3rd HOPE FOR RARE SCIENCE CONFERENCE

第三届中国罕见病科研大会

June 25-27, 2026 | Shanghai, China

CONFERENCE MANUAL

主办方 Host

HOPE FOR RARE FOUNDATION 瑞鸥公益基金会

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 Guests,

On behalf of the Organizing Committee, it is my great pleasure to warmly welcome you to the 3rd Hope for Rare Science Conference (HRSC). The Hope for Rare Foundation was established in 2022 through a joint initiative I launched with several scientists and entrepreneurs, our mission is to become an Innovation Engine for advancing rare diseases research and translational medicine. In the same year, I launched and hosted the inaugural HRSC—a biennial international academic conference dedicated to fostering academic exchange and collaboration in rare diseases research, bridging China with the global rare disease community.

The 2nd HRSC, held in 2024, received high praise from attendees for its compelling scientific program and seamless, efficient organization, quickly establishing HRSC as a premier rare diseases research conference in China. Through the sharing of expertise and open dialogue, the conference drives progress in global rare diseases research and promotes multi-stakeholder collaboration among industry, academia, research, healthcare, patients, and policymakers. This year, we are once again joining hands with the National Children's Medical Center / Children's Hospital of Fudan University and the China Organization for Rare Disorders (CORD) to co-host this high-level international academic conference.

Rare diseases represent a major medical challenge for all of humanity. In recent years, while major countries and regions around the globe have prioritized rare diseases as a health issue, and the most cutting-edge breakthroughs in biomedicine are often first applied in the rare disease space, we must still confront a stark reality: over 90% of rare diseases have no approved treatment to this day. Encouragingly, we are living in a hopeful era of transformation. In May this year, the State Council of China's "Regulations on Clinical Research and Translational Application of Novel Biomedical Technologies" officially took effect, creating, for the first time at the national level, a standardized pathway for the clinical translation of advanced therapies, and providing specific policy support for personalized treatments for rare diseases. Looking across the globe, the Children's Hospital of Philadelphia recently performed the world's first personalized gene-editing therapy for an infant with CPS1 deficiency. Rare diseases research is by no means a matter for one country alone, and drug development is not just for treating patients in a single nation. It urgently calls for close collaboration among colleagues worldwide—to break down gaps in scientific and technological development, disparities in economic levels, regional policy barriers, and geopolitical divides—so that the fruits of innovation can benefit every rare disease patient.

Over the past decade and more, China has made remarkable progress in rare diseases research and drug development. Through this conference, while deepening international exchange and collaboration, we hope to showcase to the world the vibrant strength of Chinese scientists and R&D enterprises, and contribute Chinese wisdom to the global rare diseases research endeavor. Like other foundations around the world, our foundation is also committed to making proactive efforts to advance scientific research. Just a few months ago, the first personalized gene therapy project funded by us completed the dosing of its first pediatric patient. Meanwhile, our "Homy" Familial Hypercholesterolemia Special Research Fund has already supported nine basic and clinical research projects. These explorations, though small as sparks, reflect our deep conviction: make the rare visible, and let research reach every waiting expectation.

Finally, I would like to thank every partner for your trust and companionship, and I salute all the speakers for the courage and insights you bring. Every breakthrough in this field begins with an open and honest gathering like today's. May the sparks we ignite here go beyond the laboratory, cross borders, and ultimately reach the hands of patients still waiting. I wish the conference a complete success, and I hope all of you find it a truly rewarding experience.

Kevin Huang
Chairperson of the Organizing Committee
Founder and CEO of Hope for Rare Foundation
Founder and President of Chinese Organization for Rare Disorders

HOST

HOPE FOR RARE FOUNDATION
瑞鸥公益基金会

Hope for Rare Foundation was founded in 2022 by Kevin Huang in partnership with several renowned scientists and entrepreneurs. It is the first nonprofit foundation focusing on scientific research and translational medicine of rare diseases in China. Our vision is to advance the care of ALL patients with rare diseases, and our mission is to become an Innovation Engine for advancing rare diseases research and translational medicine.

Vision: To advance the care of ALL patients with rare diseases
Mission: To be an Innovation Engine for advancing rare diseases research and translational medicine
Approach: Platforms Sponsorship Innovation

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. CORD has been operating in China for the past 13 years, with a strong international perspective and a highly professional approach.

(Alphabetical Order by Last Name) Conference Chairpersons

Guangping Gao

Professor & Chair, Department of Genetic and Cellular Medicine,
UMass Chan Medical School
Past President, American Society of Gene & Cell Therapy

Yi Wang

Director, National Children's Medical Center
President, Children's Hospital of Fudan University

Janet Woodcock

Former Acting Commissioner, U.S. Food and Drug Administration (FDA)
Former Director, FDA's Center for Drug Evaluation and Research

Xiao Xiao

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

Scientific Committee (Alphabetical Order by Last Name)

Xiaochun Cao

Co-Founder, Hope for Rare Foundation
Co-Founder, Executive Director & General Manager, Tigermed

Can Ding

Clinical Geneticist at the Amedes Genetics, Germany

Taosheng Huang

Professor of Shanghai Medical College, Fudan University
Director of Institute of Medical Genetics and Genomics, Fudan University

Xiaowei Jin

Venture Partner, Sherpa Healthcare Partners
Senior Advisor Chinese Organization for Rare Disorders

Harvey 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

Jianhua Mao

Dean and Professor of Department of Nephrology and Urology, President of Children' s Hospital of Zhejiang University School of Medicine

Weihong Tan

Academician of the Chinese Academy of Sciences
Director of the Hangzhou Institute of Medicine, CAS
Dean of the Cancer Hospital Affiliated with Hangzhou Institute of Medical Sciences, CAS

Tian Xu

Co-Founder, Hope for Rare Foundation
Chair Professor of Genetics, School of Life Sciences, Westlake University

Seng H. Cheng

Senior Vice President, Head of Research and Product Development, Alexion, AstraZeneca Rare Disease

Tao Duan

Co-Founder, Hope for Rare Foundation
Director, Department of Obstetrics & Gynecology, Shanghai East Hospital, Tongji University
Former Director, Shanghai First Maternity and Infant Hospital, Tongji University

Yichang Jia

Tenured Professor and Vice Dean, School of Basic Medical Sciences (BMS), Tsinghua University
Founder, SineuGene Therapeutics Co., Ltd.

Nianwei Lin

Co-Founder, iXCells Biotechnologies
Founder & CEO, Elpis Precision Medicine
Chief Scientific Officer, Hope for Rare Foundation

Alvin Luk

President & CMO, HanchorBio

Daniel Scherman

Director, French Foundation for Rare Diseases
Head of the Scientific Secretariat of International Rare Disease Consortium (IRDiRC)
Head of the Medicine and Life Science Division of the European Academy of Science

Zhi-Ying Wu

Qiushi Distinguished Professor, Zhejiang University

Chouwen Zhu

President, Shanghai Clinical Research and Trial Center

(Alphabetical Order by Last Name) Organizing Committee

Chairperson

Kevin Huang

Founder and CEO of Hope for Rare Foundation
Founder and President of Chinese Organization for Rare Disorders

Member

Wenhan Geng

Project Supervisor, Hope for Rare Foundation

Juan Huang

Project Manager, Hope for Rare Foundation

Zhiwei Huang

Project Assistant, Hope for Rare Foundation

Eileen Li

Senior Advisor, Chinese Organization for Rare Disorders

Li Ning

Project Supervisor, Hope for Rare Foundation

Yeyang Su

Advisor, Hope for Rare Foundation
Independent Researcher

Qi Sun

Senior Advisor, Chinese Organization for Rare Disorders
Medical Expert in PJS Patient Organizations (China & Germany)

Hanbo Wang

Advisor, Hope for Rare Foundation

Shuisong Ye

Founder, Deep Science

Boya Yu

Project Manager, Hope for Rare Foundation

Xiaowen Zhang

Advisor, Hope for Rare Foundation
Deputy General Manager, Business and Marketing Department, NewPats

Mel Zhang

Project Director, Hope for Rare Foundation

Lingling Cai
Yun Hong
Jianning Luo
Xiaopeng Song
Wanzhen Xie
Yihan Zhang

Yushi Chen
Yuhang Hu
Ke Ma
Geting Su
Jingwei Xu
Yuxiao Zhang

Zitian Cheng
Jinyue Huang
Yiming Ma
Jiao Su
Jinyu Xu
Chenxi Zhao

Yanyan Dai
Shuyu Huang
Xin Meng
Yu'ang Tang
Youyan Xu
Yanan Zhao

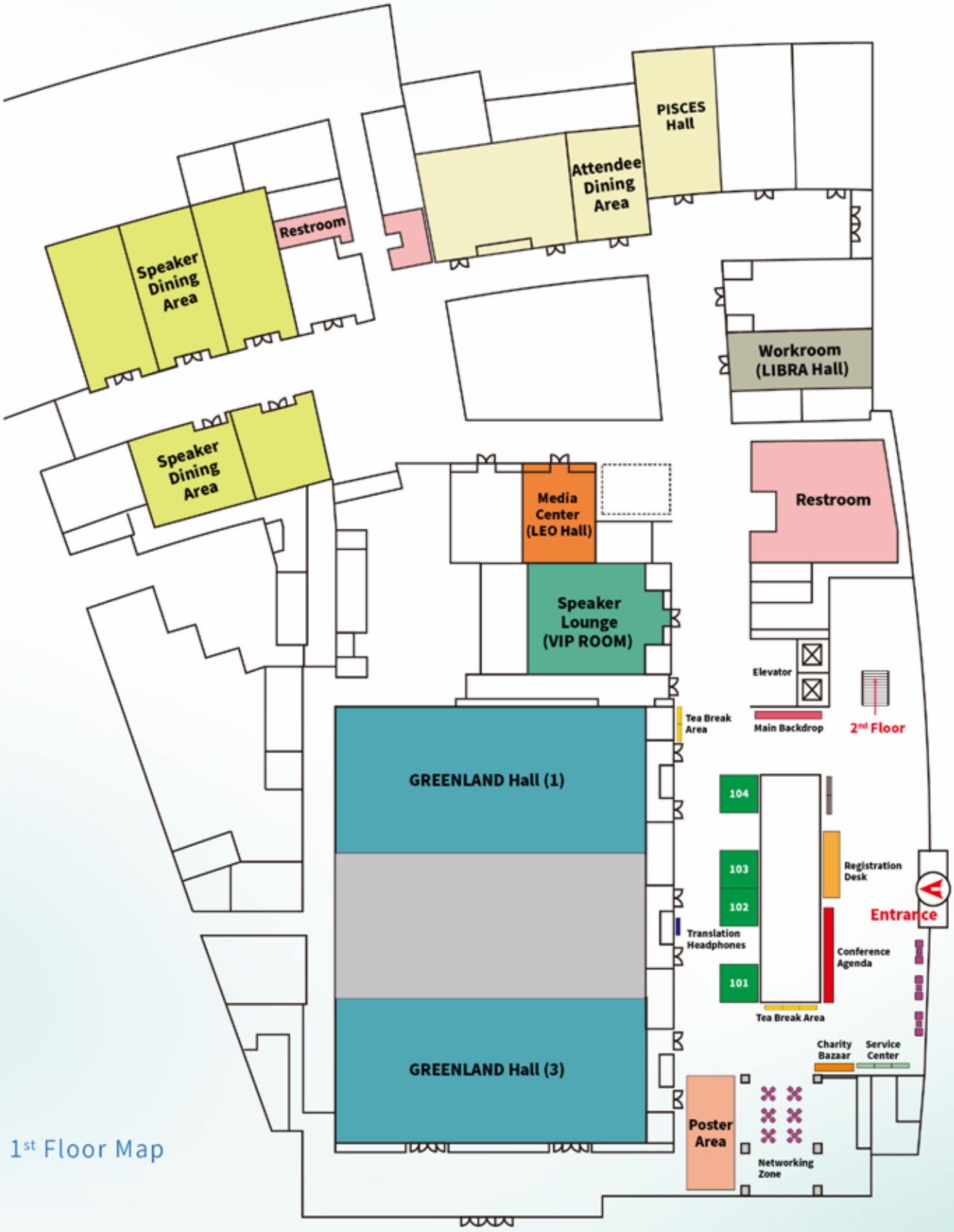
Guxiao Du
Aiwen Jian
Ling Min
Chenyan Wang
Fan Yang
Jiashuo Zhu

Siyu Duan
Haiyun Kong
Xiujuan Pu
Haiyun Wang
Guang Yang
Ziqing Zuo

Junkai Fan
Lena
Tian Qiu
Haoyang Wang
Jing Yang
Yujie Zuo

Yiting Ge
Yuanlu Li
Yuanyuan Shi
Xiaohan Wang
Jia Zeng

Convention Center & Hotel Guide Map



1st Floor Map

Convention Center & Hotel Guide Map

Booth Map

Premium Booth Area

101 PackGene Biotech
102 Hope for Rare Foundation
103 CORD
104 OBiO TECH

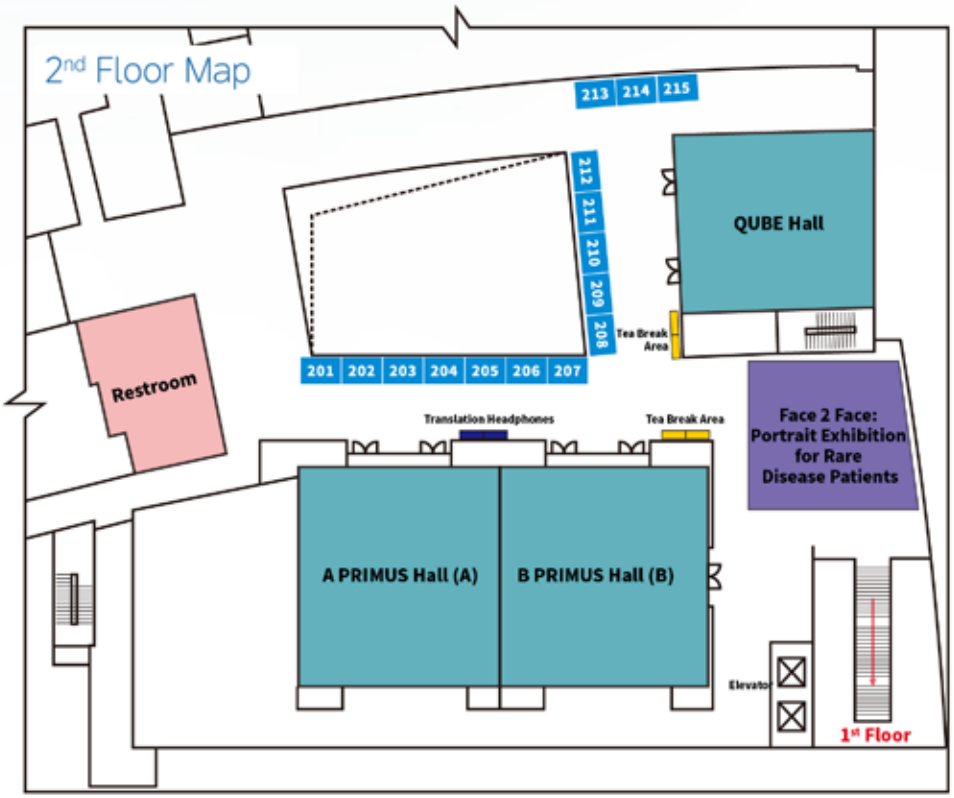
Advanced Booth Area

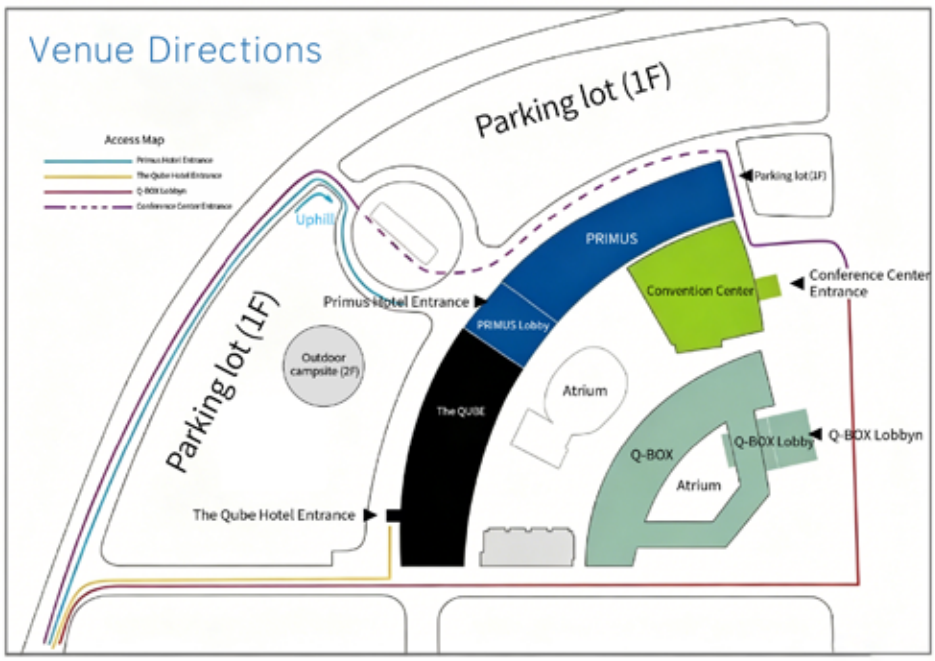
201 DYNE PHARMA
202 Chigene
203 Genevoyager
204 WuXi AppTec
205 Hunter Biotech.
206 Belief BioMed
207 Fosun Pharma
208 Jilin Huiminkangen Technology Co., Ltd.
209 Ignis Therapeutics
210 Precisiongenes
211 Shanghai Baoyuxin Medical Technology Co., Ltd.
212 Biosan
213 OAE Publishing Inc.
214 MyGenostics
215 Yoltech Therapeutics

Communication Area / Poster Area /

Tea Break Title Sponsor:

迈基诺 MyGenostics
Toward the promise of Genomics





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: June 24 14:00-19:00
June 25 07:30-19:00
June 26 07:30-19:00
June 27 07:30-14:00

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 Convention Center.
Opening Hours: June 24 14:00-19:00
June 25 07:30-19:00
June 26 07:30-21:00
June 27 07:30-18:00

Simultaneous Interpretation

The conference provides Chinese-English simultaneous interpretation services. Devices can be collected at the entrance of the Greenland Hall (1st Floor) and the Primus Hall (2nd Floor). Please return the devices promptly upon leaving the convention center.

Speaker Lounge

Located at the VIP Reception Hall on the 1st Floor. Access is restricted to speakers holding red lanyards.

Media Center

Located at the Leo Hall on the 1st Floor. Access is restricted to media personnel holding purple lanyards.

Poster Area

Located on the 1st Floor of the Convention Center, open to all attendees.

Networking Zone

Located on the 1st Floor of the Convention Center, open to all attendees.

Face 2 Face: Portrait Exhibition for Rare Disease Patients

Located in the Exhibition Area on the 2nd Floor, showcasing the life stories of 50 rare disease patients. Open to all attendees.

Charity Bazaar

Located adjacent to the Networking Zone on the 1st Floor of the Convention Center, open to all attendees.

Tea Break Area

Located next to the Greenland Hall (1st Floor), and next to the Primus Hall & Qube Hall (2nd Floor).
Opening Hours: June 26 10:00-10:30 15:30-16:00
June 27 10:00-10:30 15:30-16:00

Rock Rare · Lawn Concert

Located at the Outdoor Camp of Greenland Primus Hotel. Subject to weather changes; please confirm with the Service Center if needed.
Opening Hours: June 26, 19:00–21:00

Parking

Complimentary parking is provided during the conference. Please scan the QR code at the Service Center to validate your parking.

Dining Information

Attendees with BLUE and PURPLE Lanyards

Date	Time	Place	Food Provided
Juny 26 (Lunch)	12:00-13:30	1st Floor: near VIRGO Hall and PISCES Hall 2nd Floor: near PRIMUS Hall A	Business Boxed Meal
Juny 27 (Lunch)	12:00-13:30	1st Floor: near VIRGO Hall and PISCES Hall 2nd Floor: near PRIMUS Hall A	Business Boxed Meal

Dining is available at the designated dining areas on the 1st and 2nd floors, as indicated by directional signage

Friendly Reminders:

1. Meal Collection

Please scan the QR code using your conference badge to collect your meal. One redemption per person, per meal. If you require a vegetarian option, please inform the catering staff when collecting your tray.

2. Waste Disposal

Please dispose of your lunch boxes and cutlery in the designated trash bins after eating.

3. Welcome Dinner (June 25, 19:00)

For the Welcome Dinner, please scan your conference badge for entry.

4. Dining at Primus Hotel (2nd Floor)

Participants may register by scanning the QR code at the registration desk on the 1st floor of the Convention Center. Places are limited and available on a first-come, first-served basis.

Attendees with RED Lanyards

Date	Time	Place	Food Provided
June 25 (Lunch)	12:00 – 13:30	SCORPIO Hall	Buffet Lunch
June 25 (Dinner)	19:00 – 21:00	GREENLAND Hall 1+2	Welcome Dinner
June 26 (Lunch)	12:00 – 13:30	SCORPIO Hall	Buffet Lunch
June 26 (Dinner)	18:00 – 20:00	SCORPIO Hall	Buffet Dinner
June 27 (Lunch)	12:00 – 13:30	SCORPIO Hall	Buffet Lunch
June 27 (Dinner)	18:00 – 20:00	SCORPIO Hall	Buffet Dinner

Friendly Reminders:

1.Please scan your conference badge to access the buffet dining area.

2.If you attend satellite meetings or special sessions during lunch, please proceed to the buffet restaurant at 11:30 for an early meal.

Self-Paid Hotel Restaurant Information

Dining at Primus Hotel (2nd Floor)

The Chinese Restaurant on the 2nd Floor offers both Chinese and Western cuisine. Payment is accepted by card or cash (RMB).

· Western À la carte: 06:30 – 22:30

· Chinese À la carte: 11:00 – 14:00, 17:00 – 21:00

Private Dining Rooms:

Private rooms accommodating 8–20 guests are available for both Chinese and Western meals. Advance reservation is required.

Reservations: Dial 021-3891-8888 and ask for Primus Chinese Restaurant.

Convention Center Surroundings

Venue: Greenland International Convention Center

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

Hotel Reservation

Hotel	Negotiated Rates	Reservation Hotline
Primus Hotel Shanghai Sanjiagang (5-star)	From ¥500/night	Dial 021-3891 8888 and ask for the Reservation Department.
The Qube Hotel Shanghai Sanjiagang (4-star)	From ¥300/night	

There are two partner hotels within a 5-minute walk of the conference venue. Special conference rates are available at both hotels. Please mention **"HRSC2026"** when making reservations to enjoy the discounted rates.

Transportation

Pudong International Airport (10 km / approximately 20 minutes by car)

Shuttle Bus: Complimentary shuttle bus service is available only for participants who have booked accommodation at the conference hotels. Please contact the hotel for the shuttle schedule.

Shanghai Hongqiao International Airport (51 km / approximately 60 minutes by car)

Metro: Hongqiao Airport Terminal 2 → Metro Line 2 (toward Pudong International Airport Terminals 1 & 2) → Far East Avenue Station

Shanghai Hongqiao Train Station (56 km / approximately 70 minutes by car)

Metro: Hongqiao Railway Station → Metro Line 2 (toward Pudong International Airport Terminals 1 & 2) → Far East Avenue Station

Shanghai Station (41 km / approximately 75 minutes by car)

Metro: Shanghai Railway Station → Metro Line 1 (toward Xinzhuang) → transfer at People's Square Station → Metro Line 2 (toward Pudong International Airport Terminals 1 & 2) → Far East Avenue Station

Shanghai South Railway Station (37.5 km / approximately 45 minutes by car)

Metro: Shanghai South Railway Station → Metro Line 1 (toward Fujin Road) → transfer at People's Square Station → Metro Line 2 (toward Pudong International Airport Terminals 1 & 2) → Far East Avenue Station

Friendly Reminder: Far East Avenue Metro Station is 4.4 km from the Convention Center. Taxi service is recommended.

