Day 1 October 30, 2017

09:00-10:00 Registrations

Conference Hall: Texas D

conferenceseries.com 10:00-10:25

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

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 Rosoľanka, 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 Cermony	