2024 全球罕见病科研论坛
暨第二届中国罕见病科研及转化医学大会

GLOBAL RARE DISEASES RESEARCH SYMPOSIUM &
THE SECOND CHINA RARE DISEASES RESEARCH AND TRANSLATIONAL MEDICINE ANNUAL CONFERENCE

May 23-25, 2024 | Shanghai, China

CONFERENCE MANUAL

主办方 Hosts

HOPE FOR RARE FOUNDATION 瑞鹏公益基金会

IRDiRC INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM

复旦大学 FUDAN UNIVERSITY

联合主办方 Co-Hosts

国家儿童医学中心 复旦大学附属儿科医院

蔻德罕见病中心 Chinese Organization for Rare Disorders

CONTENTS

Welcoming Remarks

Dear Esteemed Attendees,

On behalf of the organizing committee, I sincerely welcome you to participate in the 2024 Global Rare Diseases Research Symposium and The Second China Rare Diseases Research and Translational Medicine Annual Conference. Hope for Rare Foundation is an innovative foundation founded by me together with a group of scientists and entrepreneurs in 2022, with the mission of "To be an Innovation Engine for advancing rare diseases research and translational medicine", we jointly organize this high-level scientific conference on rare diseases with Fudan University, the International Rare Diseases Research Consortium (IRDiRC), the National Children's Medical Center / Children's Hospital of Fudan University, and the Chinese Organization for Rare Disorders (CORD).

Rare diseases are the greatest medical challenge to humanity. In recent years, many countries and regions have taken rare diseases as the most important health issues, and the most advanced technologies in the field of biomedicine are firstly applied to rare diseases, but we must face the fact that more than 90% of rare diseases are still facing the reality of having no medicines. The research of rare diseases is by no means a matter for one country and region, and the research and development of drugs is not only for the treatment of patients in one country and region. International communication and cooperation have become particularly important, and we must work closely together to break down the barriers brought about by the level of scientific and technological development, the level of economic development, the differences in regional policies, geopolitical influences, and the differences in the size of patient populations in various countries.

China has made remarkable achievements in rare diseases research and drug development over the past ten years. We hope that this conference will not only strengthen international exchanges and cooperation, but also show the strength of Chinese scientists and R&D enterprises, and make China's contribution to promoting global research on rare diseases. The Hope for Rare Foundation also hopes to join other foundations around the world in making positive efforts to promote the cause of scientific research.

In closing, I would like to thank all of our partners for your support, all of our speakers for sharing your insights, and I hope that you will all gain a lot from attending the conference. Let's embark on this exciting journey together!

Kevin Huang

Chairperson of the Organizing Committee
Co-founder and Secretary-General of Hope for Rare Foundation
Founder and President of Chinese Organization for Rare Disorders

Hosts

HOPE FOR RARE FOUNDATION
瑞鸥公益基金会

Hope for Rare Foundation was founded in Hangzhou, China in 2022 by Mr. Rufang (Kevin) HUANG in partnership with 11 well-known scientists and entrepreneurs. It is the first non-profit foundation in China to focus on rare diseases research and translational medicine. Its vision is to address the medical needs of all patients with rare diseases by leveraging resources and guiding innovative technology to advance the development of both basic and translational clinical research.

IRDiRC
INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM

The International Rare Diseases Research Consortium (IRDiRC) is a global collaborative initiative launched in 2011 by the European Commission and the US National Institutes of Health to tackle rare diseases through research and accomplish the vision to enable all people living with a rare disease to receive an accurate diagnosis, care, and available therapy within one year of coming to medical attention. Through its broad representation, gathering more than 60 member organizations, IRDiRC works towards the mission of advancing diagnostics and treatments, and of understanding the impact of these, for all people living with a rare disease.

復旦大學
FUDAN UNIVERSITY

Fudan University was established in 1905 as Fudan Public School. lt was the first institution of higher education to be founded by Chinese individuals.
The two characters, 复 fù (“return”) and 旦 dàn (“dawn”) were quoted from A Commentary on The Classic of History(《尚书大传·虞夏传》), which means: “Brilliant are the sunshine and moonlight, again the morning radiance returns at dawn.” lt is an encouragement to students and scholars to make unremitting efforts in self-betterment.

Co-hosts

国家儿童医学中心
复旦大学附属儿科医院

National Children's Medical Center / Children's Hospital of Fudan University, founded in 1952, is a Grade A tertiary Pediatric hospital integrating medicine, teaching, research, prevention and management, and was approved as the National Children's Medical Center in 2017. With strong medical strength and distinctive specialty features, it currently has 50 clinical and medical technical departments. It has established close collaboration with 26 internationally renowned medical institutions and has organized large-scale international academic conferences on pediatrics for many times.

蔻德罕见病中心
Chinese Organization for Rare Disorders

Chinese Organization for Rare Disorders (CORD), founded by Kevin HUANG in 2013, is a non-profit organization specializing in fields of rare diseases. CORD works to promote exchange and cooperation among rare disease patients and organizations, medical specialists, pharmaceutical companies and governmental agencies. It is committed to enhancing public understanding of rare diseases, improving patients' access to orphan drugs, fostering formulation of rare disease policies, and initiating international exchange and cooperation.

Conference Chairpersons

Alphabetical Order by Last Name

Li Jin

Academician of Chinese Academy of Sciences
President of Fudan University
Dean of Shanghai Medical College, Fudan University

David Pearce

Chair of International Rare Disease Research Consortium
President of Innovation and Research, Sanford Health

Adam Resnick

Director, Center for Data Driven Discovery in Biomedicine
at the Children's Hospital of Philadelphia
Research Professor of Neurosurgery at the Perelman
School of Medicine at the University of Pennsylvania

Tian Xu

Co-founder, Hope for Rare Foundation
Chair Professor of Genetics and Vice President,
Westlake University

Scientific Committee

Alphabetical Order by Last Name

Chairpersons

Yi Wang

President of the National Children's Medical Center / Children's Hospital of Fudan University
Director of the Rare Disease Group of the Pediatric Branch of the Chinese Medical Association
Deputy Chairman of the Rare Disease Branch of the Chinese Hospital Association

Guangping Gao

Director, Li Weibo Institute for Rare Diseases Research, Director, Horae Gene Therapy Center and Viral Vector Core, Professor at UMass Medical School
Past President of the American Society of Gene & Cell Therapy

Members

Tao Duan

Chief Physician, Professor and Former Director of Shanghai First Maternity and Infant Hospital
Co-founder, Hope for Rare Foundation

Xin-Hua Feng

Qiushi University Professor, Distinguished Investigator and Director of Life Sciences Institute, Zhejiang University

Terence R. Flotte

Professor, Provost, Dean and Executive Deputy Chancellor of the UMass Chan Medical School
President-Elect, American Society of Gene & Cell Therapy

Taosheng Huang

Professor of Shanghai Medical College, Fudan University; Director of Institute of Medical Genetics and Genomics, Fudan University; Chairman of Medical Genetics Center, National Children's Medical Center / Children's Hospital of Fudan University

Xiaowei Jin

Venture Partner, Sherpa Healthcare Partners
Senior Consultant of the Chinese Organization for Rare Disorders

Xin Li

Qiushi Distinguished Professor, Executive Director of the Center for RNA Medicine of International School of Medicine, Zhejiang University

Harvey F. Lodish

Professor of Biology and Professor of Biological Engineering; Founding member of the Whitehead Institute, Massachusetts Institute of Technology; Member, National Academy of Sciences, US; Fellow, American Academy of Arts and Sciences

Daniel Scherman

Director, French Foundation for Rare Diseases
Professor, Université Paris Cité
Head of the Medicine and Life Science Division of the European Academy of Science

Haoyi Wang

Deputy Director of the State Key Laboratory of Stem Cell and Reproductive Biology, Leader of the Genetic Engineering Technology Research Group, Institute of Zoology, Chinese Academy of Sciences; Member, Scientific Innovation Alliance for Rare Diseases, Hope for Rare Foundation

Hongguang Xia

Professor and Vice Director, Liangzhu Laboratory

Xiao Xiao

Co-founder, Chairman and CSO of Belief BioMed Group
Co-founder, Hope for Rare Foundation

Xiangjian Zheng

Professor and Dean of Department of Pharmacology, School of Basic Medical Sciences, Tianjin Medical University
Member, Scientific Innovation Alliance for Rare Diseases, Hope for Rare Foundation

Organizing Committee

Alphabetical Order by Last Name

Chairperson

Kevin Huang

Co-founder and Secretary-General of Hope for Rare Foundation
Founder and President of Chinese Organization for Rare Disorders

Members

Wenhan Geng

Project Supervisor of Hope for Rare Foundation

Juan Huang

Project Manager of Hope for Rare Foundation
Project Manager of Chinese Organization for Rare Disorders

Linguo Li

Director of Public Policy Research Center of Rare Diseases at the Chinese Organization for Rare Disorders

Eileen Li

Chief Business Officer, RareStone Group
Senior Consultant of the Chinese Organization for Rare Disorders

Jessica Liu

Ex-head of International Clinical Operation Business Unit, Tigermed Consulting Co. Ltd.

Yeyang Su

Anthropologist
Consultant of the Hope for Rare Foundation

Qi Sun

Senior Consultant of the Chinese Organization for Rare Disorders

Hanbo Wang

Director of Business Development, Jiangsu Hengrui Pharmaceutical Co. Ltd.

Yao Wang

Vice President of CRO BU, OBiO Technology (Shanghai) Corp., Ltd.

Lei Xiao

Director, PR & Patient Support, Sperogenix Therapeutics
Senior Advisor, Chinese Organization for Rare Disorders

Shuisong Ye

Deep Science, Founder

Boya Yu

Project Manager of Hope for Rare Foundation

Lin Yuan

Staff Neonatologist, Director of Neonatal Medical Center
President Assistant, National Children's Medical Center / Children's Hospital of Fudan University,

Mel Zhang

Project Director of Hope for Rare Foundation

Yue Zhang

Researcher, Hundred Talents Program, Liangzhu Laboratory of Zhejiang University

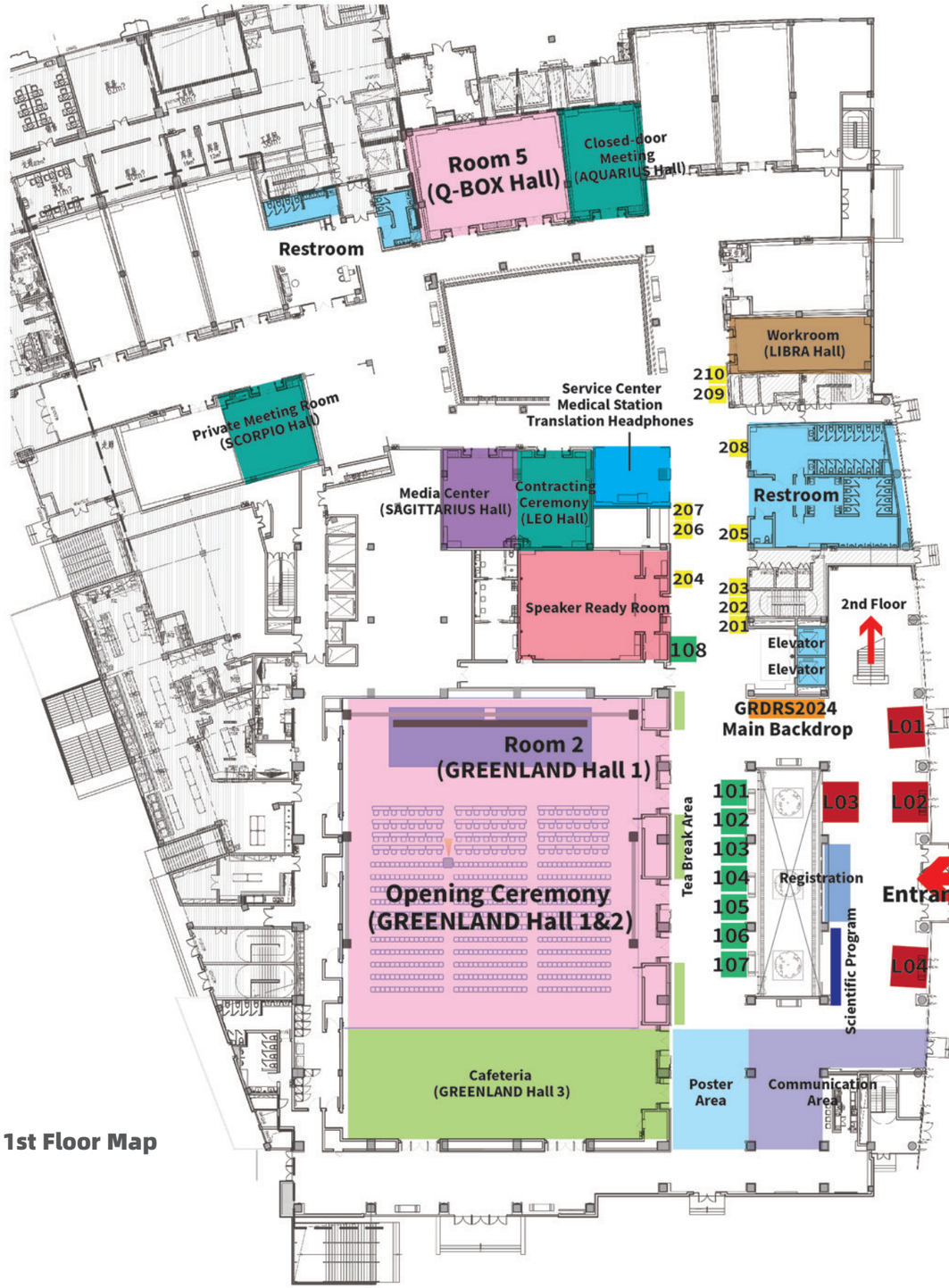
The Scientific Secretariat of IRDiRC

Team Members: Daria Julkowska, Galliano Zanello, Mary Catherine Valencia-Letinturier, Alexandra Tataru, Tanguy Onakoy, Ngangta Mbaidoum Abanga

Working Group & Volunteers

Aili
Shun Chen
Yanli Chen
Junwu Dai
Ruiyi Fan
Zikai He
Xingxing He
Qianqian Jia
Ziyi Jia
Huiling Kong
Junyao Li
Linguo Li
Yinyue Lin
Mei Lu
Junyin Luo
Fangting Wang
Jing Wang
Ping Wang
Wen Wang
Chi Yan
Mingdi Yang
Rui Zhang
Zhihao Zhai
Liming Chen
Wenrui Cui
Lilin Deng
Xiulin Fang
Hebin Gu
Yucheng Hong
Jiatong Hu
Jialei Huang
Wan Huang
Qingyi Jiang
Yewei Jiang
Haifeng Jin
Li Jin
Jia Li
Yue Li
Chao Lin
Yi Lin
Aini Liu
Dacai Liu
Jiaqi Liu
WenXing Long
Hanrui Luo
Xincheng Luo
LongHuan Piao
Aiqing Qiu
Yizhou Shao
Xing Su
Tingting Tang
Yunzhe Tang
Xiaohan Wang
YaoHui Wang
Dabin Wei
Xin Wu
Xiaoyue Xie
Yazhu Xie
Siqi Xu
Dingyi Xu
Yiran Xu
Yuheng Yan
Fan Yang
Qian Yang
Xinman Yu
Wenjia Zhang
Xiangdi Zhao

Conference Center & Hotel Guide Map



1st Floor Map

Conference Center & Hotel Guide Map

Booth Map

Premium Booth Area

L01 OBiO Technology
L02 Mindray
L03 Hope for Rare Foundation
L04 CORD

Advanced Booth Area

101 AstraZeneca
102 Sperogenix Therapeutics
103 Illumina
104 Nyuen
105 Jilin Huiminkangen Technology Co, Ltd.
106 GenSci
107 Cyagen Biosciences (Suzhou) Inc.
108 Gritgen

Standard Booth Area

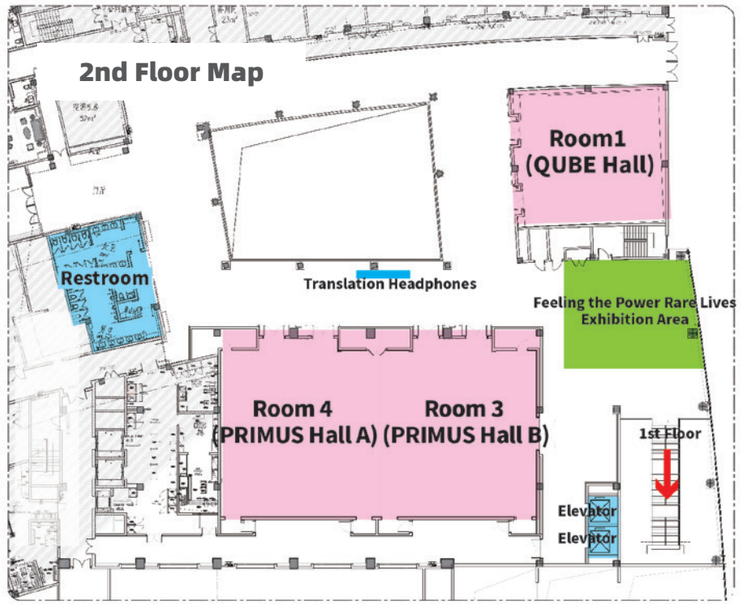
201 PackGene Biotech
202 Genevoyager
203 School of Life Sciences, Yunnan University
204 Belief BioMed
205 OAE(RDODJ)
206 Human Gene Therapy
207 Zhejiang University Press
208 VCBEAT
209 Chengyi Translation
210 Roche

Communication Area Title Sponsor
Neurophth 纽福斯

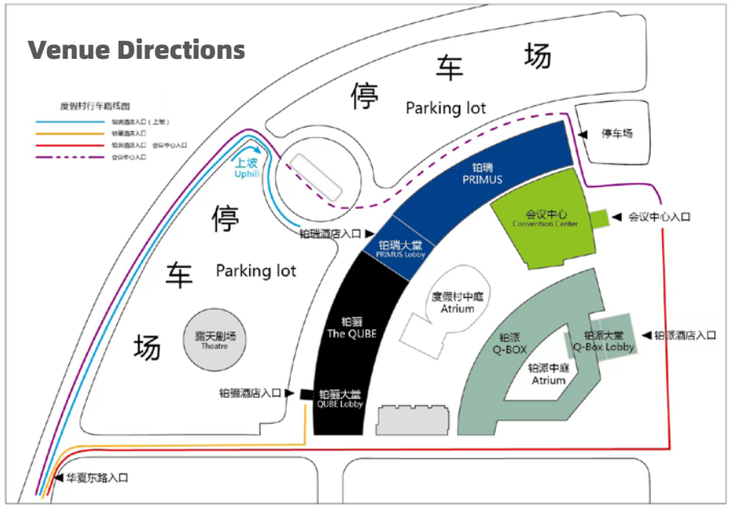
Poster Area Title Sponsor
Biogen

Tea Break Title Sponsor
华大基因 BGI

2nd Floor Map



Venue Directions



Attendee Guidelines

Registration

Please check in at the Registration desk with your registered email address and QR code to receive conference badge and materials.

Venue: Entrance of Convention Center (1st floor)

Opening Hours: May 22 14:00-19:00
May 23 07:30-19:00
May 24 07:30-18:00
May 25 07:30-18:00

Service Center & Medical Station

If you have any questions about attending the conference, please visit the Service Center for assistance. Limited food supplies are available at the Service Center. The Medical Station provides common medications for injuries and other conditions.

Venue: 1st floor of Conference Center

Opening Hours: May 22 14:00-19:00
May 23 07:30-19:00
May 24 07:30-18:00
May 25 07:30-18:00

Simultaneous Interpretation Service

The simultaneous interpretation service in English and Chinese is provided. Translation headphone can be picked up at the Service Center (1st floor) and the entrance to the PRIMUS Hall (2nd floor), please return it when you leave the Convention Center.

Speaker Ready Room

Located on the 1st floor of the Conference Center for speakers with RED lanyard to enter only.

Media Center

Located in the Sagittarius Hall on the 1st floor of the Conference Center for media with PURPLE lanyard to enter only.

Poster Area

Located on the 1st floor of the Conference Center, open to all attendees.

Communication Area

Located on the 1st floor of the Conference Center, open to all attendees.

Tea Break Area

Located in the foyer of GREENLAND Hall on the 1st floor of the Conference Center.

Opening Hours: May 24 10:00-10:30 15:30-16:00
May 25 10:00-10:30 15:30-16:00

Feeling the Power of Rare Lives Exhibition Area

Located on the 2nd floor of the Conference Center and shows the life stories of nearly 70 rare disease patients.

Parking

Complimentary parking is available at the Convention Center during the conference, and no voucher is required.

Dining Information

Attendees with BLUE and PURPLE lanyards

Date	Time	Place	Food Provided
May 24 Lunch	12:00-13:30	The food truck at the entrance of each venue	Business Boxed Meal
May 25 Lunch	12:00-13:30	The food truck at the entrance of each venue	Business Boxed Meal

The dining area: GREENLAND and Q-BOX on the first floor of the conference center, QUBE and PRIMUS on the second floor of the conference center.

Notes:

1、Please scan the code to pick up the food with the conference badge. Each person can only pick one boxed meal once (if you need vegetarian food, please tell the food truck attendant).
2、Please throw the lunch box into the trash can when finish the meal.
3、If you have paid for the Welcoming Banquet at 19:00 on May 23, please scan the code to enter with your conference badge.
4、Free portable food (such as sandwiches, fruit, yogurt, bread, etc.) will be provided for attendees who are not able to go to the hotel restaurant or eat out in the evening. Please pick it up at the service center on the 1st floor.
Opening Hours: May 23 12:00-14:00 18:00-19:00
May 24 17:00-19:30

Attendees with RED lanyards

Date	Time	Place	Food Provided
May 23 Lunch	12:30-13:30	GREENLAND 3	Buffet
May 23 Dinner	19:00-21:00	GREENLAND 1+2	Welcoming Banquet
May 24 Lunch	11:30-13:30	GREENLAND 3	Buffet
May 24 Dinner	18:00-20:00	GREENLAND 3	Buffet
May 25 Lunch	11:30-13:30	GREENLAND 3	Buffet
May 25 Dinner	18:00-20:00	GREENLAND 3	Buffet

Notes:

1、Please scan the code on the conference badge to enter the cafeteria.
2、If you attend the satellite meeting and seminar at noon, it is recommended to go to the cafeteria at 11:30 in advance.

Optional Dining Guide

If you wish to arrange lunch or dinner on your own, the Chinese restaurant at Primus Hotel is available for dining. The restaurant offers à la carte Western cuisine from 6:30 AM to 10:30 PM and à la carte Chinese cuisine from 11:00 AM to 2:00 PM. Business private rooms (for 8-20 people) need to be reserved in advance and can accommodate both Chinese and Western cuisines. Reservation hotline: 021-3891-8888, extension for Chinese Restaurant at Primus Hotel.

Conference Center Surroundings

Venue: Greenland International Convention Center

Address: Lane 6666, East Huaxia Road, Pudong, Shanghai, 201201

Hotel Reservation

Hotel	Negotiated Rates	How to Reserve
Primus Hotel Shanghai Sanjiagang (Five-star)	From 650 RMB	Dial 021-3891 8888 or 021-3891 8816, connect to the reservation department
The QUBE Hotel Shanghai Sanjiagang (Four-star)	From 400 RMB	
Q-Box Hotel Shanghai Sanjiagang (Three-star)	From 300 RMB	

To reserve a hotel near the Greenland International Convention Center, there are three options within a 5-minute walking distance to the Convention Center. The conference has negotiated rates with these hotels, and you can make your own reservation. Please confirm the payment method directly with the hotel. Please be sure to mention【GRDRS2024】, otherwise, the negotiated rates will not apply.

Transportation

Pudong International Airport (9.9 km from the venue, about 15 minutes' drive)
Shuttle service: The hotel offers complimentary shuttle service exclusively to conference attendees who have made a reservation at the hotel. For schedules, please check with the hotel's front desk.

Shanghai Hongqiao International Airport (51 km from the venue, about 1 hour's drive)
Public transportation: Take the metro at Hongqiao Airport Terminal 2 station- Line 2, to Pudong International Airport. Exit at Yuandong Avenue station.

Shanghai Hongqiao Railway Station (59 km from the hotel, about 1 hour 7 minutes' drive)
Public transportation: Take the metro at Hongqiao Railway Station- Line 2, to Pudong International Airport station. Exit at Yuandong Avenue station.

Shanghai Railway Station (42.2 km from the venue, about 1 hour 15 minutes' drive)
Public transportation: Take the metro at Shanghai Railway Station- Line 4 Inner Circle, to Baoshan Road. Get off at Century Avenue station and transfer to Line 2, to Pudong International Airport. Exit at Yuandong Avenue station.

Shanghai South Railway Station (37.5 km from the venue, about 45 minutes' drive)
Public transportation: Take the metro at Shanghai South Railway Station- Line 1, to Fujin Road. Get off at People's Square station and transfer to Line 2, to Pudong International Airport. Exit at Yuandong Avenue station.

The Yuandong Avenue station is located 4 kilometers away from the Convention Center, from which it is most convenient to take a taxi to reach the venue.

Attraction

Shanghai Disney Resort	21 km from the venue
Shanghai Museum	38 km from the venue
The Bund	36 km from the venue
The Pearl of the Orient	36 km from the venue

Shopping

Florentia Town (Shanghai Premium Outlets)	3.5km from the venue
The Bund Taikoo Li	31 km from the venue
IFC Centre	34 km from the venue

Hospital

Shanghai Pudong New District People's Hospital	9.1 km from the venue
Shanghai International Medical Center (Kangxin Highway)	24 km from the venue

Notes for Attendees

- Conference badges must be worn at all times when attending plenary sessions. Other documents and proof of identity will not be accepted.
- On-site registration and payment services will be provided for unregistered participants. Please consult the staff of sign-in desk on the first floor.
- Each participant is kindly requested to arrive on time for the sessions, in accordance with the conference agenda. Please turn off your cell phone or put it on vibrate mode when you are in the meeting rooms.
- Please take good care of your belongings during the conference.
- The service center on the first floor will provide temporary medical service for some basic and commonly used medicines. Due to the complexity of rare diseases, the organizing committee of the conference is unable to provide emergency medical assistance to patients attending the conference. Patients should assess the health situation and take necessary auxiliary or medical measures.
- The statements or opinions of conference sponsors, partners and exhibitors at this conference do not represent the endorsement or recommendation of the organizer of its products, technologies and services.
- Any form of marketing, publicity or misleading behaviors for illegal products, technologies, services, and treatment are strictly prohibited. Once found mentioned behaviors, the conference badge will be immediately disqualified.
- Not all patients or family members are willing to be photographed or identified. Obtaining their consent is the most basic form of respect for patients and their families.
- Guests and participants come from all over the world. Please respect the cultural customs and religious beliefs of different countries and regions
- The hard-working staff and volunteers are the important force to ensure the public welfare academic conference. Please give them support and understanding.

Scan QR code to view conference photos

Scan QR code to watch the live streaming of the opening ceremony

Scientific Program

May 23		
14:00-17:30	GREENLAND 1+2 (1F)	Opening Ceremony
19:00-21:00	GREENLAND 1+2 (1F)	Welcoming Banquet

May 24	QUBE (2F)	GREENLAND 1 (1F)	PRIMUS B (2F)	PRIMUS A (2F)
08:30-10:00	Cutting-Edge Research Trends in Rare Diseases I	Emerging Frontiers and Applications of Cell Therapy I	Breakthroughs from Early-Career Researchers	How Patient Organizations and Foundations Drive Drug Development
10:00-10:30	Tea Break			
10:30-12:00	Cutting-Edge Research Trends in Rare Diseases II	Emerging Frontiers and Applications of Cell Therapy II	Hope for Rare Research Funding: From Basic Research to Personalized Medicine	How Scientists and Investors Can Work Together to Accelerate Innovative Therapies
12:00-14:00	Lunch & Poster Session & Satellite Meetings			
14:00-15:30	Model Systems Used in Rare Diseases Research I	New Approaches and the Future in Gene Editing I	RNA-Based Therapeutics: From Antisense Oligonucleotides to mRNAs I	Regulatory Innovation in Rare Diseases Research I
15:30-16:00	Tea Break			
16:00-17:30	Model Systems Used in Rare Diseases Research II	New Approaches and the Future in Gene Editing II	RNA-Based Therapeutics: From Antisense Oligonucleotides to mRNAs II	Regulatory Innovation in Rare Diseases Research II
19:00-21:00	Investigator-Patient Exchange Session			

May 25	QUBE (2F)	GREENLAND 1 (1F)	PRIMUS B (2F)	PRIMUS A (2F)
08:30-10:00	Prenatal Diagnosis and Newborn Screening	Development of Vectors and Delivery Systems I	Natural History Studies & Cohort Studies of Rare Diseases I	Funding of Scientific Research in the Field of Biology and Medicine
10:00-10:30	Tea Break			
10:30-12:00	Recent Advances in Mitochondrial Diseases Research	Development of Vectors and Delivery Systems II	Natural History Studies & Cohort Studies of Rare Diseases II	Benefit-sharing: A Thorny Question for Biomedical Innovation
12:00-14:00	Lunch & Poster Session & Satellite Meetings			
14:00-15:30	Academic Lectures from Fudan University: 2024 Shanghai Medical Forum I	Gene Therapy: Advances, Challenges and Perspectives I	Novel Targets for Small Molecules and Antibodies I	International Collaboration in Rare Diseases Research & Treatment I
15:30-16:00	Tea Break			
16:00-17:30	Academic Lectures from Fudan University: 2024 Shanghai Medical Forum II	Gene Therapy: Advances, Challenges and Perspectives II	Novel Targets for Small Molecules and Antibodies II	International Collaboration in Rare Diseases Research & Treatment II Including Closing Ceremony

Scientific Program

May 26		
09:00-16:00	Tour	**Route 1** Human Phenome Institute of Fudan University **Route 2** National Children's Medical Center / Children's Hospital of Fudan University & Institute of Medical Genetics and Genomics, Fudan University **Route 3** OBiO Technology (Shanghai) Corp., Ltd. / OBiO Intelli-M

Friendly reminder: For specific itinerary and departure arrangements on the visiting day, please refer to the email notification you received. If you have any questions, you can inquire at the Convention Service Center located on the first floor.

