Tentative Program

3rd Annual Congress on

Rare Diseases and Orphan Drugs

October 30-November 01, 2017  San Antonio, USA

Theme: “Platform to share new treatments and research for Rare Diseases”

For Available Speaker Slots

rarediseases@infectiousconferences.com/rarediseases@infectiousconferences.org

19+ Interactive Sessions  15+ Keynote Lectures  75+ Plenary Lectures  5+ Workshops

Conference Secretariat
2360 Corporate Circle, Suite 400 Henderson, NV 89074-7722, USA
Email: rarediseases@infectiousconferences.com/rarediseases@infectiousconferences.org
Program at a Glance

Tentative 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

Track 1: Different types of Rare Diseases
Track 2: Clinical Research and Public Awareness
Track 3: Mystery Diagnosis of Rare Diseases

12:20-12:45  Title: The use of CRISPR/Cas9 to treat hereditary diseases: Duchenne Muscular Dystrophy, Friedreich ataxia and Alzheimer disease
             Jacques P. Tremblay, Department of Molecular Medicine, Université Laval, Québec, Canada

12:45-13:10  Title: Integrating informational resources for drug repurposing in rare diseases
             Maria Shkrob, Elsevier, USA

Lunch Break 13:10-14:10

14:10-14:35  Title: L-Cystine Diamides as a Novel Therapy for Cystinuria
             Amrik Sahota, Rutgers University, USA

14:35-15:00  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

Workshop

15:00-16:00  Title: Porphyrias – An update on the management and unmet clinical needs
             Ashwani K Singal, University of Alabama at Birmingham, USA

Networking and Refreshments Break 16:00-16:20

16:20-16:45  Title: Nephrotic syndrome: Predicting and defining therapeutic outcomes by systems biology omics approaches
             Shipra Agrawal, The Research Institute at Nationwide Childrens Hospital, USA

16:45-17:10  Title: Pediatric Rare Disease Enrollment Case Study in Latin America
             Sara G Tyfusky, 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: Lifting the burden of Rare Disease by providing access to next generation sequencing
             Romina Ortiz, Rare Genomics Institute, USA

Track 8: Orphan Drugs, Development trends and strategies
Track 9: Orphan Drugs and Ethical Issues
Track 10: Future Hereditary and Rare Diseases e Prospects of Rare Diseases

Session Introduction

             Sujatha Kannan, Johns Hopkins University School of Medicine, USA

12:15-12:40  Title: The Middle East: A mine for orphan drugs development
             Tony Zbeidy, General Manager MENA, Orphan-Europe

12:40-13:05  Title: Pharmacological Chaperone Therapy for neuromuscular Gaucher disease
             Aya Narita, Tottori University Hospital, Japan

13:05-13:30  Title: Modeling Li-Fraumeni syndrome by induced pluripotent stem cells
             Dung-Fang Lee, The University of Texas Health Science Center at Houston, USA

Lunch Break 13:30-14:30

Special Session

14:30-15:30  Title: Consumer Directed Precision Health - Convergence of Multi-omics, Environment, Life style and Behaviors
             Prafullan Jain, Vibrant Health Inc., USA

15:30-15:55  Title: Genomic approaches in modern biotechnology-from discovery, translation to implementation
             David Dongliang Ge, Apostle Inc., USA

