conferenceseries.com

2nd World Congress on

Rare Diseases and Orphan Drugs

June 11-12, 2018

Dublin, Ireland



SCIENTIFIC PROGRAM

DAY 1
Monday, 11th, June

08:30-09:00 **Registrations**

09:00-09:30 **Introduction**

09:30-09:50 COFFEE BREAK

09:50-11:50 Meeting Hall 01 **KEYNOTE LECTURES**

MEETING HALL 01

11:50-13:10 **Talks On:**

- Neglected Tropical Diseases
- Rare Pulmonary Diseases
- Rare Diseases in Neurology
- Rare Genetic Diseases
- Scope of Orphan Drugs

13:10-13:15 GROUP PHOTO

13:15-14:00 LUNCH BREAK

MEETING HALL 01

14:00-16:00 **Talks On:**

- Rare Cardiac Diseases
- Orphan Drugs Treatment for Rare Diseases
- Rare Oral Diseases
- Rare Skin Diseases
- Clinical Research on Orphan Drugs

16:00-16:20 COFFEE BREAK

DAY 2
Tuesday, 12th, June

09:00-10:30 Meeting Hall 01 — **KEYNOTE LECTURES**

10:30-10:50 COFFEE BREAK

MEETING HALL 01

10:50-12:50 **Talks On:**

- Rare Morphological Diseases
- Development of Orphan Products
- Rare Diseases in Oncology
- Orphan Drugs Market Research
- Pediatric Rare Diseases

12:50-13:35 LUNCH BREAK

MEETING HALL 01

13:35-15:55 **Talks On:**

- Current Rare Diseases Research
- Rare Diseases of Sexual Health
- Rare Hereditary Diseases
- Clinical case studies on Rare Diseases
- Imaging of Rare DiseaseS
- Other Rare Diseases

15:55-16:15 COFFEE BREAK

MEETING HALL 01 (16:15-17:00)