Special Photographic Exhibition: Feeling the Power of Rare Lives Exhibition Area

Curators: Hope for Rare Foundation & Chinese Organization for Rare Disorders (CORD)
Opening Hours: May 23-25 8:30-18:00
Venue: 2nd floor of Conference Center (near the walking stairway)

Pre-Conference Events

May 23		
09:30-11:30	Q-BOX (1F)	Tips for Starting a Biotech
09:30-11:50	LEO (1F)	Launching Ceremony of the Center for Clinical Evaluation and Translation of Advanced Therapies for Pediatric Rare and Genetic Diseases
11:30-12:30	Q-BOX (1F)	Signing Ceremony of the Cooperative Publishing Framework Agreement between Zhejiang University Press and Hope for Rare Foundation

Seminars

May 24		
08:30-12:00	Q-BOX (1F)	UCB Seminar: Innovative Breakthroughs in Rare Neurological Diseases and Immunological Disorders
12:30-14:00	Q-BOX (1F)	Scientific Innovation Alliance for Rare Diseases Programs Roadshow (Closed by Invitation)
14:00-17:40	Q-BOX (1F)	Academic Lectures from Zhejiang University Precision Medicine Project & Liangzhu Laboratory

May 25		
08:30-10:00	Q-BOX (1F)	CORD Seminar: Gene Therapy Market and Payment Innovation Solutions in China

Satellite Meetings

May 24		
12:00-13:00	QUBE (2F)	Biogen Satellite Meeting
12:30-13:30	GREENLAND 1 (1F)	Takeda Satellite Meeting
12:00-14:00	PRIMUS B (2F)	Boehringer Ingelheim Satellite Meeting
13:00-14:00	PRIMUS A (2F)	Gensci Satellite Meeting

May 25		
12:00-13:00	QUBE (2F)	Chigena Satellite Meeting
12:30-13:30	GREENLAND 1 (1F)	Ipsen Satellite Meeting

Scientific Program

Time: May 23 14:00—17:45
Venue: Greenland Hall 1+2 (1F)

Opening Ceremony	
Presenter: Chunying Zheng China Global Television Network (CGTN)	
14:00-14:10	**Opening Show** **Erhu Solo: Galloping War Horses** by **Zhixuan Zhang** (from the rare diseases community) **Chorus: Shimmering Light** by **Ke Yan, Lin Long, Wen Wang, Hui Lin** (from the rare diseases community)
14:10-14:19	**Remarks From the Organizers** **Li Jin** Academician of Chinese Academy of Sciences; President of Fudan University; Dean of Shanghai Medical College, Fudan University **David Pearce** Chair of International Rare Disease Research Consortium; President of Innovation and Research, Sanford Health **Kevin Huang** Co-founder and Secretary-General of Hope for Rare Foundation; Founder and President of Chinese Organization for Rare Disorders
14:19-14:22	**Appearance of the Conference Chairpersons**
14:22-14:40	**Remarks From the Guests**
14:40-14:50	**Group Photo Ceremony**
14:50-15:20	**Keynote Speech** **New Technologies and Building Successful Biotechnology Companies to Treat Rare Diseases** **Harvey F. Lodish** Professor of Biology and Professor of Biological Engineering, Founding member of the Whitehead Institute, Massachusetts Institute of Technology; Member, National Academy of Sciences, US; Fellow, American Academy of Arts and Sciences
15:20-15:50	**Keynote Speech** **From Genomics to Phenomics** **Li Jin** Academician of Chinese Academy of Sciences; President of Fudan University; Dean of Shanghai Medical College, Fudan University
15:50-16:20	**Panel Discussion** **Global Challenges: How to Strengthen Research, Clinical Translation, and International Cooperation on Rare Diseases** **Chair:** **Guangping Gao** Director, Li Weibo Institute for Rare Diseases Research, Director, Horae Gene Therapy Center and Viral Vector Core, Professor at UMass Medical School; Past President of the American Society of Gene & Cell Therapy **Panelists:** **P.J. Brooks** Deputy Director, Division of Rare Diseases Research Innovation, National Center for Advancing Translational Sciences, National Institutes of Health, USA **Wai-Yee Chan** Pro-Vice-Chancellor/Vice President, The Chinese University of Hong Kong; Li Ka Shing Professor of Biomedical Sciences, School of Biomedical Sciences, Faculty of Medicine, The Chinese University of Hong Kong **Terence R. Flotte** Professor, Provost, Dean and Executive Deputy Chancellor of the UMass Chan Medical School; President-Elect, American Society of Gene & Cell Therapy **Linkang Li** Executive Director of China Alliance for Rare Diseases; Vice President of Chinese Hospital Association **David Pearce** Chair of International Rare Disease Research Consortium; President of Innovation and Research, Sanford Health **Daniel Scherman** Director, French Foundation for Rare Diseases; Professor, Université Paris Cité; Head of the Medicine and Life Science Division of the European Academy of Science **Yi Wang** President of the National Children's Medical Center/Children's Hospital of Fudan University; Director of the Rare Disease Group of the Pediatric Branch of the Chinese Medical Association; Deputy Chairman of the Rare Disease Branch of the Chinese Hospital Association
16:20-16:40	**Keynote Speech** **Hope for Rare Foundation: Innovation Engine for Advancing Rare Diseases Research and Translational Medicine** **Kevin Huang** Co-founder and Secretary-General of Hope for Rare Foundation; Founder and President of Chinese Organization for Rare Disorders

Scientific Program

Opening Ceremony	
16:40-17:05	**Keynote Speech** **Research for Rare Diseases Benefits All** **Tian Xu** Co-founder, Hope for Rare Foundation; Chair Professor of Genetics and Vice President, Westlake University
17:05-17:35	**Panel Discussion** **China's Role: How China Integrate into Global Rare Diseases Research and Orphan Drugs Development** **Chair:** **Kevin Huang** Co-founder and Secretary-General of Hope for Rare Foundation; Founder and President of Chinese Organization for Rare Disorders **Panelists:** **Li Jin** Academician of Chinese Academy of Sciences; President of Fudan University; Dean of Shanghai Medical College, Fudan University **Yong-Hui Jiang** Professor of Genetics, Neuroscience, and Pediatrics, Chief of Medical Genetics, Director of Yale National Organization of Rare Disorders, Center of Excellence for Rare Disorders, Yale University School of Medicine **Xiao-Jiang Li** Professor, Guangdong-HongKong-Macao Institute of CNS Regeneration, Jinan University, Director, Guangdong Key Laboratory of Non-Human Primate Research Member, Scientific Innovation Alliance for Rare Diseases, Hope for Rare Foundation **Harvey F. Lodish** Professor of Biology and Professor of Biological Engineering, Founding member of the Whitehead Institute, Massachusetts Institute of Technology Member; National Academy of Sciences, US; Fellow, American Academy of Arts and Sciences **Adam Resnick** Director, Center for Data Driven Discovery in Biomedicine at the Children's Hospital of Philadelphia; Professor of Neurosurgery at the Perelman School of Medicine at the University of Pennsylvania **Tian Xu** Co-founder, Hope for Rare Foundation; Chair Professor of Genetics and Vice President, Westlake University **Xue Zhang** Academician of Chinese Academy of Engineering; Dean of Department of Medical Genetics, Institute of Basic Medical Sciences, Chinese Academy of Medical Sciences
17:35-17:45	**Inauguration of the Scientific Advisory Board of Hope for Rare Foundation**

Time: May 23 19:00—21:00
Venue: Greenland Hall 1+2 (1F)

Welcoming Banquet	
Presenter: Chunying Zheng China Global Television Network (CGTN)	
19:00-19:10	**Opening Show** **String Quartet** **Kunqu Opera: Peony Pavilion - Touring the Garden**
19:10-19:20	**Remarks From the Organizers** **Yi Wang** President of the National Children's Medical Center/Children's Hospital of Fudan University; Director of the Rare Disease Group of the Pediatric Branch of the Chinese Medical Association; Deputy Chairman of the Rare Disease Branch of the Chinese Hospital Association **Guangping Gao** Director, Li Weibo Institute for Rare Diseases Research, Director, Horae Gene Therapy Center and Viral Vector Core, Professor at UMass Medical School; Past President of the American Society of Gene & Cell Therapy **Kevin Huang** Co-founder and Secretary-General of Hope for Rare Foundation; Founder and President of Chinese Organization for Rare Disorders
19:20-19:30	**Remarks From the Sponsors** **Oudong Pan** President and Founder, OBiO Technology (Shanghai) Corp., Ltd.; President, Chamber of Commerce, Zhangjiang Science City; Co-founder, Hope for Rare Foundation **Jianmin Zhang** Vice President, Changchun GeneScience Pharmaceuticals Co., Ltd.
19:30-21:00	**Banquet**

Scientific Program

Time: May 24 8:30—12:00
Venue: QUBE Hall (2F)

Cutting-Edge Research Trends in Rare Diseases	
Chair: Feng Zhang Professor, Institute of Medical Genetics and Genomics, Fudan University	
8:35-9:00	**Molecular Diagnosis and Treatment of Tumors** **Weihong Tan** Academician of the Chinese Academy of Sciences; Member of the Academy of Sciences for the Developing World; Director of Hangzhou Institute of Medicine, Chinese Academy of Sciences; Director of the Institute of Molecular Medicine, Shanghai Jiao Tong University
9:05-9:30	**Mastering the Master Regulators in Pediatric Brain Cancer: The Path to Pediatric Brain Tumor Treatment** **Ruty Mehrian-Shai** Head, Brain Cancer Molecular Medicine, Sheba Medical Center
9:35-10:00	**Mitochondrial DNA in Mutation and Disease** **Zhenglong Gu** Director of Center for Mitochondrial Genetics and Health, Assistant Director for Greater Bay Area Institute of Precision Medicine (Guangzhou); Distinguished Professor, Fudan University
10:00-10:30	**Tea Break**
10:35-11:00	**Molecular Basis of Fragile X-associated Disorders** **Peng Jin** Professor and Chair, Department of Human Genetics, Emory University School of Medicine; Director of Emory Stephen T. Warren National Fragile X Research Center
11:05-11:30	**Developmental and Metabolic Impacts of the WRN Mutation in Werner Syndrome** **Wai-Yee Chan** Pro-Vice-Chancellor/Vice President, The Chinese University of Hong Kong; Li Ka Shing Professor of Biomedical Sciences, School of Biomedical Sciences, Faculty of Medicine, The Chinese University of Hong Kong
11:35-12:00	**Innate Immune Sensing of Lysosomal Dysfunction Drives Ubiquitous Lysosomal Storage Disorders** **Pinglong Xu** Qiushi Distinguished Professor, Zhejiang University, Professor, Life Sciences Institute, Zhejiang University, Director of Intelligent Medicine Institute of ZJU-Hangzhou Global Scientific and Technological Innovation Center; Member, Scientific Innovation Alliance for Rare Diseases, Hope for Rare Foundation

Scientific Program

Time: May 24 14:00—17:50
Venue: QUBE Hall (2F)

Model Systems Used in Rare Diseases Research	
Chair: Hekun Liu Professor, School of Basic Medical Sciences, Fujian Medical University	
14:05-14:30	**Human Derived IPSC-differentiated Cardiomyocytes for Muscular Dystrophy Disease Modeling** **Sophelia HS Chan** Clinical Associate Professor, Department of Pediatrics and Adolescent Medicine, School of Clinical Medicine, The University of Hong Kong
14:35-15:00	**Modeling GNAO1 Encephalopathy and Other Rare Neurological Diseases: Key Lessons From Mice and Fruit Flies** **Vladimir L. Katanaev** Full Professor at Department of Cell Physiology and Metabolism, Academic Director, HumanaFly Facility, Faculty of Medicine, University of Geneva; Academician, International Eurasian Academy of Sciences
15:05-15:30	**Genetically Modified Large Animal Models of Brain Diseases** **Xiao-Jiang Li** Professor, Guangdong-HongKong-Macao Institute of CNS Regeneration, Jinan University; Director, Guangdong Key Laboratory of Non-Human Primate Research; Member, Scientific Innovation Alliance for Rare Diseases, Hope for Rare Foundation
15:30-16:00	**Tea Break**
16:05-16:30	**Animal Models of Heart Failure and Exploration of Gene Therapy** **Xiongwen Chen** Dean and Professor, School of Pharmacy, Tianjin Medical University
16:35-17:00	**Use of Drosophila and iPSC Models to Understand Pathogenic Mechanisms of ALS and FTD** **Fen-Biao Gao** Professor and Governor Paul Cellucci Chair in Neuroscience Research, Founding Director of Frontotemporal Dementia Research Center, RNA Therapeutics Institute, UMass Chan Medical School
17:05-17:30	**Monkey Embryo Editing: Perspective on Science and Technology** **Yuyu Niu** Vice President and Professor, Kunming University of Science and Technology; Associate Director, State Key Laboratory of Primate Biomedical Research
17:35-17:50	**HUGO-GT ™ Whole Genomic DNA Humanized Model Accelerates Gene Therapy Research for Rare Diseases** **Lanqing Han** Founder & CEO, Cyagen Biosciences (Suzhou) Inc.; Director, Innovation Center for Artificial Intelligence, Research Institute of Tsinghua, Pearl River Delta

Scientific Program

Time: May 24 8:30—12:00
Venue: GREENLAND Hall 1 (1F)

Emerging Frontiers and Applications of Cell Therapy	
Chair: Liujiang Song Principal Scientist, AskBio; Adjunct Assistant Professor, University of North Carolina at Chapel Hill	
8:35-9:00	**CAR-T in Treatment of Autoimmune Diseases of the Nervous System** **Wei Wang** Professor and Chief Physician, Department of Neurology, Tongji Hospital affiliated to Tongji Medical College of Huazhong University of Science & Technology; Vice President of the Chinese Medical Association; Director of the National Medical Center for Public Health Emergency
9:05-9:30	**Allogeneic Hematopoietic Cell Transplantation in Children with Inborn Errors of Metabolism** **Hirotoshi Sakaguchi** Head, Division of Transplantation and Cellular Therapy, Children's Cancer Center, National Center for Child Health and Development, Japan
9:35-10:00	**Drug Discovery for Wolfram Syndrome Using hESCs-derived SC-β Cells and Cerebral Organoids** **Weida Li** Chief Scientist, Reg-Verse Therapeutics (Shanghai) Co. Ltd.; Professor, School of Life Sciences and Technology, Tongji University
10:00-10:30	**Tea Break**
10:35-10:50	**The Application and Development Process of Cell Therapy in Rare Diseases** **Lei Xu** Director of Cell Process Development Department, OBiO Technology (Shanghai) Corp., Ltd.
10:55-11:10	**Clinical Exploration of Off-the-shelf NK Cells in the Treatment of Rare Tumors** **Zhenwei Miao** CEO and President, iMBioRay (Hangzhou) Biomedical Co., Ltd.
11:15-11:30	**Durable Cell Gene Therapy** **Daixing Zhou** Founder & CEO, Lakeshore Biotechnology
11:30-12:00	**Panel Discussion** **Weida Li / Zhenwei Miao / Hirotoshi Sakaguchi / Liujiang Song / Wei Wang / Lei Xu / Daixing Zhou**

Time: May 24 14:00—17:30
Venue: GREENLAND Hall 1 (1F)

New Approaches and the Future in Gene Editing	
Chairs: **Daru Lu Vice Dean and Professor of Institute of Medical Genetics and Genomics, Fudan University** **Haoyi Wang Deputy Director of the State Key Laboratory of Stem Cell and Reproductive Biology, Leader of the Genetic Engineering Technology Research Group, Institute of Zoology, Chinese Academy of Sciences; Member, Scientific Innovation Alliance for Rare Diseases, Hope for Rare Foundation**	
14:05-14:30	**Genome Editing and Its Clinical Applications** **Dali Li** Researcher, School of Life Sciences, East China Normal University
14:35-15:00	**Gene Editing and Its Therapeutic Applications** **Wensheng Wei** Professor of School of Life Sciences, Investigator of Beijing Advanced Innovation Center for Genomics, Investigator of Peking-Tsinghua Center for Life Sciences, Investigator of Biomedical Institute for Pioneering Investigation via Convergence, Peking University
15:05-15:30	**An Innovative Delivery Platform for Genome and Epigenome Editing of Neurogenetic Disorders** **Yong-Hui Jiang** Professor of Genetics, Neuroscience, and Pediatrics, Chief of Medical Genetics, Director of Yale National Organization of Rare Disorders (NORD) Center of Excellence for Rare Disorders, Yale University School of Medicine
15:30-16:00	**Tea Break**
16:05-16:30	**Development of Gene Editing Technology Using Data Driven Approach** **Haoyi Wang** Deputy Director of the State Key Laboratory of Stem Cell and Reproductive Biology, Leader of the Genetic Engineering Technology Research Group, Institute of Zoology, Chinese Academy of Sciences; Member, Scientific Innovation Alliance for Rare Diseases, Hope for Rare Foundation
16:35-17:00	**Epigenetic Editing Therapy** **Albert Zheng** Principal Investigator, Institute of Zoology, Chinese Academy of Sciences; Principal Investigator, Beijing Institute of Stem Cell and Regenerative Medicine
17:05-17:30	**Base Editing and Its Application in Therapies** **Changhao Bi** Researcher, Tianjin Institute of Industrial Biotechnology, Chinese Academy of Sciences; Member, Scientific Innovation Alliance for Rare Diseases, Hope for Rare Foundation

Time: May 24 8:30—10:10
Venue: PRIMUS Hall B (2F)

Breakthroughs from Early-Career Researchers	
Chair: Boya Yu Project Manager of Hope for Rare Foundation	
8:35-8:50	**Thyrotropin- and Autoantibody-mediated Activation of the Thyrotropin Receptor** **Jia Duan** Group Leader and Investigator, Shanghai Institute of Materia Medica, Chinese Academy of Sciences
8:55-9:10	**Illuminating the Beginning of Life** **Chun So** Assistant Investigator, National Institute of Biological Sciences, Beijing; Assistant Professor, Tsinghua Institute of Multidisciplinary; Biomedical Research, Tsinghua University; Adjunct Assistant Professor, Department of Obstetrics and Gynaecology, The Chinese University of Hong Kong
9:15-9:30	**Molecular Basis of RNA Splicing and Beyond** **Rui Bai** Research Associate Professor, School of Life Sciences, Westlake University
9:35-9:50	**Unveiling Pluripotent State Transitions During Somatic Cell Reprogramming** **Xiaodong Liu** Principal Investigator, School of Life Sciences, Westlake University
9:55-10:10	**Combinatorial Design of Nanoparticles for Pulmonary mRNA Delivery and Genome Editing** **Bowen Li** Assistant Professor, Pharmaceutical Sciences, University of Toronto; Canada Research Chair in RNA Vaccines and Therapeutics

Time: May 24 10:30—12:10
Venue: PRIMUS Hall B (2F)

Hope for Rare Research Funding: From Basic Research to Personalized Medicine	
Chair: Juan Huang Project Manager of Hope for Rare Foundation; Project Manager of Chinese Organization for Rare Disorders	
10:35-10:50	**AAV1-hOTOF Gene Therapy for Autosomal Recessive Deafness 9** **Yilai Shu** Deputy Dean, Professor and Chief Physician of the Institute of Otolaryngology, Eye & ENT Hospital of Fudan University; Member, Scientific Innovation Alliance for Rare Diseases, Hope for Rare Foundation
10:55-11:10	**Exploratory Research on Gene Therapy for Neurodevelopmental Disorders** **Xiao Mao** Deputy Director of the Academic Office of the National Health Commission's Key Laboratory of Birth Defects Research and Prevention, Hunan Maternal and Child Health Hospital
11:15-11:30	**AAV Gene Therapy for Mucolipidosis Type IV** **Yang Yang** Researcher, State Key Laboratory of Biotherapy, West China Hospital, Sichuan University; General Manager, Chengdu Jinweike Biotechnology Co., Ltd.
11:35-11:50	**Adeno-associated Virus Based Gene Therapy for Autosomal Recessive Spinocerebellar Ataxia 20** **Guangzuo Luo** Professor of China Medical University Chairman of Nanjing BIONCE Biotechnology Co., Ltd.
11:55-12:10	**Novel Preclinical Model for Dravet Syndrome Reveals Disease Mechanisms** **Hong Wang** Principal Investigator, Shenzhen Institute of Advanced Technology, Chinese Academy of Sciences; Member, Scientific Innovation Alliance for Rare Diseases, Hope for Rare Foundation

Scientific Program

Time: May 24 14:00—17:40
Venue: PRIMUS Hall B (2F)

RNA-Based Therapeutics: From Antisense Oligonucleotides to mRNAs

Chair: Xin Li Qiushi Distinguished Professor, Executive Director of the Center for RNA Medicine of International School of Medicine, Zhejiang University

14:05-14:30	**The Opportunities and Challenges for ASO Drugs** **Fan Lai** Principal Investigator, School of Life Sciences, Yunnan University
14:35-15:00	**tRNA-based Gene Therapies** **Harvey F. Lodish** Professor of Biology and Professor of Biological Engineering, Founding member of the Whitehead Institute, Massachusetts Institute of Technology; Member, National Academy of Sciences, US; Fellow, American Academy of Arts and Sciences
15:05-15:30	**A Conserved Set of Ultra-Stable mRNA in Mammalian Sperm** **Xin Li** Qiushi Distinguished Professor, Executive Director of the Center for RNA Medicine of International School of Medicine, Zhejiang University
15:30-16:00	**Tea Break**
16:05-16:20	**Application of a Novel Antisense Oligonucleotide Technology in Treating Neuromuscular Diseases** **Yimin Hua** Distinguished Professor, College of Life Sciences, Nanjing Normal University; Founder, ASOcura Pharmaceuticals
16:25-16:50	**Therapeutic Potential of mRNA-based Interventions for Cardiac Rare Genetic Disease** **Jinzhong Lin** Distinguished Professor, School of Life Sciences, Director, Center for mRNA Drug Research and Development, Fudan University
16:55-17:20	**mRNA Protein Replacement Therapy for Liver Inherited Diseases** **Qiang Xia** Academician, Chinese Academy of Engineering; Dean, Chief Physician, Professor, Renji Hospital, Shanghai Jiao Tong University School of Medicine **Taihua Yang** Liver Surgeon, Renji Hospital, Shanghai Jiaotong University School of Medicine
17:25-17:40	**Suppressor tRNA: A "Broad-Spectrum" Gene Therapy Targeting Millions of Genetic Disease Patients** **Dong Wei** Co-founder, Codone Biotechnology

Time: May 24 8:30—10:10
Venue: PRIMUS Hall A (2F)

How Patient Organizations and Foundations Drive Drug Development

Chair: Kevin Huang Co-founder and Secretary-General of Hope for Rare Foundation; Founder and President of Chinese Organization for Rare Disorders

8:35-8:50	**Role of Patient Organizations and Foundations in Drug Development** **Ramaiah Muthyala** President & CEO, Indian Organization for Rare Diseases; Professor, University of Minnesota
8:55-9:10	**Success Stories on How Patient Organizations and Foundations Drive Drug Development** **Daniel Scherman** Director, French Foundation for Rare Diseases; Professor, Université Paris Cité; Head of the Medicine and Life Science Division of the European Academy of Science
9:15-9:30	**Challenges and Opportunities in the Development of Therapies for Ultrarare Diseases** **Juan Carlos López** Managing Director, Research Grants, RTW Charitable Foundation
9:35-9:50	**The Case of Fondazione Telethon: How Research Has Become Therapy** **Stefano Benvenuti** Head of Public Affairs at Fondazione Telethon ETS
9:55-10:10	**A Chinese Model of Foundation-Driven Drug Development for Rare Diseases** **Kevin Huang** Co-founder and Secretary-General of Hope for Rare Foundation; Founder and President of Chinese Organization for Rare Disorders

Scientific Program

Time: May 24 10:30—12:00
Venue: PRIMUS Hall A (2F)

How Scientists and Investors Can Work Together to Accelerate Innovative Therapies

Chair: Eileen Li Chief Business Officer, RareStone Group, Senior Consultant of the Chinese Organization for Rare Disorders

10:35-11:00	**New Business and Financing Models for Treating Rare Diseases** **Andrew W. Lo** Charles E. and Susan T. Harris Professor at the MIT Sloan School of Management; Director of the MIT Laboratory for Financial Engineering; Fellow, American Academy of Arts and Sciences
11:00-12:00	**Panel Discussion** **Xiaowei Jin** Venture Partner, Sherpa Healthcare Partners; Senior Consultant of the Chinese Organization for Rare Disorders **Xingdi Liu** Vice President, Qiming Venture Partners **Chengxi Shi** Co-founder and CEO, AstraGenomics **Ji Wang** Executive Director on the Asia Team, OrbiMed

Time: May 24 14:00—17:00
Venue: PRIMUS Hall A (2F)

Regulatory Innovation in Rare Diseases Research

Chair: Guoying Huang Professor of the National Children's Medical Center / Children's Hospital of Fudan University

14:05-14:30	**Rare and Ultra-rare Diseases, Global Regulatory Challenges and Opportunities** **Ana Hidalgo-Simon** Associate Professor, reNEW Consortium, Leiden University Medical Center; Former Head of Advanced Therapies at the European Medicines Agency (EMA)
14:35-15:00	**How to Get Beyond "One Disease at a Time": Platform Approaches to Genetic Therapies and Rare Disease Clinical Trials** **P.J. Brooks** Deputy Director, Division of Rare Diseases Research; Innovation, National Center for Advancing Translational Sciences (NCATS), National Institutes of Health, USA
15:05-15:30	**FDA Orphan Designation: Your Entry Visa to the US Market** **Timothy Cote** Former Director of the FDA Office of Orphan Products Development (OOPD); CEO & Founder of Only Orphans Cote LLC
15:30-16:00	**Tea Break**
16:05-16:30	**FDA's New Trend in Rare Disease** **Yaning Wang** CEO, Createrna Science & Technology; Former Director of the Division of Pharmacometrics in the Office of Clinical Pharmacology at FDA, US
16:35-17:00	**Russia's Access to Rare Disease Medication** **Alexey Alekhin** Associate Professor, Department of Biomedical Security, Bauman Moscow State Technical University; President, JSC Altayvitaminy

Time: May 24 19:00—21:00
Venue: Q-BOX Hall (1F)

Investigator-Patient Exchange Session

Chair: Juan Huang Project Manager of Hope for Rare Foundation; Project Manager of Chinese Organization for Rare Disorders

19:00-21:00	**Panel Discussion** **Terence R. Flotte** Professor, Provost, Dean and Executive Deputy Chancellor of the UMass Chan Medical School; President-Elect, American Society of Gene & Cell Therapy **Guangping Gao** Director, Li Weibo Institute for Rare Diseases Research, Director, Horae Gene Therapy Center and Viral Vector Core, Professor at UMass Medical School; Past President of the American Society of Gene & Cell Therapy **Steven J. Gray** Associate Professor, Department of Pediatrics, University of Texas Southwestern Medical Center **Taosheng Huang** Professor of Shanghai Medical College, Fudan University; Director of Institute of Medical Genetics and Genomics, Fudan University; Chairman of Medical Genetics Center, National Children's Medical Center / Children's Hospital of Fudan University **Daniel Scherman** Director, French Foundation for Rare Diseases; Professor, Université Paris Cité; Head of the Medicine and Life Science Division of the European Academy of Science **Haoyi Wang** Deputy Director of the State Key Laboratory of Stem Cell and Reproductive Biology, Leader of the Genetic Engineering Technology Research Group, Institute of Zoology, Chinese Academy of Sciences; Member, Scientific Innovation Alliance for Rare Diseases, Hope for Rare Foundation **Hong Wang** Principal Investigator, Shenzhen Institute of Advanced Technology, Chinese Academy of Sciences; Member, Scientific Innovation Alliance for Rare Diseases, Hope for Rare Foundation

Scientific Program

Time: May 25 8:30—10:10
Venue: QUBE Hall (2F)

Prenatal Diagnosis and Newborn Screening	
Chair: Tao Duan Chief Physician, Professor and Former Director of Shanghai First Maternity and Infant Hospital; Co-founder, Hope for Rare Foundation	
8:30-8:45	**Understanding Prenatal Screening, Newborn Screening, and the Complexities of Genetic Testing** **Sally Ann Lynch** Professor, School of Medicine, University College Dublin，Consultant in Clinical Genetics, Children's Health Ireland at Temple Street & Crumlin
8:50-9:05	**Newborn Genome Project** **Wenhao Zhou** Chief Physician, Professor and President, Guangzhou Women and Children's Medical Center, Guangzhou Medical University
9:10-9:25	**Newborn Screening Expansion for Inherited Metabolic Disorders – The Next Frontier** **Clara van Karnebeek** Professor, Amsterdam University Medical Centers (UMC)
9:30-9:45	**Precision Prenatal Diagnosis and Intrauterine Intervention for Rare Diseases** **Luming Sun** Chief Physician, Professor, Director of the Department of Fetal Medicine and Executive Director of the Prenatal Diagnostic Center, Shanghai First Maternity and Infant Hospital / Obstetrics and Gynecology Hospital of Tongji University
9:45-10:10	**Panel Discussion** **Tao Duan / Clara van Karnebeek / Sally Ann Lynch / Luming Sun / Wenhao Zhou**

