Thursday, September 14, 2017

| Time | Content | |
|---|---|---|
| 10:00-14:00<br>7th September | Registration<br>(all day) | |
| 14:00-18:00 | | Pre-Conference Workshops, ICORD Council Meeting |
| 8th September | | |
| 8:30-12:00 | Opening Ceremony Remarks<br>Dr. Manuel Posada, President, International Conference on Rare Diseases and Orphan Drugs<br>Kevin Huang. President, Chinese Organization for Rare Disorders<br>Dr. Yupei Zhao, Peking Union Medical College Hospital<br>Keynote Addresses (1.)<br>• National Health and Family Planning Commission of China (Healthcare System Perspective)<br>• Dr. Bi Jingquan China Food and Drug Administration (Regulatory Perspective)<br>Keynote Addresses (2.)<br>#1 Kevin Huang and/or Shuyang Zhang<br># 2 Dr. Tarun Weeramanthri, Assistant Director General, Public Health Division, Public Health and Clinical Services<br>Perth, Western Australia, Government Public Health Policy Imperatives of Rare Diseases<br>Sponsor speech Guest speech<br>• 陈凯先院士<br>• Shire<br><br>*10:30 – 10:50 Tea Break*<br><br>Stephen Groft – A Global View of Rare Diseases<br>Outcomes of Previous ICORD Meetings | |

| | | | |
|---|---|---|---|
| | • Cape Town South Africa<br>• Mexico City, Mexico – FEMEXER<br>• South and Central America - Virginia Llera<br>• Russia<br>• United Nations/ World Health Organization CoNGO Initiative – Anders Olausson<br>• Call to Action Proposal from Cape Town Meeting – Safiyya Dharssi, Pfizer | | |
| 12:00-13:30 | LUNCH/Poster Session | | |
| Room | Room 1 | Room 2 | Room 3 |
| 13:30-14:50 | National Rare Diseases Registry System in China: Patient Care and Innovation<br>中国国家罕见病注册系统：临床服务与科技创新并重<br>Co-Chairs:<br>Zhang Shuyang, Vice President, Peking Union Medical College Hospital（张抒扬）and Manuel Posada, Director. Institute of Rare Diseases Research, ISCIII & President of ICORD<br>1. NRDRS: Vision and Roadmap by Zhang Shuyang<br>2. Executive Report of NRDRS by Zhu Yicheng<br>3. Report on Registry of Rare Renal Tubular Diseases in China by Chen Limeng | Challenges and Opportunities for Orphan Drug Access in China and Emerging Markets<br>孤儿药市场准入的机遇与挑战：中国及其他新兴市场<br>• Co-chairs: Catarina Edfjäll, Vice President, Head of Global Regulatory Affairs, CSL Behring<br>• Other Co-Chair from China FDA or Dennis Bashaw, FDA USA<br><br>SPEAKERS:<br>• 辉瑞(暂时确认)<br>• Meryem Nimour (辉瑞)<br>• CFDA –CDE，何如意 /Bi Jingquan Director, China FDA Draft Guidance on Regulatory Requirements for Product Approvals in China – Acceptance | Cardiovascular, Pulmonary and Kidney Rare Diseases<br>心血管系统、呼吸系统及泌尿系统罕见病<br>Chair:<br>• Jing Zhicheng, Professor, Chief of Scientific Research Department,Fuwai Hospital（荆志成）<br>Speakers:<br>• Jing Zhicheng, Professor, Chief of Scientific Research Department,Fuwai Hospital（荆志成）<br>• Francis X McCormack, MD, Department of Internal Medicine, University of Cincinnati School of Medicine<br>• 李建军 Jianjun Lee, Fuwai Hospital, Beijing<br>• 李建军/阜外医院 |

