conferenceseries LLC Ltd
1968th Conference

Scientific Program

5th Annual Congress on

Rare Diseases & Orphan Drugs

August 29-30, 2018 | Boston, USA

Sponsor

SELECTA Biosciences

Day 1 August 29, 2018

Conference Hall: University Room A

08:45-9:45 Registrations

09:45-10:00 Opening Ceremony

Keynote Forum

10:00-10:50 **Introduction**
Title: A patient at the table: How partnership with patients improves research and enhances service delivery
Alastair Kent, Genetic Alliance, UK

Panel Discussion & Group Photo

Networking & Refreshment Break 10:50-11:05 @ Foyer

11:05-11:55 **Title: Measuring the impact of diagnosis and treatment of rare diseases**
Alba Ancochea, EURORDIS, Spain

Panel Discussion

Sessions: Different types of Rare Diseases | Clinical Research and Public Awareness | Mystery Diagnosis of Rare Diseases | Clinical Research on Orphan Drugs

Session Chair: Alastair Kent, Genetic Alliance, UK

Session Introduction

11:55-12:30 **Title: Re-dosing AAV gene therapy vectors – the elephant in the room**
Kei Kishimoto, Selecta Biosciences, USA

12:30-13:05 **Title: Latin America calls members of congress to legislate on rare diseases**
Fernando Ferrer, Multinational Partnerships LLC, USA

Panel Discussion

Lunch Break 13:05-14:00 @ Foyer

Workshop

14:00-15:00 **Title: Helping patients cope with, and recover from the effects of living with a rare disease: A patient's perspective**
Mark Landiak, Patient Advocate, Foundation for Sarcoidosis Research, USA

Special Session

15:00-16:00 **Title: Overview of rare disease funding at NIH**
Jason Wan, National Institutes of Health, USA

Panel Discussion

Networking & Refreshment Break 16:00-16:15 @ Foyer

Day 2 August 30, 2018

Conference Hall: University Room A

Keynote Forum

10:00-10:50 **Title: Targeting familial Alzheimer's disease**
Michael S Wolfe, University of Kansas, USA

Panel Discussion

Networking & Refreshment Break 10:50-11:05 @ Foyer

Session Introduction

Sessions: Rare Infectious Diseases and Immune Deficiencies | Rare Diseases in Cancer | Challenges in Rare Diseases Treatment | Mystery Diagnosis of Rare Diseases

Session Chair: Michael S Wolfe, University of Kansas, USA

11:05-11:40 **Title: A comparative analysis of demographic information among 12 neural intractable diseases in a national registry of rare disease in Japan**
Yoko Sato, National Defense Medical College, Japan

11:40- 12:15 **Title: Macrophage activation syndrome and acquired hemophagocytic lymphohistiocytosis in adults: A Philadelphia cohort**
Irene Tan, Temple School of Medicine, USA

Workshop

12:15-13:15 **Title: Sarcoidosis: The most common rare disease**
Mark Landiak, Patient Advocate, Foundation for Sarcoidosis Research, USA

Panel Disccussion

Lunch Break: 13:15-14:05 @ Foyer

Special Session

14:05-15:35 **Title: How to educate others about your rare disease**
Dana Mauro, National Ataxia Foundation, USA

Panel Discussion

Networking & Refreshment Break 15:35-15:50 @ Foyer

Award Ceremony

Thanks giving & Closing ceremony