Time: May 25 10:30—12:10
Venue: QUBE Hall (2F)

Recent Advances in Mitochondrial Diseases Research	
Chair: Zhenglong Gu Director of Center for Mitochondrial Genetics and Health, Assistant Director for Greater Bay Area Institute of Precision Medicine (Guangzhou), Distinguished Professor, Fudan University	
10:35-10:55	**Gene and Cell Therapies for Mitochondrial Diseases** **Shoukhrat Mitalipov** Professor and Director of the Center for Emrbyonic Cell and Gene Therapy, Oregon Health & Science University
11:00-11:20	**The Novel Approaches to Treating Mitochondrial Diseases** **Taosheng Huang** Professor of Shanghai Medical College, Fudan University; Director of Institute of Medical Genetics and Genomics, Fudan University; Chairman of Medical Genetics Center, National Children's Medical Center / Children's Hospital of Fudan University
11:25-11:45	**New Pathophysiology and Intervention Strategy of Leber Hereditary Optic Neuropathy** **Min-Xin Guan** Qiushi Distinguished Professor, Professor of Genetics, Director, Institute of Genetics, Zhejiang University; Director, Zhejiang University-University of Toronto Joint Institute of Genetics and Genomic Medicine
11:50-12:10	**Diversity of Mitochondrial Diseases in Children - A Single Center Study From Beijing Children's Hospital** **Fang Fang** Chief Physician and Professor, Department of Neurology, Beijing Children's Hospital, Capital Medical University

Scientific Program

Time: May 25 14:00—17:40
Venue: QUBE Hall (2F)

Academic Lectures from Fudan University: 2024 Shanghai Medical Forum	
Chairs: **Yi Wang President of the National Children's Medical Center / Children's Hospital of Fudan University; Director of the Rare Disease Group of the Pediatric Branch of the Chinese Medical Association; Deputy Chairman of the Rare Disease Branch of the Chinese Hospital Association** **Taosheng Huang Professor of Shanghai Medical College, Fudan University; Director of Institute of Medical Genetics and Genomics, Fudan University; Chairman of Medical Genetics Center, National Children's Medical Center / Children's Hospital of Fudan University** **Mei Tian Professor and Executive Director of the Human Phenome Institute, Fudan University**	
14:00-14:10	**Inauguration Ceremony: Institute of Medical Genetics and Genomics & iMGG Rare Diseases Research Institute, Fudan University** **Li Jin** Academician of Chinese Academy of Sciences; President of Fudan University; Dean of Shanghai Medical College, Fudan University
14:10-14:30	**The New Mendelian Disease in Human Oocyte Development** **Lei Wang** Professor, Institutes of Biomedical Sciences, Fudan University
14:30-14:50	**Harness Unintended Nucleic Acid Mutation to Precise Base Editing** **Li Yang** Distinguished Principal Investigator, Fudan University
14:50-15:10	**Breakthrough in Genetic Cholestasis** **Jianshe Wang** Director, Center for Pediatric Liver Diseases, National Children's Medical Center / Children's Hospital of Fudan University
15:10-15:30	**Fibrotic Study in Chinese Scleroderma** **Jiucun Wang** Professor and Chair of Department of Anthropology and Human Genetics, Fudan University
15:30-16:00	**Tea Break**
16:05-16:25	**The Mechanisms Underlying the Pathogenic Threshold of CAG Repeats in Huntington's Disease** **Boxun Lu** Professor, School of Life Sciences, Fudan University
16:30-16:50	**Animal Model Establishment of Amyotrophic Lateral Sclerosis Based on Genotype-Phenotype Correlation Huashan TRC Study** **Xiangjun Chen** Chief Physician and Professor, Department of Neurology, Huashan Hospital, Fudan University
16:55-17:15	**On the Way to Solutions for CDG: Personal Perspectives for Rare Disease** **Li Chen** Professor of Shanghai Medical College, Fudan University
17:20-17:40	**rAAV Capsid Mutants Eliminate Leaky Expression from DNA Donor Template for Homologous Recombination** **Chen Ling** Professor, School of Life Sciences, Fudan University

Scientific Program

Time: May 25 8:30—12:00
Venue: GREENLAND Hall 1 (1F)

Development of Vectors and Delivery Systems	
Chair: Xiao Xiao Co-founder, Chairman & CSO, Belief BioMed Group; Co-founder, Hope for Rare Foundation	
8:35-9:00	**An AI-accelerated Capsid Evolution Platform Enables the Discovery of Novel AAV with Altered Tropism** **Lijia Ma** Principal Investigator, School of Life Sciences, Westlake University; Founder, Westlake Genetech (Hangzhou) Co., Ltd.; Member, Scientific Innovation Alliance for Rare Diseases, Hope for Rare Foundation
9:05-9:30	**Novel Adeno-Associated Virus Engineering and Its Applications in Disease Therapy** **Guisheng Zhong** Associate Professor of School of Life Science and Technology, Associate Professor of iHuman Institute, ShanghaiTech University; Founder, EmayGene Biotech Co., Ltd.
9:35-9:50	**Flexible Solutions for AAV Commercial Production: From 15L Scale up to 2000L** **Weiran Shen** Vice President, OBiO Technology (Shanghai) Corp., Ltd.
9:55-10:10	**Building a Gene Therapy Platform to Accelerate Drug Development for Rare Diseases** **Xiaobin He** CEO, Genevoyager (Wuhan) Co., Ltd.
10:00-10:30	**Tea Break**
10:35-11:00	**Gene Therapy for β-thalassemia** **Yujia Cai** Principal Investigator and Assistant Dean, Shanghai Center for Systems Biomedicine, Shanghai Jiao Tong University
11:05-11:30	**Development of Next-Generation Lipid Nanoparticles for Gene Delivery** **Bowen Li** Assistant Professor, Pharmaceutical Sciences, University of Toronto; Canada Research Chair in RNA vaccines and therapeutics
11:35-12:00	**Development and Application of Tissue-selective mRNA-LNP Delivery System** **Qiang Cheng** Assistant Professor, College of Future Technology, Peking University

Scientific Program

Time: May 25 14:00—17:40
Venue: GREENLAND Hall 1 (1F)

Gene Therapy: Advances, Challenges and Perspectives	
Chair: Guangping Gao Director, Li Weibo Institute for Rare Diseases Research, Director, Horae Gene Therapy Center and Viral Vector Core, Professor at UMass Medical School; Past President of the American Society of Gene & Cell Therapy	
14:05-14:30	**Gene Therapy for Genetic Diseases: Our China Experience** **Xiao Xiao** Co-founder, Chairman & CSO, Belief BioMed Group; Co-founder, Hope for Rare Foundation
14:35-15:00	**Gene Therapy for Tay-Sachs Disease** **Terence R. Flotte** Professor, Provost, Dean and Executive Deputy Chancellor of the UMass Chan Medical School; President-Elect, American Society of Gene & Cell Therapy
15:05-15:30	**Advances in Genetic Pharmacology and Gene Therapy** **Daniel Scherman** Director, French Foundation for Rare Diseases; Professor, Université Paris Cité; Head of the Medicine and Life Science Division of the European Academy of Science
15:30-16:00	**Tea Break**
16:05-16:30	**AAV Gene Therapy for Rare Diseases: Translating from Cells to Cows and Humans** **Guangping Gao** Director, Li Weibo Institute for Rare Diseases Research, Director, Horae Gene Therapy Center and Viral Vector Core, Professor at UMass Medical School; Past President of the American Society of Gene & Cell Therapy
16:35-17:00	**Expanding Gene Therapy for Neurological Disorders** **Steven J. Gray** Associate Professor, Department of Pediatrics, University of Texas Southwestern Medical Center
17:05-17:20	**Advancing Novel Gene Therapies to Deliver Life-changing Cures** **Fenglan Wu** Co-founder and CEO, Gritgen Therapeutics Co., Ltd.
17:25-17:40	**Application of AAV Capsid Platform Delivery System in CNS Drug Development** **Huaqiong Shen** Founder and CEO, NeuShen Therapeutics

Scientific Program

Time: May 25 8:30—12:00
Venue: PRIMUS Hall B (2F)

Natural History Studies & Cohort Studies of Rare Diseases	
Chair: Yi Wang President of the National Children's Medical Center / Children's Hospital of Fudan University; Director of the Rare Disease Group of the Pediatric Branch of the Chinese Medical Association; Deputy Chairman of the Rare Disease Branch of the Chinese Hospital Association	
8:35-9:00	**Natural History & Real-World Data in Rare Diseases: An Introduction** **Linda Salem** Principal Data Scientist at F. Hoffmann-La Roche Ltd.
9:05-9:30	**A Clinical Study and Utilization of Patient Registry** **Yasuyuki Fukuhara** Chief, Division of Medical Genetics, National Center for Child Health and Development, Japan
9:35-10:00	**Natural History Study in Rare Disease Drug Development** **Karen Zhou** Senior Director, Clinical Development, Lundbeck China
10:00-10:30	**Tea Break**
10:35-11:00	**Understand Precision Medicine of Rare Diseases from the Natural History and Cohort Study of Angelman Syndrome (AS)** **Yi Wang** President of the National Children's Medical Center / Children's Hospital of Fudan University; Director of the Rare Disease Group of the Pediatric Branch of the Chinese Medical Association; Deputy Chairman of the Rare Disease Branch of the Chinese Hospital Association
11:05-11:30	**Predictive Model for Age at Onset and Prognosis of Facioscapulohumeral Muscular Dystrophy Based on a Cohort Study** **Zhiqiang Wang** Chief Physician and Professor of Neurology Department, The First Affiliated Hospital of Fujian Medical University
11:35-12:00	**Three Decades in Review: Exploring Predictors of Neurodevelopment in Phenylketonuria Patients** **Celia Azevedo Soares** Medical Genetics Attending Physician, Unidade Local de Saúde Santo António, Porto

Time: May 25 14:00—17:35
Venue: PRIMUS Hall B (2F)

Novel Targets for Small Molecules and Antibodies	
Chair: Hong Xu Professor and Former Secretary of the Party Committee of the National Children's Medical Center / Children's Hospital of Fudan University	
14:05-14:35	**Genetic Diagnosis & Target Therapy for Rare Diseases** **Qing Zhou** Qiushi Distinguished Professor, Professor at Life Sciences Institute, Zhejiang University; Member, Scientific Innovation Alliance for Rare Diseases, Hope for Rare Foundation
14:40-15:05	**Exploiting Antigen Presentation Pathways for Precision Immune Engineering** **Novalia Pishesha** Assistant Professor, Division of Immunology, Boston Children's Hospital and the Department of Pediatrics, Harvard Medical School，Co-founder & CEO, Cerberus Therapeutics
15:10-15:30	**AL01211 - A Novel Glucosylceramide Synthase (GCS) Inhibitor for Treatment of Fabry Disease** **Jianhong Zheng** Co-founder & General Manager (China), AceLink Therapeutics (Suzhou) Limited
15:30-16:00	**Tea Break**
16:05-16:30	**Novel Mechanisms and Technologies Advancing Rare Disease Therapeutics** **S. Pablo Sardi** Global Head, Rare and Neurologic Diseases Research Therapeutic Area, Sanofi
16:35-17:00	**Application of Genetics and AI to First-in-class New Drug Development** **Tony Xu** Co-founder and COO of Drug Farm
17:05-17:35	**Exploring the Establishment of Pediatric Rare Disease Tumor Models and Target Discovery: A Brief Example of NF1** **Rui Dong** Director of the Research Department, Chief Physician of Pediatric Surgery and Researcher, National Children's Medical Center / Children's Hospital of Fudan University

Scientific Program

Time: May 25 8:30—10:00
Venue: PRIMUS Hall A (2F)

Funding of Scientific Research in the Field of Biology and Medicine	
Chair: Mel Zhang Project Director of Hope for Rare Foundation	
8:35-8:55	**Private Funding Support for Biomedical Researchers** **Na Zhou** Program Officer, New Cornerstone Science Foundation
9:00-9:25	**Introducing Chen Institute's UniCasehub and Its Research Grant Program** **Yifeng Xu** Director, Brain Health Institute at National Center for Mental Disorder; Expert of Academic Committee, Tianqiao & Chrissy Chen Institute's UniCaseHub Community
9:30-9:45	**Pioneering the Future: Empowering Young Scientists in Biomedicine** **Jing-Ke Weng** Inaugural Director, Institute for Plant-Human Interface, Professor of Chemistry, Chemical Biology, and Bioengineering, Northeastern University; Member of Steering Committee, Asian Young Scientist Fellowship
9:45-10:00	**Rare Disease Research Funding: A Foundation Perspective** **Mel Zhang** Project Director of Hope for Rare Foundation

Time: May 25 10:30—12:15
Venue: PRIMUS Hall A (2F)

Benefit-sharing: A Thorny Question for Biomedical Innovation	
Chair: Yeyang Su Anthropologist, Consultant of the Hope for Rare Foundation	
10:35-10:45	**The Children's Brain Tumor Network: How to build a Data Sharing Consortium on Behalf of a Rare Disease or Cancer** **Adam Resnick** Director, Center for Data Driven Discovery in Biomedicine at the Children's Hospital of Philadelphia; Research Professor of Neurosurgery at the Perelman School of Medicine at the University of Pennsylvania
10:50-11:00	**The Changing Role of Patient Organizations as Innovators and the Role They Play** **Linguo Li** Director of Public Policy Research Center of Rare Diseases at the Chinese Organization for Rare Disorders
11:05-11:15	**Manufacturing Hope for the Rare Disease Community: Advanced Therapies and the Ethics of Care** **Dong Dong** Assistant Professor, JC School of Public Health and Primary Care, Faculty of Medicine, The Chinese University of Hong Kong; Research Associate (by courtesy), CUHK Centre for Bioethics, The Chinese University of Hong Kong; Principal Investigator, Rare Disease Real-world Data Lab, Shenzhen Research Institute, The Chinese University of Hong Kong
11:20-11:30	**Fair Price Setting as a Means to Benefit Sharing: Ethical Considerations** **Wim Pinxten** Professor at the Faculty of Health and Life Sciences, Chair of Healthcare and Ethics Group, Hasselt University
11:35-11:45	**Who Benefits? Why We Need a New Social Contract for Patient Support and Participation in Orphan Drug Research and Development** **Jin Ding** Research Fellow, iHuman & Department of Sociological Studies, University of Sheffield
11:45-12:15	**Panel Discussion** **Jin Ding / Dong Dong / Linguo Li / Wim Pinxten / Adam Resnick / Yeyang Su**

Scientific Program

Time: May 25 14:00—17:30
Venue: PRIMUS Hall A (2F)

International Collaboration in Rare Diseases Research & Treatment (Including Closing Ceremony)	
Chair: Adam Resnick Director, Center for Data Driven Discovery in Biomedicine at the Children's Hospital of Philadelphia; Research Professor of Neurosurgery at the Perelman School of Medicine at the University of Pennsylvania	
14:05-14:30	**The International Rare Disease Research Consortium (IRDiRC): Making Rare Disease Research Efforts More Efficient and Collaborative Around the World** **David Pearce** Chair of International Rare Disease Research Consortium; President of Innovation and Research, Sanford Health
14:35-15:00	**Rare Disease Diagnosis, Treatment and Research in China: Advancements and Prospects** **Shuyang Zhang** Chief Physician, Professor and President of Peking Union Medical College Hospital; Chair of the Chinese Society of Rare Diseases of the Chinese Medical Association; Chair of the National Expert Committee on Rare Diseases Clinical Care and Accessibility of the National Health Commission
15:05-15:30	**ICORD: The Value of "Collaboration" in the Development of Assistance and Research Programs for Rare Diseases** **Emilio J. A. Roldan** Secretary, the Board of the International Conference on Rare Diseases and Orphan Drugs (ICORD)
15:30-16:00	**Tea Break**
16:05-16:30	**The Children's Brain Tumor Network: How to Build a Data Sharing Consortium on Behalf of a Rare Disease or Cancer** **Adam Resnick** Director, Center for Data Driven Discovery in Biomedicine at the Children's Hospital of Philadelphia; Research Professor of Neurosurgery at the Perelman School of Medicine at the University of Pennsylvania
16:30-17:00	**Panel Discussion** **Li Jin / David Pearce / Adam Resnick / Emilio J. A. Roldan / Tian Xu / Shuyang Zhang**
17:00-17:30	**Closing Ceremony** **Announcing the Shanghai Declaration**

Pre-Conference Events

Time: May 23 9:30—11:30
Venue: Q-BOX Hall (1F)

Tips for Starting a Biotech	
Chair: Eileen Li Chief Business Officer, RareStone Group, Senior Consultant of the Chinese Organization for Rare Disorders	
9:30-9:50	**Developing and Testing tRNA- and CRISPR-based Therapies for Haploinsufficient Genetic Disorders and Genetic Diseases Caused by Nonsense Mutations** **Harvey F. Lodish** Professor of Biology and Professor of Biological Engineering, Founding member of the Whitehead Institute, Massachusetts Institute of Technology; Member, National Academy of Sciences, US; Fellow, American Academy of Arts and Sciences
9:50-10:10	**Investing in Tevard and Joining the Board of Directors** **Xiaowei Jin** Venture Partner, Sherpa Healthcare Partners; Senior Consultant of the Chinese Organization for Rare Disorders
10:10-10:30	**A Case Study in Biotech Finance: Two Dad's a Professor and $40 Million, the Emergence of Tevard Biosciences** **Warren Lammert** Co-Founder & Chairman, Tevard Biosciences; Founder & CIO, Granite Point Capital
10:30-11:30	**Panel Discussion** **Harvey F. Lodish / Xiaowei Jin / Warren Lammert** **Xiaodi Su** Executive Director, Lilly Asia Ventures **Zhiyu Yan** Co-founder, Chairman & CEO, Sperogenix Therapeutics; Co-founder, Hope for Rare Foundation

Time: May 23 11:30—12:30
Venue: Q-BOX Hall (1F)

Contracting Ceremony of the Cooperative Publishing Framework Agreement between Zhejiang University Press and Hope for Rare Foundation	
Chair: Ge Zhang Director, Science and Technology Publishing Center, Zhejiang University Press	
11:30-11:50	**Remarks** **Kevin Huang** Co-founder and Secretary-General of Hope for Rare Foundation, Founder and President of Chinese Organization for Rare Disorders
11:55-12:15	**Remarks** **Chaofu Chu** Chairman, President and Party Secretary of Zhejiang University Press
12:20-12:30	**Contracting Ceremony**

Pre-Conference Events

Time: May 23 9:30—11:50
Venue: LEO Hall (1F)

Launching Ceremony of the Center for Clinical Evaluation and Translation of Advanced Therapies for Pediatric Rare and Genetic Diseases	
Chair: Lilian Li Marketing Director of OBiO Technology	
9:35-9:45	**Remarks From the Guests** **Houjia Liu** Director of the Shanghai Biomedical Industry Promotion Center **Wei Zhu** Deputy director of Shanghai Pudong New Area Health Commission
9:45-10:00	**To Fulfill the Mission and Take the Leading Role in the Diagnosis and Treatment of Rare Diseases in Children** **Hao Zhang** President of Shanghai Children's Medical Center
10:00-10:15	**Collaborative Construction of Innovative Transformation Platforms to Advance Rare Disease Treatments** **Javier Jia** CEO of OBIO Technology
10:15-10:30	**Hope for Rare Foundation: Innovation Engine for Advancing Rare Diseases Research and Translational Medicine** **Kevin Huang** Co-founder and Secretary-General of Hope for Rare Foundation, Founder and President of Chinese Organization for Rare Disorders
10:30-10:40	**Contracting Ceremony** **Hao Zhang** President of Shanghai Children's Medical Center **Oudong Pan** President and Founder, OBiO Technology (Shanghai) Corp., Ltd.; President, Chamber of Commerce, Zhangjiang Science City; Co-founder, Hope for Rare Foundation **Tao Duan** Chief Physician, Professor and Former Director of Shanghai First Maternity and Infant Hospital; Co-founder, Hope for Rare Foundation
10:40-11:10	**Panel Discussion: Current Situation and Prospect of Clinical Research, Diagnosis and Treatment of Rare Diseases and Genetic Disorders in Children** **Chair:** **Tao Duan** Chief Physician, Professor and Former Director of Shanghai First Maternity and Infant Hospital; Co-founder, Hope for Rare Foundation **Panelists:** **Xinxing Liu** General Manager of RRGENER Therapeutics **Juan Huang** Project manager of Hope for Rare Foundation **Tianhong Xu** Founder of Base Therapeutics **Yuxuan Wu** CEO of YolTech Therapeutics **Michelle Yang** Chief Operating Officer, Clinical Research Center, GoBroad Healthcare Group
11:10-11:40	**Panel Discussion:Development and Translation of Advanced Therapies for Rare Diseases and Genetic Disorders in Children** **Chair:** **Xinyan Sun** Director, Clinical Research Center, Shanghai Children's Medical Center **Panelists:** **Zhiqi Xiong** Senior Investigator of Center for Excellence in Brain Science and Intelligence Technology, Chinese Academy of Schiences **Guangzuo Luo** Professor of China Medical University; Chairman of Nanjing BIONCE Biotechnology Co., Ltd. **Jane Zheng** Co-founder and CEO of Belief BioMed **Xingbin Liao** Deputy director, Biomedical Industry Promotion Office, Shanghai Pudong New Area Health Commission **Yuanbang Wang** Vice President of Business Development, CCB Trust
11:40-11:50	**Summary** **Lilian Li** Marketing Director of OBiO Technology

Seminars

Time: May 24 8:30—12:00
Venue: Q-BOX Hall (1F)

UCB Seminar: Innovative Breakthroughs in Rare Neurological Diseases and Immunological Disorders	
Chair: Cheng Peng External Engagement Lead, UCB China	
8:30-8:40	**Remarks (online)** **Alexandre Moreau** President of UCB China
8:40-9:10	**Thymidine Kinase 2 Deficiency (TK2d): Translational Science in Rare Disease Drug Development (online)** **Brooks Boyd** Science and Translational Science Lead, UCB
9:10-9:20	**Q&A with UCB Early Research Team (Brooks Boyd, online)**
9:20-10:00	**Advances in Screening, Diagnosis and Treatment of Neurological Disorders in China** **Yi Wang** President of the National Children's Medical Center / Children's Hospital of Fudan University; Director of the Rare Disease Group of the Pediatric Branch of the Chinese Medical Association; Deputy Chairman of the Rare Disease Branch of the Chinese Hospital Association
10:00-10:40	**MCM8-Associated Mitophagy in Neurological and Immune-Mediated Diseases** **Yuxia Zhang** PI of Guangzhou Women and Childrens Medical Center, Guangzhou Medical University
10:40-11:20	**Translating Cutting Edge Discoveries and Technologies in ATMP into Clinically Effective Cures for Rare Neurological Diseases** **Hua Lv** Vice President of Shanghai Vitalgen BioPharma
11:20-12:00	**Precision Therapy for Rare Diseases - Development of Nervous/Immune System Targeting AAV Vectors** **Lei Zhao** Head of Early Research, Cure Genetics

Time: May 24 14:00—17:40
Venue: Q-BOX Hall (1F)

Academic Lectures from Zhejiang University Precision Medicine Project & Liangzhu Laboratory	
Chairs: Xudong Fu Principal Investigator, Liangzhu Laboratory, Zhejiang University Ning Shen Principal Investigator, Liangzhu Laboratory, Zhejiang University	
14:05-14:25	**Dissection of CTLA-4 Functions Through the Study of a Disease-associated Point Mutation** **Linrong Lu** Qiushi Distinguished Professor, Zhejiang University; Executive Director and Senior Principal Investigator, Shanghai Immune Therapy Institute
14:30-14:50	**Progress in Prenatal Screening for Hereditary Birth Defects** **Yifeng Liu** Attending Physician, Associate Research Fellow, Women's Hospital, Zhejiang University School of Medicine; Assistant Director, Zhejiang Provincial Clinical Research Center for Child Health
14:55-15:15	**Mitophagy Defect Mediates the Aging-associated Hallmarks in Hutchinson–Gilford Progeria Syndrome** **Xudong Fu** Principal Investigator, Liangzhu Laboratory, Zhejiang University
15:20-15:40	**The Potential and Opportunities of Mitophagy Inducers in Treating Rare Diseases** **Mengyang Fan** Director of Medicinal Chemistry Department, Hangzhou Phecda Medicine Co., Ltd.
15:40-16:00	**Tea Break**
16:05-16:25	**Therapeutic Antisense Oligonucleotide for Hutchinson-Gilford Progeria Syndrome and Beyond** **Ning Shen** Principal Investigator, Liangzhu Laboratory, Zhejiang University
16:30-16:50	**iPSC-based Studies of Inherited Arrhythmias** **Ping Liang** Professor, Institute of Translational Medicine, The First Affiliated Hospital, Zhejiang University
16:55-17:15	**Clinical Characteristics of Patients with Hutchinson-Gilford Progeria Syndrome in China** **Jingjing Wang** Chief Physician of Children's Hospital of Zhejiang University School of Medicine
17:20-17:40	**Novel Mutations in Fabry Disease and Enzymatic Validation with iPSCs** **Lidan Hu** Distinguished Associate Research Fellow, National Clinical Research Center for Child Health, Children's Hospital of Zhejiang University School of Medicine

Seminars

Time: May 25 8:30—10:30
Venue: Q-BOX Hall (1F)

CORD Seminar: Gene Therapy Market and Innovative Payment Solutions in China

Chair: Linguo Li Director of Public Policy Research Center of Rare Diseases at the Chinese Organization for Rare Disorders

8:35-9:00	**Breakthroughs and Perspectives of Gene Therapy for Rare Diseases to Benefit Patients** **Linguo Li** Director of Public Policy Research Center of Rare Diseases at the Chinese Organization for Rare Disorders
9:05-9:30	**Hope and Value from Gene Therapy Innovations for Rare Diseases** **Zheng Liu** Management Consulting Manager at Simon-Kucher
9:30-10:30	**Panel Discussion** **Innovative Payment Solutions for Rare Disease Gene Therapy and "Going Overseas in Shanghai" Discussion** **Hao Feng** Chief Innovation Officer, MediTrust Health **Kevin Huang** Co-founder and Secretary-General of Hope for Rare Foundation; Founder and President of Chinese Organization for Rare Disorders **Qi Kang** Director, Health Management Research Office, Health Policy Research Department, Shanghai Health and Health Development Research Center **Linguo Li** Director of Public Policy Research Center of Rare Diseases at the Chinese Organization for Rare Disorders **Zheng Liu** Management Consulting Manager at Simon-Kucher **Oudong Pan** President and Founder, OBiO Technology (Shanghai) Corp., Ltd.; President, Chamber of Commerce, Zhangjiang Science City; Co-founder, Hope for Rare Foundation **Xinyan Sun** Director, Clinical Research Management Center, Shanghai Children's Medical Center **Wenjie Zheng** Head of Public Affairs Department and Patient Organization Advocacy, Takeda China

Satellite Meetings

Time: May 24 12:00—13:00
Venue: QUBE Hall (2F)

Biogen Satellite Meeting

Chair: Yi Wang President of the National Children's Medical Center / Children's Hospital of Fudan University; Director of the Rare Disease Group of the Pediatric Branch of the Chinese Medical Association; Deputy Chairman of the Rare Disease Branch of the Chinese Hospital Association

12:00-12:10	**Opening Remarks**
12:10-12:50	**Benefits of Disease-Modifying Therapy for SMA Based on Long-term Study Data** **Wenhui Li** Associate Chief Physician, Department of Neurology, National Children's Medical Center / Children's Hospital of Fudan University
12:50-13:00	**Summary**

Time: May 24 12:30—13:30
Venue: GREENLAND Hall 1 (1F)

Takeda Satellite Meeting

Chair: Linhua Yang Director of the Institute of Hematology at Shanxi Medical University, Director of Department of Hematology at the Second Hospital of Shanxi Medical University

12:30-12:35	**Opening Remarks**
12:35-13:00	**Progress in Diagnosis and Treatment of Rare Bleeding Disorders** **Jing Dai** Chief Technician, Department of Laboratory Medicine, Ruijin Hospital, Shanghai Jiao Tong University School of Medicine
13:00-13:25	**Clinical Application of Gene Therapy in Rare Diseases and Hemophilia B** **Feng Xue** Chief Physician, Center of Thrombosis and Hemostasis Diseases, Institute of Hematology & Blood Diseases Hospital, Chinese Academy of Medical Sciences
13:25-13:30	**Summary**

Time: May 24 12:00—14:00
Venue: PRIMUS Hall B (2F)

Boehringer Ingelheim Satellite Meeting

Chairs: Wenyu Wu Professor and Director of Dermatology, Huashan Hospital Affiliated to Fudan University
Hui Deng Director of Dermatology, Shanghai Sixth People's Hospital

12:00-12:05	**Opening Remarks** **Wenyu Wu** Professor and Director of Dermatology, Huashan Hospital Affiliated to Fudan University
12:05-12:40	**Disease Characterization and Diagnostic Evaluation of Generalized Pustular Psoriasis (GPP)** **Ling Han** Associate Chief Physician, Department of Dermatology, Huashan Hospital Affiliated to Fudan University
12:40-13:15	**Management of the Full Course of Generalized Pustular Psoriasis (GPP)** **Yu Gong** Deputy Director of Dermatology, Shanghai Tenth People's Hospital / Tenth People's Hospital Affiliated to Tongji University
13:15-13:50	**Reversal of GPP Biomarker Expression by Pesolizumab** **Yifeng Guo** Chief Physician, Xinhua Hospital Affiliated to Shanghai Jiao Tong University School of Medicine
13:50-14:00	**Summary** **Hui Deng** Director of Dermatology, Shanghai Sixth People's Hospital

Satellite Meetings

Time: May 24 13:00—14:00
Venue: PRIMUS Hall A (2F)

Gensci Satellite Meeting	
Chair: Yi Wang President of the National Children's Medical Center / Children's Hospital of Fudan University; Director of the Rare Disease Group of the Pediatric Branch of the Chinese Medical Association; Deputy Chairman of the Rare Disease Branch of the Chinese Hospital Association	
13:00-13:05	**Opening Remarks**
13:05-13:20	**Innovative Research and Development in Rare Diseases** **Gao Sun** Senior Director in Early Discovery, Changchun GeneScience Pharmaceuticals Co., Ltd.
13:20-13:45	**A New Chapter in the Treatment of Prader-Willi Syndrome** **Xiaoping Luo** Professor and Head of Department of Pediatrics, Tongji Hospital, Tongji Medical College, Huazhong University of Science and Technology
13:45-14:00	**Summary**

Time: May 25 12:00—13:00
Venue: QUBE Hall (2F)

Chigena Satellite Meeting	
Chair: Yi Wang President of the National Children's Medical Center / Children's Hospital of Fudan University; Director of the Rare Disease Group of the Pediatric Branch of the Chinese Medical Association; Deputy Chairman of the Rare Disease Branch of the Chinese Hospital Association	
12:00-12:05	**Opening Remarks**
12:05-12:55	**"Chigene Brain" Enhances Precision Molecular Diagnosis of Rare Diseases and Facilitates Discovery of Novel Disease-responsible Genes** **Weiyue Gu** Founder and Chairman of Beijing Chigene Translational Medicine Research Center and Beijing CAR-T Medical Technology Co., Ltd.
12:55-13:00	**Summary**

Time: May 25 12:30—13:30
Venue: GREENLAND Hall 1 (1F)

Ipsen Satellite Meeting	
12:30-12:50	**Prospect Analysis of the 3-month Release Form of Diphereline® in Children with CPP in China** **Wei Lu** Vice Director of the Department of Endocrinology and Metabolic Diseases, Director of Neonatal Disease Screening Center, National Children's Medical Center / Children's Hospital of Fudan University
12:50-13:30	**Showing of the Promotional Video**

Alphabetical Order by Last Name

Speaker Profiles

Alexey Alekhin

Associate Professor, Department of Biomedical Security, Bauman Moscow State Technical University
President, JSC Altayvitaminy

Alexey Alekhin, associate professor of Department of Biomedical Security at Bauman Moscow State Technical University. He graduated from Lomonosov Moscow State University in 2008, started career with pharmaceutical company in 2011, took position of the government official in the Russian Federation in the sphere of pharmaceutical manufacturing, GMP and licensing in 2014. After 10 years of service in the Ministry of Industry and Trade of the Russian Federation, he was appointed the president of JSC Altayvitaminy (pharmaceutical manufacturing company) in 2023.

