# Tentative Program

## 10th Annual Congress on RARE DISEASES AND ORPHAN DRUGS

September 26-27, 2019 Charlottesville | USA

## Program @ Glance

**Day 1 September 26, 2019**

- Opening Ceremony
- Plenary & 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 27, 2019**

- Opening Ceremony
- Plenary & 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
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. 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

Katarina 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

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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, Abu 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
Robert 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, PharNext, 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, Elkerlick ziekenhuis, Netherlands
WHAT YOU CAN EXPECT
GLIMPSES OF RARE DISEASES CONFERENCE
Plan Your Trip @ Virginia

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