10th Annual Congress on
Rare Diseases and Orphan Drugs

September 23-24, 2019 Toronto | Canada
# PROGRAM @ GLANCE

## Day 1 September 23, 2019
- Opening Ceremony
- Plenary & Keynote Speeches (09:00-11:30)
- Networking and Refreshments Break
- Group Photo

<table>
<thead>
<tr>
<th>Speaker Session I (11:30-13:00)</th>
<th>Different types of Rare Diseases</th>
<th>Clinical Research and Public Awareness</th>
<th>Mystery Diagnosis of Rare Diseases</th>
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<tbody>
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<td>Panel Discussion</td>
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- Lunch Break

<table>
<thead>
<tr>
<th>Speaker Session II (13:00-16:00)</th>
<th>Challenges in Rare Diseases Treatment</th>
<th>Rare Infectious Diseases and Immune Deficiencies</th>
<th>Rare Diseases in Cancer</th>
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- Networking and Refreshments Break

<table>
<thead>
<tr>
<th>Speaker Session III (16:00-18:00)</th>
<th>Rare Diseases in Aging</th>
<th>Orphan Drugs- development trends and strategies</th>
<th>Orphan Drugs and Ethical Issues</th>
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- Day 1 concludes...

## Day 2 September 24, 2019
- Opening Ceremony
- Plenary & Keynote Speeches (09:00-11:30)
- Networking and Refreshments Break

<table>
<thead>
<tr>
<th>Speaker Session I (11:30-13:00)</th>
<th>Clinical Research on Orphan Drugs</th>
<th>Orphan Drugs and Ethical Issues</th>
<th>Future Hereditary of Rare Diseases and Orphan Drugs</th>
<th>Entrepreneurs Investment Meet</th>
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<td>Panel Discussion</td>
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- Lunch Break

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<th>Speaker Session II (13:00-16:00)</th>
<th>Different types of Rare Diseases</th>
<th>Mystery Diagnosis of Rare Diseases</th>
<th>Patient organizations and their role in drug development or clinical research</th>
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<td>Panel Discussion</td>
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- Networking and Refreshments Break

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<tr>
<th>Speaker Session III (16:00-18:00)</th>
<th>Poster Presentations</th>
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<tbody>
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<td>Best Poster Award Distribution</td>
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- Day 2 concludes...
A patient at the table: How partnership with patients improves research and enhances service delivery

Alastair Kent, Genetic Alliance, UK

Measuring the impact of diagnosis and treatment of rare diseases

Alba Ancochea, EURORDIS, Spain

Targeting familial Alzheimer’s disease

Michael S. Wolfe, University of Kansas, USA

Consumer Directed Precision Health - Convergence of Multi-omics, Environment, Life style and Behaviors

Praduman Jain, Vibrent Health Inc., USA

Speeding up access to medicines for patients with unmet medical need: Integrating evidence and regulatory pathways

Stella Blackburn, QuintilesIMS, UK
**Past Keynote Speakers**

*Pediatric Rare Disease Enrollment Case Study in Latin America*

Sara Tylosky, Farmacon, USA

*Lifting the burden of Rare Disease by providing access to next generation sequencing*

Romina Ortiz, Rare Genomics Institute, USA

**Past Workshop’s**

*Helping patients cope with, and recover from the effects of living with a rare disease: A patient’s perspective*

Mark Landiak, Corporate Dynamics, Inc. USA

*Overview of rare disease funding at NIH*

Jason Wan, National Institutes of Health, USA

*How to educate others about your rare disease*

Dana Mauro, National Ataxia Foundation, USA
Demographics

Illustrated in Figure 1, 2 and 3 are the recent Demographics for Speakers, Delegates and attendees from the Continents.

Our conference is surrounded with Doctors, Deans, Professors, Students, Young researchers, Health Practitioners, Relevant Association and Societies, Business professionals all over the globe, from which most of them are decision makers.
Rare Diseases and Orphan Drugs, past Speakers & Delegates