Rui Bai

Research Associate Professor, School of Life Sciences, Westlake University

Rui Bai, Research Associate Professor at Westlake University, has been dedicated to the molecular mechanisms of spliceosomes and RNA splicing. During my research career, I and my colleagues reconstructed the near-atomic resolution structures of eight different functional spliceosomes and the first human minor spliceosome in the world by single-particle cryo-electron microscopy, and published 7 papers in Science and 3 papers in Cell as the first author and the co-first author. These structures, covering all the major states of the assembly, activation, catalysis, and dissociation of spliceosome, shed lights on molecular basis for the spliceosome of working states, and also gave rise to a complete structural view of pre-mRNA splicing for the first time in the world, which was one of the most fundamental processes in eukaryotic cells. In 2021, we captured and determined the first atomic-structure of human minor spliceosome, which catalyze splicing of the rare but essential U12-type intron. My research accomplishments thus initiate a new era for mechanistic understanding of pre-mRNA splicing and provides unprecedented opportunity for drug development against cancers and rare diseases. I earned the 2020 L'Oréal-UNESCO For Women in Science International Rising Talents rewards, the 2020 awards of the DIMITRIS N. CHORAFAS foundation, and finalist of Rising Talent from China in 2018.

Stefano Benvenuti

Head of Public Affairs at Fondazione Telethon ETS

Stefano Benvenuti is Head of Public Affairs at Fondazione Telethon (Italy) since 2021. He is vice-chair of the funders constituent committee of the International Rare Diseases Research Consortium (IRDiRC) and co-chair of the Working Group on Health Economic Value of Personalised Medicine Approaches of the International Consortium for Personalized Medicine (ICPerMed). He is also member of the Italian National Committee on Rare Disease.
After graduating in International Cooperation for Development at the University of Bologna in 2007 he started as a consultant project manager of EU funded projects. In 2010 he joined the healthcare department of Regione Veneto working as EU project specialist where he contributed to re-design the regional system of participation in EU funded programs. During this period, he also completed a master degree in Health Technology Assessment at Università Cattolica in Rome.
He joined Fondazione Telethon in 2016 to set-up the EU affairs office and coordinate the participation of Fondazione Telethon in European and International initiatives. In 2018 he took the role of Global Partnership Manager. At Fondazione Telethon he also coordinates the project "Sequencing of newborn genome: feasibility and clinical, ethical, psychological and economic implications" co-funded by the Lombardy Region.

Speaker Profiles

Speaker Profiles

Changhao Bi

Researcher, Tianjin Institute of Industrial Biotechnology, Chinese Academy of Sciences
Member, Scientific Innovation Alliance for Rare Diseases, Hope for Rare Foundation

Changhao Bi is a researcher and PI of the Synthetic Biotechnology Research Group, Tianjin Institute of Industrial Biotechnology (TIB), Chinese Academy of Sciences, and served as the Chief Scientist of the National 863 Program. He received his bachelor's and master's degrees from Nankai University, and his PhD degree from the University of Florida in 2009, and worked at the University of Delaware and Lawrence Berkeley National Laboratory. He has been working as a PI in the research group of TIB since 2014, with his main research interests in synthetic biotechnology and genome editing technology. He is now engaged in the creation and utilization of synthetic biology technology methods, genome editing technology development, artificial cell and cell machine design and construction. Several representative synthetic biology and genome editing methods have created a large impact, and hundreds of laboratories at home and abroad have requested the materials and used them. Gene editing technology is revolutionizing research and applications in life sciences. As a researcher driven by the needs of synthetic biology and biomedical applications, he has overcome the bottlenecks in base editing technology and established various broad-spectrum, efficient, and precise base editing techniques. His contributions have advanced the development of gene editing technology and gene editing therapy both internationally and domestically.

P.J. Brooks

Deputy Director, Division of Rare Diseases Research Innovation, National Center for Advancing Translational Sciences (NCATS), National Institutes of Health, USA

Philip J. (P.J.) Brooks represents NCATS in the NIH-wide Gene Therapy Working Group, the Regenerative Medicine Innovation Project and the International Rare Diseases Research Consortium (IRDiRC). He also is the working group co-coordinator for the NIH Common Fund program on Somatic Cell Genome Editing, one of the leaders of the Platform Vector Gene Therapy (PaVe-GT) pilot project and the co-chair of the Bespoke Gene Therapy Consortium.
Brooks received his Ph.D in neurobiology from The University of North Carolina at Chapel Hill. After completing a postdoctoral fellowship at The Rockefeller University, he became an investigator in the NIH intramural program, where he developed an internationally recognized research program focused on rare neurologic diseases resulting from defective DNA repair, including xeroderma pigmentosum, Cockayne syndrome and Fanconi anemia. In May 2022, Dr. Brooks was selected as the recipient of the 2022 Sonia Skarlatos Public Service Award by the American Society of Gene & Cell Therapy for consistently fostering and enhancing the field of gene and cell therapy.

Yujia Cai

Principal Investigator and Assistant Dean, Shanghai Center for Systems Biomedicine, Shanghai Jiao Tong University

Dr. Yuja Cai is a Principal Investigator and Assistant Dean of Shanghai Center for Systems Biomedicine, Shanghai Jiao Tong University. Dr. Cai is the founder and president of BDgene Therapeutics. Dr. Cai studied at Shandong University, Chinese Academy of Sciences, and Karolinska Institutet of Medicine, and received his PhD degree from Aarhus University, Denmark in 2014. He has long been engaged in basic research on gene therapy vectors and gene editing delivery technologies, as well as translational medicine research on gene therapy. He invented China's first original gene therapy vector virus-like VLP technology, initiated China's first in vivo gene editing clinical study and the world's first gene therapy clinical study with VLP vectors. The results have been published as corresponding authors in Nat Biotechnol (2021), Nat Biomed Eng (2021, 2024), Cell Stem Cell (2024 in press), etc. The VLP technology has been authorized by the national patent and realized the application of translation; the CRISPR antiviral therapy HELP has been selected as one of the "Top 10 Advances in Ophthalmology in China" in 2021. He has led or participated in the research of national key R&D projects of the Ministry of Science and Technology, Shanghai "Science and Technology Innovation Action Plan", and general programs of the Natural Science Foundation of China.

Sophelia HS Chan

Clinical Associate Professor, Department of Paediatrics and Adolescent Medicine, School of Clinical Medicine, The University of Hong Kong

Prof. Sophelia HS Chan is a Clinical Associate Professor in Paediatric Neurology at the University of Hong Kong. She graduated from the same university, where she specialized in Paediatrics. After becoming a specialist in Paediatrics, she pursued Developmental Behavioural Paediatrics and Paediatric Neurology as her subspecialties. She has completed her overseas training in Boston at the New England Medical Centre Paediatric Physical Rehabilitation Institute, Boston Children's Hospital Neuromuscular Center, and in London Dubowitz Neuromuscular Centre at Great Ormond Street Hospital for her Clinical and Research Fellowship. Her research focuses on rare neuromuscular disorders and rare neurological diseases, emphasizing treatment improvement, multi-omic diagnosis, and the use of patient-derived stem cells for disease modelling. As the principal investigator of numerous international clinical trials for spinal muscular atrophy and Duchenne muscular dystrophy including gene therapy, Prof. Chan leads the Neuromuscular Disorder Diagnostic and Treatment Program and the SMA Treatment Program in Hong Kong. She has published over 60 articles in peer-reviewed journals, including the European Journal of Neurology, Neurosurgery and Psychiatry, Journal of Neuromuscular Diseases, and Neuromuscular Disorders, GeneReviews, and has been the recipient of multiple research grants including ITF, GRF, HMRF and TDG in past 3 years, and multiple awards. Prof. Chan also serves as the Paediatric Neurology Subspecialty Board Member of the Hong Kong College of Paediatricians, the President of the Hong Kong Society of Neuromuscular Diseases, the Executive Board Member of the Asian Oceanian Myology Centre (AOMC), and the committee member of the World Muscle Society (WMS)'s Inclusion, Equity, and Diversity Committee.

Speaker Profiles

Wai-Yee Chan

Pro-Vice-Chancellor/Vice President, The Chinese University of Hong Kong
Li Ka Shing Professor of Biomedical Sciences, School of Biomedical Sciences, Faculty of Medicine, The Chinese University of Hong Kong

Professor Chan Wai-Yee obtained his BSc (Hon. 1st Class) in Chemistry from The Chinese University of Hong Kong (CUHK) in 1974 and PhD in Biochemistry from the University of Florida in 1977. From 1979 to 2009, he served consecutively as tenured Assistant to Full Professor of Pediatrics, Biochemistry and Molecular Biology at the University of Oklahoma Health Science Center, Oklahoma City, OK, and the Georgetown University Medical Center, Washington, DC, USA. In 2001, he was jointly appointed at the US National Institutes of Health and co-founded the Laboratory of Clinical Genomics. In June 2009, Professor Chan established CUHK's School of Biomedical Sciences and served as the Founding Director and Chair Professor of Biomedical Sciences. He was appointed Master of CW Chu College in January 2017, as Pro-Vice-Chancellor (Strategic Developments)/Vice President of CUHK in August 2018 and Li Ka Shing Professor of Biomedical Sciences in May 2020. He is also Director of Institute for Tissue Engineering and Regenerative Medicine (iTERM) and as Co-Director of a number of Joint Laboratories established between CUHK and Institutes of the Chinese Academy of Sciences and several Mainland universities.
Professor Chan is very active in the scientific community, both locally and internationally. He has served as President of the Association of Chinese Geneticists in America, a Member of the Development Committee of the Society for the Study of Reproduction in the USA, and the immediate past President of Hong Kong Institution of Science. Besides being a Council Member of the Shaw Prize Foundation, he also serves as Member of the Hospital Authority Board, Chair of the Hospital Governing Committee of North District Hospital, Member of the Research Grant Council, Director of the Board of the Hong Kong Genome Institute, and a Specialist for the Hong Kong Council for Accreditation of Academic and Vocational Qualifications.

Li Chen

Professor of Shanghai Medical College, Fudan University

Li Chen, Professor, Shanghai Medical School of Fudan University. Ph.D from UMDNJ in 1995, CUSBEA scholar. Deputy director of CCTMIS.
Years of successful experiences in innovative translational medicine. Participated in the early development of the first targeted anti-tumor drug Sorafenib in Onyx pharmaceuticals, co-initiated a systematic approach for anti-microbial principals in traditional herbal medicines in UCLA, led the oral healthcare products developments in GC Japan, initiated translatiinal studies for mNGS and proposed glycan BioQR hypothesis in Fudan.
Devoted to developing practical solutions for congenital disorders of glycosylation (CDG). Reported the first six cases of NGLY1-CDG in Asia and the first three cases of Mogs1-CDG in China. Proposed the concept of degenerative disorders of glycosylation and trying to build a synergy in R&D between rare disease and aging. Also interested in rare infectious disease, reported the standard whole genome sequence of Elizabethkingia meningoseptica, a conditional pathogen for a rare infection.

Speaker Profiles

Xiangjun Chen

Chief Physician and Professor, Department of Neurology, Huashan Hospital, Fudan University

Dr. Xiangjun "Bill" Chen, MD., Ph.D., is the Professor of Neurology, Huashan Hospital, Fudan University. Dr. Chen obtained Ph.D. of Clinical Medicine (neurology) from Fudan University in 2001. Since September of 2002, he had been appointed as Research Associate (Brian Popko's lab) in the Department of Neurology and the Jack Miller Centre for Peripheral Neuropathy, School of Medicine, the University of Chicago, U.S.A. His project mainly focused on exploring genes closely related to neuromuscular disorders by using forward genetic approaches. From 2007 onwards, he has joined the faculty of the Department of Neurology, Huashan Hospital, Fudan University. Meanwhile he set up the outpatient clinic for ALS/neuropathies and also runs a neuroimmunology laboratory. In 2013, He was promoted to full professor and Chief Neurologist of the department. In 2015, he was appointed to Deputy Director of the Scientific Research Department and Vice Chair of the Institute of Neurology, Fudan University. Dr. Chen has many academic titles, including the Vice chair of Immunology Committee of Chinese Stroke Society, Vice Chair of Rare Diseases subspecialty committee of Shanghai Medical Doctoral Association, Chairman of Neuroimmunology committee of Shanghai Society of Immunology.
Prof. Chen's research mainly focuses on neuroimmunology, peripheral neuropathies and motor neuron diseases (Amyotrophic Lateral Sclerosis, ALS), with special interests in clinical trials and research on: (1) Basic research and clinical trials on autoimmune diseases of nervous system; (2) molecular and genetic studies in ALS and neuropathies. He has been making many advances in the early diagnosis of autoimmune diseases in the nervous system with more than 100 papers published. He leads as Principle Investigator in various Clinical trials of both new medicine and diagnostic assays in Phase 1/2/3. And obtained many research funds from the National Natural Science Foundation and Shanghai Pujiang talent project.

Xiongwen Chen

Dean and Professor, School of Pharmacy, Tianjin Medical University

Xiongwen Chen, professor, doctoral supervisor, national talent, Tianjin level long-term introduction of high-end talent, currently the dean of Tianjin Medical University School of Pharmacy, director of Tianjin Key Laboratory of Clinical Drug Technologyr. He has received the American Heart Association (AHA) Young Scientist Award, the American Heart Association (AHA) Melvin L. Marcus Young Scientist Finalist, the HFSA Young Scientist Award, and the fellow of the American Heart Association. He was a tenured full professor at Temple University School of Medicine. His research focuses on the cellular and molecular mechanisms of heart disease and novel therapies. He presided over 5 NIH projects, 3 National Natural Science Foundation projects, 1 key research and development program sub-project of the Ministry of Science and Technology, 1 project of the Pennsylvania Department of Health, and 2 projects of the National Society of the United States; Participated in 15 NIH projects and 2 horizontal cooperation projects (total funding of 2.4 million yuan). He has in-depth and unique Research on the mechanism, prevention and treatment of heart disease, especially on the mechanism and application of cardiac calcium and β-adrenal signaling, and has published 101 original research papers in Circulation, Circulation Research, PNAS and other journals (total impact factor 1117.9; Among them, the first or corresponding author has a co-authored impact factor of 326.4), 11 reviews (co-authored impact factor of 98.3), 4 monograph chapters and more than 100 abstracts. Google Scholar has been cited 9,016 times, H-index 50, and i10 index 88. He is now a guest reviewer or editorial board member of a variety of internationally renowned academic journals, a fund reviewer of many countries/academic organizations such as China, the United States, Italy, the United Kingdom, France, and has been a research and development consultant for many world-renowned pharmaceutical companies.

Speaker Profiles

Qiang Cheng

Assistant Professor, College of Future Technology, Peking University

Dr. Cheng Qiang is an assistant professor in the Department of Biomedical Engineering, College of Future Technology, Peking University. He has been engaged in the research and development of innovative mRNA nanomedicines for more than 10 years. Using lipid nanoparticles (LNP) as a delivery platform, he is committed to achieving disease treatment and prevention through precise delivery of mRNA, and ultimately solving the unmet clinical needs of mRNA drugs. He has published more than 20 high-level research papers as the first/corresponding author (including co-authors) in journals such as Nature Nanotechnology, Nature Materials, Nature Communications, and PNAS, and has obtained more than 10 international patents. The Selective Organ Targeting (SORT) mRNA-LNP technology he led was highlighted in "Seven Technologies to Watch in 2022" by Nature, and played a key role in assisting biotech company to raise approximately US$400 million for clinical translation of mRNA-based therapy.

Timothy Cote

Former Director of the FDA Office of Orphan Products Development (OOPD)
CEO & Founder of Only Orphans Cote LLC

Dr. Timothy R. Cote, MD MPH, is a renowned national regulatory expert in orphan drug regulatory affairs. He is the founder and CEO at Only Orphans Cote, with offices in Cambridge MA and Washington DC, leading a select team of experts in rare diseases and orphan drug regulatory affairs strategy. As former Director of the Office of Orphan Product Development (OOPD) at FDA, between 2007 – 2011, Dr. Cote led the agency's efforts in implementing the Orphan Drug Act and personally signed decisions on 1400+ orphan drug designation applications.

Jin Ding

Research Fellow, iHuman & Department of Sociological Studies, University of Sheffield

Dr. Jin Ding's expertise encompasses pharmaceutical innovation, industry dynamics, patient engagement and the integration of emerging technology. Since joining University of Sheffield in 2021, Dr. Ding has been working on the Wellcome Trust funded project "Orphan drugs: high prices, access to medicines and the transformation of biopharmaceutical innovation". Her primary focus is to map the evolution and growing industrial development of orphan drugs and analyse the emergence of new orphan business strategies. Additionally, Dr. Ding lead a participatory research project, collaborating with UK rare disease patient organizations to assess the patient accessibility to medicines in the UK. Dr. Ding's research also extends to emerging technologies such as AI-powered drug repurposing and 3D-printed pharmaceuticals.
Motivated by a passion for translating research into policy impact, Dr. Ding participated in a policy note on "3D bioprinting in medicine" for the UK Parliament Office of Science and Technology, and contributed to the technical report "Innovation policy instruments (non-price) for medical innovation" for the World Health Organization.

Speaker Profiles

Dong Dong

Assistant Professor, JC School of Public Health and Primary Care, Faculty of Medicine, The Chinese University of Hong Kong
Research Associate (by courtesy), CUHK Centre for Bioethics, The Chinese University of Hong Kong
Principal Investigator, Rare Disease Real-world Data Lab, Shenzhen Research Institute, The Chinese University of Hong Kong

Dong Dong, PhD, MPhil, adopts an interdisciplinary and multimethodological approach to explore patient-centered care and conduct health services research for underprivileged and underserved populations. Her aim is to bridge theory and practice through community-academic partnerships. Rare diseases are a recurring focus of her research, with a particular emphasis on collecting real-world evidence from the perspectives and experiences of patients. Since 2014, she has employs repeated national cross-sectional study designs to evaluate the multifaceted impact of rare diseases on patients, assessing disease burden, quality of life, social support structures, diagnostic pathways, medication adherence, patient preferences, and other relevant factors. Her research also involves developing and validating measurement tools specifically tailored to capture the health-related quality of life of rare disease patients, enabling a more accurate assessment of treatment outcomes and clinical interventions' effectiveness.
Dr. Dong leads the Rare Disease Real-world Data Lab at CUHK Shenzhen Research Institute. As a Principal Investigator, she has secured major research grants totaling over US$2 million. Among her publications, over one-third of the more than 100 SCIE/SSCI indexed journal articles are related to rare diseases. Additionally, she has published two books and authored over a hundred white paper reports, commentaries, conference proceedings and papers, covering various research areas such as rare diseases, reproductive health, genetic nurturing, health equity, and bioethical issues within the field of Science and Technology Studies.
Furthermore, Dr. Dong serves as a board member of the China Alliance for Rare Diseases, the Illness Challenge Foundation, and the Beijing Aili Myasthenia Gravis Care Center. She is also a member of the Scientific and Medical Advisory Committee of the Hong Kong Alliance of Rare Diseases, as well as an international member of the MGNet Rare Disease Clinical Research Consortium and the Rare Disease Clinical Research Network of the National Institutes of Health in the United States.

Rui Dong

Director of the Research Department, Chief Physician of Pediatric Surgery and Researcher, National Children's Medical Center / Children's Hospital of Fudan University

Dong Rui engages in clinical and basic research in pediatric surgery, with a focus on the origin mechanism, diagnosis and treatment strategies of embryonic-origin diseases in pediatric surgery. He is principal investigator of more than 20 projects, including general programs and youth programs of National Natural Science Foundation of China General, Shanghai Rising-Star Program, and Shanghai Youth Top-notch Talent Program, as well as involvement in sub-projects of national key research and development programs. He has published over 50 SCI papers as first/corresponding author, with a total impact factor exceeding 500 and over 900 citations. He was awarded the third prize of the Chinese Medical Association Scientific Achievement Award, the second prize of the Ministry of Education's Higher Education Science and Technology Progress Award, the third prize of the first Women and Children's Health Science Achievement Award, and the second and third prizes of the Shanghai Medical Science and Technology Progress Award, among other scientific awards. He is recognized as "Shanghai Outstanding Young Physician" and outstanding pediatric specialist; appointed Vice Secretary-General of the Children's Hematologic Malignancy Expert Committee and Secretary-General of the Basic Research Professional Committee of the National Health Commission, as well as Vice Chairman of the Youth Committee of the Children's Oncology Professional Committee of the Chinese Research Hospital Association.

Speaker Profiles

Jia Duan

Group Leader and Investigator, Shanghai Institute of Materia Medica, Chinese Academy of Sciences

Dr. Duan, a Professor at the Shanghai Institute of Materia Medica (SIMM), Chinese Academy of Sciences, earned her pharmacy degree from Wuhan University and completed her Ph.D. at SIMM under Professor H. Eric Xu. Her research focuses on understanding the mechanisms of G protein-coupled receptor signal transduction in metabolic diseases. She has authored more than 10 research papers as the first and corresponding author in esteemed journals including Nature and Nature Communications. Moreover, she has contributed reviews to publications such as Nature Reviews Endocrinology, demonstrating his expertise in the field. Dr. Duan was swiftly promoted to Professor at SIMM because of her impactful work.

Tao Duan

Chief Physician, Professor and Former Director of Shanghai First Maternity and Infant Hospital
Co-founder, Hope for Rare Foundation

Tao Duan, chief physician of Shanghai First Maternal and Infant Hospital. Dr. Duan has engaged in obstetrics and gynecology clinical practice, teaching, scientific research and hospital manage ment for a long time. His main research interests focuses on the prenatal diagnosis of birth defects, fetal medicine and obstetric critical care. He hasstudied intensively in the areas of the prediction and prevention of pre-eclampsia, prenatal screening for chromosomal abnormalities, rapid and non-invasive diagnosis of chromosomal abnormalities and single-gene inherited diseases, He has established China's first academic journal in prenatal diagnosis field named Chinese Journal of Prenatal Diagnosis (Electronic Edition). Dr. Duan is currently a council member of Worlc Congress of Prenatal Medicine, fellow of International Academy of Perinatal Medicine, a council member of the Chinese Congress of Fetal Medicine, former chairman of the Chinese Society of Prenatal Medicine, the chairman of the Shanghai Obstetrics and Gynecology Association and the founder and co-chairman of China DOHaD, etc. He has undertaken many scientific projects such as the Natural Science Foundation of China and the Shanghai Science and Technology Commission's key research projects. He is responsible for the National Natural Science Foundation and major research, he has also published more than 20 SCI papers by the name of the first author or correspondent author.

Fang Fang

Chief Physician and Professor, Department of Neurology, Beijing Children's Hospital, Capital Medical University

Fang Fang, Professor, MD, Chief physician, Department of Neurology, Beijing Children's Hospital, Capital Medical University, National Children's Medical Center. Mainly engaged in the clinical, scientific research and teaching of pediatric nervous system diseases, and pediatric mitochondrial diseases, rare diseases, epilepsy, etc. She is a vice chairman of the Pediatric Neurology Group of the Pediatric Society of the Chinese Medical Association, vice chairman of the Pediatric Neurology Specialty of the Beijing Medical Doctor Association, vice chairman of the Pediatric Neurology group of the Pediatric Society of the Beijing Medical Association, executive director of the Beijing Antiepileptic Association.

Speaker Profiles

Xin-Hua Feng

Qiushi University Professor, Distinguished Investigator and Director of Life Sciences Institute, Zhejiang University

Xin-Hua Feng, Qiushi University Professor of Zhejiang University, is currently a member of the Academic Committee, Special Assistant to the President, Distinguished Investigator and Director of Life Sciences Institute, Zhejiang University. He graduated from Wuhan University, Institute of Genetics, Chinese Academy of Sciences, and University of Maryland, and did postdoctoral training at UCSF. He served as Professor (tenured) at Baylor College of Medicine until October 2009, when he returned to China to take up a position at Zhejiang University.
He has long been engaged in the roles and mechanisms of cell signaling networks and protein modifications in stem cells, organ development, and cancer. He has published more than 190 research papers in international mainstream journals (with a total of 25,555 citations), and enjoys a high academic reputation internationally, especially in the field of TGF-β signaling, which is in the international leading position. He has been awarded Keck Young Scholar, Leukemia & Lymphoma Society Scholar, American Cancer Society Research Scholar, National Science Fund for Distinguished Young Scholars (B), Changjiang Chair Professor. He is also a Fellow of the AAAS, a Specially Appointed Expert of the State, and a Special Expert of Zhejiang Province.

Terence R. Flotte

Professor, Provost, Dean and Executive Deputy Chancellor of the UMass Chan Medical School
President-Elect, American Society of Gene & Cell Therapy

Terence R. Flotte, MD is the Provost, Dean and Executive Deputy Chancellor of the UMass Chan Medical School, Massachusetts' only public medical school. Flotte's own research focus on cutting edge molecular therapeutics led to the first successful gene transfer with adeno-associated virus (AAV) in animals and the first ever human trial of AAV. He led the first human rAAV trials in alpha-1 antitrypsin deficiency and Tay-Sachs Disease, with the latter showing promising clinical benefits. Building on his personal commitment to novel molecular therapies and use of cutting-edge technology for human health benefit, he had exemplary success building research as dean of the UMass Chan Medical School, with impressive advancement of innovative basic and translational programs. His collaborative and imaginative leadership as well as recruitment and development of extremely talented faculty members in research programs, centers and departments in Bioinformatics, RNA Therapeutics, Systems Biology, Gene Therapy and Digital Medicine led to a rise in NIH funding rank from #39 to #21 in the US (Blue Ridge Institute) over fourteen years, an achievement rarely seen in this competitive climate. He has been a thought leader in the AAMC Council of Deans, American Society of Gene and Cell Therapy, multiple NIH committees and as Editor-in-Chief of Human Gene Therapy. Flotte has been instrumental in developing a new molecularly-savvy generation of clinicians, educators and researchers.

Yasuyuki Fukuhara

Chief, Division of Medical Genetics, National Center for Child Health and Development, Japan

Prof. Yasuyuki Fukuhara, M.D., Chief Physician of Division of Medical Genetics, National Center for Child Health and Development, Japan, graduated from Keio University School of Medicine in 1997, and has been working at the National Center for Child Health and Development since 2003, and has been the Chief Physician of Division of Medical Genetics since 2014. He specializes in pediatrics and clinical genetics.