| | | | |
|---|---|---|---|
| | 4. Announcement of Rare Disease Bank of National Gene Bank<br>5. 杨焕明院士 Report by Professor Yang Huanming<br>6. (待定人选) Progress on Registry of PAH 20min<br>7. European Rare Disease Registry by Manuel Posada<br>8. Discussion | of Data<br>• Catarina Edfjäll, Vice President, Head of Global Regulatory Affairs, CSL Behring<br>• Bruno Sepodes, COMP, EU<br>• Larry Liberti, Center for Innovation in Regulatory Science – Evolution and Harmonization of Regulatory Requirements and Procedures toward Best practices in Global Regulatory Affairs | |
| 14:50-15:20 | Panel Discussion | Panel Discussion | Panel Discussion |
| 15:20-15:40 | TEA BREAK/Poster Session | | |
| 15:40-17:00 | Importance of Patient Registries in Basic and Clinical Research, Therapeutic Development, Patient Care, and Government Healthcare Decision-Making<br>病例注册登记对医学研究、临床诊疗及卫生政策制定的重大影响<br>Chair:<br>• Gong Mengchun, Executive Director, National Rare Disease Registry System (弓孟春）<br>• Manuel Posada, Director. Institute of Rare | Policy Development for Advancing Rare Diseases and Orphan Drug Research<br>促进罕见病和孤儿药研究的支持性政策<br>Co-Chair:<br>• Huang Hui, Ph.D., Deputy Director, Scientific Research Department, Peking Union Medical College Hospital and<br>• Marlene Haffner, Former Director, Office of Orphan Products Development, FDA | Endocrine and Hematological Rare Diseases<br>内分泌系统与血液系统罕见病<br>Chair:<br>• Liu Jianmin, Professor, Director of Endocrinology Department, Rui Jin Hospital Shanghai Jiao Tong University of Medicine（刘建民）<br>SPEAKERS:<br>• Liu Jianmin, Professor, Director of Endocrinology Department, Rui Jin |

| | | | |
|---|---|---|---|
| | Diseases Research, ISCIII & President of ICORD<br><br>Speakers:<br>• Manuel Posada. Rare Diseases Registries strategy in the European Union.<br>• Domenica Taruscio, Director CNMR, ISS<br>• Peter Robinson Human Phenotype Ontology to Be Invited<br>• Ana Rath Orphanet or Sylvie Maiella，Orphanet ( 自付）or Marc, Open-linked data for RD, Orphanet （自付）<br>• 杨仁池 Hemophilia Registry in China Yang Renchi<br>• | Speakers:<br>• 陈凯先院士<br>• 张杰 CFDA<br>• Gumei Liu, FDA<br>• Xue Zhang 张学，中国遗传学会<br>• Bruno Sepodes, COMP, EU<br>• Gareth Baynam, Western Australia, Translating Policy into Clinical Services | Hospital Shanghai Jiao Tong University of Medicine（刘建民）<br>• Shi Xiaodong, Director, Children's Hospital Capital Institute of Pediatrics(师晓东)<br>• Sunita K. Agarwal, Acting Section Chief, Genetics and Endocrinology Section, Metabolic Diseases Branch, NIH |
| 17:-00-17:30 | Panel Discussion | Panel Discussion | Panel Discussion |
| 19:00-21:00 | WELCOME DINNER | | |
| 9th September | | | |
| Room | Room 1 | Room 2 | Room 3 |
| 8:30-9:50 | Collaboration among Major Stake holders<br>罕见病事业发展的突破：多方参与、紧密协作<br>Chair：<br>Xiaowei Jin，金晓玮<br>Speakers:<br>• Raj, Rainbow Across Border | Neurological, Orthopedic and Dermatological Rare Diseases<br>神经系统、骨骼及皮肤罕见病<br>Chair:<br>• Zhu Yicheng, Professor, Vice Director of Neurology, Peking Union Medical College Hospital（朱以诚） | Successful Case Study for Orphan Drug R&D(A)<br>孤儿药研发的成功案例剖析（A）<br>Co-Chairs：<br>• Emilio Roldan, Scientific Director, Gador, Buenos Aires, Argentina<br>• Harvey F. Lodish, MIT<br>Speakers: |