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<tr>
<th>Time</th>
<th>Event</th>
<th>Details</th>
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<tr>
<td>15:55-16:20</td>
<td>Title: POEMS syndrome in a 65 years old lady presenting with peripheral neuropathy and recent onset diabetes</td>
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<td>Arnab Ghosh, Armed Forces Medical College (AFMC), India</td>
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<td><strong>Networking and Refreshments Break 16:20-16:40</strong></td>
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<td>16:40-17:05</td>
<td>Title: Developing Novel, Technology-Derived Endpoints for Use in Clinical Trials</td>
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<td>Theresa Strong, Foundation for Prader Willi Research, USA</td>
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<td><strong>Poster Presentations</strong></td>
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<td>RD-01</td>
<td>Title: Complex diagnostic approach in cases of Alveolar echinococcosis</td>
<td>Katarína Šimeková, University Hospital in Martin, Slovak republic</td>
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<td>RD-02</td>
<td>Title: Rare lung dissemination of life-threatening liver parasitosis</td>
<td>Róbert Rosoľanka, University Hospital in Martin, Slovak republic</td>
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<td><strong>Panel Discussion</strong></td>
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<td><strong>Award Ceremony</strong></td>
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<td>Day 3</td>
<td><strong>Networking and Refreshments Break 10:00-10:30</strong></td>
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<tr>
<td>10:00-10:45</td>
<td>Title: Consumer Directed Precision Health - Convergence of Multi-omics, Environment, Life style and Behaviors</td>
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<td>Praduman Jain, Vibrent Health Inc., USA</td>
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<td><strong>Networking and Refreshments Break 10:45-11:05</strong></td>
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<td>11:05-11:30</td>
<td>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</td>
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<td>Zelalem Kiros Bitsue, United States of Africa Health Organization “AHO”, South Africa</td>
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<td>11:30-11:55</td>
<td>Title: A case of hoffman syndrome masquerading as pituitary tumour</td>
<td>Arnab Ghosh, Armed Forces Medical College (AFMC), India</td>
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<td><strong>Panel Discussion</strong></td>
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<td>11:55-12:20</td>
<td>Title: Cutaneous infarcts and anca positive vasculitis in aitl: A rare presentation</td>
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<td>Abhishek Mohan, Armed Forces Medical College, India</td>
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<td><strong>Networking Lunch Break 12:20-13:20</strong></td>
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<td>13:20-13:45</td>
<td>Title: Streptococcus suis: Bacteremia presenting with fever, rashes, arthritis and neurologic deficits</td>
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<td>Ahmad M. Domado, Southern Philippines Medical Center, Philippines</td>
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<td>13:45-14:10</td>
<td>Title: Hydatid cyst epidemiology in Khuzestan, Iran: A 15-years evaluation</td>
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<td>Amin Kamali, Health Center of Khuzestan Province, Iran</td>
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<td><strong>Panel Discussion</strong></td>
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<td><strong>Networking and Refreshments Break 04:00-04:20</strong></td>
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For detailed sessions & sub-sessions, kindly visit:
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Register Online:
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San Antonio officially the City of San Antonio, is the seventh most populated city in the United States of America and the second most populated city in the state of Texas, with a population of 1,409,019. It was the fastest growing of the top 10 largest cities in the United States from 2000 to 2010, and the second from 1990 to 2000. The city is located in the American Southwest, the south–central part of Texas, and the southwestern corner of an urban region known as the Texas Triangle.

San Antonio hosts over 100,000 students in its 31 higher-education institutions. Publicly supported schools include the University of Texas Health Science Center at San Antonio, the University of Texas at San Antonio, Texas A&M University–San Antonio, and the Alamo Community College District. The University of Texas at San Antonio is San Antonio’s largest university.

Venue

Hilton San Antonio Airport
611 NW Loop 410 San Antonio
Texas, USA, 78216
Conference Highlights

- Different types of Rare Diseases
- Clinical Research and Public Awareness
- Mystery Diagnosis of Rare Diseases
- Challenges in Rare Diseases Treatment
- Rare Infectious Diseases and Immune Deficiencies
- Rare Diseases in Cancer
- Rare Diseases in Aging
- Orphan Drugs- development trends and strategies
- Clinical Research on Orphan Drugs
- Orphan Drugs and Ethical Issues
- Future Hereditary of Rare Diseases and Orphan Drugs
- Entrepreneurs Investment Meet

For more about the Tracks click here to the following link
http://rarediseases.conferenceseries.com/call-for-abstracts.php
Scientific Program

Annual Congress on Rare Diseases and Orphan Drugs

October 26-27, 2016   Chicago, USA
Day 1    October 26, 2016

Registrations

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Opening Ceremony

Keynote Forum

Introduction
Title: The rare diseases clinical research network (RDCRN) program (a model for collaborative research) and resources at NCATS, NIH
Rashmi Gopal-Srivastava, NCATS, NIH, USA

Title: Inside track to an orphan designation
Timothy Coté, Coté Orphan, USA

Networking and Refreshment Break

Group Photo

Major Sessions:
Different types of Rare Diseases | Clinical Research and Public Awareness | Mystery Diagnosis of Rare Diseases

Session Chair: Amrik Sahota, Rutgers University, USA
Session Co-chair: Lisa Baumbach-Reardon, Translational Genomics Research Institute, USA

Session Introduction

Title: Tailored inhibition of cystine stone formation as a therapy for cystinuria
Amrik Sahota, Rutgers University, USA

Title: Failures in brain energy metabolism unveil therapeutic targets for Huntington’s disease
Maite A. Castro, Universidad Austral de Chile, Valdivia, CHILE

Title: Epilepsy & rescue meds in schools
Tyson E. Dewsnup, Epilepsy Association of Utah, USA

Title: Neo212: A new drug for temozolomide resistant malignant gliomas
Thomas C. Chen, Keck School of Medicine of USC, USA

Title: Management of life threatening hyperammonemia in children
Philippe Jouvet, Sainte-Justine Hospital University of Montreal, Canada