Poster Presentations

Organizing Committee Members



Tiziana Greggi

Head Rizzoli Orthopaedic Institute

Italy



Reza Nassiri

Michigan State University

USA



Bernhard R. Ruf

Head St. Georg Hospital

Germany



Laxminarayan Bhat

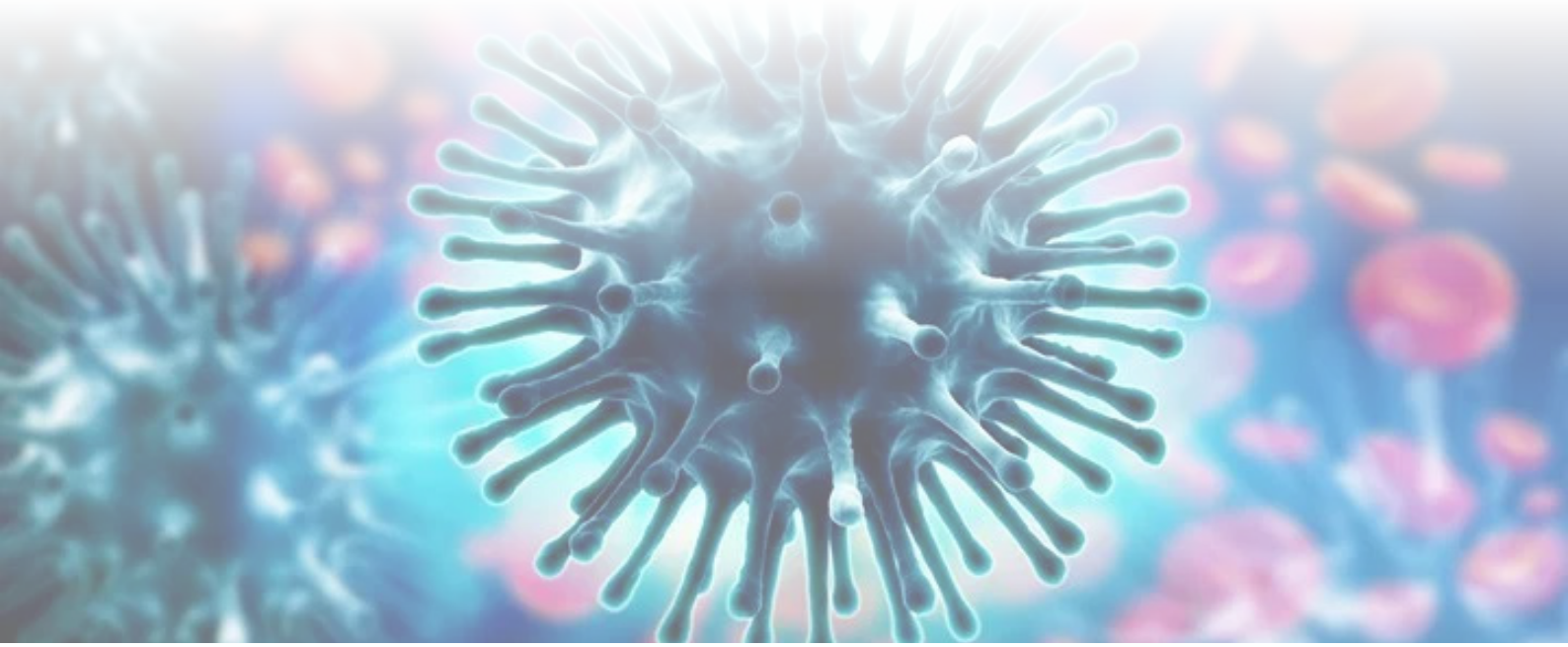
President & CEO Reviva

Pharmaceuticals, USA



Fahd Al Mulla

Kuwait University



conferenceseries.com

4th World Congress on Rare Diseases and Orphan Drugs

JUNE 11-12, 2018 DUBLIN, IRELAND

AGENDA

Title: Surgical treatment of early onset scoliosis associated with Rare Disease

Tiziana Greggi
Head Spine Surgery Deformity
Rizzoli Orthopaedic Institute
Italy

Tiziana Greggi Specialized works in Orthopedics and Traumatology, at the University of Bologna, Specialized in Biomedical Technologies, at the University of Bologna and she is the Head of Simple Departmental Structure: Spinal deformity surgery. She has worked in the Division of Spine Surgery of the Rizzoli Institute, first as an assistant and then as a senior manager. From 2000 to 2008 he received temporary positions as Director of a complex structure. From the end of this position to date has been responsible for the Organizational Module "the spinal deformities in pediatric age and associated with rare diseases" in the same Division.

Title: Serotonergic Targets in the Treatment of Pulmonary Arterial Hypertension (PAH) and Idiopathic Pulmonary Fibrosis (IPF)

Laxminarayan Bhat
Reviva Pharmaceuticals, Inc., USA

Laxminarayan Bhat, PhD, is the Founder, President, and Chief Executive Officer of Reviva Pharmaceuticals, Inc. Dr. Bhat founded Reviva in 2006 and since its inception, the company has advanced rapidly under his leadership with a portfolio of propriety compounds at different stages in a pipeline encompassing central nervous system (CNS), cardiovascular, and inflammatory diseases. Dr. Bhat has over 20 years of experience in drug discovery and development, and prior to founding Reviva, he held research positions at XenoPort, ARYx Therapeutics, and Higuchi Biosciences Center in the United States.

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

Deputy Chairman, Intensive Care Department, Consultant, Pulmonary and Critical Care Medicine, Professor, King Saud Bin Abdulaziz University for Health Sciences, Riyadh, Kingdom of Saudi Arabia, Dr. AlDawood graduate from College of Medicine in Kingdom of Saudi Arabia. Then he completed the Residency in Internal Medicine Program in McMaster University in Canada He obtained Critical Care and Respirology Fellowship Program in McMaster University, Canada (Jul 97-Jun 00).

SPEAKER SLOTS AVAILABLE

Title: Rare diagnosis in disorders/differences of sex developmenttreatening infections

Yolande van Bever,
Erasmus Medical Centre
The Netherlands

Yolande van Bever is a clinical geneticist with expertise in congenital anomalies and syndromes and with a special interest in the field of DSD. She worked in various academic hospitals in the Netherlands and abroad. Since 2004 she is an active team member not only in the clinical genetic staf, but also in various multidisciplinary teams such as the follow up team on surgical congenital anomalies, the neurofibromatosis team and the DSD team..