Attractions

Shanghai Disney Resort	21 km
Shanghai Museum (East Branch)	28 km
The Bund	36 km
Oriental Pearl Tower	36 km

Shopping Centers

Florentia Village Shanghai (Premium Designer Outlets)	4.4 km
Taikoo Li Qiantan	31 km
Shanghai IFC Mall	34 km

Hospitals

Shanghai Pudong New Area People's Hospital	9.1 km
Shanghai International Medical Center (SIMC)	24 km

Notes for Attendees

1. Badge Requirement: The officially confirmed conference badge is the sole credential for entry into the venue. Attendees must wear it visibly at all times.
2. On-site Registration: On-site registration and payment services are available for unregistered participants. Please consult the staff at the sign-in desk on the first floor.
3. Meeting Etiquette: Please observe venue rules, attend sessions punctually, and avoid being ate or leaving early. Refrain from loud conversation during sessions; kindly switch your mobile phone to silent or vibrate mode. If you need to take a call, please step outside the meeting room.
4. Personal Belongings: Please take good care of your personal belongings during the conference.
5. Medical Services: A temporary medical station providing basic and commonly used medicines is available on the first floor. Due to the complexity of rare diseases, the organizing committee is unable to provide emergency medical assistance to patients attending the conference. Patients should assess their health status and take necessary auxiliary or medical measures.
6. Statements & Opinions: The statements or opinions of conference sponsors, partners, and exhibitors do not represent the endorsement or recommendation of the organizer regarding their products, technologies, and services.
7. Prohibited Conduct: Any form of marketing, publicity, or misleading behavior for illegal products, technologies, services, and treatment methods is strictly prohibited. Once found, the conference badge will be immediately invalidated.
8. Photography & Privacy: 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.
9. Cultural Respect: Guests and participants come from all over the world. Please respect the cultural customs and religious beliefs of different countries and regions.
10. Gratitude: The hard-working staff and volunteers are the most important force ensuring the smooth operation of this public welfare academic conference. Please give them support and understanding.

Scientific Program

June 25		
14:00-18:00	GREENLAND Hall 1+2 (1F)	Open Ceremony
19:00-21:00	GREENLAND Hall 1+2 (1F)	Welcoming Banquet

June 26				
TIME	PRIMUS Hall A (2F)	GREENLAND Hall 1 (1F)	GREENLAND Hall 3 (1F)	PRIMUS Hall B (2F)
08:30-10:00	Cutting-Edge Research Trends in Rare Diseases I	New Approaches and the Future in Gene Editing I	AI Empowering Drug Development for Rare Diseases I	Development and Application of Biobanks
10:00-10:30	Tea Break			
10:30-12:00	Cutting-Edge Research Trends in Rare Diseases II	New Approaches and the Future in Gene Editing II	AI Empowering Drug Development for Rare Diseases II	Unpacking Patient-Driven Rare Disease Research Innovation
12:00-14:00	Lunch / Poster Session / Satellite Meeting			
14:00-15:30	Model Systems Used in Rare Diseases Research I	Gene Therapy: Challenges and Perspectives I	Clinical Advances in Gene Editing	Breakthroughs from Early-Career Scientists
15:30-16:00	Tea Break			
16:00-17:30	Model Systems Used in Rare Diseases Research II	Gene Therapy: Challenges and Perspectives II	Novel Targets for Small Molecules and Antibodies	Progress of the Hope for Rare Foundation's Research Projects
19:00-21:00	Investigator-Patient Exchange Session / Rock Rare · Lawn Concert			

June 27				
TIME	PRIMUS Hall A (2F)	GREENLAND Hall 1 (1F)	GREENLAND Hall 1 (1F)	PRIMUS Hall B (2F)
08:30-10:00	Undiagnosed Diseases & Medical Genetics I	RNA-Based Therapeutics: From Antisense Oligonucleotides to mRNAs I	Development of Vectors and Delivery System I	Regulatory Innovation in Advanced Therapies I
10:00-10:30	Tea Break			
10:30-12:00	Undiagnosed Diseases & Medical Genetics II	RNA-Based Therapeutics: From Antisense Oligonucleotides to mRNAs II	Development of Vectors and Delivery System II	Regulatory Innovation in Advanced Therapies II
12:00-14:00	Lunch / Poster Session / Satellite Meeting			
14:00-15:30	Natural History & Clinical Management of Rare Diseases I	Clinical Advances in Gene Therapy I	Emerging Frontiers and Applications of Cell Therapy I	Driving Drug Development: Patient Organization and Foundation
15:30-16:00	Tea Break			
16:00-17:30	Natural History & Clinical Management of Rare Diseases II	Clinical Advances in Gene Therapy II	Emerging Frontiers and Applications of Cell Therapy II	Driving Drug Development: Patient and Patient Family

Scientific Program

June 28		
09:00-13:00	Academic Visit	Route 1: School of Life Sciences, Fudan University (Jiangwan Campus)
08:00-11:00		Route 2: National Children's Medical Center | Fudan University Children's Hospital

Friendly Reminder: For specific itinerary and departure arrangements on the site visit, please refer to the email notification you received. If you have any questions, you can inquire at the Convention Service Center located on the 1st Floor.

Symposiums

June 25		
09:00-12:00	PISCES Hall (1F)	Clinical Research Advances in Developmental and Epileptic Encephalopathies Symposium (Closed-Door) Host: Chinese Organization for Rare Disorders

June 26		
08:30-12:00	QUBE Hall (2F)	Duchenne Muscular Dystrophy Symposium Hosts: Hope for Rare Foundation, Chinese Organization for Rare Disorders, China DUCHENNE Family Network
14:00-17:30	QUBE Hall (2F)	Rare Diseases Research Symposium: From Bench to Bedside Host: Institute of Medical Genetics and Genomics of Fudan University

June 27		
08:30-12:00	QUBE Hall (2F)	Rare Tumors Symposium Host: Zhejiang Cancer Hospital
14:00-17:30	QUBE Hall (2F)	Rare Diseases Symposium Hosts: Liangzhu Laboratory & Children's Hospital of Zhejiang University School of Medicine

Satellite Meetings

June 26		
12:30-13:30	PRIMUS Hall A (2F)	Biogen Satellite Meeting

June 27		
12:30-13:30	PRIMUS Hall A (2F)	BerryGenomics Satellite Meeting

Special Photographic Exhibition:" Face 2 Face: Portrait Exhibition for Rare Disease Patients "

Curators: Hope for Rare Foundation & Chinese Organization for Rare Disorders
Opening Hours: June 25-27, 08:00-18:00
Venue: 2nd Floor, Convention Center (near the stairs)

Rock Rare · Lawn Concert

Opening Hours: June 26, 19:00–21:00

Located at the Outdoor Camp of Greenland Primus Hotel. Subject to weather changes; please confirm with the Service Center if needed.

Scientific Program

Time: June 25, 14:00–17:50
Venue: GREENLAND Hall 1+2 (1F)

Opening Ceremony	
	Emcee: Weier Ge Deputy Head of Overseas Markets, Yicai Global
14:00-14:05	**Gu Zheng Performance: Excerpt from Dream in Sleeves** Performer: Hu Xiyao (living with a rare disease)
14:05-14:14	**Remarks from the Hosts** Kevin Huang Founder and CEO of Hope for Rare Foundation, Founder and President of Chinese Organization for Rare Disorders Yi Wang Director, National Children's Medical Center; President, Children's Hospital of Fudan University
14:14-14:17	**Conference Chairpersons Launched the Opening Ceremony**
14:17-14:25	**Remarks from the Guests**
14:25-14:30	**Medley Song: Daylily Flower and LAM Girl** Performers: Manman, Momo, Chun Lu, Anyue, Wen Wang (all living with rare diseases)
14:30-15:00	**Keynote Speech** **Turning Genes into Medicines** Katherine High CEO, RhyGaze AG; Emeritus Professor, Perelman School of Medicine of the University of Pennsylvania, and Children's Hospital of Philadelphia
15:00-15:30	**Keynote Speech** **Evaluating Rare Disease Treatments: Current Progress and Future Directions** Janet Woodcock Former Acting Commissioner, U.S. Food and Drug Administration (FDA) Former Director, FDA's Center for Drug Evaluation and Research
15:30-16:00	**Keynote Speech** **Kind Science and Gentle Technology: Serving the People** Yi Rao Chair Professor, Peking University
16:00-16:30	**Keynote Speech** **Exploring the Uncharted: Research Grants by Hope for Rare Foundation** Kevin Huang Founder and CEO of Hope for Rare Foundation, Founder and President of Chinese Organization for Rare Disorders
16:30-17:05	**Panel Discussion** **Regulatory Innovation and International Collaboration for Advanced Therapies** Chair: Janet Woodcock Panelists: Emmanuel Cormier Head of Regulatory Science and Innovation Task Force, European Medicines Agency Manabu Inoue Chief Medical Officer, Pharmaceuticals and Medical Devices Agency, Japan Yoko Aoi Coordination Director, Office of Review Management, Pharmaceuticals and Medical Devices Agency, Japan Yue Yang Principal Investigator and Director of Center of Excellence for Translational and Regulatory Science, School of Pharmacy, Tsinghua University
17:05-17:40	**Panel Discussion** **United in Advancing Personalized Treatments for Ultra-Rare Diseases** Chair: Eileen Li Senior Advisor, Chinese Organization for Rare Disorders Panelists: Javier García Cogorro Founder and Secretary of Fundación Columbus; Founder and General Partner of Columbus Venture Partners Al Hawkins Co-Founder & President, Amplo Biotechnology Guangping Gao Professor & Chair, Department of Genetic and Cellular Medicine, UMass Chan Medical School; Past President, American Society of Gene & Cell Therapy Juan Huang Project Manager of Hope for Rare Foundation, Project Manager of Chinese Organization for Rare Disorders Kevin Huang Founder and CEO of Hope for Rare Foundation, Founder and President of Chinese Organization for Rare Disorders Guangzuo Luo Professor, China Medical University; Founder, Bionce Biotechnology
17:40-17:50	**Appointment Ceremony of Scientific Advisory Board for Hope for Rare Foundation**

Scientific Program

Time: June 25, 2026 19:00–21:00
Venue: GREENLANE Hall 1+2 (1F)

Welcoming Banquet	
Emcee: Weier Ge Deputy Editor-in-Chief, Yicai Global	
19:00-19:10	**Opening Show** **Choral Performance: A Little Red Flower** Performers: HopeSphere (all living with rare diseases) **Guitar and Medley Song: Dear You and Push Open the Door to the World** Performers: Wen Wang, Anyue, Zhenye Zhang (all living with rare diseases)
19:10-19:25	**Remarks from the Hosts** Yi Wang Director, National Children's Medical Center; President, Children's Hospital of Fudan University Kevin Huang Founder and CEO of Hope for Rare Foundation, Founder and President of Chinese Organization for Rare Disorders Xiaochun Cao Co-Founder, Hope for Rare Foundation; Co-Founder, Executive Director and President, Tigermed
19:25-19:35	**Traditional Sichuan Opera Performance**
19:35-21:00	**Banquet**

Scientific Program

Time: June 26 08:30–12:00
Venue: PRIMUS Hall A (2F)

Cutting-Edge Research Trends in Rare Diseases	
Chair: Yongchuan Zhu Investigator, Shanghai Mental Health Center, Shanghai Jiao Tong University School of Medicine	
08:30-09:00	Elemental Genetic Disorders: Advances, Opportunities and Challenges Fudi Wang Qiushi Distinguished Professor, Zhejiang University; Director of the Institute of Nutrition and Food Safety, Zhejiang University
09:00-09:30	Defining and Targeting Intrinsically Disordered Oncoproteins Across Interaction Networks and Condensates Xiaokun Shu Distinguished Professor and Xianghui Scholar at Fudan University
09:30-10:00	Molecular Diagnosis and Reproductive Intervention for Hereditary Rare Diseases Yue-Qiu Tan Vice President and Professor, CITIC-Xiangya Reproductive and Genetic Hospital
10:00-10:30	Tea Break
10:30-11:00	Molecular Pathogenesis and Translational Therapeutics for POLG-Related Disorders Xuefeng Zhu Dean and Professor of the School of Basic Medical Sciences, North China University of Science and Technology
11:00-11:30	Stress Granule and Neurodegeneration Ge Bai Qiushi Distinguished Professor, Zhejiang University
11:30-12:00	Molecular Mechanisms of CDKL5 Deficiency Disorder Yongchuan Zhu Investigator, Shanghai Mental Health Center, Shanghai Jiao Tong University School of Medicine

Time: June 26 08:30–12:00
Venue: GREENLAND Hall 1 (1F)

New Approaches and the Future in Gene Editing	
Chair: Chengqi Yi Boya Professor, Peking University	
08:30-09:00	Precise RNA Targeting and Manipulation Chengqi Yi Boya Professor, Peking University
09:00-09:30	Early Life Genomic Therapies Tippi MacKenzie Professor, Director of The Eli and Edythe Broad Center of Regeneration Medicine and Stem Cell Research, Co-Director of the Center for Maternal-Fetal Precision Medicine, UCSF
09:30-10:00	From Gene Editing to New Frontiers: Advancing Cell and Gene Therapies Wensheng Wei Professor at the School of Life Sciences, the Biomedical Pioneering Innovation Center and the Peking University–Tsinghua University Joint Center for Life Sciences (Peking University), Director of the Genome Editing Research Center, Peking University; Lead Scientist at Changping Laboratory
10:00-10:30	Tea Break
10:30-11:00	Precise Engineering of Genome Structures Hao Yin Hongyi Distinguished Professor, Wuhan University
11:00-11:30	Enhancing Efficiency and Precision of Gene Editing for Genetic Disease Correction Chun-Qing Song Principal Investigator, Westlake University
11:30-12:00	Development and Application of Epigenetic Editing Tools Changyang Zhou Principal Investigator, Center for Excellence in Brain Science and Intelligence Technology / Institute of Neuroscience, Chinese Academy of Sciences

Scientific Program

Time: June 26 08:30–12:00
Venue: GREENLAND Hall 3 (1F)

AI Empowering Drug Development for Rare Diseases	
Chair: Lijia Ma Leading Scientist, Changping Laboratory; Founder, Westlake Genetech	
08:30-09:00	AI-Powered Discovery and Development of Drugs for Rare Diseases Lee Jia Academicians of International Eurasian Academy of Sciences, Academy of Toxicological Sciences, European Academy of Sciences and Arts; Distinguished Professor, The First Affiliated Hospital, Henan University
09:00-09:30	AI-Assisted Capsid Engineering for Next Generation Gene and Cell Therapy Lijia Ma Leading Scientist, Changping Laboratory; Founder, Westlake Genetech
09:30-10:00	Rare Disease Drug Target Discovery and First-in-Class Drug Development Cong Xu Co-Founder and Chief Operating Officer, Drug Farm
10:00-10:30	Tea Break
10:30-11:00	AI for Life Science: From Biomolecules to Cells Stan Z. Li Chair Professor in Artificial Intelligence, Westlake University; Chief Scientist, BioMap
11:00-11:30	When Neuro Disorders Become Rare for AI: Overcoming Data Scarcity and Collection Complexity in Epilepsy Alexander E. Hramov Corresponding Member of the Russian Academy of Sciences; Director and Chief Research Scientist of the Research Institute of Applied AI and Digital Solutions at Plekhanov Russian University of Economics; Chief Research Scientist at Pirogov National Medical and Surgical Center
11:30-12:00	Spatiotemporal Proteomics for Constructing Virtual Cell Models Tiannan Guo Tenured Associate Professor, School of Medicine & School of Life Sciences, Westlake University; Special Adviser to the President of Westlake University

Time: June 26 08:30–10:10
Venue: PRIMUS Hall B (2F)

Development and Application of Biobanks	
Chair: Yue Huang Director, Human Brain & Tissue Bank, China National Clinical Research Center for Neurological Diseases	
08:30-08:55	International Collaborative Cohort Studies of NHGRC Xingyu Wang Senior Researcher, National Human Genetic Resources Center; Senior Scientist, Beijing Hypertension League Institute
08:55-09:20	Introduction to SMART National Biobank of China Yiming Bao Professor, Chief Technology Officer and Chief Operating Officer, National Biobank of China, Shenzhen Medical Academy of Sciences
09:20-09:45	Human Brain Banking for Rare Neurological Disorders Yue Huang Director, Human Brain & Tissue Bank, China National Clinical Research Center for Neurological Diseases
09:45-10:10	Rare Disease Annotation System for Artificial Intelligence Diagnosis Tieliu Shi Professor, School of Life Sciences, East China Normal University

Scientific Program

Time: June 26 10:30–12:00
Venue: PRIMUS Hall B (2F)

Unpacking Patient-Driven Rare Disease Research Innovation	
Chair: Yeyang Su Advisor, Hope for Rare Foundation; Independent Researcher	
10:30-10:45	Participant and Caregiver Perspectives on Advanced Prenatal Therapies in Rare Disease Emma Canepa Clinical Trial Program Manager, Center for Maternal Fetal Precision Medicine, and Program Manager, Center for Genome Surgery at UCSF
10:45-11:00	The Importance, for Good and for Bad, of Patient-Led Research for Rare Diseases Heidi Carmen Howard Senior Researcher, Chalmers University of Technology; Head of ELSI, SciLifeLab, Sweden
11:00-11:15	The Advantages and Challenges of Rare Disease Therapeutics Development in China Xiao Xiao Co-Founder, Hope for Rare Foundation; Co-Founder, Chairman & CSO, Belief BioMed Group
11:15-11:30	The Paradoxes of Patient-Driven Rare Diseases Research and Innovation Yeyang Su Advisor, Hope for Rare Foundation; Independent Researcher
11:30-12:00	Panel Discussion Emma Canepa / Heidi Carmen Howard / Xiao Xiao / Yeyang Su

Time: June 26 14:00–17:30
Venue: PRIMUS Hall A (2F)

Model Systems Used in Rare Diseases Research	
Chair: Zhefan Stephen Chen Assistant Professor, School of Life Sciences, The Chinese University of Hong Kong	
14:00-14:30	Research and Development of Innovative Therapies for Rare Diseases Seng H. Cheng Senior Vice President, Head of Research and Product Development, Alexion, AstraZeneca Rare Disease
14:30-15:00	Modeling Rare Disease with Intellectual Disabilities in Rodents, Paving the Way from the Understanding Gene Function to Therapeutics Yann Hérault Exceptional Class Research Director, Centre National de la Recherche Scientifique (CNRS), France
15:00-15:30	Huntington Disease Animal Models and Treatments Xiao-Jiang Li Professor, Guangdong-HongKong-Macao Institute of CNS Regeneration, Jinan University; Director, Guangdong Key Laboratory of Non-Human Primate Research
15:30-16:00	Tea Break
16:00-16:30	From Genetic Targets to Therapeutic Strategies: Phenotypic Mapping and Translational Validation in Non-Human Primate Models of Rare Diseases Yuyu Niu Dean of the Medical Faculty and Vice President of Kunming University of Science and Technology
16:30-17:00	From Fruit Flies to Human Minds: Neuronal Pruning and Its Implications in Autism Fengwei Yu Distinguished Investigator, Temasek Life Sciences Laboratory, National University of Singapore
17:00-17:30	Modeling Repeat Expansion Diseases with Stem Cells: From Mechanism to Therapy Zhefan Stephen Chen Assistant Professor, School of Life Sciences, The Chinese University of Hong Kong

Scientific Program

Time: June 26 14:00–17:30
Venue: GREENLAND Hall 1 (1F)

Gene Therapy: Challenges and Perspectives	
Chair: Guangping Gao Professor & Chair, Department of Genetic and Cellular Medicine, UMass Chan Medical School; Past President, American Society of Gene & Cell Therapy	
14:00-14:30	New AAV Technologies for CNS Gene Therapy Fengfeng Bei Assistant Professor, Brigham and Women's Hospital, Harvard Medical School
14:30-15:00	Gene Therapy for IQSEC2 Disease Andrew Levy Professor, Rappaport Faculty of Medicine, Technion Israel Institute of Technology
15:00-15:30	Gene Therapy for Hearing Loss: From Research and Development to Multicenter Clinical Implementation Yilai Shu Deputy Dean, Professor and Chief Physician, Eye & ENT Hospital of Fudan University; Director, Hereditary Deafness Diagnosis and Treatment Center, Eye & ENT Hospital of Fudan University; Director, Shanghai Key Laboratory of Gene Editing and Cell Therapy for Rare Diseases
15:30-16:00	Tea Break
16:00-16:30	Overcoming Gene Therapy Limitations to Achieve Safe and Efficient Gene Transfer in Genetic Rare Diseases Giuseppe Ronzitti Research Director, French Institute of Health and Medical Research (INSERM); Director of the Research Strategy, Genethon
16:30-17:00	Human Gene Therapy: Challenges and Opportunities Guangping Gao Professor & Chair, Department of Genetic and Cellular Medicine, UMass Chan Medical School; Past President, American Society of Gene & Cell Therapy
17:00-17:30	Precision Silencing: The Therapeutic Potential of AAV-Delivered microRNA in Rare Neuromuscular Disorders Rachel Salzman CEO, Armatus Bio

Time: June 26 14:00–15:40
Venue: GREENLAND Hall 3 (1F)

Clinical Advances in Gene Editing	
Chair: Hui Yang Principal Investigator and Director of the Gene Therapy Center at the Shanghai Institute of Materia Medica, Chinese Academy of Sciences	
14:00-14:25	Development of in vivo Gene Editing Therapy Yuxuan Wu Co-Founder and CEO, YolTech Therapeutics
14:25-14:50	Arbor Biotechnologies: Leaders in Next Generation Gene Editing Don Haut Chief Business Officer, Arbor Biotechnologies
14:50-15:15	First-in-Human Results of HG204 CRISPR-Cas13 RNA-Editing Therapy for MECP2 Duplication Syndrome Hui Yang Principal Investigator and Director of the Gene Therapy Center at the Shanghai Institute of Materia Medica, Chinese Academy of Sciences
15:15-15:40	Advancing Innovative Gene Editing Therapies with tBE Lijie Wang Head of Scientific Innovation, CorrectSequence Therapeutics

Scientific Program

Time: June 26 16:00–17:40
Venue: GREENLAND Hall 3 (1F)

Novel Targets for Small Molecules and Antibodies	
Chair: Qin Guo VP, Head of Clinical Development Rare Disease, AstraZeneca R&D China	
16:00-16:25	Hypophosphatasia (HPP) Drug Development Journey Qin Guo VP, Head of Clinical Development Rare Disease, AstraZeneca R&D China
16:25-16:50	Aligning Disease Biology with Therapeutic Platforms: Emerging Modalities for Rare Disorders S. Pablo Sardi Global Head, Rare Diseases Research, Sanofi
16:50-17:15	Neurofibromatosis Type 1, Langerhans Cell Histiocytosis and Luvometinib: From Science to Clinical Practise Woody Tang Chief Medical Officer, Global R&D Center, Fosun Pharma
17:15-17:40	Strategies for the Development of Orphan Drugs Xia Chen Chief Medical Officer of Tigermed

Time: June 26 14:00–15:40
Venue: PRIMUS Hall B (2F)

Breakthroughs from Early-Career Scientists	
Chair: Min Tang Associate Professor, Shanghai University of Traditional Chinese Medicine; Chief Scientist, Cyberiad Biotechnology (Shanghai)	
14:00-14:25	Development of High-Performance Mitochondrial Base Editors for Generation and Correction of mtDNA Disease Models Liang Chen Principal Investigator, Lingang Laboratory, Shanghai; Principal Investigator (Jointly Appointed), School of Pharmacy, East China Normal University
14:25-14:50	Molecular Basis of Human Preimplantation Developmental Failure Wencheng Zhu Junior Principal Investigator, Institute of Molecular Physiology, Shenzhen Bay Laboratory
14:50-15:15	Go Beyond Building Virtual Cell with AI Yu Li Assistant Professor, Department of Computer Science and Engineering, The Chinese University of Hong Kong
15:15-15:40	Biomanufactured Human Tissues for Cancer Research and Drug Discovery Min Tang Associate Professor, Shanghai University of Traditional Chinese Medicine; Chief Scientist, Cyberiad Biotechnology (Shanghai)

Scientific Program

Time: June 26 16:00–17:40
Venue: PRIMUS Hall B (2F)