Speaker Profiles

Fen-Biao Gao

Professor and Governor Paul Cellucci Chair in Neuroscience Research, Founding Director of Frontotemporal Dementia Research Center, RNA Therapeutics Institute, UMass Chan Medical School

Dr. Gao is currently a Professor of RNA Therapeutics, a Professor of Neurology, and the Founding Director of Frontotemporal Dementia Research Center at University of Massachusetts Chan Medical School. He holds the Governor Paul Cellucci Chair in Neuroscience Research since 2017. He graduated from Peking University in 1985 and then obtained his Master's degree at Tsinghua University. He received his PhD degree studying neuronal RNA-binding proteins from Duke University in 1995 and then did postdoctoral trainings at University College London (UCL) and University of California San Francisco (UCSF).
He started his own laboratory at the Gladstone Institute of Neurological Disease and Department of Neurology at UCSF in 2000 then moved to UMass Chan in 2010 as a full professor. Dr. Gao has co-organized several international conferences and served on a few journal editorial boards and numerous grant review panels in the US and abroad including multiple study sections at the National Institutes of Health (NIH). He is currently a standing member of the Medical Advisory Council of the Association for Frontotemporal Degeneration, the Research Advisory Council of the Muscular Dystrophy Association, and the King Trust Basic Sciences Review Committee. Dr. Gao was a Sloan Research Fellow in Neuroscience, a Klingenstein Fellow in Neuroscience, and a recipient of McKnight Neuroscience of Brain Disorders Award. In 2018, Dr. Gao received a Jacob Javits Neuroscience Investigator Award from the NIH.

Guangping Gao

Director, Li Weibo Institute for Rare Diseases Research, Director, Horae Gene Therapy Center and Viral Vector Core, Professor at UMass Medical School
Past President of the American Society of Gene & Cell Therapy

Guangping Gao, PhD is the Director, Li Weibo Institute for Rare Diseases Research, Director, Horae Gene Therapy Center and Viral Vector Core, Professor of Microbiology and Physiological Systems, Penelope Booth Rockwell Professor in Biomedical Research, University of Massachusetts Medical School; Elected fellows, both the US National Academy of Inventors (NAI) and American Academy of Microbiology; Past president, American Society of Gene and Cell Therapy.
Dr. Gao has played a key role in the discovery and characterization of new family of adeno-associated virus (AAV) serotypes, which was instrumental in reviving the gene therapy field. Dr. Gao has primarily focused on novel viral vector discovery for in vivo gene delivery, vector biology and manufacturing, large animal modeling, preclinical, translational, clinical gene therapeutics and novel platform technology development for rare disease gene therapy. Dr. Gao has published 370 research papers, 6 book chapters, and 5 edited books. Dr. Gao holds 246 patents with 521 more pending. Dr. Gao had been ranked as the World Top 20 Translational Researchers for several years in a row by Nature Biotechnology.

Speaker Profiles

Steven J. Gray

Associate Professor, Department of Pediatrics, University of Texas Southwestern Medical Center

Dr. Steven Gray received a B.S. degree with honors from Auburn University followed by a Ph.D. in molecular biology from Vanderbilt University and postdoctoral training at the UNC Chapel Hill Gene Therapy Center. He is currently an Associate Professor in the Department of Pediatrics at the University of Texas Southwestern Medical Center. Dr. Gray is the director of the UTSW Viral Vector Facility and maintains affiliations with the Department of Molecular Biology, the Department of Neurology and Neurotherapeutics, the Eugene McDermott Center for Human Growth and Development, and the Hamon Center for Regenerative Science and Medicine at UT Southwestern. Dr. Gray's core expertise is in AAV gene therapy vector engineering, followed by optimizing approaches to deliver a gene to the nervous system. His research focus has also been heavily focused in developing AAV-based treatments for neurological diseases, some of which have translated into clinical trials. He is inventor on over 20 awarded or pending patents and author on over 90 publications related to gene therapy.

Zhenglong Gu

Director of Center for Mitochondrial Genetics and Health, Assistant Director for Greater Bay Area Institute of Precision Medicine (Guangzhou), Distinguished Professor, Fudan University

Prof. Zhenglong Gu, an internationally renowned geneticist, is Director of Center for Mitochondrial Genetics and Health, Assistant Director for Greater Bay Area Institute of Precision Medicine (Guangzhou), and Distinguished Professor, Fudan University, received his B.S. degree from Peking University in 1998 and Ph.D. degree from the University of Chicago in 2003, and joined the faculty of Cornell University in 2006, where he was promoted to tenured full professor in 2018. He has received numerous grants from NIH and NSF, and was honored with the 2011 "Future Leader in Nutrition" award from the International Life Sciences Institute in Washington, D.C. He has been published in many top international journals and journals. He has published more than 60 articles in top international journals, including Nature, Nature Genetics, PNAS, Genome Research, FASEB, MBE, JBC, etc., with more than 4,800 citations.

Speaker Profiles

Min-Xin Guan

Qiushi Distinguished Professor, Professor of Genetics, Director, Institute of Genetics, Zhejiang University
Director, Zhejiang University-University of Toronto Joint Institute of Genetics and Genomic Medicine

Dr. Min-Xin Guan graduated with BS in biology from Hangzhou University (previous and current Zhejiang University) in 1983. He did his postgraduate study at the Australian National University (Ph.D. 1993; Advisor: Professor G. Desmond Clark-Walker). Dr. Guan conducted postdoctoral research in the laboratory of Professor Giuseppe Attardi at the California Institute of Technology (1993-1999). In 1999, he started his independent research as an assistant professor at Cincinnati Children's Hospital Medical Center and the University of Cincinnati, eventually becoming a full professor in Division of Human Genetics, Cincinnati Children's Hospital Medical Center and University of Cincinnati College of Medicine in 2011. Since 2011, he has been joining the faculty at Zhejiang University as the founding Director of Institute of Genetics, Dean of College of Life Sciences (2011-2013), Associate Dean of Faculty of Medicine and Pharmaceutical Sciences (2015-2022).
Dr. Guan's research interests focus on human mitochondrial genetics and biomedicine. Guan's pioneering work with mitochondrial diseases included the discoveries of the mitochondrial cause of maternally inherited nonsyndromic and aminoglycoside induced hearing loss. Dr. Guan's recent pioneering work was the findings how the interactions between mtDNA mutations and nuclear modifiers manifested the deafness and Leber's hereditary optic neuropathy. Currently, Dr. Guan's lab is focusing on investigating the mechanisms underlying the aberrant mitochondrial tRNA metabolisms including the synthesis, processing, maturation, CCA addition, posttranscriptional nucleotide modification and aminoacylation of tRNA, and their impact on human diseases including deafness, optic neuropathy and hypertension. Dr. Guan has published 184 manuscripts on mitochondrial diseases in the high impact journals. Dr. Guan served as the 4th president of Asian Society of Mitochondrial Research and Medicine (2011-2014).

Lanqing Han

Founder & CEO, Cyagen Biosciences (Suzhou) Inc.
Director, Innovation Center for Artificial Intelligence, Research Institute of Tsinghua, Pearl River Delta

Lanqing (Lance) Han, a Tsinghua University alumnus with a Bachelor of Engineering (1988) and a Master of Engineering from McGill University, is a distinguished engineer turned entrepreneur. His academic journey included pivotal research on life sciences imaging equipment at MIT, igniting his passion for biotechnology. In 2006, after acquiring an MBA from Queen's University, Han founded Cyagen Biosciences, where he serves as CEO. Under his leadership, Cyagen has become a leader in providing innovative solutions for life sciences research and drug development. Beyond his corporate role, Han leads a research group at Innovation Center for Artificial Intelligence, Research Institute of Tsinghua, Pearl River Delta in Guangzhou, focusing on medical artificial intelligence and the application of large language models in bioinformatics. His work has earned international acclaim, yielding numerous scientific publications and patents.

Speaker Profiles

Xiaobin He

CEO, Genevoyager (Wuhan) Co., Ltd.

Dr. Xiaobin He, the founder of Genevoyager (Wuhan) Co., Ltd., is a highly accomplished scientist with extensive expertise in gene therapy and neuroscience. He holds a Ph.D. from the Chinese Academy of Sciences (CAS) and previously served as an Associate Researcher at the CAS Brain Research Center of Wuhan. Throughout his career, Dr. He has led and participated in numerous research projects funded by prestigious organizations such as the National Natural Science Foundation of China, the National Science and Technology Major Project of China, the 973 Program, the Brain Project, and the CAS Center for Excellence. In 2014, Dr. He established BrainVTA (Wuhan) Co., Ltd., supported by the "3551 Optics Valley Talent Schema". He has secured over 30 patents related to viral vectors and published over 20 research articles.

Ana Hidalgo-Simon

Associate Professor, reNEW Consortium, Leiden University Medical Center
Former Head of Advanced Therapies at the European Medicines Agency (EMA)

Ana Hidalgo-Simon is an experienced scientist and regulator, currently working at Leiden University Medical Center (LUMC) with the reNEW project. For the past 20 years she has worked in the European Medicines Agency (EMA), holding many key scientific and managerial positions, including establishing and leading the office for Advanced Therapies. Previous positions at EMA include head of the Specialized Scientific Disciplines Department, head of Signal Detection, and head of Risk Management. She has a degree in Medicine and Surgery from the University of the Basque Country, Spain, and a PhD from the University of London, UK. Her current mission is to increase the translation of academic research on Advanced Therapies into clinical practice in a sustainable and fair manner.

Yimin Hua

Distinguished Professor, College of Life Sciences, Nanjing Normal University
Founder, ASOcura Pharmaceuticals

Dr. Yimin Hua is founder of Suzhou ASOcura Pharmaceuticals and Nanjing Antisense Biopharm, focusing on development of novel ASO-mediated RNA processing technologies and RNA-targeted ASO drugs to treat various human diseases, including neurodegenerative diseases, cancer and chronic pain. Tremendous progress has been made in development of ASOs to treat spinocerebellar ataxia and Duchenne muscular dystrophy. Dr. Hua is the main inventor of the first and the only blockbuster ASO drug - SPINRAZA, for treating spinal muscular atrophy, which was approved in USA in December 2016 and in China in February 2019. He has published over 40 papers in well-known professional journals including Nature, which have been cited over 6,600 times. Dr. Hua is now member of board of the Chinese Society of Biochemistry and Molecular Biology, executive member of the Jiangsu Neuroscience Society, member of Tonacea RNA drug club, and leading talent of science and technology of Suzhou Industrious Park.

Speaker Profiles

Guoying Huang

Professor of the National Children's Medical Center / Children's Hospital of Fudan University

Guoying Huang, Professor of Pediatrics, Academic Chief, Pediatric Heart Center, Children's Hospital of Fudan University; Director, Shanghai Key Laboratory of Birth Defects; President, Shanghai Rare Disease Foundation; Vice Chairman, Chinese Medical Association Society of Pediatrics; Vice Chairman, Chinese Medical Association Society of Rare Disease.

Juan Huang

Project Manager of Hope for Rare Foundation; Project Manager of Chinese Organization for Rare Disorders

Juan Huang joined the Chinese Organization for Rare Disorders in 2019, and is responsible for the translation of foreign drug information, scientific research and clinical trial projects. She was responsible for introducing compassionate use of the drug of Progeria. This is the first successful project in which patients in China could use drug from abroad for free. Since 2022, Juan Huang is the project manager of Hope for Rare Foundation, responsible for the research funding project called Good Faith Moves the Mountain. This is a research program launched by Hope For Rare Foundation and aimed to provide personalized treatment to rare disease patients. The research is initiated and financed by patient

Kevin Huang

Co-founder and Secretary-General of Hope for Rare Foundation
Founder and President of Chinese Organization for Rare Disorders

Kevin Huang, a rare disease patient himself, graduated from Zhejiang University City College. He is the founder and president of Chinese Organization for Rare Disorders. He was also the one who brought the "International Rare Disease Day" to China. Through his work, rare disease is now widely known in China. Kevin has also been a champion promoting communications and facilitating
collaborations among various rare disease stakeholders. He founded the China Rare Disease Patient Organization Network; and started the China Rare Disease Summit – the most influential rare disease conference in China. He is the pioneer and practitioner and has become an iconic figure in the field of rare disease in China. In 2021, he initiated and founded the Golden Snail Award, the first award of rare diseases community in China. In 2022, he joins hands with several scientists and entrepreneurs to launch the Hope for Rare Foundation.

Speaker Profiles

Taosheng Huang

Professor of Shanghai Medical College, Fudan University
Director of Institute of Medical Genetics and Genomics, Fudan University
Chairman of Medical Genetics Center, National Children's Medical Center / Children's Hospital of Fudan University

Taosheng Huang, MD, Ph.D is a professor at the Shanghai Medical College of Fudan University, Director of the Institute of Medical Genetics and Genomics at Fudan University, and Chairman of the Genetic Center at the National Children Medical Center/ Children's Hospital of Fudan University. Formerly, he was a professor of Medical Genetics and Pediatrics and Chief of Human Genetics, at the State University of New York at Buffalo, Director of Medical Genetics and Metabolism at the John Oishei Children's Hospital, and Director of Cancer Genetics at the Great Lakes Cancer Center. He was a tenured professor of Genetics and Pediatrics at the University of Cincinnati / Cincinnati Children's Hospital, Center Director of the Mitochondrial Disease, and Director of the Molecular Diagnostic Laboratory. Dr. Huang specializes in mitochondrial diseases, As a licensed physician in both China and the United States, the director of genetic diagnostic laboratories and a tenured Professor, he has extensive experience in clinical medicine, genetic diagnosis, and translational Research.

Yong-Hui Jiang

Professor of Genetics, Neuroscience, and Pediatrics, Chief of Medical Genetics, Director of Yale National Organization of Rare Disorders (NORD) Center of Excellence for Rare Disorders, Yale University School of Medicine

Dr. Yong-Hui Jiang is Professor of Genetics, Neuroscience, and Pediatrics at Yale School of Medicine and Chief of Medical Genetics of Yale New Haven Hospital. Dr. Jiang is a physician scientist and active practicing physician at Yale Children's Hospital and Yale Hospital. He received his MD at Shanghai Medical College of Fudan University with highest honor in China and his PhD in Human and Molecular Genetics at Baylor College of Medicine under the mentorship of Arthur Beaudet. He completed a residency in pediatrics and fellowship in clinical genetics and medical biochemical genetics at the Texas Children's Hospital and Baylor College of Medicine. He is board certified in Clinical Genetics and Medical Biochemical Genetics by American College of Medical Genetics and Genomics. His clinical interest is inborn error of metabolism and genetic evaluation of neurodevelopmental disorder and rare genetic disorders. He is Director of Yale National Organization of Rare Disease (NORD) Center of Excellence for Rare Diseases. Jiang's research program focuses on to investigate molecular and neurological bases of human neurodevelopmental and neurobehavioral disorders with focus on autism spectrum disorder (ASD), Angelman and Prader, Phelan-McDermid, PURA, TUSC3, and HIST1H1E syndromes in human and model organisms. He currently leads two large NIH consortium projects including a $40 million NIH UG3/UH3 project on gene editing for neurogenetic disorders.

Speaker Profiles

Li Jin

Academician of Chinese Academy of Sciences
President of Fudan University
Dean of Shanghai Medical College, Fudan University

Dr. Li Jin, President of Fudan University and Dean of Shanghai Medical College, Director of Human Phenome Institute, Fudan University. He is an Academician of Chinese Academy of Sciences and an external member of Max-Planck Society. He received his PhD from University of Texas (UT) – Health Science Center at Houston. He was a faculty member at UT School of Public Health and Professor at University of Cincinnati College of Medicine before he joined Fudan University in 2003.
Dr. Jin's main research areas encompass genetic structure and migrations of human populations, genetics of human complex diseases and computational biology. To date, he has published more than 900 papers in international academic journals including Nature, Science, Cell, NEJM, JAMA etc. and his papers have been cited 50,000 times. He has served as editor-in-chief and editorial board member of more than 10 international journals, including Phenomics. He has won numerous awards including State Award of Natural Sciences, C.C. Tan Life Science Achievement Award, Distinguished Academic Achievement Award of Human Genome Organization (HUGO), Prize for Scientific and Technological Progress of HoLeung Ho Lee Foundation.

Peng Jin

Professor and Chair, Department of Human Genetics, Emory University School of Medicine
Director of Emory Stephen T. Warren National Fragile X Research Center

Peng Jin, Ph.D., is Robert W. Woodruff Professor of Human Genetics and Chair in the Department of Human Genetics at Emory University School of Medicine. The research program in Dr. Jin's laboratory focuses on understanding the unstable repeats and dynamic DNA/RNA modifications in the human genome and their roles in the pathogenesis of human diseases. Dr. Jin's laboratory has combined various disciplines (genetics, biochemistry, chemistry, human genetics/genomics, and bioinformatics) to understand the molecular pathogenesis of neurodevelopmental and neurodegenerative disorders. Dr. Jin is the recipient of the Beckman Young Investigator Award, Basil O'Connor Scholar Research Award from March of Dimes, Alfred P. Sloan Research Fellow in Neuroscience, NARSAD Independent Investigator Award, Simons Investigator and Fellow of American Association for the Advancement of Science (AAAS). In addition, he is among the Highly Cited Researchers recognized by Web of Science. He also serves as the Director of Emory Stephen T. Warren National Fragile X Research Center.

Xiaowei Jin

Venture Partner, Sherpa Healthcare Partners
Senior Consultant of the Chinese Organization for Rare Disorders

Dr. Xiaowei Jin is currently a venture partner at Sherpa Healthcare Partners. She has over 25 years of extensive experience in global biotech and biopharma industries, spanning from early research and discovery, preclinical and early clinical development, to business development and strategies. Her scientific expertise spans across a wide range of therapeutic areas, including CNS, cardio-metabolic diseases, inflammation and autoimmune diseases, oncology as well as infectious diseases. Dr. Jin is a passionate advocate for the rare disease community, and has been volunteering as a Senior Advisor for the Chinese Organization for Rare Disorders (CORD) since 2015, and she also serves on the board of directors of CORD-US Corporation.

Speaker Profiles

Clara van Karnebeek

Professor, Amsterdam University Medical Centers (UMC)

Professor Clara van Karnebeek works as a full professor and principal investigator at the University of Amsterdam NL, and is affiliated with the University of British Columbia in Vancouver CA. As clinician-scientist in pediatrics and biochemical genetics, she is the director of the Emma Center for Personalized Medicine in Amsterdam UMC. Her international team focuses on early diagnosis and innovative treatment of inherited metabolic diseases in a P4-medicine model. Her international team integrates genomic and metabolomics technologies to unravel the cause of degenerative brain conditions in children and adults, discovering novel genetic conditions and treatment targets. She implements this knowledge in the management of her patients, via experimental therapies and clinical trials with personalized outcomes. Translating new knowledge into expanded newborn screening, as well as useful information and action for the patient and family, using digital applications, are the ultimate goals of her multi-disciplinary team's effort. She is the Director of United for Metabolic Diseases (www.umd.nl), a Dutch consortium uniting all 6 academic metabolic expertise centers and the patient organizations to optimize research and care. She has obtained more than 25M in funding (team and personal grants), and has published over 240 peer-reviewed journal articles, multiple clinical guidelines and chapters in textbooks. She is a dedicated teacher and mentor for clinical and research trainees at different stages. For her contributions to research and clinical care and commitment she has received several (inter-)national awards.

Vladimir L. Katanaev

Full Professor at Department of Cell Physiology and Metabolism, Academic Director, HumanaFly Facility, Faculty of Medicine, University of Geneva
Academician, International Eurasian Academy of Sciences

Vladimir Katanaev graduated from Krasnoyarsk State University and Moscow State University (Pushchino branch), Russia. He received his PhD in 2000 from the Institute of Biochemistry, University of Fribourg. From 2000 to 2005 he worked as postdoct and subsequently as associate research scientist at the Department of Genetics and Development of Columbia University, New York. There he started his research on the Wnt/Frizzled signaling in Drosophila development. He continued to study this signaling cascade in Drosophila and mammalian cells as an independent Group Leader at the University of Konstanz (Germany), where he also completed his Habilitation in 2010. He joined the Department of Pharmacology and Toxicology of the University of Lausanne (Switzerland) in April 2011 as Associate Professor. In October 2018, he joined the Faculty of Medicine of the University of Geneva, where he became a Full Professor at the Department of Cell Physiology and Metabolism, working on the fundamental and translational (in the context of cancer and rare neurological diseases) aspects of Wnt and GPCR signaling pathways, as well as in bionanotechnology.
In parallel to these primary research activities, Prof. Katanaev has research projects at the School of Medicine and Life Sciences, Far Eastern Federal University (Vladivostok, Russia); Kulakov National Medical Research Center of Obstetrics, Gynecology and Perinatology (Moscow, Russia); and Minjiang University (Fuzhou, China). Prof. Katanaev is the Leader of the Working Group "Learning from nature – photonic surfaces in biological objects" of the European Action CA21159 "Understanding interaction light - biological surfaces: possibility for new electronic materials and devices (PhoBioS)" and a recipient of the 3R Prize of the University of Geneva for his work on modeling human diseases in Drosophila. He is a member of the International Eurasian Academy of Sciences and the Academic Director of the HumanaFly Facility dedicated to modeling human diseases in Drosophila.

Speaker Profiles

Fan Lai

Principal Investigator, School of Life Sciences, Yunnan University

Fan Lai, Ph.D, graduated from the University of Edinburgh in 2007. From 2007-2019, Dr. Lai was working in many different centers and Universities in Europe and US. In 2020, he came back to China and joined the Yunnan University in Kunming. His research field is to understand the RNA related mechanisms, including enhancer RNA, transcription and antisense oligonucleiotides (ASO). During his work at IONIS, he published the first mechanism paper about how ASO works in the nucleus, coupling with the transcription (MC, 2020). This paper has been awarded the paper of the year from the Oligonucleotide Therapeutics Society (OTS). Moreover, his work on enhancer RNA, transcription factor etc. has been published on many international journals, like Nature, MC, G&D, NSMB and Science Adv.

Bowen Li

Assistant Professor, Pharmaceutical Sciences, University of Toronto
Canada Research Chair in RNA Vaccines and Therapeutics

Dr. Bowen Li is a tenure-track Assistant Professor in the Leslie Dan Faculty of Pharmacy at the University of Toronto and an Affiliate Scientist at Princess Margaret Cancer Centre. He is also a chairholder of the Canada Research Chair in RNA Vaccines and Therapeutics. Dr. Li received his Ph.D. in Bioengineering from the University of Washington, Seattle, and then completed a Sanofi/Translate Bio Postdoc Fellowship under the guidance of Profs. Robert Langer and Daniel Anderson at MIT. His lab utilizes a range of interdisciplinary strategies, including combinatorial chemistry, high throughput platforms, and AI-driven design of experiments, to develop new generations of delivery systems for RNA medicines including mRNA, circular RNA, and CRISPR-Cas9 gene editing tools. His research has been published in over 40 papers in top-tier journals, such as Nat. Biotechnology, Nat. Materials, Nat. Biomedical Engineering, Nat. Medicine, Sci. Adv., among others, and led to 8 patents as well as a startup company (CynernaX). His pivotal work in mRNA delivery has be recognized by the Marsha Morton Early Career Investigator Award from Cystic Fibrosis Canada, Gairdner Early Career Investigator Award, Canadian Society for Pharmaceutical Sciences Early Career Award, Association of Faculties of Pharmacy of Canada New Investigator Research Award, J.P. Bickell Medical Research Award, Connaught New Researcher Award and Baxter Young Investigator Award.

Dali Li

Researcher, School of Life Sciences, East China Normal University

Dali Li is a professor of biochemistry and molecular biology in School of Life Sciences at East China Normal University. He is also the director of Shanghai Frontiers Science Center of Genome Editing and Cell Therapy. From 1997 to 2007, Dali Li studied in School of Life Sciences at Hunan Normal University and received his B.S and PhD degree. From 2004 to 2007, he worked in Albert B. Alkek Institute of Biosciences and Technology at Texas A&M University Health Science Center as a visiting scholar. In 2007, he became a Lecture at East China Normal University and promoted to full professor in 2014. His research focuses on developing genome editing technology and the applications of CRISPR/Cas system for therapeutics of genetic disorders. His group was one of the first team to report generation of genetically modified mouse and rat disease models through TALEN and CRISPR/Cas technology, and developed several innovative base editors, such as hyper active cytosine base editors (hyCBEs), dual base editors (A&C-BEmax), ABE with minimal bystander editing (ABE9), and a serial of TadA-derived /CGBECBEs (Td-CGBE/CBEs). Based on the advance of Cas9 technology, his group successfully developed strategies to ameliorate several genetic diseases in animal models and demonstrated the clinical application of Cas9 technology to treat β-thalassemia patients.

Speaker Profiles

Eileen Li

Chief Business Officer, RareStone Group
Senior Consultant of the Chinese Organization for Rare Disorders

Eileen Li is currently the Chief Business Officer of RareStone Group, in charge of the corporate strategy, business development, public affairs & market access, and marketing communications. Prior to RareStone Group, Eileen was the principal of IQVIA management consulting and the business leader of rare disease, pricing & market access CoE in IQVIA. She has successfully led the publication of 3 influential reports on rare disease: "China Rare Disease Drug Accessibility 2019", "Rare Disease Healthcare Security in China: City Report 2020 Edition" and "China Rare Disease High Value Drugs Healthcare Security Research Report" , which have laid a solid foundation for the rare disease studies in China. Besides, Eileen has successfully supported several specialty drugs' launch in China, with a special focus on oncology and rare disease drugs. She has accumulated rich experience in the registration & approval, pricing, reimbursement access, and the go-to-market model of specialty drugs. Prior to IQVIA, Eileen worked with Novartis US. HE&OR team.
Eileen is also an active member of several international rare disease organizations. She is currently the senior consultant of the Chinese Organization for Rare Disorders (CORD) and the project consultant of International Rare Disease Research Consortium (IRDiRC). She has obtained her M.S. degree in Social Policy from Columbia University, and her B.S. in Business Administration from the Renmin (People's) University of China.

Linguo Li

Director of Public Policy Research Center of Rare Diseases at the Chinese Organization for Rare Disorders

Linguo Li has collaborated with academic institutions, hospitals, government agencies, industry associations, non-profit organizations, top medical and health enterprises and commercial insurance companies, dedicated to pioneering innovation and accessibility research in the healthcare. She has led and participated in dozens of projects, authoring and publishing numerous healthcare-related research reports, white papers, academic articles, and international conference abstracts. She has chaired several industry summits and round-table discussions involving government officials, scholars, businesses, and patients, providing decision support for market access strategies and innovative product development for enterprises, and offering data support for policy development and public welfare.
She has been invited to serve as a reviewer for the international journal Value in Health and has lectured at School of Management at Fudan University, Steinhardt School at New York University Shanghai, and Shanghai University of Medicine and Health Sciences on multiple occasions. She has led several research projects on the life and disease burden of rare disease patients and innovative drug payment policies, such as "Report on Rare Disease Drug Payment under Common Prosperity in China" , "Solving the Last Mile Problem: A Research on the Availability of Rare Disease Drugs in National Negotiation Drugs" and "Innovative Solutions for Rare Disease Gene Therapy Access and Reimbursement in China" .

Linkang Li

Executive Director of China Alliance for Rare Diseases
Vice President of Chinese Hospital Association

Linkang Li is the Executive Director of China Alliance for Rare Diseases and Vice President of Chinese Hospital Association. Having worked in the health sector for a long time, he has in-depth knowledge and understanding of China's healthcare system and the current situation of primary healthcare. He is familiar with the internal management rules of healthcare organizations, and has participated in and pushed the National Health Commission to introduce a number of measures to prevent and control rare diseases. He has conducted research on the diagn

Speaker Profiles

Weida Li

Chief Scientist, Reg-Verse Therapeutics (Shanghai) Co. Ltd.
Professor, School of Life Sciences and Technology, Tongji University

Dr. Weida Li, Chief Scientist of Reg-Verse Therapeutics, Professor of Translational Medical Center for Stem Cell Therapy and Institute for Regenerative Medicine at Shanghai East Hospital, Frontier Science Center for Stem Cell Research, School of Life Sciences and Technology, Tongji University. His research focuses on cell therapy and novel drug development for diabetes. He has authored a series of papers in prestigious international journals such as Nature Biotechnology, Nature Communications, Science Advances, and Advanced Materials as corresponding (including co-corresponding) or first author. He has applied for several invention patents and collaborates closely with clinical departments to promote the clinical translation of stem cell medicine. As the principal investigator, he has led one project funded by the National Key R&D Program of China, three projects supported by the National Natural Science Foundation of China, two projects sponsored by the Key Project of the Science and Technology Commission of Shanghai Municipality, and an Overseas High-level Young Talents project. He has been honored with prestigious awards such as the Juvenile Diabetes Research Foundation (JDRF) Fellowship. Presently, he serves as a peer reviewer for esteemed journals, including Cell Reports, Journal of Cell Science, Journal of Diabetes, Advanced Science, Cell Research, and Cell Discovery.