| | | | |
|---|---|---|---|
| | （Singapore）<br>• Weihong Gu（顾卫红）, China-Japan Friendship Hospital<br>• Yann Le Cam, Chief Executive Officer EURORDIS<br>• Kevin Loth, Celgene, Europe（Durhane Wong-Rieger 推荐，待定）<br>• Yukiko Nishimura President/founder of NGO ASrid, Japan<br>• Olivier Menzel, Re(ACT) Initiative, Swiss Foundation for Research on Orphan Diseases (BLACKSWAN Foundation | Speakers:<br>• Wang Yi, Vice-President, Fudan University Children's Hospital (王艺)<br>• Olaf Reis, Coordinator of Neuromics European Project<br>• Cui Liying, Professor, Director of Neurology, Peking Union Medical College Hospital<br>• David Pearce, Sanford Research Institute – Animal Models for Rare Diseases<br>• Zhu Yicheng, Professor, Vice Director of Neurology, Peking Union Medical College Hospital（朱以诚）<br>• 张学军，安徽医科大学皮肤病研究所所长 Head of Skin Research Institute, Anhui Medical School,<br>• Orthopedic Speakers (TBD) | Speakers:<br>• Lu Xianping, President and Chief Research Officer, Chipscreen,Ltd.<br>• Emilio Roldan, Scientific Director, Gador, Buenos Aires, Argentina<br>Actions enhancing orphan drug discoveries and research in developing countries<br>• Joan Comello Carnice, MD, PhD<br>Director, Hospital Universitari Vall d'Hebron - Institut de Recerca (VHIR)<br>Barcelona<br>• Harvey F. Lodish, MIT<br>• Brian R. Robinson, Bluebird<br>• Dana（Alexion）<br>• Kevin ( 确定）<br>• TBD by Niemann-Pick<br>• Sandeep Sahney, JPAC |
| 9:50-10:20 | Panel Discussion | Panel Discussion | Panel Discussion |
| 10:20-10:40 | TEA BREAK | | |
| 10:40-12:00 | Patient Organization to Promote and Support Rare Disease Research and Orphan Drugs Development<br>患者组织：在推动罕见病研究与孤儿药创新中的作用<br>Co-Chairs: | Emerging Technologies the Practice of Precision Medicine in Rare Disease: The Case of NGS（已满）<br>以 NGS 为代表的推动罕见病精准诊治的新兴技术<br>Co-Chairs： | Successful Case Study for Orphan Drug R&D(B)<br>孤儿药研发的成功案例剖析（B） |

| | | | |
|---|---|---|---|
| | • Barbara Wuebbels, Audentes<br>• China Co-Chair or Lisa Phelps, VP, NORD<br>Speakers:<br>• 法布雷，Shire<br>• Barbara Wuebbels, Audentes, Partnership Role between Patient Advocacy Groups and the Pharmaceutical Industry<br>• Ning Lu, Niemann-Pick International or<br>• Preston Campbell, Cystic Fibrosis Foundation or<br>• Julia Isla, Dravet Syndrome Foundation<br>• Fabry Disease or HAE<br>• Lisa Phelps–NORD, USA | • Qi Ming, Professor, foundation medicine, Zhe Jiang University School of Medicine（祁鸣）<br>• Maja Stojiljkovic, Institute of Molecular Genetics and Genetic Engineering, University of Belgrade, Serbia<br>Speakers:<br>• Rongxia Lee,李厦戎， (Genedock)<br>• 华大基因 (BGI)<br>• Maja Stojiljkovic, Institute of Molecular Genetics and Genetic Engineering, University of Belgrade, Serbia<br>• 金域检验 (King Med Diagnositics)<br>• Qi Ming, Professor, foundation medicine, Zhe Jiang University School of Medicine（祁鸣）<br>• Interpreting Exome and Genome Sequencing Results Gareth Baynam, Western Australia UDP and David Adams NIH, NHGRI, UDN | |
| 12:00-12:30 | Panel Discussion | Panel Discussion | Panel Discussion |
| 12:30-14:00 | LUNCH/Poster Session | | |
| Room | Room 1 | Room 2 | Room 3 |