Progress of the Hope for Rare Foundation's Research Projects	
	Chair: Juan Huang Project Manager of Hope for Rare Foundation, Project Manager of Chinese Organization for Rare Disorders
16:00-16:25	SPG35: Exploration from Clinical Practice to Gene Therapy Li Cao Director of the Departments of Neurology and Genetics & Rare Diseases, Shanghai Sixth People's Hospital Affiliated to Shanghai Jiao Tong University School of Medicine; Head of Shanghai Neurological Rare Disease Biobank and Precision Diagnostic Technical Service Platform; President of the Clinical Genetics Professional Committee, Shanghai Medical Doctor Association
16:25-16:50	Exploration of Gene Therapy for Autosomal Recessive Spinocerebellar Ataxia 20 Guangzuo Luo Professor, China Medical University; Founder, Bionce Biotechnology
16:50-17:15	Development and Clinical Translation of Gene Therapy for Pediatric Rare Diseases Yang Yang Professor, State Key Laboratory of Biotherapy, Sichuan University
17:15-17:40	Development of a Highly Efficient Compact CRISPR/Cas12j System Based on Protein Conservation Yue Zhang Principal Investigator of the Hundred Talents Program, Liangzhu Laboratory, Zhejiang University; Member of the Gene Editing Technology Branch, China Association of Medical Biotechnology; Deputy Director of the Cell and Gene Therapy Platform, Liangzhu Laboratory

Time: June 26 19:00–21:00
Venue: PRIMUS Hall A (2F)

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 Guangping Gao Professor & Chair, Department of Genetic and Cellular Medicine, UMass Chan Medical School; Past President, American Society of Gene & Cell Therapy Fengfeng Bei Assistant Professor, Brigham and Women's Hospital, Harvard Medical School Wensheng Wei Professor at the School of Life Sciences, the Biomedical Pioneering Innovation Center and the Peking University–Tsinghua University Joint Center for Life Sciences, Peking University; Director of the Genome Editing Research Center, Peking University; Lead Scientist at Changping Laboratory Guangzuo Luo Professor, China Medical University; Founder, Bionce Biotechnology Yang Yang Professor, State Key Laboratory of Biotherapy, Sichuan University Wei Zhang Associate Director of ASO Core Research, n-Lorem Foundation

Scientific Program

Time: June 27 08:30–12:10
Venue: PRIMUS Hall A (2F)

Undiagnosed Diseases & Medical Genetics	
Chair: Yi Wang Director, National Children's Medical Center; President, Children's Hospital of Fudan University	
08:30-09:00	The Story Behind the Discovery of Novel Causal Genes for Skeletal Dysplasia Long Guo Professor, Department of Laboratory Animal Science, School of Basic Medical Sciences, Xi'an Jiaotong University; Director, Center for Intelligent Healthcare Investigation of Rare diseases, Translational Medicine Institute, Xi'an Jiaotong University; Visiting Professor, Center of Medical Genetics, Northwest Women's and Children's Hospital
09:00-09:30	AI-Powered RNA-Guided Diagnostics for Rare Diseases Yi Xing Francis West Lewis Chair, Children's Hospital of Philadelphia; Associate Chief Scientific Officer for Omics, Technology & Engineering, Children's Hospital of Philadelphia; Professor, Department of Pathology and Laboratory Medicine, University of Pennsylvania
09:30-10:00	Modeling and Analysis of Novel Gene Functions in Human Hereditary Diseases Feng Zhang Professor, Institute of Medical Genetics and Genomics, Fudan University
10:00-10:30	Tea Break
10:30-10:55	Common Paediatric Imprinting Diseases: Diagnostic Challenge and Management Ho-Ming Luk Chief of Service, Department of Clinical Genetics, Hong Kong Children's Hospital
10:55-11:20	Implementation of a Multicenter Project for the Diagnosis of Complex Birth Defects Yi Wang Director, National Children's Medical Center; President, Children's Hospital of Fudan University
11:20-11:45	Interpretable Diagnosis of Neuropsychiatric Disorders from Resting-State fMRI: Biologically Inspired Feature Selection and Contrastive Disentanglement Semen A. Kurkin Principal Investigator at Plekhanov Russian University of Economics
11:45-12:10	Advances in Screening and Genetic Research of Disorders of Sex Development (DSD) Hongwei Jiang Professor and Chief Physician, Vice President of the First Affiliated Hospital and Vice Dean of the Clinical Medical College of Henan University of Science and Technology

Time: June 27 08:30–12:00
Venue: GREENLAND Hall 1 (1F)

RNA-Based Therapeutics: From Antisense Oligonucleotides to mRNAs	
Chair: Oxana Iliach Vice Chair of Regulatory Scientific Committee, International Rare Disease Research Consortium (IRDiRC); Senior Director of Regulatory Strategy, Certara	
08:30-09:00	RNA-Based Therapeutics Daniel Scherman Director, French Foundation for Rare Diseases; Head of the Scientific Secretariat of International Rare Disease Consortium (IRDiRC); Head of the Medicine and Life Science Division of the European Academy of Science
09:00-09:30	A Non-Profit ASO Biotech is Transforming the Landscape of Personalized Medicines Wei Zhang Associate Director of ASO Core Research, n-Lorem Foundation
09:30-10:00	Accelerating Genetic Disease Drug Development Through Patient Partnership in China James Li Co-Founder and President, GondolaBio
10:00-10:30	Tea Break
10:30-11:00	Nucleic Acid Drug Weihong Tan Academician of the Chinese Academy of Sciences; Director of the Hangzhou Institute of Medicine, CAS; Dean of the Cancer Hospital Affiliated with Hangzhou Institute of Medical Sciences, CAS
11:00-11:30	New Avenues Towards Nucleic Acids Therapeutics Yu Wang Principal Investigator, Shenzhen Institute of Advanced Technology, Chinese Academy of Sciences
11:30-12:00	ASO-Mediated Upregulation of JAGGED1 Protein for Potential Treatment of Alagille Syndrome Lingdong Kong Senior Director, Head of Clinical Development, Suzhou Arnatar Therapeutics Co., Ltd.

Scientific Program

Time: June 27 08:30–12:00
Venue: GREENLAND Hall 3 (1F)

Development of Vectors and Delivery System	
Chair: Bowen Li Associate Professor, University of Toronto; Canada Research Chair in RNA Vaccines and Therapeutics; GSK Chair in Pharmaceutics and Drug Delivery	
08:30-08:55	Navigating AAV Gene Therapy Commercial Manufacturing: An Integrated Framework from Process Bottlenecks to Regulatory Release Suli Liu Project Leader, Shanghai OBiO Technology (Group) Corp., Ltd.
08:55-09:20	Biomimetic Glycan Repair for Precision AAV Engineering: From Deciphering Natural Glycosylation to Programmable Gene Therapy Chuanling Zhang Associate Researcher, School of Pharmacy, Peking University
09:20-09:45	Dyno Therapeutics: Advancing AI and Delivery Frontiers to Empower Patients with Genetic Agency Eric Kelsic CEO and Co-Founder, Dyno Therapeutics
09:45-10:10	AAV Capsid Engineering and Application Ye Bu CSO, PackGene Biotech
10:10-10:30	Tea Break
10:30-11:00	Development of Gene Therapy Technologies for Brain Disorders Zhonghua Lu Principal Investigator, Shenzhen Institute of Advanced Technology, Chinese Academy of Sciences
11:00-11:30	AI-Driven Development of Lipid Nanoparticles for mRNA Delivery and Gene Therapy Bowen Li Associate Professor, University of Toronto; Canada Research Chair in RNA Vaccines and Therapeutics; GSK Chair in Pharmaceutics and Drug Delivery
11:30-12:00	Nonionic Nucleic Acid Delivery System Hongzhang Deng Professor, Xidian University

Scientific Program

Time: June 27 08:30–12:00
Venue: PRIMUS Hall B (2F)

Regulatory Innovation in Advanced Therapies	
Chair: Janet Woodcock Former Acting Commissioner, U.S. Food and Drug Administration (FDA) Former Director, FDA's Center for Drug Evaluation and Research	
08:30-09:00	Advancing Innovation in Rare Diseases: Insights from the European Medicines Agency Emmanuel Cormier Head of Regulatory Science and Innovation Task Force, European Medicines Agency
09:00-09:30	Addressing the Evidence Scarcity in Rare Disease: The Bayesian Rare-Disease Integration of Evidence Framework (BRIEF) Jaime Caro Professor (adj), McGill University; Professor in Practice, London School of Economics; Chief Scientist, Evidera-Thermo Fisher Scientific
09:30-10:00	Advancing the Development of Gene Therapy for Rare Disorders Peter Marks Senior Vice President of Molecule Discovery and Head of Infectious Diseases, Eli Lilly and Company; Former Director, Center for Biologics Evaluation and Research, U.S. Food and Drug Administration
10:00-10:30	Tea Break
10:30-11:00	Accelerating Patient Access to Advanced Therapies for Rare Diseases: A PMDA Regulatory Science Perspective Manabu Inoue Chief Medical Officer, Pharmaceuticals and Medical Devices Agency, Japan
11:00-11:30	Current Status and Prospects of Patient-Centered Policies for Rare Disease Drug Registration and Medical Insurance Access Yue Yang Principal Investigator and Director of Center of Excellence for Translational and Regulatory Science, School of Pharmacy, Tsinghua University
11:30-12:00	Advancing Drug Development in Rare Diseases: Regulatory Challenges, Evidence Generation, and Innovation Antonella Isgrò Senior Clinical Assessor, Italian Medicines Agency; Member of the Haematology Working Party, European Medicines Agency

Time: June 27 14:00–17:30
Venue: PRIMUS Hall A (2F)

Natural History & Clinical Management of Rare Diseases	
Chair: Zhi-Ying Wu Qiushi Distinguished Professor, Zhejiang University; Director, Department of Medical Genetics and Center for Rare Diseases, Second Affiliated Hospital, Zhejiang University School of Medicine; Director, Zhejiang Provincial Key Laboratory of Precision Diagnosis and Treatment and Clinical Translation of Rare Diseases	
14:00-14:30	Rubinstein-Taybi Syndrome (RSTS): Natural History, Management and Treatment Oliver Bartsch Retired Associate Professor in Human Genetics, University Medical Center Mainz, Johannes Gutenberg University Mainz
14:30-15:00	Natural History and Population-Specific Integrated Staging System of Chinese Patients with Huntington's Disease Zhi-Ying Wu Qiushi Distinguished Professor, Zhejiang University Director, Department of Medical Genetics and Center for Rare Diseases, Second Affiliated Hospital, Zhejiang University School of Medicine Director, Zhejiang Provincial Key Laboratory of Precision Diagnosis and Treatment and Clinical Translation of Rare Diseases
15:00-15:30	CADASIL: A Paradigmatic Genetic Small Vessel Disease Hugues Chabriat Professor of Neurology, Head of the Translational Neurovascular Center, Coordinator of the National Reference Center for Rare Cerebrovascular and Ocular Diseases (CERVCO), Lariboisière Hospital, Université Paris Cité
15:30-16:00	Tea Break
16:00-16:30	Case-Driven Natural History Study: Exploring the Clinical Phenotypic Spectrum and Precision Interventions in WFS1-Related Disorders Yu Ding Associate Chief Physician and Deputy Director, Department of Endocrinology and Metabolism, Shanghai Children's Medical Center, Shanghai Jiao Tong University School of Medicine / National Children's Medical Center (Shanghai)
16:30-17:00	High Risk Screening and Gene Therapy in Children with Fabry Disease in China Jianhua Mao Dean and Professor of Department of Nephrology and Urology, President of Children's Hospital of Zhejiang University School of Medicine
17:00-17:30	Epigenetics and Predictive Modeling in the Clinical Cohort of Facioscapulohumeral Muscular Dystrophy (FSHD) Zhiqiang Wang Chief Physician and Professor, The First Affiliated Hospital of Fujian Medical University

Scientific Program

Time: June 27 14:00–17:30
Venue: GREENLAND Hall 1 (1F)

Clinical Advances in Gene Therapy	
Chair: Xiao Xiao Co-Founder, Hope for Rare Foundation; Co-Founder, Chairman & CSO, Belief BioMed Group	
14:00-14:30	Developing Brain-Penetrable AAV Gene Therapy for Succinic Semialdehyde Dehydrogenase Deficiency (SSADHD) Henry Lee Senior Scientist of FM Kirby Neurobiology Center, Assistant Director of Experimental Neurophysiology Core, Preclinical Science Program Manager of Translational Neuroscience Center, Boston Children's Hospital, Harvard Medical School
14:30-15:00	Development of the Next Generation of AAV Vector for Spinal Muscular Atrophy Weidong Xiao Professor of Pediatrics, Grzegorz Nalepa Scholar for Molecular Therapy, and Associate Director of the Gene and Cell Therapy Program at the Herman B Wells Center for Pediatric Research, Indiana University
15:00-15:30	Toward a Pilot Gene Therapy Trial for ColQ Congenital Myasthenic Syndrome Al Hawkins Co-Founder & President, Amplo Biotechnology
15:30-16:00	Tea Break
16:00-16:30	Gene Therapy for Rare Diseases: Our Experience in China Xiao Xiao Co-Founder, Hope for Rare Foundation; Co-Founder, Chairman & CSO, Belief BioMed Group
16:30-17:00	rAAV Gene Therapy of Fabry Disease Biao Dong Professor, State Key Laboratory of Biotherapy, Sichuan University; Chairman, Sichuan Real&Best Biotech
17:00-17:30	Targeting the Most Common Deafness Gene GJB2: A Milestone Breakthrough from R&D to First-in-Human Clinical Trial Hongxing Wang Chief Technology Officer, Euhearing Therapeutics

Time: June 27 14:00–17:30
Venue: GREENLAND Hall 3 (1F)

Emerging Frontiers and Applications of Cell Therapy	
Chair: Lizhao Feng Principal Investigator, Oujiang Laboratory	
14:00-14:30	Human iPSC-Based Cell Therapy Development for Canavan Disease Yanhong Shi Professor and Chair, Department of Neurodegenerative Diseases, Beckman Research Institute of City of Hope
14:30-15:00	Therapeutic Effects of Stem Cells and Exosomes in the Treatment of Amyotrophic Lateral Sclerosis (ALS) and Autism Zhongmin Liu Dean of the Institute of Disaster Medicine Engineering at Tongji University; Tenured Professor and Honorary President of Shanghai East Hospital Affiliated to Tongji University; Foreign Member of the Russian Academy of Engineering
15:00-15:30	Muscle Stem Cell Therapy of Duchenne Muscular Dystrophy Ping Hu Principal Investigator and Professor, Guangzhou National Laboratory
15:30-16:00	Tea Break
16:00-16:30	The Application of EPC in Acute Ischemic Stroke Yuchun Gu Chief Scientist at Allife Medicine
16:30-17:00	Development of Universal iPSC-Based Cell Therapy for Neurological Diseases Shuning Zhang Senior Vice President, Medical and Clinical Affairs, Zhejiang Hopstem Bioengineering Company Limited
17:00-17:30	Turnover and Replacement of Microglia: From Bench to Clinical Therapies Bo Peng Distinguished Professor at Fudan University

Scientific Program

Time: June 27 14:00–15:40
Venue: PRIMUS Hall B (2F)

Driving Drug Development: Patient Organization and Foundation	
Chair: Linguo Li Director of Public Policy Research Center of Rare Diseases at the Chinese Organization for Rare Disorders	
14:00-14:25	Fundación Columbus: A Non-for-Profit Model to Accelerate Treatments for Ultra-Rare Diseases Javier García Cogorro Founder and Secretary of Fundación Columbus; Founder and General Partner of Columbus Venture Partners
14:25-14:50	The Importance of Collaboration and Shared Responsibility for Gene Therapy Sustainability Sean Russell Managing Partner, PrimeRA Pharma Partners LLP; Head of Regulatory Affairs, Fondazione Telethon ETS; Board of Trustees, Eyes on the Future
14:50-15:15	Patient-Driven Innovation for Rare Diseases: The AFM-Téléthon Model Jean-François Briand Director, Operations and Scientific Innovation, AFM-Téléthon
15:15-15:40	Advances in New Drug Development for Mucinous Tumors Zhenglong Sun Principal Investigator, Interdisciplinary Research Group on Microscopic Imaging and Drug Development, Shenzhen Bay Laboratory

Time: June 27 16:00–17:40
Venue: PRIMUS Hall B (2F)

Driving Drug Development: Patient and Patient Family	
Chair: Linguo Li Director of Public Policy Research Center of Rare Diseases at the Chinese Organization for Rare Disorders	
16:00-16:25	By Patients, For Patients ™ : The Roles, Practices and Challenges of Patient-Driven Orphan Drug Development Richard Yang Rare Disease Patient; Founder and Chairman, Reflection Biotechnologies
16:25-16:50	From Diagnosis to Cure: Building a Patient-Driven Gene Therapy Program for CTNNB1 Syndrome Špela Miroševič Co-Founder and CEO, CTNNB1 Foundation
16:50-17:15	Personalized Medicine, Ultra Rare, and AI Yiwei She Founder and CEO, TNPO2 Foundation
17:15-17:40	A Journey To Cure Michael Terry Pirovolakis Founder, CureSPG50 Founder and CEO, Elpida Therapeutics

Symposiums

Time: June 25 08:30–12:00
Venue: PISCES Hall (1F)

Clinical Research Advances in Developmental and Epileptic Encephalopathies Symposium (Closed-Door) Host: Chinese Organization for Rare Disorders	
Chair: Bo Zhu Senior Advisor, Chinese Organization for Rare Disorders	
08:30-09:00	China's Rare Disease Cause and Industry: Policy Journey and Future Prospects Qi Kang Deputy Director of the Department of Health Policy Research, Shanghai Health Development Research Center (Shanghai Medical Information Center)
09:00-09:30	Advances in the Diagnosis and Treatment of Developmental and Epileptic Encephalopathy Yuwu Jiang Director of the Children's Medical Center and Pediatric Epilepsy Center, Peking University First Hospital; Dean of the Department of Pediatrics, Peking University Health Science Center
09:30-10:00	Cohort Study of Epilepsy-Related Rare Diseases Yi Wang Director, National Children's Medical Center; President, Children's Hospital of Fudan University
10:00-10:30	Tea Break
10:30-11:00	A Journey of Supporting Patients with Dravet Syndrome Ling Zhao Founder of Volunteers Group for Chinese Dravet Syndrome Patients
11:00-12:00	Panel Discussion

Symposiums

Time: June 26 08:30–12:00
Venue: QUBE Hall (2F)

Duchenne Muscular Dystrophy Symposium Hosts: Hope for Rare Foundation, Chinese Organization for Rare Disorders, China DUCHENNE Family Network	
Chair: Xinyue Han Parent of a Child with Duchenne Muscular Dystrophy (DMD); Member of the Committee, China DUCHENNE Family Network	
08:30-08:40	Opening Remarks by the Host Kevin Huang Founder and CEO of Hope for Rare Foundation, Founder and President of Chinese Organization for Rare Disorders
08:40-09:00	Two-Year Results from the Phase 1/2 Study of GNT0004, An AAV-Based Gene Therapy for Duchenne Muscular Dystrophy Giuseppe Ronzitti Research Director, French Institute of Health and Medical Research (INSERM); Director of the Research Strategy, Genethon
09:00-09:20	AAV Directed Gene Therapy of DMD with Full Dystrophin Functions Weidong Xiao Professor of Pediatrics, Grzegorz Nalepa Scholar for Molecular Therapy, and Associate Director of the Gene and Cell Therapy Program at the Herman B Wells Center for Pediatric Research, Indiana University
09:20-09:40	Progress in the Development of AAV Gene Therapy for Duchenne Muscular Dystrophy Yang Yang Professor, State Key Laboratory of Biotherapy, Sichuan University
09:40-10:00	DMD IIT and Phase 1/2 Study Exploring an Engineered Muscle Tropic AAV Capsid and a Novel Minigene Xiao Xiao Co-Founder, Hope for Rare Foundation; Co-Founder, Chairman & CSO, Belief BioMed Group
10:00-10:20	Tea Break
10:20-10:40	Safety and Efficacy of LE051: An Endogenous ADAR-Based RNA Editing Therapy for Duchenne Muscular Dystrophy Junyuan Han Vice President of Research and Development, Leaperbio Inc.
10:40-11:00	Targeting Epigenetics: Chidamide's Potential and Clinical Outlook for Duchenne Muscular Dystrophy (DMD) Song Shan Vice President, Early Stage Research, R&D Center, Shenzhen Chipscreen Biosciences Co., Ltd.
11:00-12:00	Panel Discussion Chair: Xuehui Xiao Parent of a Child with Duchenne Muscular Dystrophy (DMD); Member of the Committee, China DUCHENNE Family Network Panelists: Shekhar Natarajan Vice President of International Regulatory Affairs and Policy, Dyne Therapeutics; Chair of Therapies Scientific Committee, International Rare Disease Research Consortium (IRDiRC); Chair of R&D Focus Group, European Confederation of Pharmaceutical Entrepreneurs (EUCOPE) Cuijin Wang Attending Physician, Department of Neurology, Shanghai Children's Medical Center, Shanghai Jiao Tong University School of Medicine Giuseppe Ronzitti / Weidong Xiao / Yang Yang / Junyuan Han / Song Shan

Symposiums

Time: June 26 14:00–17:40
Venue: QUBE Hall (2F)

Rare Diseases Research Symposium: From Bench to Bedside Host: Institute of Medical Genetics and Genomics of Fudan University	
Chairs: Yu An Associate Professor and Director of Training Division, Institute of Medical Genetics and Genomics of Fudan University; Associate Professor, Human Phenome Institute of Fudan University Chenji Wang Associate Professor, School of Life Sciences, Fudan University	
14:00-14:25	FBXW4 Mutations Induced Over-Degradation of a RNA Binding Protein CCT5 Disrupts the Skeletal Development Hongyan Wang Dean of the Institute of Metabolism and Integrative Biology at Fudan University, Professor at the Obstetrics and Gynecology Hospital Affiliated with Fudan University
14:25-14:50	Genetic Determinants of Human Oocyte/Embryo Defects Qing Sang Professor, Institute of Biomedical Sciences, Fudan University
14:50-15:15	Decoding the Molecular Landscape of Epileptogenic Foci Yu An Associate Professor and Director of Training Division, Institute of Medical Genetics and Genomics of Fudan University; Associate Professor, Human Phenome Institute of Fudan University
15:15-15:40	Proteostasis Dysregulation and Neurodevelopmental Disorders Chenji Wang Associate Professor, School of Life Sciences, Fudan University
15:40-16:00	Tea Break
16:00-16:25	Implementation of the Chinese Children Genetic Kidney Disease Database in China Hong Xu Professor and Discipline Leader of Nephrology, Renal Transplantation, and Rheumatology, Children's Hospital of Fudan University
16:25-16:50	Unsolved Mysteries of Immune-Related Diseases: A Clinical Perspective Xiaochuan Wang Director and Professor, Department of Allergy and Clinical Immunology, Children's Hospital of Fudan University
16:50-17:15	Novel Mechanistic Insights into Genetic Hypotrichosis Ming Li Administrative Director and Chief Physician, Department of Dermatology, Children's Hospital of Fudan University
17:15-17:40	Clinical Hereditary Disease Testing System & ISoGenetic Intelligent Platform Li Zhang Director of Bioinformatics, Fujungenetics Technologies Inc.

