2nd World Congress on
Rare Diseases and Orphan Drugs
June 29-30, 2017  London, UK

Scientific Program
Day 1  June 29, 2017

09:00-09:30  Registrations

Kingsley Suite

09:30-10:00  Opening Ceremony

Keynote Forum

10:00-10:40  Introduction
  Title: Innovative tools for drug development and disease modeling
  Danilo A Tagle, National Institutes of Health, USA

10:40-11:20  Title: Clinical course and outcomes of critically ill patients with middle east respiratory syndrome coronavirus infection
  Abdulaziz Aldawood, King Saud Bin Abdulaziz University, Saudi Arabia

Group Photo

Network & Refreshment Break 11:20-11:40 @ Foyer

Sessions: Pediatric Rare Diseases | Therapies for Rare Diseases | Other Rare Diseases | Current Research on Rare Diseases | Rare Diseases of Immune System | Genetic Diseases and Disorders

Session Chair: Nadia Ameen, Yale University School of Medicine, USA
Session Co-chair: Wei Zheng, National Institutes of Health, USA

Session Introduction

11:40-12:10  Title: Ion transport defects in microvillus inclusion disease
  Nadia Ameen, Yale University School of Medicine, USA

12:10-12:40  Title: Drug repurposing screen for rapid identification of therapeutics for Zika virus infection
  Wei Zheng, National Institutes of Health, USA

12:40-13:10  Title: Neglected tropical diseases: Road to control, elimination and eradication by 2020
  Diego-Abelardo Alvarez-Hernandez, Anahuac University, Mexico

Lunch Break 13:10-14:10 @ Restaurant

Workshop

14:10-14:50  Title: Development of orphan medical products
  Martine Zimmermann, Alexion Pharmaceuticals Inc, Switzerland

14:50-15:20  Title: Treatment of early onset scoliosis associated with rare disease
  Stefano Giacomini, Rizzoli Orthopaedic Institute, Italy

15:20-15:50  Title: Retrodifferentiation in the treatment of a rare condition: Acquired aplastic anaemia
  Ilham Abuljadayel, TriStem Corp Ltd, UK

15:50-16:20  Title: Current challenges on chagas disease
  Jorge-Alberto Ascencio-Aragón, Anahuac University, Mexico

Network & Refreshment Break 16:20-16:40 @ Foyer

16:40-17:10  Title: Pathway-centric analysis of rare autoinflammatory diseases (AID) and drug repurposing
  Patrick J Tighe, University of Nottingham, U.K

17:10-17:40  Title: Consulting for a combination of molecular defects for variable expression
  Yingjun Xie, The Third Affiliated Hospital of Guangzhou Medical University, China

Panel Discussion
**Keynote Forum**

**Day 2  June 30, 2017**

**Kingsley Suite**

**Title:** Neurofibromatosis and spinal deformities: The severity in pediatric age and treatment  
**Stefano Giacomini,** Rizzoli Orthopedic Institute, Italy

**Network & Refreshment Break 10:45- 11:05 @ Foyer**

**Sessions:** Rare Hereditary Diseases | Rare Ophthalmological Diseases | Genetic Diseases and Disorders | Strategies for Diagnosis & Treatment | Undiagnosed Rare Diseases  
**Session Chair:** Patrick J Tighe, University of Nottingham, UK

**Session Introduction**

**10:00-10:45**

**Title:** Neurofibromatosis and spinal deformities: The severity in pediatric age and treatment  
**Stefano Giacomini,** Rizzoli Orthopedic Institute, Italy

**Network & Refreshment Break 10:45- 11:05 @ Foyer**

**11:00-11:35**

**Title:** Paracrine action of human mesenchymal stem cells for muscle diseases  
**Jong Wook Chang,** Samsung Medical Center, South Korea

**11:35-12:05**

**Title:** Splice modulation therapy for inherited retinal diseases  
**Rob W J Collin,** Radboud University Medical Center, Netherlands

**12:05-12:35**

**Title:** Management of rare diseases: An integrated approach to break down barriers and facilitate patient access to healthcare  
**Gayathri Balasubramanian,** Focus Scientific Research CenterPhamax, India

**Lunch Break 12:35-13:35 @ Restaurant**

**13:35-14:05**

**Title:** The diagnostic challenge of rare diseases: Lack of knowledge or leaking method?  
**Shmuel Prints,** Clalit Health Service, Israel

**14:05-14:35**

**Title:** Hereditary spastic paraplegias phenotype constitute part of broader rare genetic mendelian inherited disorders  
**Alice Abdel Aleem,** Weill Cornell Medical College, Qatar

**Poster Presentations**

**RDC-01**

**Title:** Patients without diagnosis: A profile  
**Isabelle Windheuser,** University Hospital Bonn, Germany

**RDC-02**

**Title:** Interdisciplinary competence unit for patients without diagnosis  
**Larissa Wester,** University Hospital Bonn, Germany

**RDC-03**

**Title:** Sclerosing angiomatoid nodular transformation of the spleen: An unusual finding in paediatric population  
**Godwin Oligbu,** St Georges Hospital London, UK

**Network & Refreshment Break @ Foyer**

**Award & Closing Ceremony**

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**Bookmark your dates**

**4th world Congress on**

**Rare Diseases and Orphan Drugs**

**June 11-12, 2018**  
**Dublin, Ireland**

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