



10<sup>th</sup> Annual Congress on

# Rare Diseases and Orphan Drugs

September 23-24, 2019 Toronto | Canada

10<sup>th</sup> Annual Congress on**Rare Diseases and Orphan Drugs**

September 23-24, 2019 Toronto | Canada

**PROGRAM @ GLANCE**

Day 1 September 23, 2019

Opening Ceremony

Plenary &amp; Keynote Speeches (09:00-11:30)

Networking and Refreshments Break

Group Photo

Speaker Session I (11:30-13:00)

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

Panel Discussion

Lunch Break

Speaker Session II (13:00-16:00)

Challenges in Rare Diseases Treatment | Rare Infectious Diseases and Immune Deficiencies | Rare Diseases in Cancer

Panel Discussion

Networking and Refreshments Break

Speaker Session III (16:00-18:00)

Rare Diseases in Aging | Orphan Drugs- development trends and strategies | Orphan Drugs and Ethical Issues

Panel Discussion

Day 1 concludes...

Day 2 September 24, 2019

Opening Ceremony

Plenary &amp; Keynote Speeches (09:00-11:30)

Networking and Refreshments Break

Speaker Session I (11:30-13:00)

Clinical Research on Orphan Drugs | Orphan Drugs and Ethical Issues | Future Hereditary of Rare Diseases and Orphan Drugs | Entrepreneurs Investment Meet

Panel Discussion

Lunch Break

Speaker Session II (13:00-16:00)

Different types of Rare Diseases | Mystery Diagnosis of Rare Diseases | Patient organizations and their role in drug development or clinical research

Panel Discussion

Networking and Refreshments Break

Speaker Session III (16:00-18:00)

Poster Presentations

Best Poster Award Distribution

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

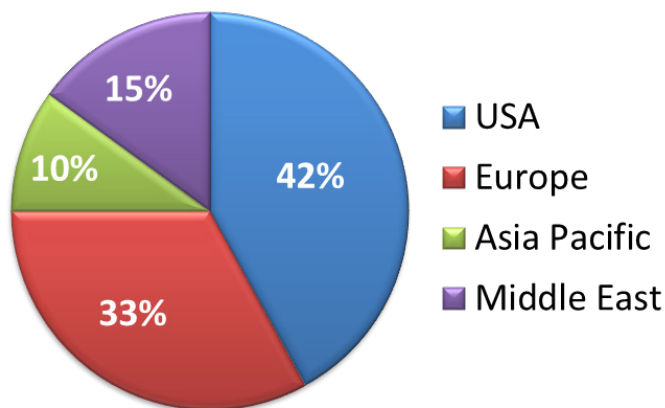


*How to educate others about your rare disease*

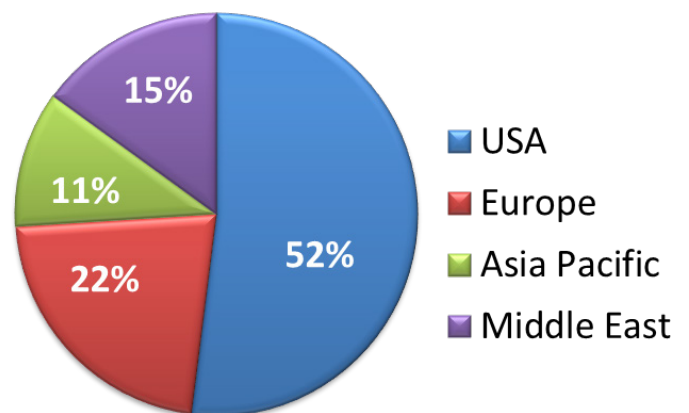
Dana Mauro, National Ataxia Foundation, USA



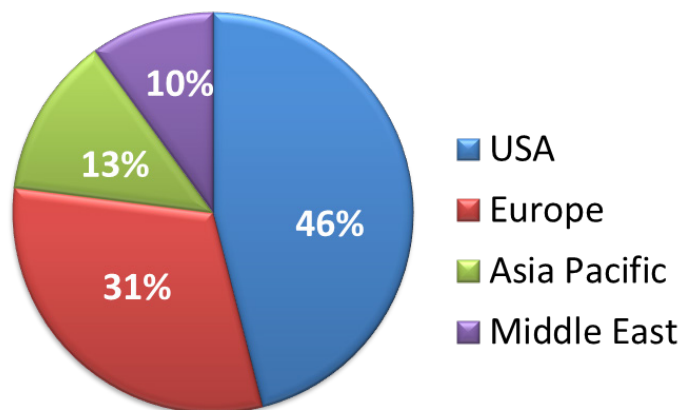
## Demographics of Speakers



## Demographics of Delegates



## Continent Demographics



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.