Abdulaziz Aldawood, King Saud Bin Abdulaziz University, Saudi Arabia
Alan B. Moy, The John Paul II Medical Research Institute and Cellular Engineering Technologies, USA
Alan Gilstrap, Akcea Therapeutics, USA
Alastair Kent, Genetic Alliance, UK
Alba Ancochea, EURORDIS, Spain
Alice Abdel Aleem, Weill Cornell Medical College, Qatar
Amrik Sahota, Rutgers University, USA
Anne Marie Finley, Biotech Policy Group LLC, USA
Annemieke Aartsma-Rus, Leiden University Medical Center, The Netherlands
April Weir Hauptman, InClin, USA
Ashwani K Singal, University of Alabama at Birmingham, USA
Aya Narita, Tottori University Hospital, Japan
Charles Du Mond, InClin, USA
Courtney Smith, Colpitts Clinical, USA
Dana Mauro, National Ataxia Foundation, USA
Daniel Cohen, Pharmnext, France
Danilo A Tagle, National Institutes of Health, USA
David Dongliang Ge, Apostle Inc., USA
Deanna Laurain, InClin, USA
Diego-Abelardo Alvarez-Hernandez, Anahuac University, Mexico
Dung-Fang Lee, The University of Texas Health Science Center at Houston, USA
Duygu Kuyuncu Irmak, INC Research, Turkey
E. Dewsnup, Epilepsy Association of Utah, USA
Fahd Al Mulla, Kuwait University, Kuwait
Fernando Ferrer, Multinational Partnerships LLC, USA
Gail Adinamis, GlobalCare Clinical Trials, LLC, USA
Gayathri Balasubramanian, Focus Scientific Research CenterPhamax, India
George Faurot, InClin, USA
Gianluca Colella, Rizzoli Orthopaedic Institute, Italy
Godwin Oligbu, St Georges Hospital London, UK
Harsha K Rajasimha, George Mason University, USA
Irene Tan, Temple School of Medicine, USA
Isabelle Windheuser, University Hospital Bonn, Germany
Jacques P Tremblay, Universite Laval, Canada
Jacques P. Tremblay, Department of Molecular Medecine, Université Laval, Québec
Janel Long-Boyle, University of California San Francisco, USA
Jason Wan, National Institutes of Health, USA
Jianmeng Chen, Food and Drug Administration, USA
John A Mauro, National Ataxia Foundation, USA
John Leaman, Selecta Biosciences, USA
Jong Wook Chang, Samsung Medical Center, South Korea
Karen S. Ho, Lineagen, Inc, USA
Karren Williams, Akcea Therapeutics, USA
Katarína Šimeková, University Hospital in Martin, Slovak Republic
Kei Kishimoto, Selecta Biosciences, USA
Kim Frieze, InClin, USA
Larissa Wester, University Hospital Bonn, Germany
Laura Sunderlin, Beazley, USA
Laxminarayan Bhat, Reviva Pharmaceuticals Inc, USA
Lei He, Food and Drug Administration, USA
Leonardo Cano, Universidad Católica Santiago de Guayaquil, Ecuador
Lisa Baumbach-Reardon, Translational Genomics Research Institute, USA
Maite A. Castro, Universidad Austral de Chile, CHILE
Manoj Krishnan, Duke-NUS, Singapore
Maria Shkrob, Elsevier, USA
Marise Abdou, Abo El-Rish Children’s Hospital, Cairo, Egypt
Mark Landiak, Patient Advocate, Foundation for Sarcoidosis Research, USA
Megan O’Boyle, Phelan-McDermid Syndrome Foundation, USA
Michael S Wolfe, University of Kansas, USA
Mingli Jiao, Harbin Medical University, China
Moamen Al Zoubi, Advocate Illinois Masonic Medical Center, USA
Moji C. Adeyeye, Roosevelt University, USA
Nadia Ameen, Yale University School of Medicine, USA
Nasir Ali Afsar, Alfaisal University College of Medicine, KSA
Ndiaye Mady, Faculty of Medicine of Thiès, Nigeria
Patrick J Tighe, University of Nottingham, U.K
Philippe Jouvet, Sainte-Justine Hospital University of Montreal, Canada
Prasanth Puthanveetil, Roosevelt University College of Pharmacy, USA
Ramune Sepetiene, Lithuanian University of Health Sciences, Lithuania
Rashmi Gopal-Srivastava, NCATS, NIH, USA
Rob W J Collin, Radboud University Medical Center, Netherlands
Róbert Rosofanka, University Hospital in Martin, Slovak Republic
Romina Ortiz, Rare Genomics Institute, USA
Ryan Clift, InClin, USA
Samuel Ayoola Abati, Lagos University, Nigeria
Sara Tylosky, Farmacon, USA
Serge Braun, Scientific Director, AFMTelethon, France
Shin’ichi TAKEDA, National Institute of Neuroscience, Japan
Shipra Agrawal, The Research Institute at Nationwide Childrens Hospital, USA
Shmuel Prints, Clalit Health Service, Israel
Stefano Giacomini, Rizzoli Orthopedic Institute, Italy
Stella Blackburn, QuintilesIMS, UK
Stephen P. Arneric, Critical Path Institute, USA
Stephen Shrewsbury, Fortuna Fix, USA
Stephen Smolinski, Selecta Biosciences, USA
Sujatha Kannan, Johns Hopkins University School of Medicine, USA
Tatjana Michel, University Hospital Tuebingen, Germany
Thomas C Chen, Keck School of Medicine of USC, USA
Timothy Coté, Coté Orphan, USA
Tony Zbeidy, Orphan-Europe, France
Wei Zheng, National Institutes of Health, USA
Xavier Paoli, Pharmnext, France
Xiaolan Zhang, Sarepta Therapeutics, USA
Yingjun Xie, The Third Affiliated Hospital of Guangzhou Medical University, China
Yoko Sato, National Defense Medical College, Japan
Yolande van Bever, Erasmus Medical Centre, The Netherlands
Yong Moon Choi, Bio-Pharm Solutions Co., Ltd, South Korea
Yunzhao Ren, Food and Drug Administration, USA
Yusuf Hovsep Eken, Elkerliek ziekenhuis, Netherlands
WHAT YOU CAN EXPECT
Glimpses of Rare Diseases Conference