiser) is Chair of Molecular Cell Biology in the School of Biological Sciences and a founder member of The British Society for Gene and Cell Therapy.

**School of Biological Sciences**

The School of Biological Sciences at Royal Holloway has earned an international reputation for the world-class quality of our research which is at the forefront of scientific discovery. We offer an exciting range of undergraduate degree programmes and research opportunities ranging from gene therapy, tropical diseases, GM technology, vaccine technology and neuroscience, to animal behaviour, conservation, ecology and biodiversity.

The School places a strong emphasis on practical teaching, with state-of-the-art equipment and an exceptionally supportive environment for all students.

It achieved 93% overall satisfaction from its students in the National Student Survey in 2016.
Programme

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<th>Time</th>
<th>Group A</th>
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<tr>
<td>09:00 - 09:30</td>
<td>Arrival and registration</td>
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<td>09:30 - 10:30</td>
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<td>Professor John Wann</td>
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<td>Moore Building Lecture Theatre</td>
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<td>10:30 - 11:30</td>
<td>Lab activity</td>
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<td>Lab activity</td>
<td>Lecture (12:00-12:50)</td>
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<td>Prof George Dickson</td>
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<td>12:30 – 13:15</td>
<td>Lunch</td>
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<td>Disease Detective</td>
<td>Speed dating</td>
<td>Evaluation and departure</td>
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<td>Bourne 203</td>
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<td>14:00 - 14:45</td>
<td>Speed dating</td>
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Our Speakers

Welcome
Professor John Wann,
Associate Dean of Research, Royal Holloway

Introduction to Rare Disease Day
Dr Rafael Yáñez, Advanced Gene and Cell
Therapy Lab, School of Biological Sciences

Syndromes Without A Name - the challenges
of having an undiagnosed genetic condition
Lauren Roberts, Director of Support,
SWAN UK National Coordinator

Living with Ataxia Telangiectasia (A-T)
Sean Kelly, Chief Executive, Action for A-T

Oculopharyngeal Muscular Dystrophy
Dr Alberto Malerba, Research Officer and
Project Manager, Royal Holloway

Genes, Gene Therapies & GM Humans
Professor George Dickson,
Professor of Molecular Cell Biology

All undergraduate degrees offered in
the School of Biological Sciences are
accredited by the Royal Society of Biology.
Our 2017 exhibitors

We warmly welcome the following partners to our Rare Disease Day

Action for A-T
AMEND
Ashford and St. Peter’s Hospitals
Beat SCAD
DEBRA
Genetic Alliance UK
Genetic Disorders UK
Muscular Dystrophy UK
Spinal Muscular Atrophy Support UK
The British Society for Gene and Cell Therapy
The Fragile X Society
The Ichthyosis Support Group
The SMA Trust
Tuberous Sclerosis Association

Royal Holloway exhibitors:
BioSoc
Barbara Zipser, Hellenic Institute, Department of History

Useful links

Rare Disease Day: royalholloway.ac.uk/rarediseaseday
School of Biological Sciences: royalholloway.ac.uk/biologicalsciences

Advanced Gene and Cell Therapy Laboratory (AGCTlab): http://agctlab.org

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