| | | | |
|---|---|---|---|
| 14:00-15:20 | • Global Collaboration and Joint Action on Rare Diseases(A)<br>• 罕见病的全球合作与共同行动（A）<br>Co-Chair: Durhane Wong-Rieger, CORD, Canada and RDI<br>Speakers:<br>• Daria Julkowska, E-Rare<br>• Christina Waters Treat NMD<br>• Roxana Merino Martinez, Karolinska Institute, Stockholm. Project manager of B3Africa B3Africa: Bridging Biobanking and Biomedical research<br>• Rachel Yang, CORD<br>• Dr. Ali Afrooz, University of Tehran, Iran Rare Diseases Foundation of Iran<br>• Zhenming Chen Professor of Epidemiology Oxford University Director, Clinical Trials and Epidemiology Service Units and China Kadoorie Biobank Prospective Study, Oxford University, UK: Biobanks and Precision Medicine<br>• Dr. Bernhard Schwartlander, WHO, Beijing – WHO and Essential Medicines | Precision Medicine in Management of Rare Diseases: Diagnosis, Prevention, and Treatment<br>精准医学与罕见病：诊断、治疗、生活质量改善与预防<br>Chair:<br>• Zhou Wenhao, Professor, Vice-President,Fudan University Pediatric Hospital（周文浩）<br>Speakers:<br>• Zhou Wenhao, Professor, Vice-President,Fudan University Pediatric Hospital（周文浩）<br>• Domenica Taruscio, UDN International Director, National Center for Rare Diseases, Istituto Superiore di Sanita, Italy<br>• Zhang Xue, Vice-President, Graduate School, Peking Union Medical College Hospital<br>• Cynthia Tifft, Deputy Director, Undiagnosed Diseases Program, NHGRI, NIH, USA<br>• Yaping Yang 杨亚平，Associate Professor, Baylor Medical College, USA<br>• Xuefan Gu 顾学范,Director, Shanghai | Cell &Gene Therapy in Rare Disease<br>针对罕见病的细胞治疗和基因治疗（已满）<br>Chair:<br>• Guangping Gao, Ph.D. Horae Gene Therapy Center University of Massachusetts Medical School（高广平）<br>Speakers:<br>• Guangping Gao, Ph.D. Horae Gene Therapy Center University of Massachusetts Medical School（高广平）<br>• Terence R. Flotte, University of Massachusetts<br>• Brian Kaspar, AveXis, INc<br>• Mark J Pykett, VMD, PhD President and CEO, Agilis Biotherapeutics<br>• Jill Weimer Sanford Research Institute, South Dakota, USA (Batten Disease)<br>• Bio Marin Speakers TBD<br>• Alexion Speakers TBD |

| | | | |
|---|---|---|---|
| | | • Peter Bauer, Institute of Medical Genetics and Applied Genomics, University Hospital of Tuebingen, Germany<br>• Helen Leonard, Australia | |
| 15:20-15:50 | Panel Discussion | Panel Discussion | Panel Discussion |
| 15:50-16:10 | TEA BREAK /Poster Session | | |
| 16:10-17:30 | Global Collaboration and Joint Action on Rare Diseases(B)<br>罕见病的全球合作与共同行动（B）<br>Chair:<br>• Durhane Wong-Rieger, CORD, Canada<br>Speakers:<br>• David Pearce, President, Sanford Research and Director CORDS Patient Registry Program<br>• David Adams or Cynthia Tifft, NHGRI, NIH, USA（待定）<br>• Ramaiah Muthyala, India Organization for Rare Disorders (IORD)<br>• Prasanna Kumar B Shirol, Organization for Rare Disorders India (ORDI) | • Practical Challenges and Solutions for Clinical Implementing Precision Medicine Programs<br>• 精准医学的临床部署：挑战与解决方案<br>Co-Chairs:<br>• Zhou Wenhao, Professor, Vice-President, Fudan University Pediatric Hospital<br>• Shen Yiping, Boston Children's Hospital(沈亦平)<br>Speakers<br>• Angela Brand, Director of the European Centre for Public Health Genomics (ECPHG)<br>• Wendy Chung, Kennedy Family Professor of Pediatrics and Medicine, Columbia University | Creating Sustainable Payment Models for Rare Disease<br>为罕见病构建可持续的支付体系<br>Chair：<br>• Junshuai Liu 刘军帅, Junshuai Liu,Labor and Social Security Office of Qingdao, China<br>Speakers:<br>• Junshuai Liu 刘军帅, Junshuai Liu,Labor and Social Security Office of Qingdao, China<br>• Li Chenghua, Taiwan 李丞华<br>• Yann Le Cam EURORDIS Sustainable Access<br>韩国医保（Durhane Wong-Rieger 推荐） |