Xiao-Jiang Li

Professor, Guangdong-HongKong-Macao Institute of CNS Regeneration, Jinan University
Director, Guangdong Key Laboratory of Non-Human Primate Research
Member, Scientific Innovation Alliance for Rare Diseases, Hope for Rare Foundation

Xiao-Jiang Li is a Professor at Jinan University in Guangzhou, China, and serves as the Director of the Guangdong Key Laboratory of Non-human Primate Research. Li obtained his Ph.D. from Oregon Health Sciences University and completed his postdoctoral training at Johns Hopkins University in the US. From 1996 to 2019, he worked in the Department of Human Genetics at Emory University, where he was promoted to a tenured full professor in 2005 and was honored with the title of Distinguished Professor since 2007. Between 2012 and 2016, he conducted research at the Institute of Genetics and Developmental Biology, Chinese Academy of Sciences, where he utilized CRISPR/Cas9 technology to establish large animal models of human diseases. In 2019, he joined Jinan University on a full-time basis and currently holds a professorship at the Guangdong-Hong Kong-Macau Central Nerve Regeneration Research Institute at Jinan University.
Professor Xiao-Jiang Li is dedicated to studying early nervous system development, aging, and neurodegenerative diseases, employing transgenic disease animal models such as mice, pigs, and monkeys. He is currently focused on elucidating pathogenic mechanisms using genetically modified large animal models. His research findings have been published in over 250 international journals, including reputable publications like "Cell" and "Nature", with cumulative citations exceeding 31300 and an H-index of 91.

Speaker Profiles

Xin Li

Qiushi Distinguished Professor, Executive Director of the Center for RNA Medicine of International School of Medicine, Zhejiang University

Xin Li, Qiushi Distinguished Professor, Executive Director of the Center for RNA Medicine of International School of Medicine, Zhejiang University. He is also the selected candidate of Zhejiang Province Overseas Talent Introduction Program (Innovation Long-term), and the selected candidate of Kunpeng Program in Zhejiang Province. He was an Associated Professor in the University of Rochester before he backed to China in 2023. He had led three NIH and one USDA research projects with a total of about one thousand dollars. His research has been published in more than 20 top journals including Nature Cell Biology, Nature Communications, etc., with an average impact factor of IF>10. He has been invited to give nearly 60 presentations at international conferences and prestigious universities.

Jinzhong Lin

Distinguished Professor, School of Life Sciences, Director, Center for mRNA Drug Research and Development, Fudan University

Dr. Jinzhong Lin earned his Ph.D. in biophysics from the Institute of Biophysics at the Chinese Academy of Sciences. He completed his postdoctoral training at Yale University under the mentorship of Professor Thomas Steitz, a 2009 Nobel laureate in Chemistry. Dr. Lin's research focuses on the ribosome, a crucial molecular machine that synthesizes proteins in cells based on the sequences encoded in messenger RNA. His work has significantly advanced our understanding of the ribosome's assembly and function at a molecular level. Currently, Dr. Lin holds a professorship at Fudan University, where he spearheads research on mRNA translation and regulation. His laboratory is dedicated to the design of mRNA molecules for therapeutic purposes. In 2019, Dr. Lin founded RNACure, a biotech startup dedicated to the development of mRNA-based therapies. His leadership in this venture culminated in the development of China's Covid-19 mRNA vaccine, which has been granted Emergency Use Authorization. This vaccine is expected to be the first mRNA product developed and authorized in China, marking a significant milestone in the country's medical and technological advancement.

Chen Ling

Professor, School of Life Sciences, Fudan University

Chen Ling, Oriental Scholars of Shanghai Universities. In 2007, he graduated with a bachelor's degree from the School of Life Sciences at Fudan University. In 2011, he obtained his Ph.D. in Biomedical Science from the University of Florida and remained at the university, serving as a postdoctoral fellow, assistant professor, and associate professor. Since 2016, he has been serving as a professor at Fudan University. His primary research focuses on the fundamental biology of recombinant adeno-associated virus (rAAV) vectors and the precise induction of gene expression. He serves as an associate editor for "Molecular Therapy-Nucleic Acids". He was awarded the Shanghai Rising-Star Program in 2016 and was appointed as a Special Appointee Professor at Shanghai Universities (Oriental Scholar) in the same year. In 2020, he was successfully reappointed to the Oriental Scholar tracking program. He has led two projects funded by the National Natural Science Foundation of China and participated in two projects under the National Key Research and Development Program of the Ministry of Science and Technology. He has published over 30 SCI papers as a first or corresponding author.

Speaker Profiles

Hekun Liu

Professor, School of Basic Medical Sciences, Fujian Medical University

Hekun Liu, Standing Member of the Chinese Medical Association Branch of Medical Genetics, Chairman of the Fujian Medical Association Branch of Medical Genetics, Executive Deputy Director of the Fujian Key Laboratory of Translational Research on Cancer and Neurodegenerative Diseases, Deputy Director and Secretary-General of the Fujian Collaborative Innovation Center for Stem Cell Translational Medicine, Deputy Chairman of the Chinese Eugenics Association Branch of Gene Diagnosis and Precision Medicine, Director of China Translational Medicine Alliance, Executive director of Fujian Anticancer Association, Executive director of Fujian Cell Biology and Translational Medicine Society, chairman of Human and Medical Genetics Branch of Fujian Genetic Society, editorial board member of Chinese Journal of Medical Genetics, Journal of Fujian Medical University and Chinese Journal of Cell and Stem Cells.

Xiaodong Liu

Principal Investigator, School of Life Sciences, Westlake University

Dr. Xiaodong Liu obtained his Bachelor of Science with Honours from Monash University, Australia, in 2011, and completed his Ph.D. at the same university in 2019. He conducted postdoctoral research in epigenetics and reprogramming at Monash University, the Australian Regenerative Medicine Institute, and the Francis Crick Institute in the UK from 2019 to 2021. Dr. Liu's research focuses on epigenetic regulation of cell fate during induced reprogramming processes. He established single-cell transcriptomic and epigenomic maps from human somatic cells to induced pluripotent stem cells, revealing dynamic regulation of cell fate during reprogramming. His work has been published in 24 academic papers, with 4 patent applications. Recognized as one of the top 10 scientific breakthroughs by Science magazine in 2021, he was also awarded Method of the Year in 2023. Dr. Liu has received fellowships from the European Molecular Biology Organization (EMBO), the Royal Commission for the Exhibition of 1851 in the UK, the Victorian Governor's Award for Medical Research, and the 2023 Alibaba Young Investigator Award, etc.

Xingdi Liu

Vice President, Qiming Venture Partners

Cindy Liu is Vice President at Qiming Venture Partners. She joined in Qiming in 2018 and focuses on therapeutics investment. Prior to joining Qiming, she worked as a management consultant at IQVIA to help pharmaceutical companies and biotechs to understand China healthcare market landscape, do product launch and growth strategy, etc. She hold master degree of Preventive Medicine, School of Public Health from Fudan University.

Speaker Profiles

Andrew W. Lo

Charles E. and Susan T. Harris Professor at the MIT Sloan School of Management
Director of the MIT Laboratory for Financial Engineering
Fellow, American Academy of Arts and Sciences

Andrew W. Lo is the Charles E. and Susan T. Harris Professor at the MIT Sloan School of Management, director of MIT's Laboratory for Financial Engineering, and principal investigator at MIT's Computer Science and Artificial Intelligence Laboratory. His healthcare-related research interests include: new financial engineering tools and business models for drug and device development and healthcare delivery, especially for rare and ultra-rare diseases; statistical methods for incorporating patient preferences into the drug approval process; predicting clinical trial outcomes via machine learning techniques; and novel reimbursement models for creating a robust gene and cell therapy ecosystem. He is a co-founder and director of BridgeBio Pharma, a director of AbCellera, Atomwise, and Vesalius, a co-founder and chairman of QLS Advisors, and a member of the advisory board to the American Cancer Society's BrightEdge Impact Fund and n-Lorem. Dr. Lo received his B.A. in economics from Yale University and his A.M. and Ph.D. in economics from Harvard University.

Harvey F. Lodish

Professor of Biology and Professor of Biological Engineering, Founding member of the Whitehead Institute, Massachusetts Institute of Technology
Member, National Academy of Sciences, US
Fellow, American Academy of Arts and Sciences

Harvey F. Lodish is a Professor of Biology and Professor of Biological Engineering at the Massachusetts Institute of Technology and Founding Member of the Whitehead Institute for Biomedical Research. He is a Member of the National Academy of Sciences, a Fellow of the American Association for the Advancement of Science, a Fellow of the American Academy of Arts and Sciences, and an Associate (Foreign) Member of the European Molecular Biology Organization. During 2004 Dr. Lodish served as President of the American Society for Cell Biology. He received the 2010 Mentoring Award from the American Society of Hematology, the 2016 American Society for Cell Biology WICB Sandra K. Masur Senior Leadership Mentoring Award, the 2020 Metcalf Lifetime Achievement Award from the International Society for Experimental Hematology, and the 2021 Coulter Award for Lifetime Achievement in Hematology from the American Society of Hematology.
He is the lead author of the widely used textbook Molecular Cell Biology. Dr. Lodish is a member of the Board of Trustees of Boston Children's Hospital. He was also Chair of the Scientific Advisory Board of the Massachusetts Life Sciences Center, the group charged with oversight of the state's 10-year, $1 billion investment in the life sciences. Dr. Lodish has been a founder and scientific advisory board member of 12 biotech companies, including Genzyme (acquired by Sanofi for US$20 billion in 2011), Millennium Pharmaceuticals (acquired by Takeda for US$8.8 billion in 2008), and Tevard Biosciences.

Speaker Profiles

Juan Carlos López

Managing Director, Research Grants, RTW Charitable Foundation

Juan Carlos López is Managing Director, Research Grants, at the RTW Charitable Foundation. A native of Oaxaca, Mexico, Juan Carlos obtained his Ph.D. degree from Columbia University (New York) in the laboratory of Nobel Laureate Eric Kandel and carried out postdoctoral research at the Instituto Cajal (Madrid). In 2000, Juan Carlos moved to London to launch the journal Nature Reviews Neuroscience, subsequently becoming its Chief Editor. Four years later, he returned to New York as Chief Editor of the prestigious journal Nature Medicine. In 2014, Juan Carlos joined Hoffmann-La Roche as Head of Academic Relations and Collaborations, leading a team in charge of fostering scientific interactions between the company and academic institutions worldwide. After leaving Roche, Juan Carlos founded Haystack Science, a consulting firm specialized in editorial services and science commercialization. In 2020, Juan Carlos returned to the pharmaceutical industry as Director of Academic Research Collaborations at Bristol Myers Squibb. Throughout his career, Juan Carlos has served on the Boards of multiple organizations in the non-profit and biotechnology sectors, most recently on the Board of Directors of Keystone Symposia.

Daru Lu

Vice Dean and Professor of Institute of Medical Genetoics and Genomics, Fudan University

Daru Lu, Ph.D of Genetics, Distinguished Professor and Vice Dean of Institute of Medical Genetics and Genomics, Fudan university; Director of MOE Gene Technology Engineering Research Center, Director of the Key Laboratory of Birth Defects and Reproductive Health of the National Health Commission; Vice Chairman of the Chinese Genetic Society/Chairman of the Genetic Diagnosis Branch, Executive Director of the Chinese Society of Environmental Mutants; At present, he mainly interested in genetic disease gene screening and molecular mechanisms, gene editing and gene therapy research. He has published over 200 SCI papers cited over 10,000 times, and edited 6 textbooks and works; He has won two national awards, including the second prize of National Technological Invention Award, the second prize of National Natural Science Award, and two National Education Achievement Awards, six first prizes of provincial and ministerial scientific and technological achievements, and won the China Youth Science and Technology Award, the national personnel of the National Hundred and Ten Thousand Talents Project, the special allowance of the State Council, the national outstanding teachers, the national exemplary individual of teacher ethics, the excellent discipline leader of Shanghai, the famous teacher of higher education in Shanghai.

Boxun Lu

Professor, School of Life Sciences, Fudan University

Dr. Boxun Lu is currently a full professor at Fudan University, China. He obtained his Ph.D. degree at the University of Pennsylvania and then became a Presidential Postdoctoral Fellow at Novartis. At the end of 2012, Dr. Lu started his lab at Fudan University, studying Huntington's disease and other neurodegenerative disorders with a focus on degrading the pathogenic proteins for potential treatment for these diseases. He led the studies of ATTECs (Autophagy-tethering compounds) tackling various targets such as polyQ proteins and organelles such as lipid droplets and mitochondria. He published over 50 papers in top-tier journals including Nature, Cell, Neuron, Nature Neuroscience, etc. He has also obtained several awards such as the Newton Advance Fellow from the Academy of Medical Sciences (UK), the Xplorer Prize, the New Cornerstone Investigator, the CC'Tan Award of Life Sciences, etc.

Speaker Profiles

Guangzuo Luo

Professor of China Medical University
Chairman of Nanjing BIONCE Biotechnology Co., Ltd.

Guangzuo Luo, Professor of China Medical University, Chief Scientific Officer of Bionce Biotechnology, mainly engaged in translation and industrialization of gene therapy. He had worked at Children's Hospital of Philadelphia, Spark Therapeutics, and SIO Gene Therapy for many years. He participated in the chemistry, manufacturing, and controls (CMC) of LUXTURNA, the first in vivo gene therapy drug approved by FDA (2017) and BEQVEZ, a gene therapy drug approved by Health Canada (2024). Currently, he focuses on the development of recombinant adeno-associated virus, lentivirus and oncolytic adenovirus vectors for gene therapy of ophthalmology, central nervous system, and malignant solid tumors, including technology development, drug design and efficacy research, process development, large-scale GMP production and quality control, non-clinical research, etc. Recently, he has published many high-quality papers in scientific journals in the field of cell biology and gene therapy.

Sally Ann Lynch

Professor, School of Medicine, University College Dublin
Consultant in Clinical Genetics, Children's Health Ireland at Temple Street & Crumlin

Prof. Sally Ann Lynch research interests are in rare diseases. She is the author of >200 peer-reviewed publications. She has recently published a paper on the cumulative prevalence of rare disorders in Ireland PMID: 35853949, this won best research paper at the Irish hospital awards Dec 2022. Current research includes developing a Clinical genetic specific process map to facilitate identification of risk areas and target controls to make mainstreaming safer. She is also the Principal investigator on a pilot study introducing carrier testing for Galactosemia to the Irish Traveller population, an endogamous minority ethnic group from Ireland.
She developed a microsite, http://www.ucd.ie/medicine/rarediseases/ which contains information for use by Health care professionals on common genetic topics such as consanguinity, inheritance patterns, chromosomal translocations, variant of unknown significance and copy number variation. These videos are awavilable on YouTube and have been uploaded on the Eurogems.org & have had >1.75 million views to date. She is the Irish co-ordinator on the European reference network ERN-ITHACA and one of two Clinical Genetic reps on UEMs. She is a member of IRDiRC diagnostic scientific & the ESHG educational committees. Prof Lynch won the Health Research Charities Ireland inaugural research impact award in December 2022.

Lijia Ma

Principal Investigator, School of Life Sciences, Westlake University
Founder, Westlake Genetech (Hangzhou) Co., Ltd.
Member, Scientific Innovation Alliance for Rare Diseases, Hope for Rare Foundation

Dr. Lijia Ma earned her Ph.D. in bioinformatics in 2009. Since 2018, Dr. Ma leads the Laboratory of Functional Genomics and Gene Therapy at Westlake University. She is also the founder of Westlake Genetech, a biotech startup focused on AI-accelerated gene therapy.

Speaker Profiles

Xiao Mao

Deputy Director of the Academic Office of the National Health Commission's Key Laboratory of Birth Defects Research and Prevention, Hunan Maternal and Child Health Hospital

Xiao Mao, Ph D. in neurology, associate professor. Dr. Mao focuses on genetic research related to neurodevelopmental disorders (NDDs), with a primary emphasis on the identification and pathological mechanisms of novel disease-causing genes. Since 2017, Dr. Mao has discovered and published six new disease-causing genes associated with NDDs, including CDK19, CAPZA2, LMBRD2, MOCS3, MAST3, and MAST4. Notably, CDK19 and CAPZA2 are the first disease-causing genes identified in China for major disorders such as epileptic encephalopathy and isolated intellectual disability.

Ruty Mehrian-Shai

Head, Brain Cancer Molecular Medicine, Sheba Medical Center

Dr. Ruty Mehrian-Shai incorporates the state-of-the-art technologies into her work in order to develop new drugs and apply them to brain cancer translational research. Preclinical studies show promising results for a novel therapeutic target for medulloblastoma pediatric brain cancer with poor prognosis. Dr Shai develops also precise molecular diagnostic and classification tools to be implemented in precision medicine. For example, brain tumor markers that can be quantified in liquid biopsy blood samples to monitor tumor evolution in real time and reduce the need for surgical biopsies for better patient care. Her discovery in adult brain cancer of a new treatment that re-activates the mechanism of self-killing in brain cancer cells is being evaluated in a clinical trial.
Her graduate work was done at the University of Southern California (USC). Subsequently she had postdoctoral training at University of California Los Angeles (UCLA) and served 4 years as an Assistant Professor at USC. During her postdoctoral training she participated at the UCLA/Cedars Sinai intercampus medical genetics program. Later on she participated at the USC/Norris Comprehensive Cancer Center training: Cancer Clinical Trials Design and Statistical Considerations. Dr Shai is also GCP certified to design, conduct and manage clinical trials.

Zhenwei Miao

CEO and President, iMBioRay (Hangzhou) Biomedical Co., Ltd.

Zhenwei Miao, CEO and President of iMBioRay Biomedical. Ph.D from Peking University, postdoctoral fellow from Vanderbilt University, global ADC leader and well-known expert in the field of antibody-coupled NK cells, former Chief Scientist of Enanta and Director of Chemistry Department of Ambrx.
Dr. Miao founded iMBioRay in 2014 to develop antibody-coupled NK cell products with ADC strategy, which is dedicated to solving the clinical unmet needs of oncology and immune diseases, chronic diseases, CNS diseases and other non-oncology diseases, mainly focusing on the global cutting-edge antibody-coupled NK cell therapeutic drugs and cell connector development and application, covering solid tumors, hematological tumors, viral infections diseases and other indications.

Speaker Profiles

Shoukhrat Mitalipov

Professor and Director of the Center for Emrbyonic Cell and Gene Therapy, Oregon Health & Science University

Dr. Shoukhrat Mitalipov is the Director and Professor of the Center for Embryonic Cell and Gene Therapy at Oregon Health & Science University. His research is focused on investigating and developing novel cell and gene therapy approaches in reproductive medicine. His laboratory pioneered the concept of mitochondrial/cytoplasmic replacement therapy, and they are exploring applications of direct reprogramming somatic cells to haploid oocytes as a future infertility therapy to restore reproductive potential of patients. He is also investigating novel gene editing strategies that would allow repairing gene defects in gametes or early preimplantation embryos in order to prevent transmission of heritable genetic disorders.
Dr. Mitalipov earned his MS in Animal Science from Timiriasev Academy and PhD in Stem Cell and Developmental Biology from Research Center for Medical Genetics in Moscow. He completed his postdoctoral fellowship at Utah State University. Among his many commitments, he serves on several NIH study sections (DEV2, DEV1, CMIR) and CIRM GWG and currently is a member of the Board of Directors for the Academy of Sciences of Kazakhstan. Among his many honors, he has received the American Society for Reproductive Medicine Distinguished Researcher Award (awarded for outstanding contributions to clinical or basic research in reproduction) and was identified by Time magazine as one of the 50 most influential people in health care in 2018.

Ramaiah Muthyala

President & CEO, Indian Organization for Rare Diseases
Professor, University of Minnesota

Prof. Ramaiah Muthyala is an associate professor in the Department of Experimental Clinical Pharmacology, an Adjunct Professor in the Department of Medicine, Graduate Faculty, Bioinformatics, and Computational Biology, formerly Senior Assistant Director, Center for Drug Design, and associate director for the Center for Orphan Drug Research, University of Minnesota, USA, spanning 23 years academic research. His current research interests are developing therapeutics for rare diseases, specifically blood disorders, neurological disorders, and drug-resistant infections. Before his academic appointment, he worked in the pharma industry in the USA and India for 22 years in drug discovery and development.
Dr. Muthyala is President & CEO of the Indian Organization for Rare Diseases, a not-for-profit umbrella organization representing 7000 rare diseases and 90 million patients and their families from India. He has raised awareness of rare diseases, advocated public policy, and promoted diagnosis and treatments since 2005. His contributions have been noted in the India Drugs and Cosmetic Act, Ministry of Health and Family Welfare, and Central Drugs Standard Control Organization. He is a member of Rare Diseases International, International Rare Diseases Research Consortium, Asia-Pacific Alliances for Rare Disease Organizations, National Organization for Rare Disorders, USA, and European Organization for Rare Disorders.

Speaker Profiles

Yuyu Niu

Vice President and Professor, Kunming University of Science and Technology
Associate Director, State Key Laboratory of Primate Biomedical Research

Dr. Yuyu Niu is the Vice President of Kunming University of Science and Technology, Associate Director and Professor of State Key Laboratory of Primate Biomedical Research. Research in Dr. Niu's laboratory is focused on early embryo development and cell reprogramming, particularly preimplantation embryo development which plays an important role in cell differentiation and organ initiation and therefore has a predominant relationship with various human diseases. Over the last decade, Dr. Niu has made significant contributions to non-human primate genetic engineering and early embryo development, including 1) the first transgenic rhesus monkey in China (PNAS 2010), 2) the first monkey model with genome engineered by precision gene-targeting methods (Cell 2014; Cell 2017), and 3) the continuous development of monkey embryos in vitro beyond early gastrulation and to 20 days post fertilization (Science 2019).

David Pearce

Chair of International Rare Disease Research Consortium
President of Innovation and Research, Sanford Health

David Pearce is President of Innovation & Research for Sanford Health. He completed his undergraduate Bachelor of Science Degree with honors in biological sciences at Wolverhampton Polytechnic in 1986. He gained his PhD in 1990 at the University of Bath, UK, and did postdoctoral training at the University of Rochester, U.S., and Oxford University, UK.
Dr. Pearce has been researching Neuronal Ceroid Lipofuscinosis (Batten disease) since 1997. His research has led to the first clinical trial for Juvenile Batten disease. He has published over 100 research papers on Batten disease. He also oversees a national registry for rare diseases known as the Coordination of Rare Diseases at Sanford (CoRDS). He has served on numerous National Institutes of Health (NIH) review committees, has organized rare disease workshops for the National Institute for Neurological Disorders and Stroke (NINDS) arm of NIH and is currently the chair of the consortium assembly for the International Rare Diseases Research Consortium (IRDiRC). As President of Innovation & Research at Sanford, he oversees the development of research programs, including more than 450 researchers, eight research centers and more than 300 ongoing clinical trials.

Wim Pinxten

Professor at the Faculty of Health and Life Sciences, Chair of Healthcare and Ethics Group, Hasselt University

Wim Pinxten is professor of medical ethics at Hasselt University (Belgium). He has a degree in religious studies (MA), law (BA), applied ethics (MA), and biomedical sciences (PhD). He is chair of the research group "Healthcare and Ethics" at the Faculty of Medicine and Life Sciences. His research focuses on research ethics, scientific integrity, and ethical issues related to research and healthcare for vulnerable populations. He lectures medical ethics for medical students. He is member of the Belgian Advisory Committee on Bioethics, the Research Ethics Committee and the Committee on Scientific in Integrity of Hasselt University, and vice chair of the Institutional Review Board of the Institute of Tropical Medicine Antwerp. Besides, he serves as a ethics advisor for several international research projects.

Speaker Profiles

Novalia Pishesha

Assistant Professor, Division of Immunology, Boston Children's Hospital and the Department of Pediatrics, Harvard Medical School
Co-founder & CEO, Cerberus Therapeutics

Novalia (Nova) Pishesha earned her PhD in Biological Engineering from MIT in 2018 under the mentorship of Prof. Harvey Lodish, where her research focused on the engineering of red blood cells for the treatment of autoimmune diseases, hyperlipidemia, and the development of biodefense strategies against lethal bioweapons. Following her graduation, Nova was elected a Junior Fellow at the Harvard Society of Fellows and continued her research in the laboratories of Professors Hidde Ploegh, Aviv Regev, and Sangeeta Bhatia at the Boston Children's Hospital, the Broad Institute, and the Koch Institute, respectively. Her work has since revolved around alpaca-derived single domain antibody fragment (nanobody)-based platform to create novel therapeutics for immune modulation, specifically for treating various autoimmune diseases and enhancing vaccine efficacy. In 2022, she co-founded and served as CEO of a biotech company, Cerberus Therapeutics, based on this technology.
Nova has been recognized as one of the 2021 MIT Technology Review Innovators Under 35 for the Asia Pacific region and was listed in The Boston Globe's STAT+ Wunderkinds. She is also a recipient of the National Multiple Sclerosis Foundation Career Transition Award. In January 2024, Nova initiated her own laboratory at the Boston Children's Hospital and Harvard Medical School, where she serves as an Assistant Professor in the Division of Immunology. Her lab is dedicated to advancing the understanding and application of immune engineering to combat pathogenic immunity.

Adam Resnick

Director, Center for Data Driven Discovery in Biomedicine at the Children's Hospital of Philadelphia
Research Professor of Neurosurgery at the Perelman School of Medicine at the University of Pennsylvania

Adam Resnick is the Director of Data Driven Discovery in Biomedicine (D3b) at Children's Hospital of Philadelphia (CHOP) responsible for leading a multidisciplinary team to build and support a scalable, patient-focused healthcare and educational discovery ecosystem on behalf of accelerated discovery and clinical translation for all children. The D3b Center is comprised of a trans-disciplinary team that spans the clinical research unit, biospecimen research unit; molecular diagnostics research unit, pre-clinical research unit, bioinformatics unit, translational imaging unit, and the advanced data applications and platform technologies unit. Together, the Center's resources support the development and implementation of data platforms to accelerate translation on behalf of children.

Speaker Profiles

Emilio J. A. Roldan

Secretary, the Board of the International Conference on Rare Diseases and Orphan Drugs (ICORD)

Emilio J. A. Roldan, got his MD, PhD, and Clinical Pharmacologist degrees at the University of Buenos Aires. As researcher he was trained at the Physicochemical Department, Faculty of Pharmacy & Biochemistry, and then at The Institute of Metabolic Research (IDIM), Buenos Aires. Roldan is author or co-author of several publications and invention patents, receiving national and international awards. Roldan joined several medical and scientific societies at both the national and international fields, being active as speaker and organizing events. As pharmacologist Roldan joined the industry at Parke & Davis; then at Gador SA achieving the position of Scientific Director; and in 2021 become Scientific Director of Qualix DoT LS, an R&D hub devoted to repurposing drugs for unsatisfied rare and neglected conditions. Currently working on ectopic calcifications, rare skeletal conditions, retinoblastoma, and others.
Roldan was early involved with rare diseases (RDs) NGO's activities being part of the 2008 1st Latin-American RDs congress organizing board (2008, Buenos Aires). Then at the board of 2010 ICORD congress at Buenos Aires, first international RDs event in Latin-American. Roldan is a board member of ICORD since 2010. He created the Discoveries and Innovations on Orphan Drugs (D&IOD) meetings aiming to work on the translation of knowledge to the industry in the less developed countries. Today, is the scientific coordinator of CERyDH a private start-up seeking collaboration for RDs projects and events in the Latin-American region.

Jonathan Rothberg

Member, National Academy of Engineering, US
Adjunct Professor of Genetics, Yale University School of Medicine

Jonathan Rothberg earned a B.S. in chemical engineering from Carnegie Mellon and an M.S., M.Phil, and Ph.D. in biology from Yale. Rothberg is a member of the National Academy of Engineering, the Connecticut Academy of Science and Engineering, is a trustee of Carnegie Mellon. and an Adjunct Professor of Genetics at the Yale. He and his wife started the nonprofit Rothberg Institute for Childhood Diseases which works on treatments for tuberous sclerosis, a rare disease that affects one of their children. Jonathan sponsors the Rothberg Catalyzer Prize at four universities.
Jonathan is a scientist and entrepreneur who has dedicated his life to inventing technologies and building companies at the intersection of medicine, engineering, biology, and artificial intelligence. He was awarded the National Medal of Technology and Innovation, the nation's highest honor for technological achievement, by President Obama for inventing and commercializing high-speed DNA sequencing. He is the founder of multiple life science and medical device companies including CuraGen, 454 Life Sciences, Ion Torrent, RainDance Technologies, ClariFI, Butterfly Network, Quantum-Si, Hyperfine, Detect, Liminal Sciences, Identifeye Health, AI Therapeutics, Protein Evolution Inc., and 4Catalyzer. Jonathan Rothberg is focused on founding companies and building technologies to democratize medicine and that will be there to save the lives of the people he loves.

Speaker Profiles

Hirotoshi Sakaguchi

Head, Division of Transplantation and Cellular Therapy, Children's Cancer Center, National Center for Child Health and Development, Japan

Hirotoshi Sakaguchi is the head of the Division of Transplantation and Cellular Therapy of Children's Cancer Center, National Center for Child Health and Development in Japan. Sakaguchi worked at Japanese Red Cross Nagoya First Hospital from April 2005 to March 2009. Then he went to the Department of Pediatrics at Nagoya University Hospital and worked there from April to September of 2009. From October 2009 to September 2010, he worked at the Department of Hematology and Oncology at Children's Medical Center of Japanese Red Cross Nagoya First Hospital. He went back to the Department of Pediatrics at Nagoya University Hospital in October 2010. From October 2013 to the December of 2020, he returned to the Department of Hematology and Oncology at Children's Medical Center of Japanese Red Cross Nagoya First Hospital. Now he is working at Children's Cancer Center at National Center for Child Health and Development since January 2021.

