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1718<sup>th</sup> Conference

**Scientific Program** 

# 4<sup>th</sup> World Congress on Rare Diseases and Orphan Drugs

June 11-12, 2018 | Dublin, Ireland



**UK: Conference Series IIc LTD** 

47 Churchfield Road, London, W3 6AY Toll Free: +0-800-014-8923 http://rarediseases.conferenceseries.com/europe/

#### 09:00-09:30 Registrations

#### Day 1 June 11, 2018

#### **Manor Suite**

### conferenceseries.com 09:30-09:50

### **Opening Ceremony**

Keynote Forum		
09:50-10:00	Introduction	
	Title:Serotonergic targets in the treatment of pulmonary fibrosis (PF) and pulmonary	
10:00-10:40	arterial hypertension (PAH)	
	Laxminarayan Bhat, Reviva Pharmaceuticals Inc, USA	
Networking & Refreshment Break: 10:40-11:00 @ Foyer		
11:00-11:40	Title: Surgical treatment of early onset scoliosis associated with rare disease	
	Gianluca Colella, Rizzoli Orthopaedic Institute, Italy	
	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	
Sessions: Rare Genetic Diseases   Pediatric Rare Diseases   Rare Hereditary Diseases   Diagnosis and Treatment for Rare Diseases   Clinical case studies on Rare Diseases		
Session Chair: Karren Williams, Akcea Therapeutics, USA		
	Alan Gilstrap, Akcea Therapeutics, USA	
	Session Introduction	
12:20-12:50	Title: Rare diagnosis in disorders/differences of sex development	
	Yolande van Bever, Erasmus Medical Centre, The Netherlands	
Workshop		
12:50-13:20	Title: Surgical treatment of scoliosis in rare diseases: Arthogryposis	
	Gianluca Colella, Rizzoli Orthopaedic Institute, Italy	
	Lunch Break: 13:20- 14:20 @ Peacock Restaurant	
14:20- 15:00	Title: Pulmonary arterial hypertension and pulmonary fibrosis: Treatments, unmet	
	needs, and future directions	
	Laxminarayan Bhat, Reviva Pharmaceuticals, Inc, USA	
	Session Title: Synthetic messenger RNA-based therapeutic strategy for treatment of alpha-1-	
15:00-15:30	antitrypsin deficiency	
	Tatjana Michel, University Hospital Tuebingen, Germany	
	Title: Evaluating the impact of peer support and connection on the quality of life of	
15:30-16:00	patients with familial Chylomicronemia Syndrome	
	Alan Gilstrap, Akcea Therapeutics, USA	
	Networking & Refreshment Break: 16:00-16:20 @ Foyer	
	Session	
16:20-16:50	Title: Rare diseases in Kuwait: The experience of Genatak Center for Genomic Medicine	
	Fahd Al Mulla, Kuwait University, Kuwait	
	Panel Discussion	

Day 2 June 12, 2018		
Manor Suite		
Sessions: Curr	rent Rare Diseases Research   Orphan Drugs Market Research   Rare Diseases of Sexual	
Health   Scop	e of Orphan Drugs  Rare Diseases in Neurology	
Session Chair	: Karren Williams, Akcea Therapeutics, USA	
Session Chair	: Alan Gilstrap, Akcea Therapeutics, USA	
	Session Introduction	
10:00-10:30	Title: Results of the investigation of findings and observations captured in burden of illness	
	survey in FCS patients (in-focus) study: European respondents	
	Karren Williams, Akcea Therapeutics, USA	
	Networking & Refreshment Break: 10:30- 10:50 @ Foyer	
10:50- 11:20	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	
	Workshop	
11:20- 13:00	Title: Synthetic messenger RNA (mRNA) as a therapeutic tool	
	Tatjana Michel, University Hospital Tuebingen, Germany	
	Lunch Break 13:00 - 14:00 @ Peacock Restaurant	
14:00- 14:30	Title: Rare diseases in west Africa	
	Ndiaye Mady, Faculty of Medicine of Thiès, Nigeria	
	Poster Presentations 14:30-15:30	
	Panel Discussion	



9<sup>th</sup> World Congress on

## **Rare Diseases and Orphan Drugs**

June 17-18, 2019 Rome, Italy

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