

3rd Annual Congress on
Rare Diseases &

Orphan Drugs

Oct 30-Nov 01, 2017 San Antonio, USA

Moderator

Thank You for supporting Rare Diseases 2017



Tony Zbeidy

Orphan-Europe, France

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Conference Series-America

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Opening Ceremony

Keynote Forum

10:25-10:30	Introduction
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

Panel Discussion

Networking and Refreshments Break 11:15-11:35 @ Texas D Foyer

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: Mitochondrial bioenergetics in porphyria: Studies in peripheral blood cells Ashwani K Singal, University of Alabama at Birmingham, USA

Panel Discussion

Lunch Break 13:05-14:05 @ Brazos

Sessions: Different types of Rare Diseases | Challenges in Rare Diseases Treatment | Mystery Diagnosis of Rare Diseases | Clinical Research and Public Awareness

Session Chair: Amrik Sahota, Rutgers University, USA

Session Introduction

14:05-14:30	Title: The use of CRISPR/Cas9 to treat hereditary diseases: Duchenne Muscular Dystrophy, Friedreich ataxia and Alzheimer disease Jacques P Tremblay, Universite Laval, Canada
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

Panel Discussion

Networking and Refreshments Break 16:10-16:30 @ Texas D Foyer
Workshop

16:30-17:30	Title: Porphyrias – An update on the management and unmet clinical needs Ashwani K Singal, University of Alabama at Birmingham, USA
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Panel Discussion

Day 2 October 31, 2017

Conference Hall: Texas D

Keynote Forum

	Introduction
10:00-10:45	Title: Pediatric rare disease enrollment case study in Latin America Sara Tylosky, Farmacon, USA

Panel Discussion

Networking and Refreshments Break 10:45-11:05 @ Texas D Foyer

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
11:50-12:35	Title: Lifting the burden of rare disease by providing access to next generation sequencing Romina Ortiz, Rare Genomics Institute, USA

Sessions: Orphan Drugs- development trends and strategies | Clinical Research on Orphan Drugs | Rare Diseases in Cancer

Session Chair: Tony Zbeidy, Orphan-Europe, France

Session Introduction

12:35-13:00

Title: The Middle East: A mine for orphan drugs development

Tony Zbeidy, Orphan-Europe, France

Lunch Break 13:00-14:00 @ Brazos

Special Session

14:00-15:00

Title: Consumer directed precision health - Convergence of multi- omics, environment, life style and behaviors

Praduman Jain, Vibrent Health Inc., USA

15:00-15:25

Title: Pharmacological chaperone therapy for neuronopathic gaucher disease

Aya Narita, Tottori University Hospital, Japan

15:25-15:50

Title: Modeling Li-Fraumeni syndrome by induced pluripotent stem cells

Dung-Fang Lee, The University of Texas Health Science Center at Houston, USA

15:50-16:15

Title: Genomic approaches in modern biotechnology-from discovery, translation to implementation

David Dongliang Ge, Apostle Inc. , USA

Panel Discussion

Networking and Refreshments Break 16:15-16:35 @ Texas D Foyer

Poster Judge : Harsha K Rajasimha, George Mason University, USA

Poster Session (16:35:17:00)

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 Rosol'anka, University Hospital in Martin, Slovak Republic

Panel Discussion

Award Ceremony

Day 3 November 01, 2017

Conference Hall: Texas D

Keynote Forum

Introduction

10:00-10:45

Title: Consumer directed precision health - Convergence of multi- omics, environment, life style and behaviors
Praduman Jain, Vibrent Health Inc., USA

Panel Discussion

Networking & Refreshment Break 10:45-11:05 @ Texas D Foyer

11:05-11:30

Title: A case of hoffman syndrome masquerading as pituitary tumour

Arnab Ghosh, Armed Forces Medical College(AFMC), India

11:30-11:55

Title: Cutaneous infarcts and antineutrophil cytoplasm antibodies positive vasculitis in angioimmunoblastic T-cell lymphoma: A rare presentation

Abhish Mohan, Armed Forces Medical College, India

11:55-12:20

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

Panel Discussion

Lunch Break 12:20-13:20 @ Brazos

Networking & Refreshment Break 04:00-04:20 @ Texas D Foyer

Closing Ceremony