Linda Salem

Principal Data Scientist at F. Hoffmann-La Roche Ltd.

Linda Salem, Pharm D, PhD. Real-World Evidence (RWE) expert with over 10 years experience in international pharmaceutical companies. She has worked on various therapeutic areas, among which several Rare Diseases programs such as Huntington's disease, SMA and DMD, leading RWE generation activities supporting drugs' clinical development plans and post-marketing activities. She is a pharmacist and pharmaco-epidemiologist by training.

S. Pablo Sardi

Global Head, Rare and Neurologic Diseases Research Therapeutic Area, Sanofi

Dr. Pablo Sardi is the Global Head of Rare and Neurologic Diseases Research Therapeutic Area at Sanofi. He leads early exploratory and discovery activities, overseeing a team focused on transformative medicines for patients with rare and neurologic diseases, including rare metabolic, lysosomal, renal, hematologic, skeletal, muscular, and neurologic diseases such as multiple sclerosis, ALS/FTD, Parkinson's disease, and dementias. Dr. Sardi holds a PharmD, an MS in Biochemistry, and a PhD in Pharmacology from the University of Buenos Aires. His research focuses on genetic neurological diseases, leading to discoveries of therapeutic targets and biomarkers that enable the clinical development of therapies. His team has advanced multiple candidates into clinical development for several rare and common diseases, including lysosomal, bone, blood, muscle, and neurological diseases. Dr. Sardi has authored multiple publications and patents and serves on scientific committees and advisory boards, demonstrating his commitment to advancing novel therapeutics.

Speaker Profiles

Daniel Scherman

Director, French Foundation for Rare Diseases
Professor, Université Paris Cité
Head of the Medicine and Life Science Division of the European Academy of Science

Dr. Daniel Scherman holds the position of Exceptional-Class Research Director at the Centre of National Scientific Research (CNRS), situated at the Pharmacy Faculty of Université Paris Cité. He serves as the Director of the Foundation for Rare Diseases and holds the role of Editor-in-Chief of the Rare Disease and Orphan Drug Journal. Dr. Scherman boasts expertise in various fields such as Rare Diseases, Gene therapy, Gene and Drug delivery, Biotherapy, Bioimaging, Neuropharmacology, and Cell Biology. In addition to his primary roles, he holds various additional functions, including being the Head of Medicine and Life Sciences Division of the European Academy of Sciences (EURASC) and a Corresponding Member of the French National Academy of Pharmacy. Furthermore, he serves as the Editor of the "Advanced Textbook on Gene Transfer, Gene Therapy And Genetic Pharmacology" (World Scientific) and has previously contributed as a member of the Scientific Committee of the American Society of Gene & Cell Therapy.

Huaqiong (Joan) Shen

Founder and CEO, NeuShen Therapeutics

Dr. Joan Shen is a physician certified by the American Board of Psychiatry and Neurology. She holds a PhD in life science from Indiana University School of Medicine where she also completed her clinical residency training and three postdoctoral trainings in endocrinology, psychopharmacology, and clinical pharmacology. In the US, she was the clinical lead for CNS drug development at Eli Lilly & Co, Wyeth, and Pfizer, with increasing responsibilities. She is currently a guest professor at Peking University. Perviously she served as an adjunctive professor of Indiana University School of Medicine.
With wealthy global R&D experiences, Joan Shen has a track record of success as a pharma executive. Before founding NeuShen Therapeutics, she served as head of R&D and CEO of I-Mab Biopharma. During her tenure, I-Mab was successfully listed on Nasdaq and formed a global collaboration with AbbVie for a CD-47 compound, Lemzoparlimab. Prior to I-Mab, Joan was the head of development of Janssen Pharmaceutical Company of J&J China where she led to multiple successful NDA/BLA approvals by China NMPA. She also built the largest clinical team among China's domestic pharmas and led clinical trials in Australia, US, and China as the chief medical officer (CMO) of Hengrui Pharmaceutical. Joan was selected by Endpoints News as one of the 20 women in Biopharma R&D in 2021. She was listed in Forbes' Top 50 Women in Tech in China in 2021. She was named "Woman Leader of the Year" by Bay-Helix in 2022.

Weiran Shen

Vice President, OBiO Technology (Shanghai) Corp., Ltd.

Dr. Shen is the vice president of R&D department in OBiO Technology. He leads vector design, innovation, novel vector development, stable and producer cell line selection, in vitro/vivo model construction and improves potency-cost ratio in GCT products by virological and cellular mechanistic designing. Dr. Shen worked as associated director and director in Kriya Therapeutics, Scientist II in Sangamo Therapeutics and group leader in Gene Therapy Program, University of Pennsylvania. He has master of science degree in Neuroscience and doctor degree in Microbiology.

Speaker Profiles

Chengxi Shi

Co-founder and CEO, AstraGenomics

Dr. Chengxi Shi is the co-founder and CEO of AstraGenomics, a biotech company focusing on the development of innovative genome editing technologies. He holds a Ph.D degree in biomedicine with the research topic on epigenetic regulation of chromatin structure.

Yilai Shu

Deputy Dean, Professor and Chief Physician of the Institute of Otolaryngology, Eye & ENT Hospital of Fudan University
Member, Scientific Innovation Alliance for Rare Diseases, Hope for Rare Foundation

Dr. Yilai Shu, Professor, Chief Physician, and Doctoral Supervisor, is a recipient of both National Natural Science Foundation of China for National Science Fund for Distinguished Young Scholars and Excellent Young Scientists Fund Program. Now, he serves as the deputy dean of the Institute of Otolaryngology and director of the Diagnosis and Treatment Center for Hereditary Deafness at Eye & ENT Hospital of Fudan University. He has been honored with numerous accolades, including Shanghai Shu Guang Scholar, Shanghai Pujiang Talent, China Hospital Association Hospital Science and Technology Innovation Award for R&D Innovation, Huaxia Youth Medical Science and Technology Award, Second Prize of Shanghai Medical Science and Technology Award, Excellent May 4th Youth Medal in Shanghai and Shanghai Silver Snake Award. His research team are primarily dedicated to studies on the mechanisms, prevention, and clinical translation of deafness, and has successfully conducted the world's first gene therapy clinical trial for hereditary deafness, achieving a satisfying treatment results with improved hearing threshold, auditory and speech rehabilitation for multiple pediatric patients. This is a significant breakthrough in the field of auditory research.

Chun So

Assistant Investigator, National Institute of Biological Sciences, Beijing
Assistant Professor, Tsinghua Institute of Multidisciplinary Biomedical Research, Tsinghua University
Adjunct Assistant Professor, Department of Obstetrics and Gynaecology, The Chinese University of Hong Kong

Dr. Chun So (Nick) joined the National Institute of Biological Sciences, Beijing as a principal investigator in 2022. He received his B.Sc. (first class honor) in Cell and Molecular Biology from The Chinese University of Hong Kong in 2016 and his Ph.D. (summa cum laude) in Physics of Biological and Complex Systems from Georg-August-Universität Göttingen in 2019. During his studies, he received more than 15 awards and scholarships, including the Croucher Scholarship for Doctoral Study and the Max Planck Croucher Postdoctoral Fellowship. He previously conducted his doctoral and postdoctoral research in Dr. Melina Schuh's lab at the Max Planck Institute for Biophysical Chemistry (now Max Planck Institute for Multidisciplinary Sciences), and published his work on journals such as Science (2019, 2022a, 2022b), Nature Biotechnology, Nature Protocols, Trends in Cell Biology. His work on female meiosis was highly recognized and awarded for the DAMO Academy Young Fellow from Alibaba DAMO Academy, "Innovators Under 35 China" from the MIT Technology Review., the Otto Hahn Medal and the prestigious Otto Hahn Award from the Max Planck Society and the Nikon Young Scientist Award from the German Society for Cell Biology.

Speaker Profiles

Celia Azevedo Soares

Medical Genetics Attending Physician, Unidade Local de Saúde Santo António, Porto

Celia Azevedo Soares MD-PhD is a clinical geneticist working with patients with inherited disorders, who participates in research projects related to Human Genetics and Neurodevelopment. She performed her PhD thesis in the development of the earliest projections from the eye to the brain, and retinal neurogenesis, at the Mason Lab, Columbia University, as a visiting MD/PhD student from Minho University. She also completed a 2-years postgraduation in methods of clinical research with Harvard Medical School – Portugal. She has been an active member of multiple professional societies including the European MD/PhD Association, Portuguese Post Graduate Society, and Young Geneticists Network. Presently, she is a member of the EduComm of the European Society of Human Genetics and the Scientific Committee of the non-profit RD-Portugal.

Liujiang Song

Principal Scientist, AskBio
Adjunct Assistant Professor, University of North Carolina at Chapel Hill

Dr. Liujiang Song is a Principal Scientist at AskBio, a wholly-owned, independently operated subsidiary of Bayer AG. Additionally, she serves as an adjunct assistant professor at UNC at Chapel Hill. Her previous positions include Research Assistant Professor at the UNC and Lecturer at Hunan Normal University. Dr. Song completed a 5-year medical training at Huan University of Chinese Medicine, and she earned a PhD through a joint program between Central South University and the University of Florida, specializing in Adeno-Associated Virus (AAV) vector development. She conducted postdoctoral research at the University of Utah and UNC's Gene Therapy Center, focusing on AAV biology and gene therapy for ocular diseases. She has received honors such as the Distinguished Postdoc Award in Gene Therapy (Pfizer-NCBiotech), Postdoc Award for Research Excellence (UNC), and ASGCT Career Development Award. Dr. Song is a frequent speaker at scientific conferences, including ASGCT annual meetings, and serves as an ad hoc reviewer for journals such as Molecular Therapy-Methods & Clinical Development, Human Gene Therapy, Pharmaceutics, and Genes. Her training and research experience have reinforced her expertise and passion for cell and gene therapy.

Yeyang Su

Anthropologist, Consultant of the Hope for Rare Foundation

Yeyang Su, independent researcher. With educational and research backgrounds in genetics (M.S., China), bioethics (M.A., Erasmus Mundus programme) and social anthropology (Ph.D., the UK), Dr Su has made enquiries into biomedical innovation and policy for nearly two decades. While incorporating diverse users' perspectives and impacts on biomedical innovation, Dr Su gazed at the difference and the distance between biological potentiality and medicinal actuality, and has accordingly, developed the concepts of "toolised medicine" and "tooling work". This pair of concepts shed light on the perceived 'failure' of the 'translational research' model, and call for concretising 'patient-centred' ideal in innovation practices, along with bettering the innovation ecosystem for their sustainable development.

Speaker Profiles

Luming Sun

Chief Physician, Professor, Director of the Department of Fetal Medicine and Executive Director of the Prenatal Diagnostic Center, Shanghai First Maternity and Infant Hospital / Obstetrics and Gynecology Hospital of Tongji University

Luming Sun has completed three years of clinical specialist training in maternal-fetal medicine at the University of Ottawa/ Toronto, Canada, and is certified in obstetrics and gynecology ultrasound and fetal cardiac ultrasound by the American Registry for Diagnostic Medical Sonography (ARDMS). She is a member of the Prenatal Diagnostic Expert Group of the National Health Commission of China, Vice Chairman of the Fetal Medicine Group of the Perinatal Branch of the Chinese Medical Association, Standing Committee of the Birth Defects Prevention and Control Committee of the Chinese Preventive Medicine Association, Member of the Medical Geneticists Branch of the Chinese Medical Doctor Association, Deputy Director of the Birth Defects Prevention Committee of the China Healthy Birth Science Association, Director of the Specialized Committee on Prevention and Control of Birth Defects of the Shanghai Preventive Medicine Association, Deputy Director of the Fetal Medicine and Intrauterine Pediatrics Specialized Committee of the Shanghai Medical Association, Deputy Editor-in-Chief of Journal Maternal-Fetal Medicine, Editorial Director of Chinese Journal of Prenatal Diagnosis.

Weihong Tan

Academician of the Chinese Academy of Sciences
Member of the Academy of Sciences for the Developing World
Director of Hangzhou Institute of Medicine, Chinese Academy of Sciences
Director of the Institute of Molecular Medicine, Shanghai Jiao Tong University

Dr. Weihong Tan is currently a member of the Science and Technology Committee of the Ministry of Education, a member of the Advisory Committee of the Department of Chemistry of the National Natural Science Foundation of China, and a member of the Expert Committee on Nanotechnology of the Ministry of Science and Technology. He is also deputy editor-in-chief of the Journal of the American Chemical Society, J. Am. Chem. Soc., and the deputy editor-in-chief or editor of ACS Nano, Chem Sci and National Science Review. Long-committed to education and research on biochemistry, biochemical analysis and molecular medicine, he is the first in the world to put forward a new concept of nucleic acid aptamer living cell screening. He developed molecular probes and targeted drugs for specific recognition of malignant tumor and other pathological cells, established a variety of new methods and technologies for the diagnosis and treatment of major diseases based on functional nucleic acids, provided new strategies, new tools and new materials for diagnostic medicine. He has published more than 700 scientific papers in global renowned journals such as Science, PNAS and Nature, with the H index at 170. For the tenth consecutive year from 2014 to 2023, he was selected in the Thomson Reuters global list of Highly Cited Researchers. He was awarded second prize in the 2014 & 2020 National Natural Science (first author), the 2018 Ho Leung Ho Lee Foundation Prize, the 2019 Ralph N. Adams Prize for Achievement in Bioanalytical Chemistry, and more than 20 research achievements at home and abroad.

Speaker Profiles

Mei Tian

Professor and Executive Director of the Human Phenome Institute, Fudan University

Prof. Mei Tian, MD., PhD., is a Professor of Diagnostic Imaging and Nuclear Medicine, the Distinguished Professor of Fudan University, and the Executive President of Human Phenome Institute, Fudan University. Prof. Tian's clinical interests include precise diagnosis of pediatric epilepsy, Alzheimer Disease, and metastatic tumors. To advance precise imaging diagnosis, Prof. Tian conducts extensive research focusing on research on human phenome, development of novel molecular imaging technologies including molecular imaging probes, positron emission tomography (PET)-related instrumentation, and artificial intelligence (AI)-assisted imaging acquisition and analysis. Prof. Tian has published more than 100 papers, edited three national textbooks, and one English monograph. She also educates nuclear medicine specialists of the future. More than 40 of her trainees are now nuclear medicine physicians or radiologists in major general hospitals in China. Prof. Tian has earned some honors, including the Distinguished Professorship of Chang Jiang Scholar Program (Ministry of Education of China), and the Outstanding Young Scholar Grant (NSFC, 2), Women in Science Award and Distinguished Young Scientist of China Association of Science and Technology. Prof. Tian is serving as the President of the World Molecular Imaging Society (WMIS), Deputy President of the Chinese Society for Cognitive Science (CSCS), Secretary-General of the International Human Phenome Consortium (IHPC). She is an elected Fellow of the World Molecular Imaging Society (WMIS), Fellow of the Royal Society of Chemistry (RSC). She has long been serving as the Associate Editors or Regional Editor of the most prestigious journals in the field of nuclear medicine and molecular imaging, including the official journals of the Society of Nuclear Medicine and Molecular Imaging, Association of European Society of Nuclear Medicine, Japanese Society of Nuclear Medicine, and the World Molecular Imaging Society.

Haoyi Wang

Deputy Director of the State Key Laboratory of Stem Cell and Reproductive Biology, Leader of the Genetic Engineering Technology Research Group, Institute of Zoology, Chinese Academy of Sciences
Member, Scientific Innovation Alliance for Rare Diseases, Hope for Rare Foundation

Dr. Wang has an interdisciplinary training in genetics, molecular biology, and stem cell biology. As a doctoral and post-doctoral researcher, Dr. Wang has worked on the development of a variety of genome engineering technologies, including transposon-based "Calling Card" method for determining the genome-wide binding locations of transcription factors, TALEN-mediated genome editing in human pluripotent stem cells and mice, CRISPR-mediated multiplexed genome editing in mice, and CRISPR-mediated gene activation in human cells. In 2014, he established his own lab at the Institute of Zoology, Chinese Academy of Sciences. Since then, Wang lab has developed Casilio method to regulate gene transcription, novel TnpB genome editors, as well as human naive pluripotent stem cell model for studying human X chromosome inactivation.

Speaker Profiles

Hong Wang

Principal Investigator, Shenzhen Institute of Advanced Technology, Chinese Academy of Sciences
Member, Scientific Innovation Alliance for Rare Diseases, Hope for Rare Foundation

Dr. Hong Wang received her undergraduate degree from Peking University. She then completed her Master's degree in Medical Neuroscience from the Humboldt University of Berlin. She completed her Ph.D. training at the European Molecular Biology Laboratory (EMBL) and received her Ph.D. degree from the University of Heidelberg. During her postdoctoral research at Heidelberg University, she developed a strong interest in studying the role of the brain in thermoregulation. Since 2017, she has been a group leader at the Shenzhen Institute of Advanced Technology, Chinese Academy of Sciences. Her group has established and characterized the preclinical rat model of Dravet syndrome. Her current work focuses on understanding the molecular mechanisms underlying Dravet syndrome and the development of gene therapies for Dravet syndrome using transgenic rats.

Ji Wang

Executive Director on the Asia Team, OrbiMed

Ji Wang joined OrbiMed in 2020 and is an Executive Director on the Asia team, focuses on venture and growth-stage investment.
Prior to joining OrbiMed, Ji served as an investment Vice President focusing on pharmaceutical, biotechnology and medical service sectors at Hony Capital Hong Kong. Ji received master degrees in both Biotechnology Management and Finance from Johns Hopkins University and University of Virginia, and a B.S in Economics from Boston University.

Jian-She Wang

Director, Center for Pediatric Liver Diseases, National Children's Medical Center / Children's Hospital of Fudan University

Jian-She Wang, Professor, Department of Pediatrics, Shanghai Medical College, Fudan University; Director, Department of Infectious Diseases, National Medical Center for Children, Children's Hospital of Fudan University. He first identified MYO5B, USP53, and ZFYVE19 as the new pathogenic genes of cholestasis, RINT1 as the new pathogenic gene of fever-related recurrent liver failure worldwidely. As first or corresponding author, He published on world-renown journals like Lancet, Hepatology, American Journal of Human Genetics, Journal of Medical Genetics etc.

Speaker Profiles

Jiucun Wang

Professor and Chair of Department of Anthropology and Human Genetics, Fudan University

Dr. Jiucun Wang is professor of Genetics and the Director of the Department of Anthropology and Human Genetics, School of Life Sciences, Fudan University, Shanghai. Dr. Wang has been working on the pathogenesis of systemic sclerosis (SSc), fibrosis from the perspectives of genetics, epigenetics, and molecular mechanism for about 15 years. She has found several susceptible genes and methylated sites, and found FGF6 as a novel gene in the regulation of iron metabolism.
She has published more than 200 peer-reviewed papers. She is leading two major project from National Science Foundation of China (NSFC), one Innovation Fund for Medical Sciences from Chinese Academy of Medical Sciences (CAMS), and one Shanghai Municipal Science and Technology Major Project. Dr. Wang is currently Co-Director of one Innovation Research Unit of CAMS; and the Deputy Director of Institute of Rheumatology, Immunology and Allergy, Fudan University. She serves as the Associate Board Director and Executive Committee Member of the International Network of Scleroderma Clinical Care and Research (InSCAR).

Lei Wang

Professor, Institutes of Biomedical Sciences, Fudan University

Lei Wang Ph.D. is a Professor of Medical Genetics in Fudan University. Wang's research focused on reproductive genetics. He is investigating on genetic basis and molecular mechanism of diseases responsible for female infertility, including oocyte maturation arrest, early embryonic arrest, Polycystic Ovary Syndrome, et al. He was the first to show oocyte maturation arrest (MIM:616780) is a new Mendelian disease and he identified the first and second disease-causing genes (TUBB8 and PATL2) for the disease. Besides, he also identified a few novel genes responsible for other kinds of female infertility. His work incorporate disease pedigree, population cohort, cell model and mouse model to identify pathogenic genes responsible for diseases related to female infertility and its molecular mechanism. The findings will provide molecular biomarkers for quality evaluation of oocytes and embryos, help patients having precision genetic diagnosis and pave the way for the future treatment.

Speaker Profiles

Wei Wang

Professor and Chief Physician, Department of Neurology, Tongji Hospital affiliated to Tongji Medical College of Huazhong University of Science & Technology
Vice President of the Chinese Medical Association
Director of the National Medical Center for Public Health Emergency

Wei Wang, Professor, Chief Physician, Doctoral Supervisor. Recipient of National Science Foundation for Distinguished Young Scholars (2008), Distinguished Professor of Changjiang Scholars (2009), National Health System Young Position Master (1999), Medical Leader of Hubei Province (2013). Former Executive Vice President of Huazhong University of Science & Technology and Party Secretary of Tongji Hospital. He is now the vice president of the Chinese Medical Association, the director of the National Center for Medicine in Serious Public Health Events, the vice president of the Neurology Committee of the Chinese Medical Doctor Association, the editor-in-chief of the Journal of Neural Injury and Functional Reconstruction, the editor-in-chief of the textbook "Neurology" (4th edition).
He has conducted a series of researches on the pathogenesis and treatment strategies of major neurological diseases, and has achieved innovative results. He has published 135 SCI papers in Lancet, BMJ, Science Immunology, PNAS, Lancet Digital Health, Lancet Resp Med, Lancet Public Health, Brain, Ann Neurol, etc., with a cumulative impact factor of 1560 and total citations of 8,098. As the projects leader, he won one second prize of National Natural Science Award, one first prize of Natural Science Award of Ministry of Education, and one first prize of Natural Science of Hubei Province. His research work has been reported by domestic and international media, including People's Daily, Reuters, New York Times, and Agence France-Presse.

Yaning Wang

CEO, Createrna Science & Technology
Former Director of the Division of Pharmacometrics in the Office of Clinical Pharmacology at FDA, US

Dr. Yaning Wang is the CEO of Createrna Science & Technology. He was the Director of the Division of Pharmacometrics in the Office of Clinical Pharmacology at US FDA until September 2021 and oversaw reviews, research projects, and policy development for all disease areas. During his service at FDA, Dr. Wang was involved in the approval of many new drugs and the publication of various guidance. He also received numerous awards, including Award of Merit (highest award at FDA), FDA Outstanding Service Award and many other awards. Before joining FDA, Dr. Wang received his Ph.D. in Pharmaceutics and master's degree in Statistics from the University of Florida in 2003. He also obtained a master's degree in Biochemistry (1999) from National Doping Control Center and a bachelor's degree in Pharmacy (1996) from Peking University in China. Dr. Wang served as a committee member for multiple Ph.D. candidates from various universities. He mentored more than 70 former research fellows (visiting scholars, post-doctoral scholars, and Ph.D. candidates) at FDA. Dr. Wang is a regulatory expert lecturer for three new drug development and regulatory courses. He served as a board member of the International Society of Pharmacometrics (ISoP) and is a fellow of ISoP. He is a member of the Advisory Committee for Chinese Pharmacometrics Society and a member of the Editorial Advisory Board for the Journal of Pharmacokinetics and Pharmacodynamics. Dr. Wang is an Adjunct Professor in multiple universities. Dr. Wang has published over 120 papers and given over 400 presentations at various national and international meetings as an expert in new drug development and regulation. He was selected to be one of the top Healthcare Technology Leaders of Washington, DC for 2023. He is the external advisor for Chinese National Medical Products Administration and Chinese Ministry of Science and Technology. He also serves as a senior consultant to multiple investment companies and banks.

Speaker Profiles

Yi Wang

President of the National Children's Medical Center / Children's Hospital of Fudan University，Director of the Rare Disease Group of the Pediatric Branch of the Chinese Medical Association，Deputy Chairman of the Rare Disease Branch of the Chinese Hospital Association

Yi Wang, chief physician, professor, doctoral supervisor, outstanding academic leader in Shanghai,the chairman of the Pediatric Branch of the Shanghai Medical Association, and the president of the Shanghai Anti-Epilepsy Association. She is committed to the research on the mechanisms and prevention of childhood neurodevelopmental disorders and rare diseases. As the chief scientist of the National Key R&D Program and the head of the special project of the National Health Commission, she has presided over 14 national and provincial scientific research projects, published 211 academic papers, including over 100 SCI papers. She has led the development of 8 clinical guidelines and expert consensus, participated in the compilation of more than 20 books and textbooks. She serves as the deputy editor-in-chief of the journal Intractable Rare Diseases Research, an editorial board member and reviewer of academic journals such as NEJM, MED, Autism, and has presided over/participated in 14 international multicenter clinical trials, responsible for the first IND clinical research of SMA gene therapy in China.

Zhiqiang Wang

Chief Physician and Professor of Neurology Department, The First Affiliated Hospital of Fujian Medical University

Zhiqiang Wang, M.D., Ph.D., is Chief Physician, Professor, and Doctoral Supervisor of Neurology Department, The First Affiliated Hospital of Fujian Medical University, member of the Myopathy Group of the Neurology Branch of the Chinese Medical Association, Deputy Director of the DMD/BMD Study Group of the China Alliance for Rare Disease, Member of the Medical Genetics Branch of Fujian Medical Association, director of the Genetics Society of Fujian, and a high-level talent in Fujian Province. Dr. Wang specializes in the diagnosis and treatment of neuromuscular diseases, peripheral nerve diseases, and neurogenetic diseases. He has accumulated rich experience in neuropathology and molecular diagnostics, with a focus on the diagnosis and pathogenesis of diseases such as muscular dystrophy, metabolic myopathy, and inflammatory myopathy. He has led three projects funded by the National Natural Science Foundation of China and has published over 20 SCI papers as corresponding author or first author in journals such as Neurol, Ann Neurol, The Lancet Regional Health, Acta Neuropathol, and Brain.

Speaker Profiles

Dong Wei

Co-founder, Codone Biotechnology

Dr. Dong Wei is a Co-founder of Codone Biotech, and a seasoned biotech executive with over 20 years of global research & development and management experiences in the industry. He has an MBA from the Wharton School of University of Pennsylvania, a Ph.D. in Microbiology from Michigan State University and a B.S. in Genetics from Peking University. Dr. Wei was CEO of EdiGene, where in four and half years he transformed the company into a clinical-stage biotech with multiple therapeutic gene-editing platforms, obtaining the 1st IND approval and executing the 1st Phase I clinical trial of a gene-editing therapy in China. Before that, he was Chief Operating Officer at Treos Bio, Global Development Leader of late-stage development programs at Shire (now part of Takeda), Senior Director of Business Operation and Program Management at Johnson & Johnson Innovation Center at California, and held various R&D and business positions with increasing responsibilities at global biopharmaceutical companies including BioMarin, Elan, and Janssen, leading a number of clinical-stage programs across therapeutic areas including oncology, immunology, neurodegenerative diseases, and orphan genetic diseases. Prior to that, he was Senior Consultant and Manager at Deloitte Consulting, advising global life science and healthcare clients in Strategy & Operation areas, and worked on oncology drug discovery at Chiron as well as genomic technologies at Applied Biosystems. And he has published many papers and obtained many patents.

Wensheng Wei

Professor of School of Life Sciences, Investigator of Beijing Advanced Innovation Center for Genomics, Investigator of Peking-Tsinghua Center for Life Sciences, Investigator of Biomedical Institute for Pioneering Investigation via Convergence, Peking University

Wensheng Wei, Professor of Biomedical Pioneering Innovation Center, Peking-Tsinghua Center for Life Sciences, and the School of Life Sciences at Peking University, the director of Peking University Genome Editing Research Center, and the lead scientist of Changping Laboratory. The research of Wei group is mainly focused on the development of eukaryotic gene editing tools with the emphasis on high-throughput functional genomics, gene and cell therapy, and the novel vaccination approach based on circular RNAs.

Jing-Ke Weng

Inaugural Director, Institute for Plant-Human Interface, Professor of Chemistry, Chemical Biology, and Bioengineering, Northeastern University
Member of Steering Committee, Asian Young Scientist Fellowship

Jing-Ke Weng received his B.S. (2003) in Biotechnology from Zhejiang University. He received his Ph.D. (2009) in Biochemistry from Purdue University, and was a pioneer postdoctoral fellow at the Salk Institute and HHMI between 2009 and 2013. He was a member of the Whitehead Institute, and an Assistant and Associate Professor of Biology at MIT between 2013 and 2023. Currently, he is the Inaugural Director of the Institute for Plant-Human Interface, and a Professor of Chemistry, Chemical Biology, and Bioengineering at Northeastern University. Dr. Weng's research focuses on understanding the origin and evolution of plant specialized metabolism, and how to harness plant biochemistry to advance human health and sustainability on planet Earth. Dr. Weng has won numerous awards in his career, including Smith Family Foundation Odyssey Award (2018), Scialog Fellow (2018), Beckman Young Investigator Award (2016), Alfred P. Sloan Research Fellow (2016), Searle Scholar (2015), Pew Scholar in the Biomedical Sciences (2014), American Society of Plant Biologists Early Career Award (2014), and Tansley Medal for Excellence in Plant Science (2013).

Speaker Profiles

Fenglan Wu

Co-founder and CEO, Gritgen Therapeutics Co., Ltd.

Fenglan Wu, Profound industrial and financial background in the pharmaceutical field. 15 years of compound experience in pharmaceutical R&D, company operation, and financial marketing. Formerly served as the research scientist at GSK, senior scientist at Zai Lab, and vice president of China Renaissance Investment Group. Ph.D. of Pharmacy from Shanghai Jiao Tong University, Master of Immunology from Shanghai Jiao Tong University, Bachelor of Pharmacy from Tongji Medical College.

