

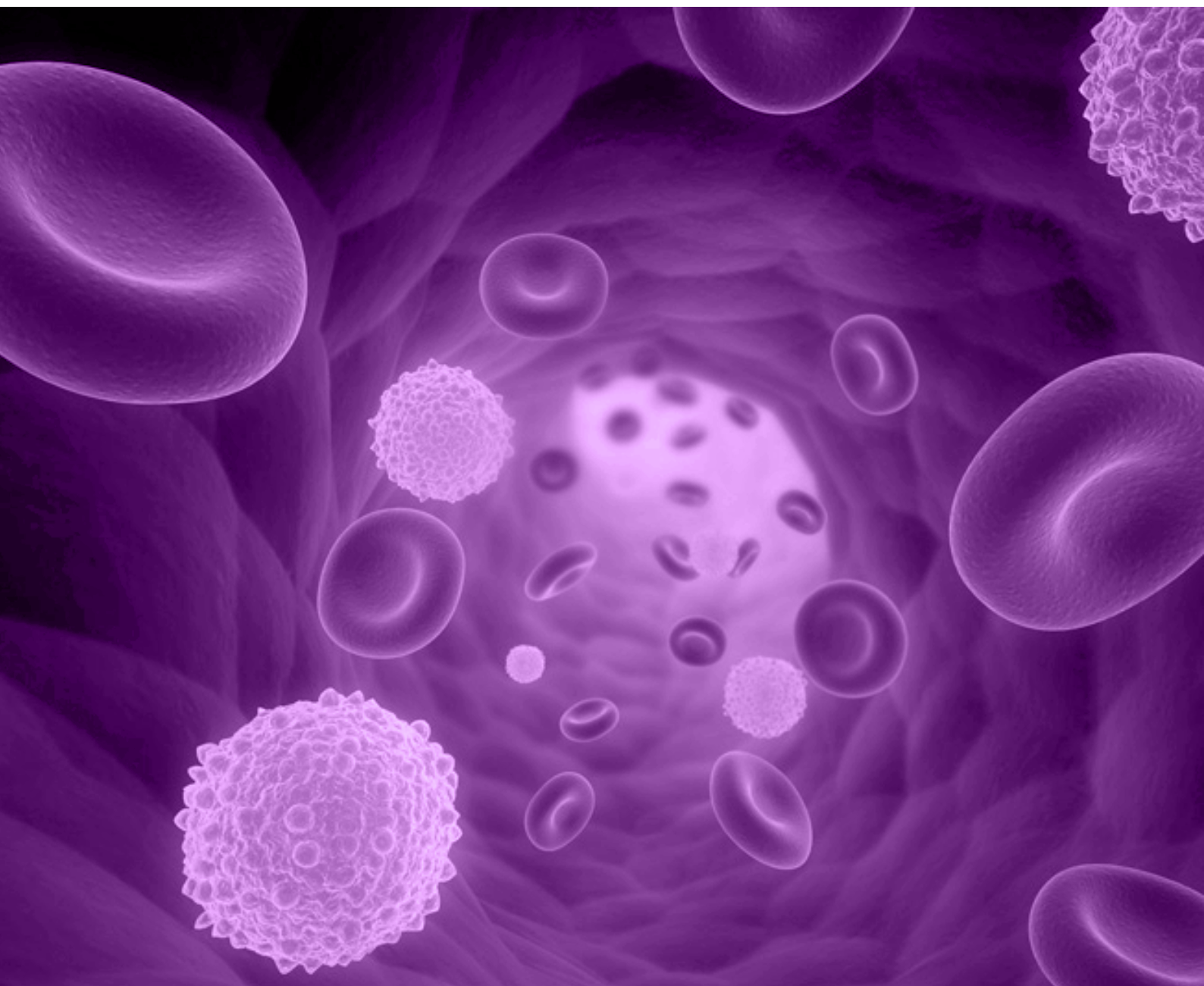
**conference**series.com  
1005<sup>th</sup> Conference

*Scientific Program*

2<sup>nd</sup> World Congress on

# Rare Diseases and Orphan Drugs

June 29-30, 2017 London, UK



**Conference Series-America**

One Commerce Center-1201, Orange St. #600, Wilmington, Zip 19899, Delaware, USA  
Toll Free: 1-888-843-8169, P: 702-508-5200, F: +1-650-618-1417

**Conference Series-UK**

Kemp House, 152 City Road, London EC1V 2NX, UK  
Toll Free: +0-800-014-8923

09:00-09:30 Registrations

**Kingsley Suite**

**conference**series.com 09:30-10:00

**Opening Ceremony**

**Keynote Forum**

**Introduction**

10:00-10:40

**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**

**Day 2 June 30, 2017**

**Kingsley Suite**

**Keynote Forum**

**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**

**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**

**11:05-11:35** 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**

**Bookmark your dates**

4<sup>th</sup> world Congress on

**Rare Diseases and Orphan Drugs**

June 11-12, 2018 Dublin, Ireland

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