# THE XLP RESEARCH TRUST SYMPOSIUM 2018

A Symposium for Researchers and Clinicans on XLP1 and XIAP



### Agenda @5<sup>th</sup> October 2018

09:00 - 09:30 **Registration** 

09:30 – 09:45 **Welcome** David Hartley, The XLP Research Trust

### XLP1

09:45 – 10:05 CD8 defects in XLP1 and Activated P13 Kinase Syndrome

Dr. Pamela Schwartzberg, NHGRI and NIAID, Bethesda, USA

10:05 – 10:25 Diacylglycerol kinase-alpha is an effector of SAP/NCK1/WASP/Arp2/3 signalling pathway regulating cytoskeleton remodelling and Restimulation Cell Death (RICD)

Dr. Andrea Graziani, Università Vita-Salute S. Raffaele, Milan, Italy

10:25 – 10:45 Novel DGKa Inhibitors for XLP1 therapy via virtual screening

Assoc. Prof. Gianluca Baldanzi, Dept. Translational Medicine, Novara, Italy

### 11:15 – 11:35 Modification of cellular and humoral immunity by somatically reverted T cells in XLP1

Prof. Hirokazu Kanegane, Tokyo Medical and Dental University, Tokyo, Japan

#### 11:35 – 12:05 Developing novel T cell therapies for XLP1

Dr. Neelam Panchal, UCL-GOSICH, London, UK

### 12:05 – 12:25 TALEN-mediated genome editing via AAV6 donor in T cells for the treatment of X-linked lymphoproliferative disease (XLP)

Dr. Ben Houghton, UCL Great Ormond Street Institute of Child Health, London, UK

12:25 - 13:30 Lunch

#### 13:30 – 13:50 Update on The XLP Research Trust

David Hartley, Chairman & Co-Founder, The XLP Research Trust, UK

#### **XIAP**

## 13:50 – 14:10 Critical role of T-cell expansion in immunity to EBV revealed by genetic defects with high predisposition to EBV infection

Dr. Sylvain Latour, Institute Imagine, Paris, France

### 14:10 – 14:30 Determining anti-viral immune responses and cell death pathways in XIAP deficiencies

Dr. Lynn Wong, Institute of Experimental Immunology, University of Zurich, Switzerland

### 14:30 – 14:50 Dysregulated IL 1-beta secretion in XIAP deficiency is abrogated by the natural flavonoid quercetin

Dr. Rebecca Marsh, Cincinnati Children's Hospital, USA

#### 14:50 - 15:15 Tea Break

#### 15:15 – 15:35 XIAP deficiency and inflammatory bowel disease

Dr. Holm Uhlig, Oxford University, UK

### 15:35 – 15:55 An update on outcome following Haematopoietic stem cell transplantation in XIAP deficiency

Dr. Austen Worth, Great Ormond Street Hospital, London, UK

### 15:55 – 16:15 Correction of innate immune responses in XIAP deficiency using gene modified haematopoietic stem cells

Yusef Topal, UCL Great Ormond Street Institute of Child Health, UK

#### 16:15 – 16:35 Launch and demonstration of the XLP Registry

Dr. Claire Booth, UCL Great Ormond Street Institute of Child Health, London, UK

David Hartley, Chairman & Co-Founder, The XLP Research Trust, UK

#### 16:45 Wrap Up & Mingle

We'd again like to thank the Jeffrey Modell Foundation for awarding is a WIN grant to reduce the cost of the symposium.