Hongguang Xia

Professor and Vice Director, Liangzhu Laboratory

Professor Hongguang Xia has made important breakthroughs and progress in the field of mitophagy by focusing on the function and mechanism of mitophagy regulating of diseases.He led the development of mitophagy inducer candidate TJ0113, which has received clinical approval from the Chinese NMPA and the U.S. FDA, and has completed the phase Ⅰ clinical study with positive clinical results. The company he incubated has completed four rounds of financing, with an amount of more than 200 million yuan. Professor Hongguang Xia has published more than 50 high-level papers such as Cell, presided over 5 projects of the National Natural Science Foundation and 3 provincial major scientific research projects, with a total funding of more than 28 million yuan. He was appointed as "Distinguished Expert of Zhejiang Province" and supported by "National Science Fund for Excellent Young Scholars" and " Zhejiang Province for Distinguished Young Scholars".

Qiang Xia

Academician, Chinese Academy of Engineering
Dean, Chief Physician, Professor, Renji Hospital, Shanghai Jiao Tong University School of Medicine

Dr. Xia Qiang, Academician of the Chinese Academy of Engineering, is the president of Renji Hospital, affiliated with Shanghai Jiao Tong University School of Medicine. He is also the director of the Shanghai Organ Transplantation Research Institute, director of the Shanghai Organ Transplantation and Immunology Engineering Technology Research Center, leader of liver surgery, chief physician, professor, doctoral supervisor, Shanghai science and technology elite, Shanghai leading figure, and outstanding discipline leader in Shanghai. Currently, he serves as a member of the Children's Committee of the International Liver Transplantation Society, standing committee member of the Organ Transplantation Physician Branch of the Chinese Medical Doctor Association, chairman of the Children's Organ Transplantation Professional Committee, leader of the Children's Transplantation Study Group of the Organ Transplantation Branch of the Chinese Medical Association, and chairman of the Organ Transplantation Division of the Shanghai Medical Association. He has hosted 21 projects, including the National Key R&D Program and National Natural Science Foundation Major Research Plan, and serves as the editor-in-chief of Translational Research. He has published more than 170 papers as the corresponding author in internationally authoritative journals such as Cell, Gut, Gastroenterology, Journal of Hepatology, and Hepatology. As the first principal investigator, he has won one second prize of the National Science and Technology Progress Award and three first prizes at the provincial and ministerial levels.

Speaker Profiles

Xiao Xiao

Co-founder, Chairman & CSO, Belief BioMed Group
Co-founder, Hope for Rare Foundation

Dr. Xiao Xiao has nearly 40 years of experience in the development and clinical transformation of virus vectors and gene therapy drugs. He has achieved remarkable scientific research results with broad adoption in the field of gene delivery vectors, including but not limited to the invention of the "three-plasmid transfection" technology for producing recombinant adeno-associated viruses (rAAVs), the establishment of production system for clinical batch rAAV, and the development of tissue-targeted vectors (Capsid engineering). With extensive expertise and practical experience, he has been dedicated to gene therapy research in multiple therapeutic areas. The gene therapy drugs for duchenne muscular dystrophy (DMD) and limb girdlemuscular dystrophy (LGMD2i) developed by him have been clinically transformed by Pfizer and Bayer respectively. He has led over 20 scientific research projects, published over 200 academic papers, and his national and international invention patentapplications totaled over 100.
In 2018, Dr. Xiao Xiao co-founded Belief BioMed, a high-tech company that engages inthe research and development, production and clinical application of gene therapy products. The company's pipeline is widely distributed in the fields of rare and common diseases. In April 2023, the company reached a key milestone of dosing completion for all subjects in its Phase III registrational trial for BBM-H901, a gene therapy drug independently developed and produced by the company and expected to become the first AAV gene therapy to be approved for treating hemophilia B in China. The clinical study results of BBM-H901 have been published in two prestigious international journals including The Lancet Haematology and New England Journal of Medicine, and BBM-H901 has received breakthrough therapy designation from China CDE and orphan drug designation from the U.S. FDA.

Hong Xu

Professor and Former Secretary of the Party Committee of the National Children's Medical Center / Children's Hospital of Fudan University

Hong Xu, MD, PhD is Professor of Pediatrics, Children's Hospital of Fudan University. She is the Chair of Chinese Pediatric Nephrologist Association, Chair of Rare Disease Specialized Committee of Shanghai Medical Doctor Association and Vice Chair of Nephrology Society of Shanghai Medical Association. She also serves as the Director of Shanghai Renal Development and Pediatric Nephrology Institution, former council member of International Pediatric Nephrology Association (IPNA), Chief of IPNA Fellowship Training Center (Shanghai China).
Dr. Xu received her M.D in 1984 and PhD in 1991 at Shanghai Medical School of Fudan University. She performed her post doctor program at School of Medicine, Kobe University, Japan. Her research interests include the integration management program for CKD children, developing renal replacement therapy program for ESKD children in China, genetics and pathogenesis of steroid resistant nephrotic syndrome and CAKUT, clinical treatment of nephrotic syndrome, lupus, enuresis, and so on. Her Basic Researches on "ANGPTL3 and proteinuria" , "New genes and mechanism of kidney malformation" and "Prediction model and AI of pediatric kidney disease" are funded by the Chinese National Natural Science Funds and National Key Research and Development Program. She conducted numerous collaboration studies and workshops on urine screening, CAKUT, nephrotic syndrome, pediatric dialysis and enuresis. She was also the President of the 16th Congress of International Pediatric Nephrology Association in August 2013 and got the First Prize of 2021 Shanghai Medical Science and Technology Award.

Speaker Profiles

Lei Xu

Director of Cell Process Development Department, OBiO Technology (Shanghai) Corp., Ltd.

Lei Xu, Director of Cell Process Development Department, OBiO Technology. This department focus on the development of processes for CAR-T, U-CART, CAR-NK, γδT, Treg, iPSCs, primary stem cells, and other terminally differentiated cells. She has 5 years of experience in CAR-T project process development, having worked at JW and Fosun Kite's process development department. She has led the process development, IND filing, IIT production, and participated in the BLA filing and post-marketing changes for CAR-T projects. During studies at East China University of Science and Technology, she gained seven years of experience in basic research on mesenchymal stem cells. Studied on "Isolation and Tri-lineage Differentiation Identification of Stem Cells, Isolation and Dedifferentiation Studies of Chondrocytes" and "Co-culture and Mechanism Research of Stem Cells and Chondrocytes", and published four SCI articles as the first author.

Pinglong Xu

Qiushi Distinguished Professor, Zhejiang University, Professor, Life Sciences Institute, Zhejiang University, Director of Intelligent Medicine Institute of ZJU-Hangzhou Global Scientific and Technological Innovation Center
Member, Scientific Innovation Alliance for Rare Diseases, Hope for Rare Foundation

Dr. Pinglong Xu graduated with a Bachelor in Biochemistry from Sichuan University in 1998 and obtained his Ph.D. in Biochemistry & Molecular Biology from the Shanghai Institute of Biochemistry and Cell Biology in 2003. He pursued postdoctoral research at the University of California, San Francisco (UCSF) from 2004 to 2010, under supervision by Dr. Rik Derynck, one of the founders of TGF-β signaling. In 2011, he was appointed as an assistant research professor at UCSF and joined Zhejiang University in 2013.
Dr. Xu's lab employs multidisciplinary approaches to systematically elucidate the signaling mechanisms, biological functions, disease implications, and intervention strategies of nucleic acid sensing, an innate immune system. Dr. Xu identified the cGAS-STING-PERK signaling pathway, proposed that nucleic acid sensing activation is a universal mechanism leading to lysosomal storage disorders, discovered biological functions of nucleic acid sensing related to protein synthesis, neuronal loss, liquid-liquid phase separation, mitochondrial dynamics, and cellular transdifferentiation, and revealed key mechanisms by which nucleic acid sensing induces cellular senescence and fibrosis in the lungs, kidneys, and liver, and advanced theories on how oncogenes, blood glucose levels, nutritional microenvironments, and phase separation control innate immune responses. His achievements have been published as the corresponding author in prestigious journals such as Nat Cell Biol (4), Mol Cell (4), Cell Host Microbe, Genes Dev, PLoS Biol, and Sci Adv. Dr. Xu received funding from the National Science Fund for Distinguished Young Scholars in 2017 and for the Innovative Research Group Project in 2023. Dr. Xu has been honored with the Gu Xiaocheng Lectureship, the Promega Innovation Award in Cell Biology, and the Qiushi Distinguished Professorship at Zhejiang University.

Speaker Profiles

Yifeng Xu

Director, Brain Health Institute at National Center for Mental Disorder
Expert of Academic Committee, Tianqiao & Chrissy Chen Institute's UniCaseHub Community

Dr. Yifeng is a distinguished psychiatrist, scholar, and leader in the field of mental health in China. He is the former president of Shanghai Mental Health Center (2011-2021) and now serves as honorary dean of the Institute of Psychology and Behavioral Science, Shanghai Jiao Tong University, director of WHO/Shanghai Collaborating Center for Research and Training on Mental Health, and dean of Institute of Mental Health, Fudan University. His academic interests are social psychiatry, health management, and global mental health. He has made great achievements in China's mental health service reform, as well as the local reform in Shanghai, and is one of the chief architects of the "686 Project". He is the head of the Department of Psychiatry, and a member of the Shanghai Jiao Tong University Academic Committee, as well as the Academic Degree Evaluation Committee. In addition, he is the Editor-in-Chief of General Psychiatry and the past Chairman of the Chinese Psychiatrist Association. Dr. Xu maintains active basic and applied research programs, with interests centered on the clinical and environmental determinants of major psychoses, especially schizophrenia and depression. He has participated in multiple national and international research programs as a principal investigator. He has published more than 180 peer-reviewed journal articles, and edited/translated dozens of books, textbooks, and monographs. His work has appeared in the Social Science & Medicine, Progress in Neuro-Psychopharmacology and Biological Psychiatry, BMC Medical Ethics, Journal of Affective Disorders, PLoS One, British Journal of Psychiatry, World Journal of Biological Psychiatry, Psychiatry Research, Nature Genetics, Schizophrenia Research, and Lancet Psychiatry.

Tian Xu

Co-founder, Hope for Rare Foundation
Chair Professor of Genetics and Vice President, Westlake University

Prof. Tian Xu received his B.S. degree from Fudan University in 1982 and Ph.D. degree from Yale University in 1990. He was a postdoctoral fellow at University of California, Berkeley from 1990 to 1993. From 1993 to 2018, he was an Assistant Professor, Associate Professor (tenured in 2001), C.N.H. LONG Professor of Genetics at Yale University. Prof. Xu also served as the Vice Chairman of the Department of Genetics at Yale from 2003 to 2018 and the Special Advisor to President of Yale University from 2002 to 2013. He was also an Investigator of the Howard Hughes Medical Institute from 1997 to 2018. Prof. Xu joined Westlake University as a Chair Professor of Genetics and Vice President in April, 2018.
Prof. Xu's work mainly focuses on mechanism of growth control and new genetic and biotech methods. He pioneered genetic dissection of growth control and identified all the key growth regulators and pathways including PTEN/TSC/mTor and Lats/Hippo. His studies have not only elucidated fundamental principles of developmental biology, but also revealed pathogenic mechanisms and contributed to the development of multiple drugs. Xu has developed multiple widely used genetic methods including mammalian piggyBac transposon and mosaic analysis. He serves and served as the Editorial Board Member of multiple journals including Cell, Annual Review of Genetics, Molecular Cancer Research, Integrative Biology and The International Journal of Biological Sciences, and is the Founding Editor and the Editorial Board Member of Disease Models and Mechanisms.

Speaker Profiles

Li Yang

Distinguished Principal Investigator, Fudan University

Li Yang, PhD, Distinguished Principal Investigator, Fudan University. He focuses on developing computational pipelines, together with deep-sequencing technologies and diverse biochemical methods, to decode expression, function and potential application of long noncoding RNAs, including circular RNAs, and on inventing and applying the-state-of-the-art base editing technologies to recode genetic information for basic research and gene therapeutics. He has published over 120 peer-reviewed articles including Cell, Nat Biotechnol, Genome Res and etc, with Google Scholar citation > 23,000 and H-index=57. He is one of Most Cited Chinese Researchers in four continuous years from 2020 to 2023 by Elsevier and one of Highly Cited Researchers from 2022 to 2023 by Clarivate.

Taihua Yang

Liver Surgeon, Renji Hospital, Shanghai Jiaotong University School of Medicine

Taihua Yang, Liver surgeon, postdoctoral. During his PhD and postdoctoral period, he has been devoted to the development of gene therapy strategies for chronic liver disease, under supervisor: Prof. Michael Ott & PD. Dr. Amar Deep Sharma; successfully developed HNF4A-based liver fibrosis protein replacement therapy using mRNA technology, published as independent first author in Journal of Hepatology in 2021. In terms of delivery system, 2 national invention patents have been applied for. Yang was selected into the "Super Postdoctoral Program" of Shanghai City, presided over 1 project of Shanghai City Qiming Star (Sailing Program), 1 project of Postdoctoral Program and 1 project of National Natural Science Foundation of China.

Yang Yang

Researcher, State Key Laboratory of Biotherapy, West China Hospital, Sichuan University
General Manager, Chengdu Jinweike Biotechnology Co., Ltd.

Yang Yang, PhD, is a Professor and doctoral supervisor at the National Key Laboratory of Biotherapy, Sichuan University. He engaged in research and clinical translation of gene therapy for rare genetic diseases. Dr. Yang has published over 60 papers in journals such as Nat Biotech, Blood, Sci Adv and STTT. He is a Co-founder and general manager of Chengdu Genevector Biotechnology Co., Ltd.

Speaker Profiles

Boya Yu

Project Manager of Hope for Rare Foundation

Boya Yu, Project Manager of Hope for Rare Foundation. He is in charge of the Research Grants for Rare Diseases, the International Communication Scholarship, and the Funding for Rare Disease Book Publishing at the Foundation, and is responsible for program planning and speaker invitations at the GRDRS2024 conference. He studied at Wuhan University (Pharmacology), Sun Yat-sen University (Psychology), and California Institute of Technology (Neuroscience), and has a broad background in biology and medicine. He joined the Hope for Rare Foundation in 2022 due to his longstanding interest in public welfare and recognition of the value of advancing rare diseases research.
He is also a popular science author and translator. In the course of more than ten years engaged in science communication, he has published articles in the media such as "DUKU", "Southern Weekend", "Beijing News" and social media, accumulating more than a million words; and translated several well-known popular science books, including "In Search of Memory", "Survival of the Friendliest", "The Selfish Ape", "The Disordered Mind", and "Breaking the Age Code", accumulating more than a million words. He is committed to enhancing the scientific literacy and critical thinking skills of Chinese readers.

Feng Zhang

Professor, Institute of Medical Genetics and Genomics, Fudan University

Dr. Feng Zhang is currently Professor of Genetics at the Institute of Medical Genetics and Genomics, Fudan University. He also serves as the Deputy Director of State Key Laboratory of Genetic Engineering (China). During 2007-2009, Dr. Zhang was a postdoctoral associate in the Lupski lab at Baylor College of Medicine, where he studied the mutational mechanisms of copy number variation in the human genome. In the end of 2009, Dr. Zhang established his own research group at Fudan University and focused his researches on the genetic variants in human infertility (e.g., asthenoteratospermia, premature ovarian insufficiency) and birth defects (e.g., congenital scoliosis). Dr. Zhang has published more than 180 papers in scientific journals, including the selected papers in New England Journal of Medicine, Nature Medicine, Nature Genetics, Cell Research, Nature Communications, Science Advances, American Journal of Human Genetics, Human Molecular Genetics and etc. Dr. Zhang has been awarded many honors, including the Changjiang Scholar (Ministry of Education of China, 2019), the Outstanding Young Scholar (National Natural Science Foundation of China, 2016), and the Outstanding Achievements Award of WuXi PharmaTech Life Science and Chemistry Awards (2015).

Mel Zhang

Project Director of Hope for Rare Foundation

Mel Zhang, Project Director of Hope for Rare Foundation. Working in the field of social innovation for over fifteen years, previously employed at a science communication institution. She joined the Hope for Rare Foundation full-time in 2022, is fully responsible for the management and operation of projects including research funding, industry promotion, and institutional fundraising.

Speaker Profiles

Shuyang Zhang

Chief Physician, Professor and President of Peking Union Medical College Hospital
Chair of the Chinese Society of Rare Diseases of the Chinese Medical Association
Chair of the National Expert Committee on Rare Diseases Clinical Care and Accessibility of the National Health Commission

Dr. Shuyang Zhang is the President of Peking Union Medical College Hospital (PUMCH) and the Deputy Secretary of the PUMCH Party Committee. She also serves as the Vice President of Chinese Academy of Medical Sciences & Peking Union Medical College (CAMS&PUMC), and the Member of the Standing Committee of CAMS&PUMC Party Committee. Dr. Zhang is a chief physician, professor, and doctoral supervisor of internal medicine. She mainly engages in basic and clinical research and new drug development of cardiovascular diseases and rare diseases. She is a representative to the 14th National People's Congress. She also serves as the Standing Committee Member of the Chinese Medical Association, the Chair of the Chinese Society of Rare Diseases of CMA, the Chair of the National Expert Committee on Rare Diseases Clinical Care and Accessibility of the National Health Commission, and the Chair of the National Expert Committee on the Drug Administration and Pharmacotherapeutics of the National Health Commission. She leads 16 national and provincial-level scientific research projects, including the Key R&D Program of the Ministry of Science and Technology. She edited the planning textbook "Rare Diseases" and 11 monographs, and presided over the formulation of the "Guidelines for the Diagnosis and Treatment of Rare Diseases (2019 Edition)" and 8 guidelines for special diseases.

Xue Zhang

Academician of Chinese Academy of Engineering
Dean of Department of Medical Genetics, Institute of Basic Medical Sciences, Chinese Academy of Medical Sciences

Xue Zhang, Professor of Medical Genetics, Doctoral Supervisor, Academician of Chinese Academy of Engineering, Member of Chinese Academy of Medical Sciences. Currently, he is the Party Secretary of Harbin Medical University and the President of Heilongjiang Academy of Medical Sciences. He received his undergraduate degree in Clinical Medicine from China Medical University in 1986, Master's Degree in Genetics from China Medical University in 1989, and Ph.D. degree in Cell Biology from China Medical University in 1994. He was appointed as the Vice President of the Chinese Academy of Medical Sciences and Peking Union Medical College in April 2017. He was appointed as the President and Deputy Party Secretary of Harbin Medical University and the President of Heilongjiang Academy of Medical Sciences in September 2018. In December 2022, he became Party Secretary of Harbin Medical University.
He is mainly engaged in the molecular genetics of single-gene and genomic diseases, and has discovered the causative genes of single-gene diseases such as familial acne inversa and the causative DNA rearrangements of genomic diseases such as congenital generalized hypertrichosis, and revealed two new mechanisms of gene repressive upstream open reading frame-causing mutations and palindrome-mediated interchromosomal insertions, and published a series of papers and articles in Science, Nature Genetics, and Am J Hum Genet, etc.

Speaker Profiles

Albert Zheng

Principal Investigator, Institute of Zoology, Chinese Academy of Sciences
Principal Investigator, Beijing Institute of Stem Cell and Regenerative Medicine

Professor Zheng, Principal Investigator at the Institute of Zoology at the Chinese Academy of Sciences and Beijing Institute of Stem Cell and Regenerative Medicine, graduated with a Ph.D. from Computational and Systems Biology Program at the Massachusetts Institute of Technology. He researched a wide range of topics, including epigenetic and RNA alternative splicing mechanisms in reprogramming, stem cell differentiation, cancer metastasis and erythropoiesis, as well as the development and utilization of CRISPR technology. The main research results include: identification of H3K27ac as a marker of active enhancers; analysis of RNA alternative splicing in cancer metastasis and erythropoiesis, and identification of splicing factors that regulate these processes; construction of CRISPR-on gene activation tool. After graduating from MIT in 2014, he joined the Jackson Laboratory and became one of the first JAX Scholars to continue working on improving CRISPR/Cas technologies. Since July 2015, he has established an independent laboratory at the Jackson Laboratory and later at Arizona State University to develop CRISPR/Cas and other synthetic biology technologies, such as Casilio, CASFx, CREST, TALEsense systems, etc. In 2024, Prof Cheng joined the Chinese Academy of Sciences.

Jianhong Zheng

Co-founder & General Manager (China), AceLink Therapeutics (Suzhou) Limited

Dr. Zheng joined AceLink Therapeutics in 2018 as cofounder, General Manager China. She has more than 20 years of experience in drug discovery and clinical development. She started her drug discovery journey at Zen Sun Biotechnology, China, a start-up company focusing on developing innovative drugs for cancer and cardiovascular diseases, as Senior Director of R&D, building Company's R&D center, taking the first drug candidate Neuregulin for IND filing. She then took the position of Senior Director of R&D at IgCon Therapeutic, Shanghai, China, a start-up company utilizing the proprietary yeast two hybrid platform and library to identify antibody leads for therapeutic uses and to generate high affinity humanized antibodies. She further expanded her career experience into regulatory and clinical development as Chief Consultant with Rightway Consulting Inc., specializing in regulatory consultation and medical writing in clinical and health-related areas, bringing global drugs to Chinese market, including treatments for kidney diseases, inflammatory diseases, and biological products. Dr. Zheng holds a Bachelor of Science degree in Microbiology from Fudan University, and PhD in Molecular Microbiology and Immunology from University of Missouri-Columbia.

Xiangjian Zheng

Professor and Dean of Department of Pharmacology, School of Basic Medical Sciences, Tianjin Medical University
Member, Scientific Innovation Alliance for Rare Diseases, Hope for Rare Foundation

Xiangjian Zheng's research focuses on the molecular mechanisms of cardiovascular development and disease. He specializes in the construction and use of genetically engineered model animals, as well as various cellular and molecular techniques to analyze the pathogenesis of human diseases and explore drug targets. Prof. Xiangjian Zheng has been working on the pathogenesis of causative genes of cavernous cerebrovascular malformations (CCM) for a long time. He has systematically analyzed the molecular signaling pathways of the causative genes of CCM, established animal models of CCM diseases and their detection methods, and explored the drug therapies for CCM diseases (Dev Cell 2015, Nature 2016, JOVE 2017, Scie Adv 2018, Plos Biol 2020, Stroke 2021, JCBFM 2022, JCI Insight 2023), and is an internationally recognized expert in the field of cerebral cavernous malformation research. Based on the long-term accumulation in the field of CCM, his team is working on finding therapeutic drugs for CCM diseases. In addition his team is also working on the regulatory role of CCM signaling pathways in liver development and metabolism, and renal tubular development and functional maintenance (JCI Insight 2021, CMGH 2022).

Speaker Profiles

Guisheng Zhong

Associate Professor of School of Life Science and Technology, Associate Professor of iHuman Institute, ShanghaiTech University
Founder, EmayGene Biotech Co., Ltd.

Professor Guisheng Zhong obtained a bachelor's degree in Physics and a master's degree in Biophysics in China. He pursued his Ph.D. in Neurobiology from Cornell University in the United States, followed by five years of postdoctoral research at Professor Zhuang Xiaowei's laboratory at Harvard University. Since 2014, Professor Guisheng Zhong has led an independent research group at ShanghaiTech University. Under his leadership, the team has focused on both fundamental and translational research in otology. He established a comprehensive adenovirus-associated virus (AAV) vector development platform, AAVMeta. Through rational design and high-throughput screening methods, they successfully developed the first AAV vector with high transduction efficiency in the auditory system in China, named AAV-ie (inner ear). This groundbreaking achievement was published on Nature Communications. This breakthrough has garnered recognition from both academia and industry. Utilizing the AAV-ie vector, Professor Zhong's team achieved hair cell regeneration in vivo in mice, a milestone in promoting the practical application of hair cell regeneration strategies in the clinic. The vector has currently applied for international patents, indicating its potential for the gene therapy of auditory diseases. Since establishing the research group, Professor Guisheng Zhong has published over 30 articles as corresponding author in journals such as Cell Discovery, Nature Communications, Science Advances, and more.

Daixing Zhou

Founder & CEO, Lakeshore Biotechnology

Dr. Daixing Zhou is a trailblazer in the field of genomics. He has devoted his career to developing human genome sequencing technologies and their medical applications. He was one of the core team members at Solexa Inc. (acquired by Illumina Inc. in 2007) and contributed significantly to the development and commercialization of next generation sequencing (NGS) technologies, which dramatically lower the human genome sequencing cost from $3B USD in 2001 to $300 USD today. Returning to China in 2010, Dr. Zhou co-founded Berry Genomics to apply NGS to medical applications. He pioneered the non-invasive prenatal testing (NIPT), which revolutionizes the field of prenatal genetic disease screening. He took the company public in 2017 and continued serving the company as CEO for four years. In 2021, Dr. Zhou con-founded Lakeshore Biotech, dedicating to finding cures for patients. Dr. Zhou earned his Ph.D. from University of Maryland at Baltimore and completed his postdoc training at Duke University.

Karen Zhou

Senior Director, Clinical Development, Lundbeck China

Karen Zhou joined Lundbeck China in 2018 as the head of Clinical Development, leading the Clinical Research and Clinical Operation team. Before joined in Lundbeck, Karen worked in MSD, Sanofi-aventis and Novatis, and rotated in Novartis HQ. She has more than 20 years' experience of clinical research and drug development strategy planning. Karen graduated from Capital Medical University and practiced as cardiologist in Xuanwu Hospital of Capital Medical University. She also obtained a master degree in epidemiology from University of Toronto.

Speaker Profiles

Na Zhou

Program Officer, New Cornerstone Science Foundation

With over 15 years' experience working in health-related philanthropic organizations, Ms. Zhou currently serves in the role of Program Officer at the New Cornerstone Science Foundation where she manages the review and selection process for award candidates in the medical sciences field. Her duties also involve leading the Foundation's international benchmarking as well as its external communications. In her position as the Program Director at the China Medical Board (CMB), a Rockefeller Foundation-endowed philanthropic organization, Ms. Zhou led strategy development, programming and operations for all CMB's China Program portfolios, covering health policy, health systems and medical education. Ms. Zhou was trained in medicine, economics and English at Peking University where she received two bachelor's degrees and holds a master's degree in health policy from the London School of Hygiene and Tropical Medicine and London School of Economics and Political Science.

Qing Zhou

Qiushi Distinguished Professor, Professor at Life Sciences Institute, Zhejiang University
Member, Scientific Innovation Alliance for Rare Diseases, Hope for Rare Foundation

Qing Zhou's research focuses on the genetic analysis of monogenic disorders of inflammation and elucidating the genetic basis of autoinflammatory diseases, investigations into the mechanisms by which mutations discovered by our study cause autoinflammation, development of targeted therapies for the patients with autoinflammatory diseases, and design of the new drug based on the disease causal genes/mutations. Papers published in Nature, NEJM, Immunity, Nature Genetics, ARD, A&R, Science Advances, JACI, PNAS, AJHG, JASN, etc.

Wenhao Zhou

Chief Physician, Professor and President, Guangzhou Women and Children's Medical Center, Guangzhou Medical University

Professor Wenhao Zhou is the president of Guangzhou Women and Children's Medical Center, clinical professor of Pediatrics, doctor supervisor at Guangzhou Medical University. Professor Zhou served as the President of the Neonatology Group of the Pediatric Society of the Chinese Medical Association, Vice President of the Medical Genetics Society of the Chinese Physician Association, President-to-elect of the Rare Disease Society of the Shanghai Medical Association. Professor Zhou is a world renowned practitioner and researcher in neonatology. His research focuses on clinical management of critical neonatal illnesses, neonatal brain diseases and rare diseases. He has established the China Neonatal Neurocritical Care Alliance and the China Neonatal Genome Project. He is the principle investigator 15 National Major Research and Development Grants and NSFC Key Projects. He is the author of more than 185 peer-reviewed scientific publications. He received the First Prize of Shanghai Science and Technology Progress Award, the Chinese Medical Award, and the Second Prize in Science and Technology of the Ministry of Education.

鸣谢支持 | Acknowledgement

学术支持单位 | Supporting Organizations

排名不分先后 | Listed in No Particular Order

复旦大学医学遗传研究院
Institute of Medical Genetics and Genomics, Fudan University
上海医学论坛
Shanghai Medical Forum
浙江大学精准医学会聚研究计划
Precision Medicine Convergence Research Project, Zhejiang University
天津医科大学药学院
School of Pharmacy, Tianjin Medical University
武汉大学医学部
Wuhan University School of Medicine
天津大学医学部
Division of Medicine, Tianjin University
上海交通大学药学院
School of Pharmacy, Shanghai Jiao Tong University
复旦大学人类表型组研究院
Human Phenome Institute, Fudan University
天桥脑科学研究院
Tianqiao & Chrissy Chen Institute
良渚实验室
Liangzhu Laboratory
云南大学生命科学学院
School of Life Sciences, Yunnan University
武汉大学生命科学学院
College of Life Sciences, Wuhan University
东南大学生命科学与技术学院
School of Life Science and Technology, Southeast University
中国农业大学生物学院
College of Biological Sciences, China Agricultural University

大会指定学术期刊 | Official Scientific Journal

Human Gene Therapy

大会赞助商 | Sponsors

- **钻石赞助商 Diamond Partner**
- **黄金赞助商 Gold Partners**
- **白银赞助商 Silver Partners**
- **青铜赞助商 Bronze Partners**

媒体支持 | Media Partners

- **战略合作媒体 Strategic Media Partners**
- **深度合作媒体 Advanced Media Partners**
- **支持媒体 Media Support**