Symposiums

Time: June 27 08:30–12:10
Venue: QUBE Hall (2F)

Rare Tumors Symposium Host: Zhejiang Cancer Hospital	
Chairs: Huarong Tang Chief Physician, Zhejiang Cancer Hospital Qiang Wen Deputy Director (Acting) of the Pediatric Oncology Department and Associate Chief Physician at Zhejiang Cancer Hospital	
08:30-08:55	Pimicotinib Versus Placebo for Tenosynovial Giant Cell Tumour (MANEUVER): An International, Randomised, Placebo-Controlled, Phase 3 Trial Xiaohui Niu Director of Bone and Soft Tissue Tumor Diagnosis and Treatment Research Center, Beijing Jishuitan Hospital
08:55-09:20	Non-Viral Gene Therapy Strategies for Rare Diseases Jie Song Principal Investigator, Hangzhou Institute of Medicine, Chinese Academy of Sciences
09:20-09:45	Prospects for Cervical Tumor Interception Strategies in Peutz-Jeghers Syndrome Yu Kang Chief Physician and Director of the Phase I Clinical Trial Unit, Obstetrics & Gynecology Hospital of Fudan University
09:45-10:10	WGAN-Enhanced Dual-Stream GCN for Proteomics Uncovers Diagnostic Biomarkers of Gastric-Type Adenocarcinoma of the Uterine Cervix Huarong Tang Chief Physician, Zhejiang Cancer Hospital
10:10-10:30	Tea Break
10:30-10:55	Antibody Drug Conjugates for Treating Childhood Cancers Peng Guo Professor, Hangzhou Institute of Medicine, Chinese Academy of Sciences
10:55-11:20	Salivary Gland Cancer: Past and Present Meiyu Fang Chief Physician, Professor and Director of the Department of Head and Neck and Rare Cancers, Zhejiang Cancer Hospital
11:20-11:45	Progress in the Diagnosis and Treatment of Langerhans Cell Histiocytosis Yamin Tan Director and Chief Physician of the Hematology Department, Zhejiang Cancer Hospital
11:45-12:10	Diagnosis, Treatment and a Case Report of Uterine Adenosarcoma in Children Qiang Wen Deputy Director (Acting) of the Pediatric Oncology Department and Associate Chief Physician at Zhejiang Cancer Hospital

Symposiums

Time: June 27 14:00–17:30
Venue: QUBE Hall (2F)

Rare Diseases Symposium Hosts: Liangzhu Laboratory & Children's Hospital of Zhejiang University School of Medicine	
Chairs: Xudong Fu Principal Investigator of the Hundred Talents Program, Liangzhu Laboratory, Zhejiang University Liang Gong Principal Investigator of the Hundred Talents Program, Liangzhu Laboratory, Zhejiang University	
14:00-14:30	AI-Driven Therapeutic Antisense Oligonucleotide for Processing-Deficient Progeroid Laminopathies Ning Shen Principal Investigator of the Hundred Talents Program, Liangzhu Laboratory, Zhejiang University
14:30-15:00	From Enzyme Replacement to Functional Cure: Exploring Gene Therapy for Fabry Disease Zhihong Lu Chief Physician, Children's Hospital of Zhejiang University School of Medicine
15:00-15:30	The Role of Epigenetic Factor SP100 in HGPS and Aging Xudong Fu Principal Investigator of the Hundred Talents Program, Liangzhu Laboratory, Zhejiang University
15:30-16:00	Tea Break
16:00-16:30	Exploration of the Safety and Efficacy of Gene Therapy for Mucolipidosis Type IV Xin Zhang Chief Physician, Children's Hospital of Zhejiang University School of Medicine
16:30-17:00	Sequential Sequencing Reveals the Architecture and Complexity of Genomic Variants in Patients with Alport Syndrome Liang Gong Principal Investigator of the Hundred Talents Program, Liangzhu Laboratory, Zhejiang University
17:00-17:30	Precision Diagnosis and Treatment of Rare Diseases: Insights from the Natural History and Cohort Studies of Spinal Muscular Atrophy Shanshan Mao Chief Physician and Professor, Children's Hospital of Zhejiang University School of Medicine

Satellite Meetings

Time: June 26 12:30–13:30
Venue: PRIMUS Hall A (2F)

Biogen Satellite Meeting	
Chair: Yi Wang Director, National Children's Medical Center; President, Children's Hospital of Fudan University	
12:30-12:35	Opening Remarks Yi Wang Director, National Children's Medical Center; President, Children's Hospital of Fudan University
12:35-13:00	Beyond the Natural History: An Evidence-Based Analysis of Long-Term Treatment Benefits in SMA Shanshan Mao Chief Physician and Professor, Children's Hospital of Zhejiang University School of Medicine
13:00-13:25	Every Neuron Matters: Long-Term Benefits of Nusinersen in Patients with Type 1 SMA Yunhong Wu Chief Physician and Director of the Department of Neurology, Shanxi Children's Hospital
13:20-13:30	Closing Remarks Yi Wang Director, National Children's Medical Center; President, Children's Hospital of Fudan University

Time: June 27 12:30–13:30
Venue: PRIMUS Hall A (2F)

BerryGenomics Satellite Meeting	
Chair: Yi Wang Director, National Children's Medical Center; President, Children's Hospital of Fudan University	
12:30-12:35	Opening Remarks Yi Wang Director, National Children's Medical Center; President, Children's Hospital of Fudan University
12:35-13:00	Precision Diagnosis and Treatment of Hereditary Deafness Yilai Shu Deputy Dean, Professor and Chief Physician, Eye & ENT Hospital of Fudan University; Director, Hereditary Deafness Diagnosis and Treatment Center, Eye & ENT Hospital of Fudan University; Director, Shanghai Key Laboratory of Gene Editing and Cell Therapy for Rare Diseases
13:00-13:25	Applications of Artificial Intelligence in Genetic Disease Diagnosis and Genetic Counseling Yulin Jiang Director of Prenatal Diagnosis Center, Deputy Director of Obstetrics Center, Peking Union Medical College Hospital
13:25-13:30	Closing Remarks Yi Wang Director, National Children's Medical Center; President, Children's Hospital of Fudan University

(Alphabetical Order by Last Name) Speaker Profiles

Yu An

Associate Professor and Director of Training Division, Institute of Medical Genetics and Genomics of Fudan University
Associate Professor, Human Phenome Institute of Fudan University

Yu An, Director of Training Division at the Institute of Medical Genetics, and Associate Research Professor at the Human Phenome Institute, Fudan University; Visiting Scholar at the Center for Genomic Medicine, Massachusetts General Hospital and Harvard Medical School.
Her research focuses on the genetic mechanisms and etiology of genetic disorders, including the genetic basis of birth defects, molecular pathogenesis of neurodevelopmental disorders, genotype–phenotype correlations, identification of mutation spectra, and genetic counseling research.
She serves as editor-in-chief of the graduate textbook Genetic Counseling, and has received the Graduate Teaching Achievement Award of Fudan University, as well as grants from the Fudan University Graduate Course Supporting Textbook Program and the Professional Degree Course Development Program.
She is a Young Editorial Board Member of the Journal of Translational Genetics and Genomics (JTGG). She also serves as Secretary-General and Committee Member of the Genetic Diagnosis Branch of the Genetics Society of China, Council Member of the Shanghai Society of Genetics, Vice Chair of the Clinical Genetics and Genetic Counseling Committee of the Shanghai Society of Genetics, Young Council Member of the Mitochondrial Biology Branch of the Biophysical Society of China, and Expert in Genetic Disease Molecular Diagnostics Quality Control at the Shanghai Center for Clinical Laboratory.

Yoko Aoi

Coordination Director, Office of Review Management, Pharmaceuticals and Medical Devices Agency, Japan

Dr. Yoko Aoi is a coordination director of Office of Review Management. After joining PMDA, Dr. Aoi was involved in review and scientific advice on new drugs for rheumatoid arthritis, asthma, COPD etc. (2011-2016), planning and coordinating international activities such as PMDA Asia Training Center (2016-2019) and review and scientific advice on oncology drugs (2019-2025). In addition, Dr. Aoi is leading Orphan drugs WG which investigates problems in development and application review, and proposes support measures in order to contribute to the orphan drug development. Moreover, Dr. Aoi was involved in ICH E17 EWG/IWG as Deputy Topic Leader from 2014 to 2020. Dr. Aoi holds a PhD from Tokyo Dental and Medical University.

Ge Bai

Qiushi Distinguished Professor, Zhejiang University

Professor Ge Bai earned his Ph.D. from the Shanghai Institute of Biochemistry and Cell Biology, Chinese Academy of Sciences. He subsequently conducted postdoctoral research in the laboratory of Professor Sam Pfaff at the Salk Institute for Biological Studies/HHMI. In 2017, he joined Zhejiang University as a faculty member. His laboratory currently focuses on motor circuit development and related disorders. His work has been published in leading journals including Cell (2023, 2011), Nature (2015), Neuron (2024, 2011), and Developmental Cell (2007). Dr. Bai has received several honors, including the First Prize of the Shanghai Natural Science Award, and has been supported by the Original Exploration Project of the National Natural Science Foundation of China (NSFC) and the National Talent Project.

Speaker Profiles (Alphabetical Order by Last Name)

B

Yiming Bao

Professor, Chief Technology Officer and Chief Operating Officer, National Biobank of China, Shenzhen Medical Academy of Sciences

Yiming Bao, Chief Technology Officer and Chief Operating Officer, National Biobank of China, Shenzhen Medical Academy of Sciences. Dr. Bao earned his Bachelor' s degree in Biochemistry from Peking University and his PhD in Genetics from the John Innes Centre, UK. He has previously served as Director of the National Genomics Data Center, Professor at the China National Center for Bioinformation, Distinguished Core Researcher of the Chinese Academy of Sciences, and Staff Scientist at the National Center for Biotechnology Information, USA. His research focuses on the storage, management, sharing and utilization of biological samples and bioinformatics big data. He serves as the Chief Scientist for two National Key R&D Programs and one Strategic Priority Research Program (Category B) of the Chinese Academy of Sciences. He is a recipient of the NIH Merit Award from the United States. His team' s achievements have been repeatedly selected among the Top 10 Advances in Bioinformatics in China. He is recognized as a recipient of the Special Government Allowance.

Oliver Bartsch

Retired Associate Professor in Human Genetics, University Medical Center Mainz, Johannes Gutenberg University Mainz

Dr. Oliver Bartsch is a 2026-retired associate professor of human genetics at the Johannes Gutenberg University Mainz, Germany. After attending a seminar with visiting professors of medicine from the PRC at the Free University of Berlin in 1977, he became part of one of the first Western travel groups to visit China since 1949. In 1978, he traveled to Beijing, Wuhan, and Guangzhou. After graduating from medical school at the FU Berlin, he had postgraduate medical training of pediatrics and fellowships of human genetics at the University Hospitals of Frankfurt, Lübeck and Dresden. Among his main interests are the scientific investigation of hereditary and congenital diseases and the application of this knowledge in genetic diagnostics and counseling. Since the mid-1980s he has been integrating latest diagnostic methodologies into clinical practice, including biochemical diagnostics, fluorescence in situ hybridization (FISH) and molecular genetic diagnostics.

Dr. Bartsch is author of 170 peer-reviewed articles. A passionate medical doctor, researcher and mentor, he is committed to training the next generation of medical geneticists. In 1993 he founded the German-Austrian-Swiss FISH External Quality Assessment scheme, heading it until 2008/2009 when he co-founded the CEQA (European) scheme for FISH diagnostics and merged the two. In 1996, he became a founding member of the Romanian-German Symposium on Medical Genetics at Oradea, Romania, of which he is a co-director since 2002. He was awarded prizes in Germany and, in 2019, the highest prize of the Romanian Society of Medical Genetics, the Golden Chromosome.

Dr. Bartsch continues to contribute to the genetics community through board memberships, advisory roles and collaborative research networks.

(Alphabetical Order by Last Name) Speaker Profiles

B

Fengfeng Bei

Assistant Professor, Brigham and Women's Hospital, Harvard Medical School

Dr. Fengfeng Bei received his B.Sc. in Biology from Tsinghua University, his M.Sc. from King's College London, and his Ph.D. in Clinical Neuroscience from University College London. Following postdoctoral training at Boston Children's Hospital, he joined Harvard Medical School, where he is currently an Assistant Professor of Neurosurgery and Principal Investigator at Brigham and Women's Hospital.

Dr. Bei's research focuses on gene therapy for neurological diseases, including AAV vector engineering, neural repair and regeneration, and oncolytic viral immunotherapy for brain cancers. Through collaborative efforts, his laboratory also conducts translational and early-stage clinical research targeting rare genetic disorders and brain cancers. His work has been published in leading journals such as Cell, Neuron, and Nature Biomedical Engineering.

Beyond academia, Dr. Bei has co-founded the biotechnology companies Brave Bio and XinGene Therapeutics, and works closely with global industry partners to advance gene therapy development. He is also actively engaged in rare disease advocacy, collaborating with international patient organizations to initiate and accelerate gene therapy programs for underserved conditions.

Jean-François Briand

Director, Operations and Scientific Innovation, AFM-Téléthon

Jean-François Briand is Director of Operations and Scientific Innovation at AFM-Téléthon, a leading French patient association dedicated to rare diseases research and treatment development, with a strong focus on neuromuscular disorders. In this role, he leads a team across four departments: Research and Development, Scientific Evaluations, Health Data & Medical Research, and International Scientific Networks, implementing the scientific strategy defined by the board of directors. Prior to AFM-Téléthon, Jean-François served as Head of Molecular Biology Oncology Department at Cytomics Pharmaceuticals, where he led research and development of drug candidates in oncology and nosocomial infections targeting the Ubiquitin-Proteasome pathway. Before he was working at BioProtein Technologies on recombinant complex proteins produced in transgenic animals. He holds a PhD in Microbiology and Virology from the University of Paris and Pasteur Institute as well as an executive education in Leadership and Entrepreneurship from HEC Paris. He has contributed to several publications and is named inventor on patents in the field of cancer treatment and drug screening methods.

Ye Bu

CSO, PackGene Biotech

Ye Bu, Ph.D. from Peking University, was a Postdoctoral Fellow at Johns Hopkins University School of Medicine and a Research Scientist in the Department of Biomedical Engineering at Purdue University. He currently serves as Chief Scientist at PackGene Biotech, leading the R&D and CRO business divisions. With over 15 years of extensive research experience, his expertise spans molecular biology, biochemistry, virus production, cell culture, and animal disease models.

Speaker Profiles (Alphabetical Order by Last Name)

C

Emma Canepa

Clinical Trial Program Manager, Center for Maternal Fetal Precision Medicine, and Program Manager, Center for Genome Surgery at UCSF

Emma Canepa, MS, CCRP, is a Clinical Research Program Manager at the University of California, San Francisco Center for Maternal Fetal Precision Medicine. She operationalizes and oversees cutting-edge investigator-initiated clinical trials in rare fetal and pediatric genetic diseases, serving as a "lighthouse" for families facing devastating diagnoses while guiding cross-functional teams through complex research. Her work bridges science, ethics, and humanity, drawing on skills in project management, communication, and risk management to ensure the responsible translation of basic science into clinical care. Grounded in reproductive justice and patient-centered care, she integrates qualitative inquiry to center patient and family experiences. Over nearly a decade at UCSF, she has helped build organizational infrastructure for growing research teams and has guided studies from protocol development through FDA and IRB submission, implementation, and publication.

Li Cao

Director of the Departments of Neurology and Genetics & Rare Diseases, Shanghai Sixth People' s Hospital Affiliated to Shanghai Jiao Tong University School of Medicine
Head of Shanghai Neurological Rare Disease Biobank and Precision Diagnostic Technical Service Platform
President of the Clinical Genetics Professional Committee, Shanghai Medical Doctor Association

Li Cao, Director of the Departments of Neurology and Genetics and Rare Diseases at Shanghai Sixth People' s Hospital Affiliated to Shanghai Jiao Tong University School of Medicine, has been recognized as a Shanghai Outstanding Academic/Technical Leader and a Shanghai Leading Talent in Health and Medical Sciences, and has been selected for the Shanghai "Double Hundred Talent Program" for Peak and Plateau Disciplines. He serves as President of the Clinical Genetics Professional Committee of the Shanghai Medical Doctor Association, Secretary of the Rare Diseases Branch of the Shanghai Medical Doctor Association, a member of the Neurogenetics Group of the Neurology Branch of both the Chinese Medical Association (CMA) and the Chinese Medical Doctor Association (CMDA), Vice-Chair of the Neuroimmunology, Genetics and Biochemistry Group of the Neurology Branch of the Shanghai Medical Association, and a member of the Neurology Branch of the Shanghai Medical Doctor Association. His long-term research focuses on clinical and translational studies of movement disorders, leukoencephalopathies, and neurogenetic diseases, and he has published more than 160 papers in journals such as Science, Brain, Neurology, and Advanced Science. He pioneered hematopoietic stem cell transplantation (HSCT) for hereditary diffuse leukoencephalopathy with spheroids (HDLS) in China, completing over 30 cases, cloned multiple pathogenic genes for paroxysmal movement disorders, and conducted AAV9-mediated gene therapy for spastic paraplegia type 35 (SPG35).

(Alphabetical Order by Last Name) Speaker Profiles

Jaime Caro

Professor (adj), McGill University
Professor in Practice, London School of Economics
Chief Scientist, Evidera-Thermo Fisher Scientific

J. Jaime Caro, MDCM, FRCPC, FACP, Professor in practice, LSE; Professor of Medicine, Epidemiology, Biostatistics (adj), McGill University; Honorary Full Professor with the Saw Swee Hock School of Public Health, National University of Singapore; Chief Scientist, Evidera. For Germany's IQWiG, Dr. Caro proposed an innovative approach to health technology assessment, involving efficiency frontiers. His recent work involves the development of a Bayesian framework approach to integrating all evidence for a rare disease treatment (BRIEF); development of DICE 2.0, a unified approach to modeling that enables rapid, standardized and less error-prone forecasting models; a broader approach than QALYs to value health benefits, the BADIE. He led ISPOR-SMDM's task forces for Good Modeling Practices and Modeling Quality Assurance and now co-chairs a second edition.

Hugues Chabriat

Professor of Neurology, Head of the Translational Neurovascular Center, Coordinator of the National Reference Center for Rare Cerebrovascular and Ocular Diseases (CERVCO), Lariboisière Hospital, Université Paris Cité

Hugues Chabriat, MD, PhD is Professor of Neurology at Université Paris Cité and senior neurologist at Assistance Publique–Hôpitaux de Paris, based at Lariboisière Hospital in Paris. He is internationally recognized for his work on cerebral small vessel diseases (cSVD), with a particular focus on hereditary vascular disorders of the brain, including CADASIL and COL4A1-related angiopathies.

He leads the Translational Neurovascular Center and coordinates the French National Reference Center for Rare Cerebrovascular and Ocular Diseases (CERVCO). He also co-leads the GENOVASC research team at the Paris Brain Institute, where his work combines clinical neurology, neuroimaging, genetics, and translational neuroscience to investigate the mechanisms of microvascular brain injury and their consequences for stroke and cognitive decline.

Prof. Chabriat has authored more than 300 peer-reviewed publications in leading international journals and has contributed to international recommendations on cerebral small vessel diseases. His current work focuses on MRI biomarkers of microvascular damage, genetic determinants of vascular brain injury, and the mechanisms linking vascular pathology with cognitive decline.

Speaker Profiles (Alphabetical Order by Last Name)

C

Liang Chen

Principal Investigator, Lingang Laboratory, Shanghai
Principal Investigator (Jointly Appointed), School of Pharmacy, East China Normal University

Liang Chen is a Principal Investigator and doctoral supervisor at Lingang Laboratory, with research focusing on gene editing technologies and gene therapy, he serves as a project leader for National High-Level Talent Program, National Key R&D Program of China, National Natural Science Foundation of China for Excellent Young Scholars and National Laboratory Key Program. Through modifying the catalytic substrates of deaminases and screening DNA damage repair factors, he has developed novel CRISPR-derived base editors such as Td-CGBE/CBE and ACBEs that enable new types of base conversion and highly precise nucleotide editing, providing safer and more effective tools for gene therapy. Recently, he has developed ultra-active and accurate mitochondrial base editors and conducted the first closed-loop therapeutic study of mitochondrial diseases through an "editing–modeling–correction" paradigm. As first or corresponding author (including co-first/co-corresponding), he has published 8 papers (4 ESI 1% highly cited papers) in high-level journals such as Nature Biotechnology (5 papers), Nature Chemical Biology, and Nature Cell Biology, these studies have been highlighted by Nature, Nature Reviews Genetics, Nature Biotechnology, etc. He has filed 16 patents including 4 PCT applications, of which three have been granted. He has won the Wu Rui Award of the Wu Rui Memorial Foundation, Shanghai Science & Technology 35 under 35 and Shanghai Outstanding Individual in Employment and Entrepreneurship, and serves as a young committee member of the Cell and Gene Therapy Branch of the Chinese Society for Cell Biology.

Xia Chen

Chief Medical Officer of Tigermed

Dr. Chen Xia holds an MD from Peking Union Medical College, a PhD in Clinical Pharmacology from Leiden University, and an MS in Quantitative Pharmacology from the University of Manchester.
Her professional experience includes serving as a Physician in the Department of Neurology and Head of the Phase I Unit at Peking Union Hospital, working as an Early-Stage Clinical Trial Researcher at the Human Medicine Research Center in Leiden, the Netherlands, acting as Director of the Institutional Office and Phase I Center at Beijing Tiantan Hospital, and serving as an External Clinical Reviewer at the Center for Drug Evaluation.
With over 20 years of experience in drug clinical development, she has contributed to the review of approximately 200 IND projects and 20 NDA projects at China CDE. She has been with Tigermed for over four years, overseeing medical strategies and protocols for more than 800 projects across a wide range of therapeutic areas and treatment types.

(Alphabetical Order by Last Name) Speaker Profiles

Zhefan Stephen Chen

Assistant Professor, School of Life Sciences, The Chinese University of Hong Kong

Dr. Zhefan Stephen Chen is currently an Assistant Professor at School of Life Sciences, The Chinese University of Hong Kong (CUHK). He completed his Ph.D. in Biochemistry at CUHK under the supervision of Professor Ho Yin Edwin Chan. Dr. Chen was awarded a Postdoctoral Fellowship under a Clinical Neurosciences program between CUHK and University of Oxford, where he was mentored by Prof. Kevin Talbot, Head of the Nuffield Department of Clinical Neurosciences. During his doctoral study and postdoctoral training, Dr. Chen investigated the pathogenic mechanisms of repeat expansion neurodegenerative diseases, including amyotrophic lateral sclerosis and polyglutamine diseases. The research program of Dr. Chen' s laboratory focuses on studying the neurobiology of primary cilium, a solitary hair-like cellular organelle. His group employs human stem cell-derived neuronal models, integrating molecular biology, cell biology, neurophysiology, and bioinformatics approaches to investigate the role of primary cilium in human neurodevelopment and neurological disorders.

Seng H. Cheng

Senior Vice President, Head of Research and Product Development, Alexion, AstraZeneca Rare Disease

Seng H. Cheng is responsible for drug discovery, process development and clinical supply as well as clinical biomarkers and bioanalytical discovery, rare disease diagnostics and bioinformatics, supporting Alexion' s pipeline from target discovery through post-marketing.

Seng was previously Senior Vice President and Chief Scientific Officer of the Rare Disease Research Unit at Pfizer. Prior to joining Pfizer, he held roles including Group Vice President of Genetic Diseases Science at Genzyme as well as the Global Head of Research of Rare Diseases at Sanofi. He has contributed to the development of several medicines for patients with rare diseases, co-authored over 280 research articles and reviews and is a named co-inventor on 64 issued US patents in the area of biotechnology.

Seng obtained his BSc and PhD degrees in Biochemistry at the University of London and trained as a postdoctoral fellow in the field of tumor biology at the National Institute of Medical Research, UK.

Speaker Profiles (Alphabetical Order by Last Name)

C
D

Emmanuel Cormier

Head of Regulatory Science and Innovation Task Force, European Medicines Agency

Dr. Emmanuel Cormier brings nearly 25 years of global leadership across drug and vaccine development, cultivating a global mindset and flexible, cross-functional expertise spanning discovery, CMC, clinical development, regulatory science, and both pharma and medical devices. He has forged strong public-private partnerships to address complex unmet medical needs, including pandemics. Recently, he has focused on health data, AI, real-world evidence and the European Health Data Space, leading EU-funded public-private initiatives. Since November 2023, he leads the European Medicines Agency' s Task Force on Regulatory Science and Innovation.
Previously, he served as Scientific Director, Regulatory Affairs – Policy Innovation at Johnson & Johnson MedTech (2022–2023) and held multiple leadership roles at Janssen Vaccines, including Head of Clinical Immunology (2016–2023) and Head of Molecular and Bioassays (2013–2016). Earlier, he was Associate Director, Clinical Research and Immunology at the International AIDS Vaccine Initiative in London (2007–2013) and Senior Scientist, Small Molecule Drug Discovery at Progenics Pharmaceuticals in the USA (2002–2007). He holds a Ph.D. in Molecular Virology from the University of Paris VII and an M.Sc. in Microbiology and Medical Virology from the University of Paris VI.

