

Annual Congress on

Emerging Orphan Drugs and Rare Diseases

October 30 - 01 November, 2017 San Antonio, Texas, USA

Theme: "The developmental and regulatory landscape of orphan drugs in perspectives of gaming and abuse"



****For Available Speaker Slots****

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Conference Secretariat

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Scientific Program	
Day 1: October 30, 2017	
09:00-10:00	Registrations
10:00-10:25	Opening Ceremony
10:25-10:30	Introduction
Keynote Forum	
10:30-11:15	Title: Rare diseases and orphan drugs: Passion and compassion or growing market with career opportunities for scientists and technologists? Harsha K Rajasimha, George Mason University, USA
Networking and Refreshments Break @11:15-11:35	
11:35-12:20	Title: Speeding up access to medicines for patients with unmet medical need: Integrating evidence and regulatory pathways Stella Blackburn, QuintilesIMS, UK
12:20-13:05	Title: Lifting the burden of Rare Disease by providing access to next generation sequencing Romina Ortiz, Rare Genomics Institute, USA
Sessions: Different types of Rare Diseases Challenges in Rare Diseases Treatment Mystery Diagnosis of Rare Diseases Clinical Research and Public Awareness	
Session Introduction	
13:05-13:30	Title: The use of CRISPR/Cas9 to treat hereditary diseases: Duchenne Muscular Dystrophy, Friedreich ataxia and Alzheimer disease Jacques P. Tremblay, Université Laval, Canada
Lunch Break @ 13:30-14:30	
14:30-14:55	Title: Integrating informational resources for drug repurposing in rare diseases Maria Shkrob, Elsevier, USA
14:55-15:20	Title: L-Cystine Diamides as a Novel Therapy for Cystinuria Amrik Sahota, Rutgers University, USA
15:20-15:45	Title: Using Quantitative Pharmacology to Overcome Challenges in Hematopoietic Cell Transplantation: application towards primary immune deficiencies and inborn errors of metabolism Janel Long-Boyle, University of California San Francisco, USA
15:45-16:10	Title: Nephrotic syndrome: Predicting and defining therapeutic outcomes by systems biology omics approaches Shipra Agrawal, The Research Institute at Nationwide Childrens Hospital, USA
Networking and Refreshments Break @ 16:10-16:30	
16:30-16:55	Title: Pediatric Rare Disease Enrollment Case Study in Latin America Sara G Tylosky, Farmacon, USA
Panel Discussion	
Day 2: October 31, 2017	
Keynote Forum	
10:00-10:45	Title: Mitochondrial bioenergetics in porphyria: Studies in peripheral blood cells Ashwani K Singal, University of Alabama at Birmingham, USA
Networking and Refreshments Break 10:45-11:05	
11:05-11:50	Title: OP-101: A novel therapy for treatment of childhood cerebral Adrenoleukodystrophy Sujatha Kannan, Johns Hopkins University School of Medicine, USA
Sessions: Orphan Drugs- development trends and strategies Clinical Research on Orphan Drugs Rare Diseases in Cancer	
Session Introduction	
11:50-12:15	Title: The Middle East: A mine for orphan drugs development Tony Zbeidy, General Manager MENA, Orphan-Europe
Workshop	
12:15-13:15	Title: Porphyrias – An update on the management and unmet clinical needs Ashwani K Singal, University of Alabama at Birmingham, USA

Lunch Break @ 13:15-14:15	
14:15-14:40	Title: Pharmacological Chaperone Therapy for neuronopathic Gaucher disease Aya Narita, Tottori University Hospital, Japan
14:40-15:05	Title: Modeling Li-Fraumeni syndrome by induced pluripotent stem cells Dung-Fang Lee, The University of Texas Health Science Center at Houston, USA
15:05-15:30	Title: Genomic approaches in modern biotechnology-from discovery, translation to implementation David Dongliang Ge, Apostle Inc. , USA
Special Session	
15:30-16:30	Title: Consumer Directed Precision Health - Convergence of Multi- omics, Environment, Life style and Behaviors Praduman Jain, Vibrent Health Inc., USA
Networking and Refreshments Break @ 16:30-16:50	
16:50-17:15	Title: POEMS syndrome in a 65 years old lady presenting with peripheral neuropathy and recent onset diabetes Arnab Ghosh, Armed Forces Medical College(AFMC), India
Poster Presentations	
RD-01	Title: Complex diagnostic approach in cases of Alveolar echinococcosis Katarína Šimeková, University Hospital in Martin, Slovak republic
RD-02	Title: Rare lung dissemination of life-threatening liver parasitosis Róbert Rosolanka, University Hospital in Martin, Slovak republic
Panel Discussion	
Day 3: November 1, 2017	
Keynote Forum	
10:00-10:45	Title: Consumer Directed Precision Health - Convergence of Multi- omics, Environment, Life style and Behaviors Praduman Jain, Vibrent Health Inc., USA
Networking and Refreshments Break @ 10:45-11:05	
11:05-11:30	Title: The role of auto zelk bridge soft wares, hard wares, and Bioelectronics in biomedical and space science advanced research and new vaccine and drug for cure discovery and development Zelalem Kiros Bitsue, United States of Africa Health Organization "AHO", South Africa
11:30-11:55	Title: A case of hoffman syndrome masquerading as pituitary tumour Arnab Ghosh, Armed Forces Medical College(AFMC), India
11:55-12:20	Title: Cutaneous infarcts and anca positive vasculitis in airt: A rare presentation Abhish Mohan, Armed Forces Medical College, India
Networking Lunch Break @ 12:20-13:20	
Panel Discussion	
Award Ceremony	