Lunch Break

Title: Defending the Dream; perils beyond science and finance
Laura Sunderland, Beazley, USA

Title: Engaging families in research to drive progress: The phelan-mcdermid syndrome international registry (PMSIR) and the phelan-mcdermid foundation data network (PMS_DN)
Megan O’Boyle, Phelan-McDermid Syndrome Foundation, USA

Title: Fever of unknown origin: Case study
Yusuf Hovsep Eken, Elkerliek ziekenhuis, Netherland

Title: A 20 days old patient with genital asymmetry
Marise Abdou, Abo El-Rish Children’s Hospital, Cairo, Egypt

Title: Association between fbn1 polymorphisms and tgf- β1 concentration within aneurysms and dissections of ascending thoracic aorta
Ramune Sepetiene, Lithuanian University of Health Sciences, Kaunas, Lithuania

Major Sessions:
Challenges in Rare Diseases Treatment | Rare Diseases in Cancer | Rare Infectious Diseases and Immune Deficiencies

Session Chair: Lisa Baumbach-Reardon, Translational Genomics Research Institute, USA

Title: On-going exon 53 skipping clinical trial for Duchenne Muscular Dystrophy
Shin’ichi TAKEDA, National Institute of Neuroscience, Japan

Title: Rare disease research: Opportunities and challenges
Lisa Baumbach-Reardon, Translational Genomics Research Institute, USA

Networking and Refreshment Break

Title: COST Action BM1207: Involving all stakeholders to overcome challenges of genetic therapy development for Duchenne Muscular Dystrophy
Annemieke Aartsma-Rus, Leiden University Medical Center, The Netherlands
Panel Discussion

Day 2 October 27, 2016

Keynote Forum

Title: How patient organizations can change the game in innovation and drug marketing: Example of AFMTelethon
Serge Braun, Scientific Director, AFMTelethon, France

Major Sessions:
Orphan Drugs, Development Trends and Strategies | Orphan Drugs and Ethical Issues | Future Hereditary and Rare Diseases & Prospects of Rare Diseases
Session Chair: Alan B. Moy, The John Paul II Medical Research Institute and Cellular Engineering Technologies, USA

Session Introduction
Title: Network pharmacology based repurposed drugs combination for orphan diseases treatment
Daniel Cohen, Pharnext, France

Networking and Refreshment Break
Title: JBPOS0101: A new generation mGluR- and BBB-targeted broad spectrum antiepileptic drug for the treatment of super-refractory status epilepticus
Yong Moon Choi, Bio-Pharm Solutions Co., Ltd, South Korea

Title: Cellular therapies for rare disease
Stephen Shrewsbury, Fortuna Fix, USA

Title: Digital measurements of health- regulatory science challenges opportunities in rare diseases
Stephen P. Arneric, Critical Path Institute, Tucson, AZ, USA

Title: Novel viral-free and oncogene-free induced pluripotent stem cell for orphan disease cell therapies
Alan B. Moy, The John Paul II Medical Research Institute and Cellular Engineering Technologies, USA

Title: Orphan drugs: Getting arms around rare diseases
Duygu Kuyuncu Irmak, INC Research, Turkey

Lunch Break

Title: Pricing, public health, & politics: Policy considerations for orphan products
Anne Marie Finley, Biotech Policy Group LLC, USA

Title: The role of clinical genomic testing in treatment discovery for rare neurodevelopmental diseases
Karen S. Ho, Lineagen, Inc, USA

Title: Rare disease studies facilitated by taking study visits to the patients
Gail Adinamis, GlobalCare Clinical Trials, LLC, USA

Title: A robust reprogramming approach to create viral-free and oncogene-free, orphan-disease specific induced pluripotent stem cells from peripheral blood mononuclear cells
Alan B. Moy, The John Paul II Medical Research Institute and Cellular Engineering Technologies, USA

Title: Challenges and opportunities in development of orphan drugs
Moji C. Adeyeye, Roosevelt University, USA

Title: Potential treatments for rare diseases: Cell therapy, gene therapy and genome editing
Jacques P. Tremblay, Department of Molecular Medicine, Université Laval, Québec, Canada

Networking and Refreshment Break

Poster Presentations
Title: An online survey of neurologists about charcot-marie-tooth disease type 1A
Xavier Paoli, Pharnext, France

Title: The first case report of Raoultella planticola liver abscess
Moamen Al Zoubi, Advocate Illinois Masonic Medical Center, Chicago, USA

Title: Rapid progression osteolysis in Gorham Stout Syndrome: A case report and literature review
Leonardo Cano, Universidad Católica Santiago de Guayaquil, Ecuador

Award Ceremony