Hongzhang Deng

Professor, Xidian University

Hongzhang Deng, Professor and Doctoral supervisor, joined Xidian University in 2021 through a talent recruitment program. Currently, he is mainly engaged in research on the development of machine learning-assisted drug/gene delivery carrier systems and disease immunotherapy. He has published 33 papers in the first-tier journals of the Chinese Academy of Sciences as the first author or corresponding author, including Nat. Mater., PNAS, Nat. Commun., Adv. Mater., Matter, Nano Today, Nano Letters, Adv. Funct. Mater., and Biomaterials. Among them, 7 papers have been selected as ESI highly cited papers, and he has been granted 5 national invention patents.

(Alphabetical Order by Last Name) Speaker Profiles

Yu Ding

Associate Chief Physician and Deputy Director, Department of Endocrinology and Metabolism, Shanghai Children' s Medical Center, Shanghai Jiao Tong University School of Medicine / National Children' s Medical Center (Shanghai)

Yu Ding serves as a member of several professional committees: the Subspecialty Group of Endocrinology, Genetics and Metabolism under the Pediatricians Branch of the Chinese Medical Doctor Association; the Subspecialty Group of Disorders of Sex Development under the Adolescent Health and Medicine Professional Committee of the Chinese Medical Doctor Association; the Special Disease Research Collaborative Group of the Subspecialty Groups within the 19th Committee of the Pediatrics Branch of the Chinese Medical Association; the Endocrinology, Genetics and Metabolism Group of the Pediatrics Branch of the Shanghai Medical Association; and the Child Growth and Special Food Professional Committee of the China Association for Birth Health and Child Development. She has received the Medical Clinical Youth Science and Technology Innovation Award and an award from the Shanghai Women Physicians' Association. As a principal investigator, she has led projects funded by the Pudong New Area Science and Technology Commission and the Youth Project of the Medical-Engineering Cross Research Fund at Shanghai Jiao Tong University. She is also co-author of two monographs, Diagnosis and Management of Typical Cases in Pediatric Endocrinology and Rare Diseases, and chief editor of a set of popular science picture books.

Biao Dong

Professor, State Key Laboratory of Biotherapy, Sichuan University
Chairman, Sichuan Real&Best Biotech

Biao Dong holds a PhD in Biology from the Chinese University of Hong Kong and is a National High-Level Overseas Talent. His main research areas are rAAV gene therapy for genetic diseases and aging-related diseases. He has published over 60 SCI papers in journals such as Nature Aging, and has led one National Key Research and Development Program project and two National Natural Science Foundation of China (NSFC) projects, while also participating in two other National Key Research and Development Program projects. He has established an internationally advanced rAAV gene therapy platform and invented a large-scale production system that significantly reduces costs. Sichuan Zhishan Weixin Biotechnology Co., Ltd., founded in June 2018, has obtained three IND approvals for rAAV gene therapy in China and the United States.

Meiyu Fang

Chief Physician, Professor and Director of the Department of Head and Neck and Rare Cancers, Zhejiang Cancer Hospital

Meiyu Fang, Member of Oncology Special Committee of Capacity Building and Continuing Education Committee of National Health Commission; Member of the Standing Committee of CSCO melanoma Committee; Member of the Standing Committee of CSCO Head and Neck Cancer Committee; Member of the Committee of Multiple Primary/Unknown Primary Cancer, Chinese anti-Cancer Association; Member of Rare Diseases Committee of Zhejiang Medical Association.
She is engaged in medical oncology and basic research of oncology, and is currently engaged in the medical oncology of rare malignant tumors such as head and neck malignant tumors, malignant melanoma, urinary system and bone /soft tissue tumors.

Speaker Profiles (Alphabetical Order by Last Name)

Lizhao Feng

Principal Investigator, Oujiang Laboratory

Dr. Lizhao Feng is a Principal Investigator at Oujiang Laboratory and a high-level young talent recognized at both national and Zhejiang provincial levels. He has long focused on human iPSC-based stem cell therapy, the developmental regulation of oligodendrocyte progenitor cells (OPCs), and translational research on demyelinating diseases. During his time at the City of Hope National Medical Center in the United States, he participated in iPSC-derived NPC/OPC cell therapy projects, gaining comprehensive translational experience spanning disease modeling, cell preparation and quality assessment, in vivo efficacy validation in animal models, and preclinical pipeline advancement. After joining Oujiang Laboratory, he established a research group on iPSC-based stem cell therapy and clinical translation, conducting systematic investigations into OPC developmental regulation, white matter diseases, rare neurogenetic disorders, brain organoid/myelination organoid models, and cell/gene therapy platform development. His work is dedicated to advancing innovative therapies for major brain diseases from mechanistic discovery toward clinical translation.

Xudong Fu

Principal Investigator of the Hundred Talents Program, Liangzhu Laboratory, Zhejiang University

Dr. Xudong Fu is a Hundred Talents Program Researcher at the Liangzhu Laboratory of Zhejiang University. He has been awarded the National Science Fund for Excellent Young Scholars (overseas). His major academic achievements include identifying key metabolic regulatory mechanisms that govern cell fate during aging, elucidating the mechanisms that determine the fate of totipotent stem cells, and participating in the application for 7 patents, with 3 patents authorized. He has published multiple papers as corresponding or first author in international journals such as Developmental Cell, Cell Metabolism, Nature Cell Biology, Science Advances, and Aging Cell. His work has been covered by media outlets including National Geographic and Scientific American, and has been commented on by Nature and Science.

Guangping Gao

Professor & Chair, Department of Genetic and Cellular Medicine, UMass Chan Medical School
Past President, American Society of Gene & Cell Therapy

Guangping Gao, PhD is Professor and Chair of the Department of Genetic and Cellular medicine, Director, Li Weibo Institute for Rare Diseases Research, Director, Horae Gene Therapy Center and Viral Vector Core, Professor of Microbiology, Penelope Booth Rockwell Professor in Biomedical Research at University of Massachusetts Chan 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 421 research papers, 6 book chapters, and 5 edited books. Dr. Gao holds 275 patents with 596 more pending. Dr. Gao had been ranked as the World Top 20 Translational Researchers for several years in a row by Nature Biotechnology.

(Alphabetical Order by Last Name) Speaker Profiles

Javier García Cogorro

Founder and Secretary of Fundación Columbus
Founder and General Partner of Columbus Venture Partners

Javier García is a Founding Partner at Columbus Venture Partners. Prior to this role, he spent 25 years at Eli Lilly and Company, where he held various executive positions in Business Development and Information Systems. Since 2009, he has focused on venture capital investment, particularly in private companies within the biopharma, life sciences, and MedTech sectors. He currently serves on the board of directors of several biotechnology companies, including Lark Health, Jurata, Cocoon, and Quatre Labs.
Javier García is also the Founder and Secretary of Fundación Columbus, a foundation dedicated to developing advanced therapies for ultra-rare diseases. He sits on the board of trustees of the National Alliance for Hispanic Health and the Health Foundation of the Americas, both based in Washington, D.C., and is also a trustee of the Fundación Querer. He holds a degree in Mathematics (Statistics and Operations Research) from the Complutense University of Madrid and earned his MBA from ICADE (Spain). In 2023, he was awarded the Tambor de Oro (Golden Drum), the highest civic honour granted by the City of San Sebastián.

Liang Gong

Principal Investigator of the Hundred Talents Program, Liangzhu Laboratory, Zhejiang University

Dr. Gong is a Principal Investigator at Zhejiang University School of Medicine, Liangzhu Laboratory, and the First Affiliated Hospital of Zhejiang University, and a high-level young talent in Zhejiang Province. His research focuses on third-generation nanopore sequencing, combining cutting-edge technologies such as epigenetics and 3D genomics with bioinformatics tools to study the structure, regulation, and function of genomic variants in genetic diseases. He has published multiple papers in top journals including Nature Methods, Nature Genetics, Nature Communications, and PNAS. He has led projects funded by the National Natural Science Foundation of China (NSFC), the Zhejiang Provincial Natural Science Foundation (Major Project for Young Scientists), and is a key member of the National Key Research and Development Program of China Young Scientists Project, as well as a project leader of the NSFC Research Project for International Senior Scientists. He serves as a member of the Computational Synthetic Biology Committee of the Bioinformatics Society of China and the 3D Genomics Committee of the Genetics Society of China.

Yuchun Gu

Chief Scientist at Allife Medicine

Yuchun Gu went to the University of Birmingham in 1997 to pursue a PhD degree, and received his PhD in Molecular Physiology in 2000, where his doctoral dissertation won the UK's Best Doctoral Dissertation Award of that year. Subsequently, from 2000 to 2002, he conducted postdoctoral research at the Imperial College School of Medicine, University of London; from 2002 to 2004, he worked as a Research Fellow in the laboratory of Professor Roger Hardie, FRS, at the University of Cambridge. In October 2004, he was appointed as a faculty Principal Investigator at the University of Birmingham Medical School. In April 2011, he returned to China and became a Professor at the Institute of Molecular Medicine, Peking University, where he also served as the Director of the Molecular Pharmacology Laboratory. From January 2017 to July 2022, he held the position of Professor and Director of the Institute of Regenerative Medicine, Aston University Medical School, UK. In 2025, he was elected a Fellow of the Royal Society of Medicine, UK. He is currently the Chief Scientist at Allife Medicine.

Speaker Profiles (Alphabetical Order by Last Name)

Long Guo

Professor, Department of Laboratory Animal Science, School of Basic Medical Sciences, Xi' an Jiaotong University
Director, Center for Intelligent Healthcare Investigation of Rare diseases, Translational Medicine Institute, Xi' an Jiaotong University
Visiting Professor, Center of Medical Genetics, Northwest Women' s and Children' s Hospital

Long Guo, Professor and Doctoral Supervisor at the School of Basic Medical Sciences, Director of the Center for Intelligent Healthcare Investigation of Rare Diseases, Head of the Zebrafish Research Platform, Xi' an Jiaotong University. He serves as the Deputy Head of the Youth Working Group of the Medical Geneticist Branch, Chinese Medical Doctor Association, and as the Secretary-General of the Medical Geneticist Branch, Shaanxi Medical Doctor Association. He was formerly a Young Principal Investigator at the RIKEN Center for Integrative Medical Sciences in Japan. He has been selected for Xi' an Jiaotong University' s "Young Talent Support Program" (Class A) and the Shaanxi Province High-level Talent Program. As first author or last corresponding author, he has identified four novel disease-causing genes and defined four new rare genetic disorders, including one named after him, "Guo-Campos type spondyloepiphyseal dysplasia" (OMIM #620663). He has published over 20 papers in journals such as Am J Hum Genet and Nat Commun. He has led projects including the General Program and International Cooperation Project of the National Natural Science Foundation of China (NSFC). His awards include the JSBMR Distinguished Scientist Award (2023) and the ECTS East-meets-West Award (2022, 2024). He is also a Visiting Professor at the Center of Medical Genetics, Northwest Women' s and Children' s Hospital.

Peng Guo

Professor, Hangzhou Institute of Medicine, Chinese Academy of Sciences

Dr. Peng Guo received his Ph.D. in Analytical Chemistry from the University of Florida in 2011. From 2011 to 2021, he worked at Harvard University and Boston Children' s Hospital as a postdoctoral fellow and instructor. In 2021, he joined the Hangzhou Institute of Medicine, Chinese Academy of Sciences, as a Principal Investigator. His main research interests include cancer target discovery, antibody-drug conjugates, and exosome-based therapeutics. To date, he has published more than 50 research and review articles, with representative work appearing in journals such as PNAS, Nature Communications, Science Advances, Cancer Research, and Clinical Cancer Research.

Qin Guo

VP, Head of Clinical Development Rare Disease, AstraZeneca R&D China

Qin Guo holds an MD in Psychiatry from Shanghai Medical College, Fudan University. She has extensive clinical experience with a background in neurology. Since 2011, she has worked in clinical development in the pharmaceutical industry, focusing on neuroscience at multinational companies including Roche and Eli Lilly. After 2019, she transitioned to innovative biotech and domestic enterprises—serving as clinical development lead for neuroscience at AffaMed and subsequently overseeing clinical development across multiple therapeutic areas at Jemincare. In November 2021, she joined the AstraZeneca Global R&D China Center as the Clinical Development Lead for Rare Diseases.

(Alphabetical Order by Last Name) **Speaker Profiles**

Tiannan Guo

Tenured Associate Professor, School of Medicine & School of Life Sciences, Westlake University
Special Adviser to the President of Westlake University

Dr. Tiannan Guo completed his clinical medicine training (1999–2006) at Tongji Medical College, Huazhong University of Science and Technology, and simultaneously pursued studies in biology (2001–2005) at Wuhan University. Subsequently, he earned a Ph.D. in cancer proteomics (2008–2012) in Singapore and conducted postdoctoral research at ETH Zurich (2012-2017). After a brief tenure as Scientific Director of ProCan, Children's Medical Research Institute, The University of Sydney School in 2017, Dr. Guo relocated to Westlake University in August 2017 as a tenure-track Assistant Professor, achieving promotion to tenured Associate Professor in January 2023. He also directs the Westlake Center for Intelligent Proteomics at Westlake Laboratory.

Dr. Guo and his team have developed high-throughput proteomic technologies for micro-scale tissue samples and spatial subcellular proteomics, supporting efforts to address major health challenges including cancer, infectious diseases, and aging. They have constructed MassNet, a billion-scale AI-friendly mass spectrometry corpus, and developed a suite of proteome-focused pre-trained models. In addition, Dr. Guo has led the development of a strategic roadmap for artificial intelligence-driven proteomics, proposed a novel framework for constructing AI-enabled virtual cells, and established ProteinTalks—the first foundational virtual cell model based on perturbed proteomics. His team has also developed multiple clinical kits based on mass spectrometry proteomics, and initiated the international consortiums for AI Proteomics and virtual yeast.

Dr. Guo holds the presidency of the Chinese Proteomics Society, and serves as a Council Member of the Human Proteome Organization (HUPO). He is also an Associate Editor of Molecular & Cellular Proteomics, an Editorial Board Member of Genomics, Proteomics & Bioinformatics and Cell Systems, an Advisory Expert of Cell Reports Medicine, and a Scientific Advisor for Vita and Molecular Systems Biology.

Junyuan Han

Vice President of Research and Development, Leaperbio Inc.

Dr. Junyuan Han joined EdiGene in 2023. Previously, he worked as a Senior Study Director in the Department of Toxicology and Safety Pharmacology at Pharmaron (Beijing) Biotechnology Co., Ltd., and as Associate Director of the Department of Pharmacology and Toxicology at Guangzhou Inmagene Biopharmaceutical Co., Ltd. He has 12 years of experience in pharmacology and toxicology for new drug R&D and has undertaken more than 50 safety evaluation studies. He has successfully completed IND-enabling studies and submissions for Class 1 innovative drugs in compliance with FDA, OECD, and NMPA requirements, and has led dual China-U.S. IND filings for small-molecule and antibody drugs in therapeutic areas including ophthalmology, oncology, and anti-infectives.

Speaker Profiles (Alphabetical Order by Last Name)

Xinyue Han

Parent of a Child with Duchenne Muscular Dystrophy (DMD)
Member of the Committee, China DUCHENNE Family Network

Xinyue Han, parent of a child with Duchenne Muscular Dystrophy (DMD), expert patient, member of the Committee of the China DUCHENNE Family Network, and business supervisor at a central state-owned enterprise.
The China DUCHENNE Family Network is a nationwide, nonprofit, and mutual-aid patient community voluntarily joined by families of DMD patients. Its vision is to help DMD patients grow up healthily, and its mission is to unite the strength of DMD patient families, advance diagnosis, treatment, and drug development, and ultimately improve the quality of treatment and quality of life for patients.

Don Haut

Chief Business Officer, Arbor Biotechnologies

Don Haut, PhD, is a seasoned biopharmaceutical executive with over two decades of experience scaling both public and private biotechnology and medical technology organizations. He currently serves as the Chief Business Officer at Arbor Biotechnologies, a clinical-stage company developing next-generation genetic medicines through its proprietary gene editing platform.
Over the course of his career, Dr. Haut has successfully executed transactions totaling more than $10 billion. His leadership experience spans a variety of innovative modalities and therapeutic areas, having served as the CEO of Carmine Therapeutics, where he led the development of non-viral gene therapies, and as the Chief Business Officer for AskBio, EG 427, Sherlock Biosciences, and Histogenics. His tenure at AskBio included the company's $4 billion acquisition by Bayer AG. Additionally, he served as a Vice President at The Medicines Company, overseeing new business ventures and sales operations.
Dr. Haut's foundational career included senior roles at 3M Company, Smith & Nephew, and Monsanto, as well as a tenure as a consultant with McKinsey & Company. Beyond his executive leadership, he provides extensive governance expertise as the Chairman of the Board for Immvention Therapeutix and Orthox Ltd., and as a board member for Aspect Biosystems and Xiros Ltd. He holds a PhD in Molecular Microbiology and Immunology from the University of Missouri-Columbia and an MBA from Washington University's Olin School of Business.

Al Hawkins

Co-Founder & President, Amplo Biotechnology

Al Hawkins is a U.S. based entrepreneur who has been translating new therapies from academia to startup companies for 25 years. He has developed multiple rare disease gene therapy products through clinical trials; he was formerly Co-Founder and Chairman of Abeona Therapeutics, developing therapies for lysosomal storage diseases and Co-Founder and CEO of Milo Biotechnology, which advanced clinical therapies for muscular dystrophy. He is currently a co-founder of several cell and gene therapy companies via Saule Gene & Cell Therapy. Al received a Science Masters from MIT HST and a Business Masters from the University of Wisconsin-Madison.

(Alphabetical Order by Last Name) Speaker Profiles

Yann Hérault

Exceptional Class Research Director, Centre National de la Recherche Scientifique (CNRS), France

Yann Hérault is a distinguished French scientist and Exceptional Class Research Director at the Centre National de la Recherche Scientifique (CNRS). A biologist and mouse geneticist by training, he earned his Ph.D. from the University of Lyon in 1993 and completed a postdoctoral fellowship at the University of Geneva, where he studied Hox gene regulation under Prof. Denis Duboule. Since 2000, Hérault has led research teams and infrastructure projects, notably at the Institut de Génétique et de Biologie Moléculaire et Cellulaire (IGBMC) in Strasbourg, where he currently heads the Mouse Clinical Institute (ICM/ICS).

Hérault' s research focuses on understanding the genetic and molecular mechanisms underlying rare disease leading to neurodevelopmental disorders, particularly those caused by gene dosage effects. His work has significantly advanced the study of Down syndrome (trisomy 21), 16p11.2 deletion/duplication syndromes, Koolen de Vries syndrome, Dyrk1a syndrome and Ptchd1 syndrome, and other intellectual disabilities linked to copy number variations. He has developed innovative mouse and rat models to dissect the pathophysiological mechanisms of these conditions, identifying key genes and proposing novel therapeutic strategies. His lab' s contributions include the discovery of genes involved in cognitive deficits and the development of pharmacological approaches to mitigate learning and memory impairments in Down syndrome models.

Beyond his scientific achievements, Hérault has played a pivotal role in establishing CELPHEDIA, the French national infrastructure for Model Organism, and INFRAFRONTIER the European research infrastructure for disease models. His leadership extends to the development of the International Mouse Phenotyping Consortium (IMPC), and the International Mammalian Genome Society (IMGS), further solidifying France' s position in global biomedical research. His work continues to bridge fundamental research and clinical applications, aiming to improve the lives of individuals with neurodevelopmental disorders.

Katherine High

CEO, RhyGaze AG
Emeritus Professor, Perelman School of Medicine of the University of Pennsylvania, and Children' s Hospital of Philadelphia

Dr. Katherine High is Chief Executive Officer of RhyGaze, Inc., a biotech company developing treatments for inherited and acquired retinal diseases, based in Philadelphia and Basel. She previously held faculty roles at the Perelman School of Medicine at the University of Pennsylvania and was an Investigator of the Howard Hughes Medical Institute at the Children' s Hospital of Philadelphia (CHOP).

Dr. High served on the U.S. FDA Advisory Committee on Cell, Tissue and Gene Therapies and is a past president of the American Society of Gene & Cell Therapy. In 2013, she co-founded Spark Therapeutics, where she was President and Chief Scientific Officer. There, she led development of Luxturna, the first FDA-approved gene therapy for a genetic disease, and of Beqvez for hemophilia B. Following Spark' s acquisition by Roche, she joined AskBio, a Bayer subsidiary, as President of Therapeutics.

Dr. High holds a chemistry degree from Harvard University and an MD from the University of North Carolina. She is a member of the National Academy of Medicine and the National Academy of Sciences and serves on the boards of CRISPR Therapeutics and Incyte. She is also an advisor in Life Sciences to GV (Google Ventures).

Speaker Profiles (Alphabetical Order by Last Name)

Heidi Carmen Howard

Senior Researcher, Chalmers University of Technology
Head of ELSI, SciLifeLab, Sweden

Heidi Carmen Howard is a bioethicist who uses interdisciplinary ELSI approaches with a large emphasis on empirical research and policy analysis to study the challenges and implications of emerging technologies (i.e. genomics, AI) and their responsible translation for end users and society. Themes she has worked on include participant-centred research initiatives, consumer genetics, consent for high-throughput sequencing, ELSI of human genome editing, approaches for meaningful public engagement, and publics' views on medical data sharing. Recently, she was a research scientist at Google DeepMind, where she innovated methodologies to evaluate AI models for Chemical, Biological, Radiological and Nuclear (CBRN) informational risks. Her research on commercialisation of genomics and consent in research and clinical genomics has help guide meaningful ways of communicating with publics and policies for newborn screening, among others. Dr. Howard also has important experience in stakeholder engagement for policy creation. Heidi has an H-index of 46; has co-authored over 90 articles in international peer-reviewed journals, including journals such as Science, Nature, and Nature Genetics. She was an active member of the Policy and Ethic Committee (PEC) of the European Society of Human Genetics for over a decade and continues to head their AI Ethics working group. She has been invited to consult for organisations such as the Council of Europe Bioethics Committee (on human genome editing) and been part of experts group such as the National Academies of Science, Engineering and Medicine on AI and Biology.

Alexander E. Hramov

Corresponding Member of the Russian Academy of Sciences
Director and Chief Research Scientist of the Research Institute of Applied AI and Digital Solutions at Plekhanov Russian University of Economics
Chief Research Scientist at Pirogov National Medical and Surgical Center

Alexander E. Hramov is a Corresponding Member of the Russian Academy of Sciences, Doctor of Science in Physics and Mathematics, and Professor. He earned his diploma with honors, Ph.D., and D.Sc. from Saratov State University. He is Director and Chief Research Scientist of the Research Institute of Applied AI and Digital Solutions at Plekhanov Russian University of Economics, and Chief Research Scientist at Pirogov National Medical and Surgical Center.

His interdisciplinary research spans physics, neuroscience, machine learning, and biomedicine, focusing on predictive medicine, clinical decision support, neurorehabilitation, and artificial intelligence. His work addresses brain connectivity in disorders of consciousness, depression, autism, epilepsy, and Alzheimer's, using interpretable machine learning and reservoir computing for diagnostics. His work also includes brain–computer interfaces, neurostimulation, and hardware–software systems for cognitive and motor rehabilitation.

He has led over 70 research projects, including major grants from the Russian Science Foundation and the Megagrant program. He previously headed neurotechnology centers at Immanuel Kant Baltic Federal University and Innopolis University, and held professorships at Saratov universities. He has authored 368 scientific works, 8 monographs, and 36 patents, with an H-index of 51 (Scopus). He is an Institute of Electrical and Electronics Engineers (IEEE) member, serves on the International Physics and Control Society (IPACS) Supervisory Board, and on editorial boards for 12 Russian and international scientific journals.

(Alphabetical Order by Last Name) Speaker Profiles

Ping Hu

Principal Investigator and Professor, Guangzhou National Laboratory

Ping Hu, Ph.D., Principal Investigator, Guangzhou National Laboratory. Dr. Hu earned her Bachelor's degree from Peking University; her Ph.D. degree from Cold Spring Harbor Laboratory/State University of New York Stony Brook. She performed her postdoctoral research in University of California, Berkeley/Howard Hughes Medical Institute. She was principal Investigator, Shanghai Institute of Biochemistry and Cell Biology, Chinese Academy of Sciences from 2011 to 2021. From 2021, she has been Principal Investigator in Guangzhou National Laboratory. Dr. Hu focuses on the research of regulation of muscle regeneration, cell and gene therapy of muscular diseases.