Title: Synthetic messenger RNA-based therapeutic strategy for treatment of alpha-1-antitrypsin deficiency

Tatjana Michel
University Hospital
Tuebingen,
Germany

Tatjana Michel works with synthetic modified mRNA treatment strategy since 2013. Her work is focused on the development and evaluation of mRNA-based drugs for rare monogenetic diseases like alpha-1-antitrypsin deficiency or familial hypercholesterolemia. Prior to that, she worked on strategies for cell transdifferentiation for heart regeneration and investigated immunogenic effects caused through synthetic nucleic acids

Title: Results of the investigation of findings and observations captured in burden of illness survey in FCS patients (in-focus) study: European

Karren Williams
Akcea Therapeutics
USA

Dr.Karren Williams has experience in the pharmaceutical industry with previous leadership roles at Akcea Therapeutics

SPEAKER SLOTS AVAILABLE

Title: Hepatitis c virus testing and treatment among persons receiving buprenorphine in an office -based program for opioid use disorders in nigeria

Samuel Ayoola Abati
lagos university,
Nigeria

Samuel Ayoola is lead PA and both a pathology residency and a pathologists' assistant program instructor with expertise in macroscopic pathology.

Title: Evaluating the impact of peer support and connection on the quality of life of patients with familial Chylomicronemia Syndrome

Alan Gilstrap, Akcea Therapeutics USA

Alan Gilstrap has experience in the pharmaceutical industry with previous leadership roles at Akcea Therapeutics

Title: Rare diseases in Kuwait: The experience of Genatak Center for Genomic Medicine Infectious Diseases

Fahd Al Mulla, Kuwait University, Kuwait

Fahd Al-Mulla is Professor of molecular pathology and heads a Molecular Pathology Unit, aiming delivering state-of-the-art diagnostic, targeted or tailored therapy and research facilities. Currently, his mandate is to build collaborative partnerships, invest institutional outputs, generate capital and resources, and promote public awareness as regards to the importance of scientific research outputs in resolving society's problems and in expediting the development process.

SPEAKER SLOTS AVAILABLE

Title: Surgical treatment of scoliosis in rare diseases: Arthogryposis

Tiziana Greggi, Rizzoli Orthopaedic Institute,Italy

Tiziana Greggi Specialized works in Orthopedics and Traumatology, at the University of Bologna, Specialized in Biomedical Technologies, at the University of Bologna and she is the Head of Simple Departmental Structure: Spinal deformity surgery. She has worked in the Division of Spine Surgery of the Rizzoli Institute, first as an assistant and then as a senior manager. From 2000 to 2008 he received temporary positions as Director of a complex structure. From the end of this position to date has been responsible for the Organizational Module "the spinal deformities in pediatric age and associated with rare diseases" in the same Division.

Title: unmet medical needs and future directions to PAH and IPF

Laxminarayan Bhat Reviva Pharmaceuticals, Inc., USA

Laxminarayan Bhat, PhD, is the Founder, President, and Chief Executive Officer of Reviva Pharmaceuticals, Inc. Dr. Bhat founded Reviva in 2006 and since its inception, the company has advanced rapidly under his leadership with a portfolio of propriety compounds at different stages in a pipeline encompassing central nervous system (CNS), cardiovascular, and inflammatory diseases. Dr. Bhat has over 20 years of experience in drug discovery and development, and prior to founding Reviva, he held research positions at XenoPort, ARYx Therapeutics, and Higuchi Biosciences Center in the United States.

Title: Challenges in using patient reported outcome measures in rare diseases research

York Health Economics Consortium, UK

Andria Hanbury is a psychologist with a background in quantitative and qualitative methods and applied health services research. Andria works within the Outcomes Research Team at York Health Economics Consortium, leading on NHS and public sector projects and proposals focussed on the use of patient reported outcome measures. Andria previously worked at the University of York, as the lead researcher on an implementation science research program. Andria has also worked as a Lecturer in Psychology and as an Advisor in Quantitative Research Methods for the Higher Education Academy,

SPEAKER SLOTS AVAII

Dublin Attractions



Rare Diseases Congress Reminisce













Glimpses of Rare Diseases Congress 2017