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

# 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, Pharnext , 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. Dewsnap, 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

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

<b>Michael S Wolfe, University of Kansas, USA</b>	<b>Shipra Agrawal, The Research Institute at Nationwide Childrens Hospital, USA</b>
<b>Mingli Jiao, Harbin Medical University, China</b>	<b>Shmuel Prints, Clalit Health Service, Israel</b>
<b>Moamen Al Zoubi, Advocate Illinois Masonic Medical Center, USA</b>	<b>Stefano Giacomini, Rizzoli Orthopedic Institute, Italy</b>
<b>Moji C. Adeyeye, Roosevelt University, USA</b>	<b>Stella Blackburn, QuintilesIMS, UK</b>
<b>Nadia Ameen, Yale University School of Medicine, USA</b>	<b>Stephen P. Arneric, Critical Path Institute, USA</b>
<b>Nasir Ali Afsar, Alfaisal University College of Medicine, KSA</b>	<b>Stephen Shrewsbury, Fortuna Fix, USA</b>
<b>Ndiaye Mady, Faculty of Medicine of Thiès, Nigeria</b>	<b>Stephen Smolinski, Selecta Biosciences, USA</b>
<b>Patrick J Tighe, University of Nottingham, U.K</b>	<b>Sujatha Kannan, Johns Hopkins University School of Medicine, USA</b>
<b>Philippe Jouvett, Sainte-Justine Hospital University of Montreal, Canada</b>	<b>Tatjana Michel, University Hospital Tuebingen, Germany</b>
<b>Prasanth Puthanveetil, Roosevelt University College of Pharmacy, USA</b>	<b>Thomas C Chen, Keck School of Medicine of USC, USA</b>
<b>Ramune Sepetiene, Lithuanian University of Health Sciences, Lithuania</b>	<b>Timothy Coté, Coté Orphan, USA</b>
<b>Rashmi Gopal-Srivastava, NCATS, NIH, USA</b>	<b>Tony Zbeidy, Orphan-Europe, France</b>
<b>Rob W J Collin, Radboud University Medical Center, Netherlands</b>	<b>Wei Zheng, National Institutes of Health, USA</b>
<b>Róbert Rosol'anka, University Hospital in Martin, Slovak Republic</b>	<b>Xavier Paoli, Pharnext, France</b>
<b>Romina Ortiz, Rare Genomics Institute, USA</b>	<b>Xiaolan Zhang, Sarepta Therapeutics, USA</b>
<b>Ryan Clift, InClin, USA</b>	<b>Yingjun Xie, The Third Affiliated Hospital of Guangzhou Medical University, China</b>
<b>Samuel Ayoola Abati, Iagos university, Nigeria</b>	<b>Yoko Sato, National Defense Medical College, Japan</b>
<b>Sara Tylosky, Farmacon, USA</b>	<b>Yolande van Bever, Erasmus Medical Centre, The Netherlands</b>
<b>Serge Braun, Scientific Director, AFMTelethon, France</b>	<b>Yong Moon Choi, Bio-Pharm Solutions Co., Ltd, South Korea</b>
<b>Shin'ichi TAKEDA, National Institute of Neuroscience, Japan</b>	<b>Yunzhao Ren, Food and Drug Administration, USA</b>
	<b>Yusuf Hovsep Eken, Elkerliek ziekenhuis, Netherland</b>





# WHAT YOU CAN EXPECT





# GLIMPSES OF RARE DISEASES CONFERENCE





# PLAN YOUR TRIP @ TORONTO



## CONTACT US

### For Queries

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