She has published over 60 papers in prestigious journals such as Cell, Dev Cell, Mol Cell, Cell Res, EMBO J., and Genes & Dev. She holds more than 20 national and international patents and leads over 20 national and province research projects. She is a recipient of the State Council Special Government Allowance and has been honored with awards including the Chinese Academy of Sciences "Hundred Talents Program," the Ministry of Human Resources and Social Security "Award for Outstanding Overseas Returnees," Shanghai "Pujiang Talent" Award, Guangdong "Specially Introduced High-Level Talent" Award, First Prize of Shanghai Science and Technology Innovation Award, the Yaokang Outstanding Women Scientist Fund from the Genetics Society of China, the Biophysical Society of China, and the Chinese Society for Cell Biology, as well as the First Prize of the Chinese Medical Science and Technology Award from the Chinese Medical Association.

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 Mountains. 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 families and Hope For Rare Foundation leads and builds a joint force with experts and researchers from related domains. Juan Huang is also the mother of a child with a rare disease.

Speaker Profiles (Alphabetical Order by Last Name)

Kevin Huang

Founder and CEO 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.

Yue Huang

Director, Human Brain & Tissue Bank, China National Clinical Research Center for Neurological Diseases

Prof. Huang has long been engaged in clinical and basic research on neurodegenerative diseases. She has published more than 100 SCI papers with an H-index of 36. She is committed to the development of the Tiantan Brain Health Volunteer Service Team to advance the cause of brain health.
She has served as a reviewer for over 50 international journals including Annals of Neurology and Neurology. She is also an expert reviewer for the Netherlands Organisation for Scientific Research Research Infrastructure (NOW-RI) projects, National Natural Science Foundation of China (NSFC) Young Scientists Fund, International Cooperation Projects of the Ministry of Science and Technology, as well as the Ministry of Industry and Information Technology of China, Ningbo Yongjiang Talent Introduction Programme, and Xueshuqiao Talent Program.
Dr. Huang is a member of the Scientific Issue Committee of the International Parkinson and Movement Disorder Society (MDS), a member of the Sigma Xi, The Scientific Research Honor Society (USA), a member of the Neurodegenerative Diseases Branch of the Chinese Neuroscience Society, and a member of the Chinese Huntington' s Disease Association. He previously served as President of the first Executive Committee of the Chinese Biomedical Scientists Association in Australia.

(Alphabetical Order by Last Name) Speaker Profiles

Oxana Iliach

Vice Chair of Regulatory Scientific Committee, International Rare Disease Research Consortium (IRDiRC)
Senior Director of Regulatory Strategy, Certara

Dr. Oxana Iliach has more than 20 years of experience in the healthcare industry, including over 15 years in regulatory affairs. She specializes in developing and executing regulatory strategies for drugs for rare diseases (including pediatrics) and biosimilars, with a focus on Chemistry, Manufacturing and Control (CMC). Her specific expertise lies in biologics and cell and gene therapy products. Dr. Iliach has experience working with the FDA, EMA, Health Canada, and other smaller regulatory agencies. She holds an MSc in Chemistry and a PhD in Pharmaceutical Science.
Currently, Dr. Iliach serves as Vice Chair of the Regulatory Scientific Committee (RSC) at the International Rare Diseases Research Consortium (IRDiRC) and as Senior Director of Regulatory Strategy at Certara. She is also a member of the Canadian Organization for Rare Disorders (CORD). In academia, she is a professor at Seneca College in Toronto, Canada, where she teaches a course on clinical trials regulations, and a part-time lecturer at Northeastern University, where she teaches regulatory courses.

Manabu Inoue

Chief Medical Officer, Pharmaceuticals and Medical Devices Agency, Japan

Manabu Inoue, MD, PhD, is a board-certified neurologist and stroke specialist with more than 22 years of experience in clinical medicine, academic research, and regulatory science. He currently serves as Chief Medical Officer (CMO) at the Pharmaceuticals and Medical Devices Agency (PMDA) of Japan, where he provides senior medical leadership in the evaluation of drugs, medical devices, and emerging technologies, and contributes to regulatory policy development and international harmonization. From 2011 to 2015, Dr. Inoue served as a Visiting Professor at the Stanford Stroke Center in the United States. He is fluent in Japanese, English, and German. Dr. Inoue also holds a specially appointed senior clinical position at the National Cerebral and Cardiovascular Center (NCVC), Japan. His expertise focuses on acute ischemic stroke, advanced neuroimaging including CT and MR perfusion imaging, large vessel occlusion, endovascular thrombectomy, and the clinical and regulatory implementation of artificial intelligence–based diagnostic tools. He has participated in numerous national and international randomized controlled trials and has authored many peer-reviewed publications.

Speaker Profiles (Alphabetical Order by Last Name)

Antonella Isgrò

Senior Clinical Assessor, Italian Medicines Agency
Member of the Haematology Working Party, European Medicines Agency

Antonella Isgrò, MD, PhD, is a senior clinical assessor at the Italian Medicines Agency (AIFA) and member of the Haematology Working Party (HAEMWP) at the European Medicines Agency (EMA). She received her MD in Medicine and Surgery at University of Naples "Federico II" (Italy) and completed her specialization in Allergy and Clinical Immunology, PhD in Immunological Science and a Master in Pediatric Hematology at the University of Rome "La Sapienza" (Italy).
From 2005 to 2019, Dr. Isgrò worked at Mediterranean Institute of Haematology (IME), University of Rome "Tor Vergata" (Italy), where she engaged in clinical activities on allogeneic stem cell transplantation of thalassemia and sickle cell patients and focused her research activities on hemoglobinopathies, conducting studies on immunological reconstitution after bone marrow transplant, thus combining clinical practice with research.
Over the past two decades, Dr. Isgrò contributed to the diagnosis and treatment of haematological and onco-haematological diseases, in both adult and paediatric populations, regarding the hemoglobinopathies, primary and acquired immunodeficiencies, and other rare haematological disorders.
In 2019, she recruited by AIFA, where she is engaged in the evaluation of centralized procedures of medical products in the fields of immunological and haemato-oncologic diseases, and as a clinical expert in scientific advice procedures, orphan designations, clinical trials, and early access applications.
She is author and co-author of 82 papers on international peer-reviewed journals; 109 papers in international and national congress (Orcid: 0000-0002-0639-2389).

Lee Jia

Academicians of International Eurasian Academy of Sciences, Academy of Toxicological Sciences, European Academy of Sciences and Arts
Distinguished Professor, The First Affiliated Hospital, Henan University

Dr. Jia is the Founding Chair of the AI Functional Pharmaceutics of Chinese Pharmaceutical Association (CPA) and AI-pharmaceutics Committee Member of the International Pharmaceutical Federation (FIP). He has played a pivotal role in advancing interdisciplinary pharmaceutical research. Jia has authored 257 peer-reviewed publications in journals such as Nature, Science, N Engl J Med, Nat. Comm., STTT (IF52.7), Nano Today, and PNAS, with >20,000 citations and an h-index of 70 (Google Scholar). He is one of the world top 2% scientists selected by Stanford University & Elsevier/Mendeley Data. He has delivered 115 keynote and invited presentations.
Jia was a Section Chair of the American Association of Pharmaceutical Scientists (AAPS, USA; 2009-2012); Vice-President, Fujian Pharmacological Society (2014-2024), China; Vice-President, Fujian Biomedical Engineering Society, China (2021-present); Commission Member of Free Radical Biology and Medicine, the Biophysical Society of China. He is the Associate Editor for Current Drug Metabolism (2005-present); Associate Editor, Drug Metabolism and Bioanalysis Letters (2023-). He is an elected Academician of the International Eurasian Academy of Sciences, elected Academician of Academy of Toxicological Sciences (ATS), a Fellow of AAPS, Senior Expert Committee of the All-China Federation of Returned Overseas Chinese, and a Council Member of the Chinese Pharmaceutical Association (CPA). He represents CPA in global AI-pharmaceutical activities of the International Pharmaceutical Federation (FIP) headquartered in the Netherlands.

(Alphabetical Order by Last Name) Speaker Profiles

Yulin Jiang

Director of Prenatal Diagnosis Center, Deputy Director of Obstetrics Center, Peking Union Medical College Hospital

Yulin Jiang, MD, is a Professor and Chief Physician in the Department of Obstetrics and Gynecology at Peking Union Medical College Hospital, where he also serves as Director of the Prenatal Diagnosis Center and Deputy Director of the Obstetric Center. He holds several key academic positions, including Member of the National Expert Group for Prenatal Diagnosis Technology; Vice Chairman and Secretary-General of the Professional Committee for Birth Defect Prevention, Chinese Association of Eugenics and Genetics; Deputy Head of the Prenatal Diagnosis Group, Medical Genetics Branch, Chinese Medical Association; and Deputy Secretary-General of the Chinese Association of Eugenics and Genetics.

Dr. Jiang has been a primary author of several major industry standards, guidelines, and consensus statements, such as the Industry Standard for Prenatal Serological Screening and Cytogenetic Prenatal Diagnosis of Common Fetal Chromosomal Abnormalities, the Guidelines for the Application of Chromosomal Microarray in Prenatal Diagnosis in China (2023), the Expert Consensus on the Clinical Application of Carrier Screening for Common Recessive Monogenic Disorders Before and in Early Pregnancy, and the Clinical Application Standards for Cell-Free Fetal DNA Prenatal Testing Technology. His contributions have been recognized with numerous awards, including a Third Prize of the Chinese Medical Science and Technology Award (2019), a Third Prize of the Huaxia Medical Science and Technology Award (2020), and a Second Prize of the Beijing Municipal Science and Technology Award (2022). He has led and completed multiple national-level research projects in the field of prenatal diagnosis of genetic diseases and currently serves as the Chief Scientist for a key research and development project of the 14th Five-Year Plan. His main areas of expertise include prenatal genetic diagnosis, genetic counseling and maternal-fetal medicine, and intrauterine fetal therapy.

Yuwu Jiang

Director of the Children's Medical Center and Pediatric Epilepsy Center, Peking University First Hospital
Dean of the Department of Pediatrics, Peking University Health Science Center

Yuwu Jiang, Vice chair of Chinese Association Against Epilepsy (CAAE); Vice chair of Chinese Pediatric Society (CPS); Executive board Member of ICNA (International Child Neurology Association); National delegate of China in Asian Oceanian Child Neurology Association (AOCNA); Member of ILAE (International League Against Epilepsy) Terminology Commission; Member of ILAE Medical Therapy in Children Task Force of Pediatrics Commission.

Speaker Profiles (Alphabetical Order by Last Name)

Chaowen Jin

Deputy Secretary-General of the CDKL5 Baby Care Home China

CDKL5 Baby Care Home hopes to unite all patients, as well as social and charitable groups that care about us, to leverage all social resources, and to create a fast and effective channel for the rare disease group of CDKL5 syndrome in all aspects including diagnosis, treatment, rehabilitation institutions and home-based rehabilitation, family psychological care, social welfare assistance, and community integration, so as to ensure that affected families receive active treatment and support. At the same time, it aims to raise public awareness of this disease and secure the broadest social attention, laying the foundation for future early screening, rational allocation of medical resources, rapid advancement of effective treatment plans, and standardized procedural progress in diagnosis, treatment, rehabilitation, and assistance for affected children.

Qi Kang

Deputy Director of the Department of Health Policy Research, Shanghai Health Development Research Center (Shanghai Medical Information Center)

Qi Kang, Ph.D., is an Associate Researcher and Deputy Director of the Department of Health Policy Research at the Shanghai Health Development Research Center (Shanghai Medical Information Center). He also serves as a board member of the Shanghai Rare Disease Prevention and Treatment Foundation and has been selected for the Shanghai Health Outstanding Young Talents Program. His main research areas include rare disease policy, research hospitals, health planning, and industry.

In the field of rare diseases, he has organized several surveys on disease burden and quality of life among rare disease patients in China, including two provincial-level surveys in Shanghai and Guangdong, as well as single-disease surveys on mucopolysaccharidosis, hypophosphatemic rickets, isolated methylmalonic acidemia, and propionic acidemia, among others. He has undertaken over 20 rare disease–related research projects funded by the Shanghai Philosophy and Social Sciences Planning Office and other sources, and has published more than 20 papers on rare diseases.

Yu Kang

Chief Physician and Director of the Phase I Clinical Trial Unit, Obstetrics & Gynecology Hospital of Fudan University

Yu Kang, M.D., Ph.D. Chief Physician, Obstetrics and Gynecology Hospital of Fudan University. She focuses on targeted therapy, immunotherapy, and the development and application of innovative drugs for recurrent and drug-resistant gynecologic malignancies. She has established a nationwide multi-center cohort of hereditary ovarian cancer susceptibility gene carriers and a cohort of Peutz-Jeghers syndrome-associated cervical lesions, and has developed an AI-powered genetic counseling agent for gynecologic tumors, aiming to promote standardized precision diagnosis and treatment, prevention, family inheritance blocking, and source control of hereditary gynecologic cancers.

Dr. Kang has led multiple projects funded by the National Natural Science Foundation of China (NSFC) and provincial/ministerial research programs. She has published 55 SCI-indexed papers and contributed to 12 monographs. She is co-editor of Practical Gynecologic Oncology Genetics, Expert Consensus on Genetic Counseling for Gynecologic Tumors, and the Guidelines for the Management of Genetic Testing and Genetic Counseling in Gynecologic Oncology. She serves on the editorial board of Gynecologic Oncology, the official journal of the American Society of Gynecologic Oncology (SGO).

(Alphabetical Order by Last Name) Speaker Profiles

Oleg E. Karpov

Academician of the Russian Academy of Sciences
Professor and General Director of the Pirogov National Medical and Surgical Center

Oleg Eduardovich Karpov, MD, PhD, Professor, and Academician of the Russian Academy of Sciences, is the General Director of the Pirogov National Medical and Surgical Center in Moscow. A graduate with honors from I.P. Pavlov Ryazan Medical Institute in 1989 (General Medicine), he completed surgical internship, residency, and postgraduate training. He received his Doctor of Medical Sciences degree in 2005. He was awarded the title of Professor in 2007 and elected Academician of RAS in 2022 (Department of Nanotechnologies and Information Technologies).
His career began as a surgeon in Ryazan, advancing to Deputy Chief Physician for Surgery at Ryazan Regional Clinical Hospital. After serving as Head of the Ryazan Region Healthcare Department, he took on senior leadership roles at the Pirogov Center, becoming General Director in 2006. Professor Karpov has authored over 400 scientific publications, including 8 monographs and 15 educational manuals, and holds 12 patents. He serves as Editor-in-Chief of the journal Physician and Information Technologies, and is an editorial board member of several other medical journals.

Eric Kelsic

CEO and Co-Founder, Dyno Therapeutics

Eric Kelsic is a scientist and entrepreneur leading the transformation of gene therapy through artificial intelligence. As the CEO and Cofounder of Dyno Therapeutics, he is pioneering an AI-powered platform to solve the industry's greatest bottleneck: the safe and effective in vivo delivery of genetic medicines. Eric' s work is rooted in a "physics-first" philosophy, viewing biological systems as programmable machines. Prior to founding Dyno, he was a researcher in George Church' s lab at the Wyss Institute of Harvard Medical School, where he led the team that developed Dyno' s core technology. During this time, he published foundational research in Science, measuring the first comprehensive fitness landscape of the AAV capsid and co-discovering the AAV MAAP gene. Under his leadership, Dyno has raised over $100M in financing (including a Series A led by a16z) and established a dominant partnership network with global leaders like Astellas, Roche, and NVIDIA. Eric holds a PhD in Systems Biology from Harvard University and a BS in Physics from Caltech. He has been recognized as one of Endpoint News' "20 under 40" next-gen biotech leaders and Xconomy' s Startup of the Year.

Lingdong Kong

Senior Director, Head of Clinical Development, Suzhou Arnatar Therapeutics Co., Ltd.

Dr. Lingdong Kong is currently the Head of Clinical Development at Suzhou Arnatar Therapeutics Co., Ltd. With 18 years of experience in the pharmaceutical industry, he has previously worked at both multinational and domestic pharmaceutical companies, including Novartis, Wyeth, UCB, GeneScience, Visen, etc. He has served in various leadership roles, such as Medical Director, Head of Clinical sciense, Head of Medical Affairs and so on.

Speaker Profiles (Alphabetical Order by Last Name)

Semen A. Kurkin

Principal Investigator at Plekhanov Russian University of Economics

Semen A. Kurkin is a Principal Investigator at Plekhanov Russian University of Economics. He holds a Doctor of Sciences degree in Mathematical Modeling (2017) and a Ph.D. in Radiophysics (2011) from Saratov State University, where he also earned his M.S. in Physics (2008). His academic career at Saratov State University spanned over a decade, progressing from Assistant Lecturer to Professor in the Faculty of Nonlinear Processes. He additionally headed the Department of Electron Devices and Units at Yuri Gagarin State Technical University. He later held a professorship at Innopolis University (2019–2022) and worked as a Principal Investigator at Immanuel Kant Baltic Federal University (2021–2025).

Dr. Kurkin's research sits at the intersection of neuroscience, nonlinear dynamics, and data science. He specializes in the analysis of multimodal brain signals—including EEG, MEG, EMG, fNIRS, and fMRI—and in the development of brain-computer interfaces. His methodological expertise covers neural source localization techniques, complex network analysis, and the application of artificial neural networks to decode brain activity. With a strong foundation in mathematical modeling, his broader work also encompasses data mining and the study of complex systems, aiming both to uncover fundamental principles of brain organization and to advance next-generation neurotechnologies.

Henry Lee

Senior Scientist of FM Kirby Neurobiology Center, Assistant Director of Experimental Neurophysiology Core, Preclinical Science Program Manager of Translational Neuroscience Center, Boston Children's Hospital, Harvard Medical School

Henry Lee, MPhil, PhD, is a molecular neurobiologist who received training in University College London (UCL), University of Pennsylvania (UPenn), and Harvard University. Dr. Lee is currently a Research Associate in Neurology at Harvard Medical School and a Senior Scientist/Assistant Director/Program Manager at Boston Children's Hospital. Dr. Lee specializes in neuronal inhibition, brain plasticity, and functional recovery in neurodevelopment and brain trauma. His research has discovered mechanisms through which phosphorylation-dependent turnover of potassium chloride co-transporter KCC2 controls Cl^- mediated GABAergic inhibition (Lee et al., Nat Neurosci, 2011). He also used novel genetic mouse models to dissect a paracrine signaling mechanism underlying critical period (CP) plasticity control (Spatazza, Lee et al., Cell Rep, 2013; Lee et al., Mol Psychiatry, 2017). Dr. Lee's translational research included functional recovery after nerve damage (Bei, Lee et al., Cell, 2016), traumatic brain injury (Hsieh, Lee et al., Cerebral Cortex, 2016), and rare GABA metabolic disorders such as succinic semialdehyde dehydrogenase deficiency (SSADHD) (Lee et al., J Child Neurol., 2021; Lee et al., JIMD, 2024). Dr. Lee is co-inventor on patents for gene therapy in SSADHD, and co-founder of Galibra Neuroscience, leveraging brain-penetrating AAV capsids for broad CNS delivery. His work bridges basic neuroscience with clinical translation for rare pediatric neurologic diseases. Dr. Lee was a recipient of a Partnership Award from the Citizens United for Research in Epilepsy (CURE), an NIH NINDS IGNITE Award, and a Young Investigator Award from the American Epilepsy Society (AES) among other academic honors.

(Alphabetical Order by Last Name) Speaker Profiles

Andrew Levy

Professor, Rappaport Faculty of Medicine, Technion Israel Institute of Technology

Andrew Levy earned his B.A. Summa Cum Laude in Molecular Biophysics and Biochemistry from Yale University in 1982, followed by an M.D.-Ph.D. from the Johns Hopkins Medical Institutions in 1990, where his Ph.D. research was conducted under Nobel laureate Daniel Nathans. He completed his medical residency in Internal Medicine at Johns Hopkins Hospital (1990–1992) and a fellowship in Cardiovascular Disease at Brigham and Women's Hospital, Harvard Medical School (1992–1996). In 1997, he joined the faculty of the Technion Israel Institute of Technology Faculty of Medicine, where he has been a tenured full professor since 2010. He has authored over 190 peer-reviewed publications garnering more than 17,000 citations, and currently receives grant support from the Israel Science Foundation and the U.S.-Israel Binational Science Foundation.

Bowen Li

Associate Professor, University of Toronto
Canada Research Chair in RNA Vaccines and Therapeutics
GSK Chair in Pharmaceutics and Drug Delivery

Dr. Bowen Li is the GSK Chair Professor in Pharmaceutics and Drug Delivery, Canada Research Chair in RNA Vaccines and Therapeutics, and a tenured Associate Professor in the Leslie Dan Faculty of Pharmacy at the University of Toronto. Dr. Li received his Ph.D. in Bioengineering from the University of Washington, Seattle, and then completed a Postdoc Fellowship under the guidance of Profs. Robert Langer and Daniel Anderson at MIT. His laboratory integrates artificial intelligence with molecular bioengineering and biomaterials to design nucleic acid molecules and nonviral delivery systems, enabling prediction and control of their behavior across molecular, cellular, and in vivo scales to advance disease treatment. His work has led to more than 70 publications in journals such as Cell, Nature Biotechnology, Nature Nanotechnology, Nature Materials, Nature Biomedical Engineering, et al and more than 10 patents.
Dr. Li's research contributions have been recognized through awards including the Breakthrough T1D Innovative Award (2026), Connaught Innovation Award (2026), Terry Fox New Investigator Award (2026), Oxford-Harrington Rare Disease Scholar Award (2025), UofT Innovator of the Year (2025), National Sanitarium Association Scholar Award (2025), Ontario Early Researcher Award (2025), Stem Cell Network Early Career Researcher Award (2025), AFPC New Investigator Research Award (2025), NIH R01 grant (2024), CIHR Project Grants (2022-2025), AAPS Emerging Leader Award (2024), CSPS Early Career Award (2024), Cystic Fibrosis Canada - Marsha Morton Early Career Investigator Award (2024), Biomaterials Science Emerging Investigator Award (2024), Cystic Fibrosis Foundation Path-to-A-Cure Research Awards (2023, 2022), Gairdner Early Career Investigator Award (2022), and the Connaught New Researcher Award (2022), among others.

Speaker Profiles (Alphabetical Order by Last Name)

Eileen Li

Senior Advisor, Chinese Organization for Rare Disorders

Eileen Li, General Manager of ResMed Greater China. Prior to joining ResMed, Eileen was CEO and a founding member of RareStone Group, served successively as Vice President of Corporate Development, Chief Business Officer (CBO), President and CEO, with overall responsibility for corporate strategy, business development, portfolio strategy, R&D, commercialization, market access, and public relations.

Eileen used to serve as Principal (Partner) at IQVIA Management Consulting (previously named IMS Consulting Group), leading their Rare Disease, Pricing & Market Access Center of Excellence. During her tenure, she spearheaded three landmark reports that shaped China's rare disease landscape. Eileen has successfully guided multiple specialty drug new launches in China, particularly in oncology and rare diseases areas, with deep expertise across the product lifecycle from regulatory approval to commercialization plan, market access, and go-to-market strategies. Her earlier career includes health economics research at Novartis Pharmaceuticals US.

An active contributor to global rare disease initiatives, Eileen currently serves as Senior Advisor for the Chinese Organization for Rare Disorders (CORD) and Project Consultant for the International Rare Disease Research Consortium (IRDiRC), having previously held a director position at the Asia Pacific Alliance of Rare Disease Organizations (APARDO).

She holds an M.S. in Social Policy from Columbia University and a B.S. in Business Administration from Renmin University of China.

James Li

Co-Founder and President, GondolaBio

James Li, Ph.D., is Co-Founder and President of GondolaBio, a clinical-stage biopharmaceutical company and sister company of BridgeBio Pharma focused on developing transformative medicines for patients living with genetic diseases. GondolaBio was founded to advance a broad portfolio of genetically validated therapeutic programs with speed, scientific rigor, and a patient-first mission. The company' s pipeline spans more than 20 genetic diseases across multiple therapeutic areas, including hematology, neurology, pulmonology, cardiology, nephrology, and endocrinology, and includes programs across small molecules, oligonucleotides, antibodies and other therapeutic modalities.

As President, Dr. Li helps shape GondolaBio' s strategy, culture, and operating model, with a focus on advancing high-quality science efficiently toward meaningful patient impact. He works closely with academic collaborators, patient communities, and industry partners to translate emerging human biology into potential new medicines for diseases with high unmet need.