| | | | |
|---|---|---|---|
| | • K.P. Tsang, Hong Kong Alliance for Rare Diseases, China | • Bo Zheng,郑波博士，TBD by NIKYANY）(待定)<br>• Wei Zhang,张巍博士，嘉检医学 Amcare Genomics<br>• Dennis Bashaw, FDA<br>• Dr. Jeff Gulcher(Wuxi Pharma)<br>• （备选） | |
| 17:30-18:00 | Panel Discussion | Panel Discussion | Panel Discussion |
| | | 10th September | |
| Room | Room 1 | Room 2 | Room 3 |
| 8:30-9:50 | Our Rare Story *<br>“我们的故事”<br><br>Speakers: 2 个中国人，3 个外国人）<br>• 小星星 | Innovative Service Model for Patients and Families: The Unique Challenges in Rare Diseases<br>面向患者及家庭的服务模式创新：罕见病领域的独特挑战<br>Chair:<br>• Duan Tao, Professor, Chief Physician, Obstetrical Department, Shanghai First Maternity and Infant Hospital（段涛）<br>Speakers:<br>• Kevin Huang -根底健<br>Patients Like Me<br>• Duan Tao, Professor, Chief Physician, Obstetrical Department,Shanghai First Maternity and Infant Hospital（段涛） | Annual Meeting of Rare Diseases Society, Chinese Research Hospital Association<br>中国研究型医院学会罕见病分会年会 |

| | | | |
|---|---|---|---|
| | | • Tarun Weeramanthri, Assistant Director General, Public Health Division, Public Health and Clinical Services，Australia<br>• Morehealth 推荐<br>• Ana Rath, Orphanet, Inserm, Paris, France<br>• Janine Lewis, Genetic and Rare Diseases Information Center (GARD), NIH Bethesda, MD | |
| 9:50-10:20 | | Panel Discussion | |
| 10:20-10:40 | | TEA BREAK | |
| 10:40-12:00 | | Traditional Chinese Medicine in the Management of Rare Diseases<br>传统中医在罕见病领域的应用<br>Chair:<br>● Xiaochun Liang, Peking Union Medical College Hospital(梁晓春)<br>Speakers:<br>● Chen Lee 李忱<br>● Jinzhou TIAN, 田金洲 北京中医药大学东直门医院教授（副院长）<br>● Zhenhua DONG, 董振华 北京协和医院中医科教授<br>樊永平， 北京天坛医院中医科主任 | Annual Meeting of Rare Diseases Society, Chinese Research Hospital Association<br>中国研究型医院学会罕见病分会年会 |
| 12:00-12:30 | | Panel Discussion | |

| | |
|---|---|
| 13:00-15:00 | LUNCHEON<br>CONCLUSIONS FROM INDIVIDUAL TRACKS - Rachel Yang, Menchun Gong, and Steve Groft<br>PRIZE AWARDING CEREMONY - Menchun Gong,<br>BEIJING DECLARATION – Kevin Huang<br>CLOSING CEREMONIES AND WELCOME TO ICORD 2018 INDIA - Manuel Posada |