Before co-founding GondolaBio, Dr. Li served as Chief Business Officer at BridgeBioX, where he oversaw research and early development programs and led new company formation, asset acquisition, and diligence efforts. Previously, he was an Engagement Manager at McKinsey & Company, advising pharmaceutical and biotechnology clients on strategic initiatives, portfolio strategy, and asset optimization.

Dr. Li holds a B.S. in Molecular Biophysics and Biochemistry from Yale University and a Ph.D. in Biochemistry, with a minor in Management Science and Engineering, from Stanford University.

(Alphabetical Order by Last Name) Speaker Profiles

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".

Ming Li

Administrative Director and Chief Physician, Department of Dermatology, Children's Hospital of Fudan University

Ming Li, Chief Physician, Professor, PhD Supervisor, Postdoctoral Mentor; Director, Department of Dermatology, Children's Hospital of Fudan University. Dr. Li is supported by Shanghai Eastern Talent Program, Pujiang Talent Program, Outstanding Academic Leader of Shanghai Health Commission, and Clinical Scientist of Fudan University Shanghai Medical College. He serves as Vice Chair of the Pediatric Dermatology Group, Chinese Medical Association; Vice Chairman of Shanghai Society of Dermatology and Venereology.

His research focuses on genetic and allergic skin diseases. He has identified 6 novel disease-causing genes and named 2 new syndromes. He has led 19 grants including NSFC projects, published >100 SCI papers in EMBO Mol Med, Am J Hum Genet, etc. He has won 13 scientific awards and was awarded "Top Ten Medical Professionals" of Fudan University Shanghai Medical College.

Speaker Profiles (Alphabetical Order by Last Name)

Stan Z. Li

Professor in Artificial Intelligence, Westlake University
Chief Scientist, BioMap

Stan Z. Li, IEEE Fellow, IAPR Fellow, is a Chair Professor of Artificial Intelligence at Westlake University. He previously served as a Lead Researcher at Microsoft Research Asia and a Senior Researcher at the National Laboratory of Pattern Recognition, Institute of Automation, Chinese Academy of Sciences. He has published over 500 papers and authored 10 books, with over 85,000 citations and an H-index of 156 on Google Scholar. He led the development of the world's first real-time face recognition system, which Bill Gates demonstrated in a CNN interview. He has also designed and developed multiple national-level face recognition systems that have been successfully implemented and applied. He served as an Associate Editor for top-tier AI journals, including IEEE TPAMI, and General Chair, Program Chair, or Program Committee Member for over 100 international conferences. His current research focuses on AI for Life Science — AI virtual cell and AI virtual metabolism in particular, since he joined Westlake University in 2019.

Xiao-Jiang Li

Professor, Guangdong-HongKong-Macao Institute of CNS Regeneration, Jinan University
Director, Guangdong Key Laboratory of Non-Human Primate Research

Xiao-Jiang Li obtained his Ph.D. from Oregon Health Sciences University in 1991 and completed his postdoctoral training at Johns Hopkins University in 1995. 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.
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 35250 and an H-index of 97.

Yu Li

Assistant Professor, Department of Computer Science and Engineering, The Chinese University of Hong Kong

Yu Li is an Assistant Professor in the CSE department at CUHK. He works at the intersection between machine learning, healthcare and bioinformatics, developing new machine learning methods to resolve computational problems in biology and healthcare, which leads to works published in top venues, such as Nature Biotechnology and Nature Methods, and featured by Nature technology features and Science Expert Voices. He was selected to the MIT Technology Review Innovators Under 35 (TR35) China, APEC Asia-Pacific Leaders Under 30 in 2024, and Forbes 30 Under 30 Asia list, Healthcare & Science in 2022. He obtained his Ph.D. in Computer Science from KAUST in 2020. Before that, he got the Bachelor degree in Biosciences with the First-class Honorary from USTC. He received Vice-Chancellor's Exemplary Teaching Award from CUHK in 2024.

(Alphabetical Order by Last Name) Speaker Profiles

Hong Liu

Professor, Shanghai Institute of Materia Medica, Chinese Academy of Sciences

Prof. Liu and her team, focusing on the fundamental scientific questions of "innovative drug research and target discovery," have built and developed a foundational platform and technology system for drug discovery and mechanism research that integrates multiple disciplines, including medicinal chemistry, AI drug design, and chemical biology. Her team has proactively constructed a "drug-like" scaffold compound library. For the discovery of innovative drugs against major diseases such as neuropsychiatric disorders and viral infections, her team has advanced 12 drug candidates to receive 21 clinical trial approvals globally, with 7 progressing into Phase II/III clinical studies, and achieved 10 technology transfers. As the corresponding or co-corresponding author, Prof. Liu has published over 400 papers in top-tier journals such as Science and Nature, which have been cited more than 25,000 times, with an H-index of 67. She has been recognized as an Elsevier "Highly Cited Chinese Researcher" for six consecutive years, and holds 188 authorized domestic and international invention patents. She has received numerous honors including the Fourth National Innovation Competition Award, and has been elected a fellow of the Chinese Chemical Society, the American Institute for Medical and Biological Engineering (AIMBE), and the Royal Society of Chemistry (RSC).

Suli Liu

Project Leader, Shanghai OBiO Technology (Group) Corp., Ltd.

Suli Liu brings over 15 years of biopharmaceutical commercialization experience, with a focus on end-to-end AAV gene therapy CMC from IND to BLA. She has established scalable, cost-optimized cGMP platforms with proven regulatory robustness, enabling successful regulatory submissions. By leveraging Quality by Design (QbD), she resolves critical scale-up bottlenecks—such as empty capsid reduction, lot-to-lot consistency, and impurity clearance—to drive per-dose cost-of-goods reduction and accelerate time-to-market. She delivers integrated CDMO pathways that span from technical feasibility through to commercial accessibility.

Speaker Profiles (Alphabetical Order by Last Name)

Zhongmin Liu

Dean of the Institute of Disaster Medicine Engineering at Tongji University
Tenured Professor and Honorary President of Shanghai East Hospital Affiliated to Tongji University
Foreign Member of the Russian Academy of Engineering

Zhongmin Liu, Chief Physician, Professor, and Doctoral Supervisor, is a Foreign Member of the Russian Academy of Engineering and an Officer of the French Legion of Honour. He currently serves as Dean of the Institute of Disaster Medicine Engineering at Tongji University, Tenured Professor and Honorary President of Shanghai East Hospital Affiliated to Tongji University, Chairman and Legal Representative of Shanghai East United Hospital, President and Legal Representative of Jiaozhou Hospital of Tongji University-affiliated Shanghai East Hospital, and Chairman of Shanghai Tongjin Stem Cell Technology Co., Ltd. He is a Changjiang Scholar of the Ministry of Education and was listed among the world's top 2% scientists in 2024/2025.

Professor Liu has extensive influence in academic and stem cell fields. He is the Founding Chairman of the Disaster Medicine Branch of the Chinese Medical Association and the Disaster Preventive Medicine Branch of the Chinese Preventive Medicine Association, the 5th President of the Cardiovascular Surgery Branch of the Chinese Medical Doctor Association, Council Member of the World Association for Disaster and Emergency Medicine, and President of the Asia-Pacific Disaster Medicine Association. In the stem cell field, he serves as Chairman of the China Stem Cell Industry Alliance, Director of the Shanghai Institute of Stem Cell Clinical Translation, and Head of the Clinical-grade Stem Cell Resource Bank of the National Stem Cell Translational Resource Bank. He has received the Second Prize of the National Science and Technology Progress Award, the Ho Leung Ho Lee Foundation Prize for Scientific and Technological Progress, the Guanghua Engineering Science and Technology Award, and the First Prize of the Chinese Medical Science and Technology Award.

Zhihong Lu

Chief Physician, Children's Hospital of Zhejiang University School of Medicine

Zhihong Lu, Chief Physician in the Department of Nephrology, Children's Hospital of Zhejiang University School of Medicine. She specializes in standardized full course management of pediatric chronic kidney disease and has in depth research experience in the diagnosis and treatment of Fabry disease.

Zhonghua Lu

Principal Investigator, Shenzhen Institute of Advanced Technology, Chinese Academy of Sciences

Dr. Zhonghua Lu is a Principal Investigator at the Shenzhen Institute of Advanced Technology (SIAT), Chinese Academy of Sciences. The major focus of his lab is to develop gene therapy approaches for brain disorders. His lab is also interested in developing nonhuman primate and rodent animal models for brain disorders, especially neurodevelopmental disorders, such as autism spectrum disorders, schizophrenia, and epilepsy, and study disease mechanisms. He has authored over 50 peer-reviewed publications in journals such as Cell, Nature, and Cell Reports, and is a recipient of the 2023 "Top 10 Scientific Advances in Life Sciences of China" award.

(Alphabetical Order by Last Name) # Speaker Profiles

Ho-Ming Luk

Chief of Service, Department of Clinical Genetics, Hong Kong Children's Hospital

Dr. Ho-Ming Luk obtained his basic medical and doctoral degree from the University of Hong Kong. He was trained in Paediatrics in Queen Mary Hospital and Genomic Medicine in Clinical Genetic Service, Department of Health Hong Kong and Guy's and St Thomas' Hospital UK, He is now working as Chief of Service in Department of Clinical Genetics at Hong Kong Children's Hospital, Hospital Authority and the Clinical Lead in the Hong Kong Genome Project/Hong Kong Children's Hospital Partnering Center. He is the fellow of Royal College of Paediatrics and Child health (UK), Royal College of Pathologists (UK) and Royal College of Physicians (London). He is Specialist in Genetics and Genomics (Paediatrics) under Hong Kong Medical Council and Chairman in Genetics and Genomics (Paediatrics) subspecialty board under Hong Kong College of Paediatricians.

His main clinical activities and researches are the usage of cutting-edge technologies in diagnosis, management and prevention for prenatal, paediatric and adult genetic and genomic diseases. He has been published more than 120 articles in local, regional and international peer viewed journals.

Guangzuo Luo

Professor, China Medical University
Founder, Bionce Biotechnology

Guangzuo Luo, Professor at China Medical University and Founder of Bionce Biotech. Contributed to the CMC development of LUXTURNA (2017), the first FDA-approved gene therapy drug, and BEQVEZ (2024), a gene therapy for hemophilia B. As the founder, established Nanjing Bionce Biotech Co., Ltd., dedicated to the development of viral vector-based gene therapies. Led the team to obtain two investigational new drug (IND) approvals for gene therapy products from the National Medical Products Administration (NMPA) of China. Recently published multiple high-quality papers as corresponding author in the field of gene therapy.

Lijia Ma

Leading Scientist, Changping Laboratory
Founder, Westlake Genetech

Dr. Lijia Ma earned her Ph.D. in bioinformatics in 2009 from the Beijing Institute of Genomics at the Chinese Academy of Sciences. In 2010, she joined the University of Chicago as a postdoctoral scholar and was appointed as a research scientist in 2014. Dr. Ma has significantly contributed to several NIH cross-center projects, including ENCODE and modENCODE.

In 2018, she became a faculty member at Westlake University, where she established the Laboratory of Functional Genomics and Systems Biology. Her research centers on designing high-throughput genomic approaches to decode and edit the human genome and developing new technologies to enhance the efficacy and safety of gene and cell therapies.

In 2026, Dr. Ma took on the role of Leading Scientist at Changping Laboratory. Additionally, she is a scientific co-founder and board member of Westlake Genetech, a biotech startup focused on creating AI-accelerated gene editing platforms and advancing next-generation gene therapy products.

Speaker Profiles (Alphabetical Order by Last Name)

Tippi MacKenzie

Professor, Director of The Eli and Edythe Broad Center of Regeneration Medicine and Stem Cell Research, Co-Director of the Center for Maternal-Fetal Precision Medicine, UCSF

Tippi MacKenzie is a pediatric and fetal surgeon at UCSF focused on diagnosing and treating genetic diseases before birth, and is regarded as a leader in the emerging field of fetal molecular therapies.

Dr. MacKenzie runs a translational research lab examining fetal immunology and maternal-fetal tolerance, with the ultimate goal of inventing new fetal therapies for patients with genetic diseases or pregnancy complications. She has moved two fetal molecular therapies from the laboratory to the clinic as FDA-approved phase 1 clinical trials: (1) in utero hematopoietic stem cell transplantation to treat fetuses with alpha thalassemia and (2) in utero enzyme replacement therapy to treat fetuses with lysosomal storage disorders. In 2015, she co-founded the UCSF Center for Maternal-Fetal Precision Medicine to accelerate the processes that link basic research to clinical trials to improve maternal, fetal, and neonatal health. In 2021, she was appointed as the Director of the Eli and Edythe Broad Center of Regeneration Medicine and Stem Cell Research, where her vision is to strengthen stem cell science through the application of gene editing and cell engineering techniques to develop therapies for patients. In both leadership roles, she is committed to strengthening the bridges between scientists and physicians to promote the translation of our fundamental discoveries to help people.

Dr. MacKenzie trained at Harvard College, Stanford University, Brigham and Women's Hospital, and Children's Hospital of Philadelphia. She was elected to the American Society for Clinical Investigation in 2021 and to the National Academy of Medicine in 2022.

Jianhua Mao

Dean and Professor of Department of Nephrology and Urology, President of Children's Hospital of Zhejiang University School of Medicine

Jianhua Mao is a Professor, Chief Physician, and Tutor of PhD., serving as the Dean of the Department of Nephrology and Urology, and President of the Children's Hospital of Zhejiang University School of Medicine. He is a Consultant to the Nephrology Group of the Pediatric Branch of the Chinese Medical Association, Vice Chairman of the Nephrologists Committee of the Pediatric Branch of the Chinese Medical Association, and also serves as a member and Secretary-General of the Standing Committee of the Pediatric Clinic and Health Branch of the China Eugenics Science Association.

Dr. Mao has long been engaged in research on the genetic background and immune mechanisms of idiopathic nephrotic syndrome in children. He has led eight projects funded by the National Natural Science Foundation of China and has received two second-place prizes for science and technology from Zhejiang Province. He has published over 170 papers in prestigious journals such as JAMA Pediatrics, JASN, and Advanced Science. He serves on the editorial boards of Acta Physiologica (Oxf), Pediatric Nephrology, World Journal of Pediatrics, Pediatric Investigation, Chinese Journal of Nephrology, and Chinese Journal of Pediatrics.

(Alphabetical Order by Last Name) Speaker Profiles

Shanshan Mao

Chief Physician and Professor, Children's Hospital of Zhejiang University School of Medicine

Shanshan Mao, Chief Physician, Professor, and Doctoral Supervisor at the Children's Hospital, Zhejiang University School of Medicine, holds an M.D. and Ph.D. and previously conducted postdoctoral research at the Neuroscience Institute of the University of Michigan. She is a Committee Member of the Clinical Epidemiology Group of the Chinese Pediatric Society of the Chinese Medical Association, a Council Member of the Pediatric Clinical and Health Care Branch of the China Healthy Birth Science Association, the Deputy Head of the Genetic Rare Diseases Group of the Pediatrics Branch of Zhejiang Medical Doctor Association, and the Head of the SMA Diagnosis and Treatment Expert Panel of Zhejiang Province. Her main research interests are pediatric neurological genetic rare diseases and neuromuscular diseases. She has served as the principal investigator of three projects funded by the National Natural Science Foundation of China. She has published 30 papers in the field of SMA, and has contributed to the development of 10 standards, guidelines, or expert consensuses as corresponding author, lead writer, or expert panel member.

M

Peter Marks

Senior Vice President of Molecule Discovery and Head of Infectious Diseases, Eli Lilly and Company
Former Director, Center for Biologics Evaluation and Research, U.S. Food and Drug Administration

Peter Marks, MD, PhD, received his graduate degree in cell and molecular biology and his medical degree at New York University and completed Internal Medicine residency and Hematology/Medical Oncology training at Brigham and Women's Hospital in Boston. He has worked in academic settings teaching and caring for patients and in industry on drug development. In 2012, he joined the Food and Drug Administration and served as Director of the Center for Biologics Evaluation and Research from 2016 to 2025. He is currently Senior Vice President for Molecule Development and Head of Infectious Disease at Eli Lilly and Company, has published extensively, and is a member of the National Academy of Medicine.

Speaker Profiles (Alphabetical Order by Last Name)

Špela Miroševič

Co-Founder and CEO, CTNNB1 Foundation

Dr Špela Miroševič, PhD, is Co-Founder and CEO of the CTNNB1 Foundation, a non-profit leading development of the first AAV9-based gene therapy for CTNNB1 syndrome, a rare neurodevelopmental disorder. Her work spans translational research and regulatory development of advanced therapy medicinal products. She holds a PhD in Biomedicine from the University of Ljubljana. Early in her career, she worked in psychosocial oncology, inspired by her mother's illness, collaborating with Professor David Spiegel at Stanford on supportive-expressive group therapy for metastatic breast cancer.

After her son was diagnosed with CTNNB1 syndrome in 2020, she redirected her focus to rare genetic neurodevelopmental disorders and founded the CTNNB1 Foundation to coordinate an international translational research program. She has been directly involved in all stages of gene therapy development: preclinical studies, manufacturing, biodistribution and toxicology, quality control, CMC documentation, and preparation of the full regulatory dossier (IMPD, IB, clinical trial protocol). This work led to regulatory approval of the first clinical trial for CTNNB1 syndrome.

She coordinates research led by Dr Damjan Osredkar, including genotype-phenotype studies, the multicentre Dragonfly natural history study, clinical practice guidelines, and qualitative research on family experiences. She has organised three International CTNNB1 Syndrome Conferences. In Slovenia, she advocated for the "Urban Lex" amendment, enabling public co-financing of early-stage gene therapy and a €1 million national investment in the CTNNB1 programme. The Foundation has raised over €4 million to date. Her current research interests focus on translational strategies for rare neurodevelopmental disorders and European policy frameworks supporting equitable access to advanced therapies.

Shekhar Natarajan

Vice President of International Regulatory Affairs and Policy, Dyne Therapeutics
Chair of Therapies Scientific Committee, International Rare Disease Research Consortium (IRDiRC)
Chair of R&D Focus Group, European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)

Shekhar Natarajan is a seasoned biotechnology executive with nearly three decades of experience in regulatory affairs, quality, and compliance across the full product lifecycle, from preclinical development to commercialization. He is Vice President of International Regulatory Affairs and Policy at Dyne Therapeutics, focusing on genetically driven neuromuscular diseases including Duchenne Muscular Dystrophy (DMD), Myotonic Dystrophy Type 1 (DM1), and Facioscapulohumeral Muscular Dystrophy (FSHD).

A recognized leader in rare disease drug development, he chairs the Therapies Scientific Committee of the International Rare Disease Research Consortium (IRDiRC), working with regulators, industry leaders, and patient advocates to accelerate therapy development. He also serves on IRDiRC's Regulatory Convergence Task Force and the Expert Advisory Group of the British Pharmacopoeia Commission. Through EUCOPE, he supports the European Medicines Agency (EMA) as an industry expert on regulatory initiatives.

Shekhar is an active member of multiple regulatory and scientific organizations and holds Fellowships of the Royal Society of Chemistry (FRSC) and the Organisation for Professionals in Regulatory Affairs (FTOPRA).

(Alphabetical Order by Last Name) Speaker Profiles

Xiaohui Niu

Director of Bone and Soft Tissue Tumor Diagnosis and Treatment Research Center, Beijing Jishuitan Hospital

Dr. Xiaohui Niu is a Chief Physician, Professor, and Doctoral Supervisor who serves as the Director of the Bone and Soft Tissue Tumor Diagnosis and Treatment Research Center at Beijing Jishuitan Hospital. He holds numerous prominent leadership positions, including Chairman of the Sarcoma Expert Committee and Vice Chairman of the Melanoma Expert Committee of the Chinese Society of Clinical Oncology (CSCO), and former Chairman of the Sarcoma Professional Committee of the Chinese Anti-Cancer Association (CACA). He is also a Standing Committee Member of the Tumor Multidisciplinary Diagnosis and Treatment Committee of the Chinese Medical Doctor Association, Deputy Head of the Bone Tumor Professional Group of the Orthopedic Branch of the Chinese Medical Doctor Association, Vice-chairman of the Bone Tumor Professional Group of the Orthopedics Branch of the Chinese Medical Association, and Head of the Bone Oncology Group of the Orthopedic Branch of the Beijing Medical Association. Internationally, Dr. Niu serves as a Board Member of both the International Society for Limb Salvage (ISOLS) and the Asia Pacific Bone and Soft Tissue Tumor Society (APMSTS), and is the former Chairman and Executive Director of the East Asian Bone and Soft Tissue Tumor Group (EAMOG). In addition, he is the Deputy Editor-in-Chief of the Chinese Journal of Bone and Joint. He has served as a reviewer for 34 SCI journals and has published 282 papers as the first author or corresponding author, including 48 SCI articles.

Yuyu Niu

Dean of the Medical Faculty and Vice President of Kunming University of Science and Technology

Yuyu Niu currently serves as the Dean of the Medical Faculty and Vice President of Kunming University of Science and Technology (KUST). He is a Changjiang Scholar Distinguished Professor appointed by the Ministry of Education, the Chief Scientist of the National Key R&D Program of China, and a member of the Expert Panel for the National Key Special Project on "Stem Cell Research and Organ Repair."

Professor Niu has long been dedicated to research on human development and the creation and mechanism study of disease models. He has pioneered the fields of non-human primate genetic engineering models and advanced stem cell research in China. His work focuses on exploring the molecular regulatory mechanisms of cell fate transitions during development and disease, uncovering the potential links between abnormal developmental trajectories of tissues and organs and the onset of complex diseases.

He has published over 80 research papers in prestigious journals such as Cell and Science. His representative achievements have been selected as one of the "Top Ten Advances in Life Sciences in China," a major breakthrough in basic research at the "13th Five-Year Plan Scientific and Technological Innovation Achievement Exhibition," one of the "Top 10 Technological Breakthroughs in the World" by the MIT Technology Review, and a globally influential scientific event by the journal Nature.

Speaker Profiles (Alphabetical Order by Last Name)

Bo Peng

Distinguished Professor at Fudan University

Bo Peng is a distinguished professor at Fudan University. His research is centered on microglia in the central nervous system, with a particular focus on elucidating the mechanisms of their turnover and replacement. Based on the study of microglial repopulation, he has pioneered microglia replacement for the treatment of neurological disorders. In 2020, his team achieved the world' s first efficient microglia replacement in animal models. After that, his group published the world' s first clinical trial of microglia replacement therapy in 2025, demonstrating its efficacy and paving a new path for treating neurological diseases. His key research findings have been published as last author in high-impact journals including Science (2025), Nature Neuroscience (2018), Nature Aging (2023), Neuron (2021), Cell Stem Cell (2025), Cell Reports (2020), Nature Communications (2022), and eLife (2023). Prof. Peng has received numerous awards, including the Asian Young Scientist Fellowship (AYSF), Takeda Innovators in Science Award with Nature, Zhong Nanshan Youth Science and Technology Innovation Award, Chinese Medical Science and Technology Award for Young Scientists, and the Huaxia Medical Science and Technology Youth Award.

Terry Pirovolakis

Founder, CureSPG50
Founder and CEO, Elpida Therapeutics

Terry Pirovolakis, a co-founder of CureSPG50 alongside his wife Georgia, encountered the formidable challenge of addressing his child' s SPG50 diagnosis in 2019. Leveraging robust fundraising and community backing, he spearheaded ground-breaking research, successfully treating his own child within three years through a Canadian CTA and two more children via an FDA IND.

Furthermore, Mr. Pirovolakis established Elpida Therapeutics, a non-profit, committed to addressing ultra-rare, non-commercially viable conditions through gene therapies. Collaborating with industry leaders, Elpida initially focuses on SPG50, CLN7 and CMT4J, with plans to address two more ultra-rare diseases in 2026, allocating profits to sustain and expand programs. Pirovolakis extends his impact by assisting other patient foundations, conducting Gene Therapy 101 classes, and engaging in collaborations, all driven by the overarching goal of saving as many children as possible.

(Alphabetical Order by Last Name) Speaker Profiles

Yi Rao

Chair Professor, Peking University

Yi Rao is a Chair Professor, Founding Director of PKU-IDG/McGovern Institute for Brain Research, Director Emeritus of PKU-THU Center for Life Sciences (CLS), Peking University; Founding Honorary Director, Chinese Institute for Brain Research (CIBR), Beijing; and Founding Director Emeritus, Chinese Institutes for Medical Research (CIMR), Beijing. He received his Ph.D. from the University of California, San Francisco, completed postdoctoral training at Harvard University, and previously taught at the University of Washington and Northwestern University. He returned to China full-time in 2007. He is a founder of the Future Science Prize of China, a co-founder of the XPLORER Prize of China, and served as a member of the medical advisory committee of the Gairdner International Award of Canada.

His previous research focused on the molecular mechanisms of behavior and cognition. In 2019, he proposed the concept of chemoconnectome (CCT). He developed CCT approaches, generated the necessary genetic tools, investigated the functional roles of genes and cells in the CCT, manipulated chemical transmission, and revealed neural circuits. In humans, he uses genetics, genomics, and functional magnetic resonance imaging to study genes and brain regions important for cognition. His research subjects include flies, frogs, mice, rats, monkeys, and humans, with the goal of understanding fundamental mechanisms both those conserved among animals and those unique for humans.

His current research employs biochemistry, molecular biology, genetics, and biophysical imaging to study novel neurotransmitters, new endogenous ligands for GPCRs and their functions, protein kinases and their physiological and pathological roles, the molecular mechanisms of sleep, the molecular mechanisms and therapeutic approaches for multiple sclerosis, and the treatment of Alzheimer' s disease.

Giuseppe Ronzitti

Research Director, French Institute of Health and Medical Research (INSERM)
Director of the Research Strategy, Genethon

Dr. Ronzitti is an accomplished scientist in the field of gene therapy, with a strong track record of innovation and leadership in AAV-based therapeutic strategies. Over the course of his career, he has authored 64 original research articles and 27 reviews, book chapters, and commentaries, achieving a cumulative impact factor of approximately 750 and more than 5,600 citations. He is also a prolific inventor, holding 21 patents related to AAV gene therapy, eight of which have been granted and five licensed to biotechnology companies.

Currently, Dr. Ronzitti serves as the scientific lead on two clinical trials targeting Crigler–Najjar syndrome, including a Phase III study and a Phase I/II trial exploring the use of imlifidase to overcome pre-existing anti-AAV immunity. Notably, his team reported the first-in-human data demonstrating the feasibility of treating patients with anti-AAV antibodies at ESGCT 2025. In parallel, he is advancing three additional AAV-based gene therapy programs from preclinical proof-of-concept studies originating in his laboratory.

Since 2018, Dr. Ronzitti has led the Immunology and Liver Gene Transfer team at Genethon. Appointed as a tenured INSERM Research Director in 2019 within the UMR_S951 "INTEGRARE" unit, he also joined Genethon' s management committee in 2025, contributing to the institute' s scientific strategy. He oversees a multidisciplinary team of over 30 researchers focused on rare genetic diseases, structured around metabolic diseases, AAV technology, and immunology. Throughout his career, Dr. Ronzitti has consistently secured competitive funding from European, governmental, non-profit, and industrial sources, reflecting the impact and translational potential of his research.

Speaker Profiles (Alphabetical Order by Last Name)

Sean Russell

Managing Partner, PrimeRA Pharma Partners LLP
Head of Regulatory Affairs, Fondazione Telethon ETS
Board of Trustees, Eyes on the Future

Sean is an experienced biotech executive and regulatory affairs leader who has been specifically dedicated to commercial cell and gene therapy product development since 2011. Sean currently holds multiple leadership positions within the advanced therapies and pharmaceutical regulatory affairs space as Managing Partner for PrimeRA Pharma Partners, Head of Regulatory Affairs for Fondazione Telethon, Member of the Board of Directors for The Organisation for Professionals in Regulatory Affairs (TOPRA) and Member of the Board of Trustees for a rare disease charity, Eyes on the Future.
Prior to founding PrimeRA Pharma Partners, Sean was a full-time Senior Vice President, Regulatory Affairs for Achilles Therapeutics, a UK headquartered autologous cell therapy company working in oncology. Sean's regulatory affairs leadership facilitated Achilles going from concept to initiating clinical trials in under 3 years in two transatlantic multicentre clinical trials.
Sean's experience in cell and gene therapy regulatory affairs spans all development stages, from concept to withdrawal from the market and across many different product types (including somatic cell therapy, ex vivo genetically modified cells and in vivo gene therapy). Most recently Sean has led the EU MAA and US BLA processes on behalf of the Italian non-profit organization, Fondazione Telethon, to achieve the first approval of a gene therapy product by a non-profit organization.
In addition to specific project work, Sean also participates in various industry activities including as Vice-Chair of the Alliance for Regenerative Medicine (ARM) EU Regulatory Advisory Group.

Rachel Salzman

CEO, Armatus Bio

Dr. Rachel Salzman is Chief Executive Officer at Armatus Bio, a privately held preclinical stage biotech focusing on precision therapies for genetic neuromuscular disorders. For over 20 years, Dr. Salzman has provided drug development expertise in the rare disease space where complex biological and business issues intersect with serious unmet medical need. Prior to joining Armatus, Rachel was Executive Vice President of Portfolio, External Affairs & Development at Alcyone Therapeutics. She co-founded SwanBio Therapeutics in 2017, subsequently acquired by Spur Therapeutics, and served as CEO and Director through 2019. She then founded UltraSquared Bio, a not-for-profit organization dedicated to bringing gene therapies to ultra-rare populations where traditional business cases are not tractable, and in this capacity was awarded a prestigious Termeer Foundation Fellowship. Prior to her time at Swan, she was the Chief Science Officer (CSO) of The Stop ALD Foundation. Dr. Salzman's impact is a result of her unique leadership ability and successful aggregation of resources and commitment from investigators, technologists, and investors. Dr. Salzman has been an active member of ASGCT for over 20 years and has served on multiple committees and task forces, in addition to the Board of Directors. She currently represents ASGCT at the National Academy of Sciences' Forum on Regenerative Medicine. Dr. Salzman received her DVM from Oklahoma State University and a bachelor's degree in animal science from Rutgers University.

(Alphabetical Order by Last Name) Speaker Profiles

Qing Sang

Professor, Institute of Biomedical Sciences, Fudan University

Dr. Qing Sang' s research interests include the genetic mechanisms of female infertility, especially focused on human oocyte and early embryo defects. He has published more than 60 papers in journals including New England Journal of Medicine, Science, Journal of Clinical Investigation, Science Translational Medicine, and Genome Biology, and was invited to write a review for Science. He leads projects such as the National Science Fund for Distinguished Young Scholars, the National Science Fund for Excellent Young Scholars, and key research and development programs of the Ministry of Science and Technology. Recipient of the First Prize of Shanghai Natural Science Award, the First Prize of Natural Science of the National Maternal and Child Health Science and Technology Award, and the title of Shanghai Excellent Academic Leader. He serves as a committee member of the Reproductive Biology Branch of the Zoological Society of China, the Reproductive Biology Branch of the Chinese Society for Cell Biology, and the Reproductive Science Committee of the Chinese Physiological Society. He also acts as an editorial board member for BMC Medicine and Journal of Assisted Reproduction and Genetics.

S. Pablo Sardi

Global Head, Rare Diseases Research, Sanofi

Dr. S. Pablo Sardi, PharmD, PhD, is a globally recognized leader in translational science for rare, genetic, and neurologic diseases. As Global Head of Rare Diseases Research at Sanofi, he directs discovery and early development across a broad portfolio including lysosomal, metabolic, renal, hematologic, neuromuscular, neurodevelopmental, and other rare disorders. His scientific impact also spans common neurologic conditions such as Parkinson' s disease, ALS/FTD, and multiple sclerosis, which continues to inform his strategic perspective. He remains active in advisory roles and global consortia advancing disease-modifying approaches for prevalent neurodegenerative diseases.

Dr. Sardi has guided multidisciplinary teams across small molecules, biologics, gene therapy, vaccines, and oligonucleotides, advancing over twenty programs in parallel and contributing to therapies now reaching patients worldwide. His work covers the full R&D continuum from mechanistic discovery to clinical enablement. With more than 100 publications and patents, he is internationally regarded for contributions to lysosomal biology, neurodegeneration, genetic biomarkers, and AI-enabled translational science. He has held key advisory roles in the Michael J. Fox Foundation' s PPMI and AMP-PD programs.

Dr. Sardi holds a PharmD, MS in Biochemistry, and PhD in Pharmacology from the University of Buenos Aires, and completed postdoctoral training in neurobiology at Harvard Medical School.

Speaker Profiles (Alphabetical Order by Last Name)

Daniel Scherman

Director, French Foundation for Rare Diseases
Head of the Scientific Secretariat of International Rare Disease Consortium (IRDiRC)
Head of the Medicine and Life Science Division of the European Academy of Science

Prof. Daniel Scherman is Director of the Foundation for Rare Diseases (Fondation Maladies rares) and Research Director at the CNRS (French National Scientific Research Center) within the Pharmacy Faculty of Paris University. He serves as Scientific Secretary of the International Rare Disease Research Consortium (IRDiRC) and heads the Medicine and Life Sciences Division of the European Academy of Sciences (EURASC). He is a Corresponding Member of the French National Academy of Pharmacy and a member of the Scientific Committee of the American Society of Gene and Cell Therapy (ASGCT). He is also the founding Editor-in-Chief of the Rare Disease and Orphan Drug Journal.
Prof. Scherman has dedicated most of his scientific career to gene therapy, nanomedicine, pharmacology, and bioimaging. He has published 578 articles (h-index 77, 26,600 citations) as well as 65 reviews or book chapters. He has edited several books, including Gene Transfer, Genome Editing and Gene Therapy (World Scientific, 2025) and the two editions of the Advanced Textbook on Gene Transfer, Gene Therapy and Genetic Pharmacology (World Scientific, 2019; Imperial College Press, 2014).
Previously, Prof. Scherman served as Chief Scientific Officer of Genethon, the gene therapy laboratory of AFM-Téléthon. His scientific contributions have been recognized with numerous awards, including the Grand Prix Emilia Valori Prize of the French Academy of Sciences (2017), the German Experimental Uveitis Research Award, the Academic Palms National Medal, the Prize of the French National Academy of Medicine, the CNRS Silver Medal (2000), and the Neurobiology Prize from the Foundation for Medical Research.

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Song Shan

Vice President, Early Stage Research, R&D Center, Shenzhen Chipscreen Biosciences Co., Ltd.

Dr. Song Shan holds a Ph.D. in Biochemistry and Molecular Biology from Peking University and joined Chipscreen Biosciences in 2001. With over 20 years of experience in preclinical R&D of novel drugs in oncology, metabolism, immunology, and CNS, she has led or participated in the preclinical development of multiple Class I innovative drugs, including Chidamide and Chiglitazar Sodium, both of which have been approved globally for multiple indications. Dr. Shan serves as Principal Investigator for several national and provincial research projects, including the National Major New Drug Innovation Project. He has filed over 300 invention patents, with more than 100 granted, and is a recipient of the China Patent Gold Award in 2017 as a co-inventor of Chidamide.

(Alphabetical Order by Last Name) Speaker Profiles

Yiwei She

Founder and CEO, TNPO2 Foundation

Yiwei She is the founder and CEO of the TNPO2 Foundation, a not-for-profit organization dedicated to building accessible and affordable pathways to early diagnosis and treatment for children with ultra-rare diseases.

Yiwei established the TNPO2 Foundation in 2022 after her son, Leo Wei Church, was diagnosed with an ultra-rare and severe neurodevelopmental disease caused by a mutation in a copy of his Transportin-2 (TNPO2) gene. A former academic mathematician and a founding engineer at the technology company, Landing.AI, Yiwei leveraged her training, background, and keen attention to detail to read and understand Leo' s medical reports, which instilled the confidence to pursue the relatively uncharted path of developing a personalized medicine for her son.

Steeped in her personal experience and key learnings, Yiwei is now on a mission to reshape the medical system for ultra-rare patients, relying on modern science, medicine, and technology to abbreviate the diagnostic and therapeutic odyssey. Through the TNPO2 Foundation, she has launched Project Baby Lion, a data-backed plan to create scalable solutions, from rapid NICU diagnosis to personalized therapeutics development and beyond.

Yiwei obtained a B.S. in Mathematics from Northwestern University and a Ph.D. in Mathematics from the University of Chicago.

Ning Shen

Principal Investigator of the Hundred Talents Program, Liangzhu Laboratory, Zhejiang University

Dr. Ning Shen is a Hundred Talents Program Researcher at the Liangzhu Laboratory of Zhejiang University. Focusing on "AI-driven precision medicine", the Shen Lab develops artificial intelligence algorithms and integrative analysis methods for disease multi-omics data. By combining these computational tools with an organoid experimental platform, the group conducts research and development for new drugs and precision treatments.

As the corresponding author, Prof. Shen has published papers in Nature Genetics (2026), Nature Communications (2024, 2025), and Genome Medicine (2023, 2024) etc, and has been granted 10 international patents. Additionally, Prof. Shen has been invited to give oral presentations at major conferences, including ISMB/ECCB 2025 and the 12th International Scientific Meeting of the Progeria Research Foundation, and serves as the Principal Investigator for both the General Program and the Original Exploration Program funded by the National Natural Science Foundation of China (NSFC).

Speaker Profiles (Alphabetical Order by Last Name)

Tieliu Shi

Professor, School of Life Sciences, East China Normal University

Dr. Tieliu Shi received his Master' s degree in Computer Science and his Ph.D. in Molecular Biology from the University of Louisville, USA. He joined East China Normal University as a full professor in 2008.
His research focuses primarily on the standardization of clinical information and the application of AI in clinical big data; the study of disease mechanisms by integrating clinical information and multi-omics data.
He has applied AI to life sciences, completing the standardization of a catalog of rare diseases and birth defects, establishing a comprehensive annotation platform and phenotypic system for rare diseases and birth defects, and developing a clinical diagnostic system for rare diseases. He has published over 160 peer-reviewed papers.
Currently, he serves as a council member of the Shanghai Society for Plant Physiology and the Shanghai Society for Bioinformatics; and on the editorial boards of "Science China-Life Sciences" and "Pediatric Investigation" journals.

Yanhong Shi

Professor and Chair, Department of Neurodegenerative Diseases, Beckman Research Institute of City of Hope

Yanhong Shi, Ph.D., is Professor and Chair of Department of Neurodegenerative Diseases, and Director of Division of Stem Cell Biology Research at Beckman Research Institute of City of Hope. Dr. Shi is also the Herbert Horvitz Endowed Professor in Neuroscience, a beneficiary of the Christopher Family Endowed Innovation Fund for Alzheimer' s Disease Research in Honor of Vineta Christopher, and an elected fellow of the American Institute for Medical and Biological Engineering (AIMBE). The Shi lab at Beckman Research Institute of City of Hope is focused on human induced pluripotent stem cell (iPSC)-based disease modeling, drug discovery, and cell therapy development for debilitating diseases, including Alzheimer' s disease, Canavan disease, and cancer. The Shi lab has developed cGMP manufacturing processes for human iPSC derivation, differentiation, and genetic engineering, and has demonstrated robust disease-modifying effects of human iPSC-derived cells as a cell therapy for Canavan disease in preclinical studies. Dr. Shi holds multiple patents on human iPSC-based technologies or cellular products and has published rigorously on human iPSC-related studies. Lab Website: https://www.cityofhope.org/yanhong-shi

(Alphabetical Order by Last Name) Speaker Profiles

Xiaokun Shu

Distinguished Professor and Xianghui Scholar at Fudan University

Xiaokun Shu is a Distinguished Professor and Xianghui Scholar at Fudan University, where he joined in 2026. Previously, he was a tenured full Professor at the University of California, San Francisco (UCSF). Dr. Shu received his B.S. in Theoretical Physics from Sichuan University, his M.S. in Condensed Matter Physics from Fudan University, and his Ph.D. in Biophysics from the University of Oregon. He subsequently carried out postdoctoral training in the laboratory of Nobel laureate Roger Y. Tsien.

Dr. Shu' s research lies at the interface of physics, chemistry, and biology, with a long-standing focus on intrinsically disordered proteins and regions (collectively IDRs). He aims to establish a conceptual framework linking "sequence–multiscale dynamic interaction networks–function" in IDRs, thereby extending the classical "sequence–structure–function" paradigm that primarily describes proteins with well-defined three-dimensional structures. His work seeks to elucidate how dynamic interaction networks and multiscale molecular organization of IDRs regulate cellular functions and drive disease processes.

In parallel, his laboratory develops innovative biosensors and chemogenetic tools for visualizing and controlling IDR interaction networks and phase behavior with high spatiotemporal precision, enabling mechanistic studies of cell signaling, transcriptional regulation, biomolecular condensation, and disease-associated processes. His work has been published in leading journals including Science, Cell, Nature Chemical Biology, Nature Structural & Molecular Biology, Molecular Cell, and PNAS. He is the recipient of the NIH Director' s New Innovator Award and the NIH MIRA Award, and is an inventor on multiple patents.

Yilai Shu

Deputy Dean, Professor and Chief Physician, Eye & ENT Hospital of Fudan University
Director, Hereditary Deafness Diagnosis and Treatment Center, Eye & ENT Hospital of Fudan University
Director, Shanghai Key Laboratory of Gene Editing and Cell Therapy for Rare Diseases

Yilai Shu, Professor and Chief Physician, Deputy Dean of Eye & ENT Hospital of Fudan University, Director of Hereditary Deafness Diagnosis and Treatment Center, and Postdoctoral Fellow at Harvard Medical School. He has long focused on the pathogenesis, treatment strategies, and clinical translation of deafness, achieving a series of original breakthroughs with global impact. He elucidated the key pathogenic mechanisms of multiple congenital deafness genes, developed an OTOF gene therapy drug for congenital deafness, led the world' s first clinical trial of gene therapy for congenital deafness, successfully restoring hearing and speech in deaf-mute patients, achieving etiological cure and paradigm shift in deafness treatment. He has promoted nationwide application of this therapy and led the formulation of the first international expert consensus. His findings have been published in journals including The Lancet (cover story), Nature, Nature Medicine, etc. He received the International "Award for Clinical Innovation" from the most authoritative association in the field—Association for Research in Otolaryngology (the first Asian scientist to receive this honor). He serves as Principal Investigator of National Natural Science Foundation of China (NSFC) projects (The Innovative Research Group Project Leader, Distinguished Young Scholar, Excellent Young Scholar) as well as major projects of the Shanghai Municipal Education Commission and Shanghai Science and Technology Commission.

Speaker Profiles (Alphabetical Order by Last Name)

Chun-Qing Song

Principal Investigator, Westlake University

Dr. Song received her Ph.D. (2014) from China Agricultural University and the National Institute of Biological Sciences (NIBS). Then, she finished her postdoctoral training (2015-2019) at the RNA Therapeutics Institute at UMass Medical School. In Oct 2019, Dr. Song joined Westlake University as an assistant professor.
Song' s lab focuses on understanding the mechanisms underlying liver-related diseases—including liver cancer and tissue regeneration—and on advancing CRISPR-based technologies for live-cell DNA imaging, genetic disease correction, aging rejuvenation, and cancer inhibition.

Jie Song

Principal Investigator, Hangzhou Institute of Medicine, Chinese Academy of Sciences

Jie Song, Principal Investigator at the Hangzhou Institute of Medicine, Chinese Academy of Sciences; Adjunct Professor at Shanghai Jiao Tong University; Recipient of the National "Ten Thousand Talents" Leading Talent, National Science Fund for Excellent Young Scholars, and National "Young Thousand Talents" Program.
The research group currently focuses on intelligent drug delivery systems based on DNA structures and gene therapy. The group has published over 100 papers in top-tier international journals including Science, Science Advances, Nature Communications, and JACS. For more details, please visit the lab website: https://songresearchgroup.com/.

Yeyang Su

Advisor, Hope for Rare Foundation
Independent Researcher

Yeyang Su is an independent researcher focusing her inquiry into "the contemporary". She holds master' s degrees in human genetics (Chinese Academy of Sciences), bioethics (Erasmus Mundus Master of Bioethics) and social research methods (University of Sussex), as well as a doctorate in social anthropology (University of Sussex). As part of her inquiry into the contemporary, Dr. Su has conducted years-long, interdisciplinary research into biomedical and AI-related innovation activities in China and beyond. She is a member of iGEM Human Practice Committee, Global Forum for Research Ethics & Integrity, and the host of the podcast "Hang on in there".

(Alphabetical Order by Last Name) Speaker Profiles

Zhenglong Sun

Principal Investigator, Interdisciplinary Research Group on Microscopic Imaging and Drug Development, Shenzhen Bay Laboratory

Dr. Zhenglong Sun is the Principal Investigator of the Interdisciplinary Research Group on Microscopic Imaging and Drug Development and Director of the Bio-Imaging Core Facility at Shenzhen Bay Laboratory. He has long been engaged in interdisciplinary research at the intersection of biomedicine and microscopic optics. His research interests include: (1) investigating the pathological mechanisms of mucinous tumors and developing new drugs; and (2) developing novel optical microscopy imaging technologies and instruments.

Since 2018, he has established a laboratory dedicated to mucinous tumor research and developed a novel mucolytic drug, PX, which has been evaluated in two investigator-initiated clinical trials (IITs). This drug is for the treatment of mucinous tumors such as pseudomyxoma peritonei, colorectal mucinous adenocarcinoma, and mucinous ovarian cancer, as well as respiratory diseases associated with mucus. Over the past five years, he has independently led seven research projects in the fields of oncology drug development and imaging technology and has published 24 research papers, including 14 as first author or corresponding author.

Weihong Tan

Academician of the Chinese Academy of Sciences
Director of the Hangzhou Institute of Medicine, CAS
Dean of the Cancer Hospital Affiliated with Hangzhou Institute of Medical Sciences, CAS

Dr. Weihong Tan is a Professor of Chemistry and Molecular Medicine at the Chinese Academy of Sciences (CAS). He earned his Ph.D. in physical chemistry at the University of Michigan. He is currently the Inaugural Director of the Hangzhou Institute of Medicine, CAS. He is also the Dean of the Institute of Molecular Medicine at Shanghai Jiaotong University, Renji Hospital. He was recognized as a Distinguished Professor and a V.T. and Louis Jackson Professor in the Department of Chemistry and School of Medicine at University of Florida for about 25 years. He serves as Vice President of the Chinese Chemical Society and the Founding Director of the Division of Molecular Medicine.

Professor Tan' s general research focus is in the fields of bioanalytical chemistry, chemical biology and molecular medicine. He specializes in molecular elucidation of diseases, aptamer research, DNA nanotechnology, and cancer theranostics. The total citations of his publications exceed 117,000, equating to an h-index of 181.

Speaker Profiles (Alphabetical Order by Last Name)

Yamin Tan

Director and Chief Physician of the Hematology Department, Zhejiang Cancer Hospital

Yamin Tan, Director of Hematology Department, Chief Physician, and Master's Supervisor at Zhejiang Cancer Hospital. Engaged in clinical diagnosis, treatment, and basic research of benign and malignant blood diseases for a long time. Her main research areas include hematopoietic stem cell transplantation, cellular immunotherapy, and benign and malignant hematological diseases. She has extensive clinical work experience. As a major contributor, she received a National Science and Technology Progress Second Prize. As a technical backbone, she has won the first prize of the Excellent Research Achievement Award for Higher Education Institutions and the first prize of the Science and Technology Progress Award issued by the Ministry of Education. She also won the first prize of Zhejiang Provincial Government Science and Technology Progress Award for establishing the latest strategy of targeted therapy for chronic myeloid leukemia and allogeneic hematopoietic stem cell transplantation. As the project leader, she has received two general project grants from the National Natural Science Foundation of China and two general project grants from the Zhejiang Provincial Natural Science Foundation.

Yue-Qiu Tan

Vice President and Professor, CITIC-Xiangya Reproductive and Genetic Hospital

Dr. Yue-Qiu Tan is a Professor of Central South University and Vice President of CITIC-Xiangya Reproductive and Genetic Hospital. He is also a Deputy Director of the National Health Commission Key Laboratory of Human Stem Cells and Reproductive Engineering, and Deputy Director of the Institute of Reproductive and Stem Cell Engineering, Xiangya School of Basic Medical Sciences, Central South University.

His research focuses on clinical and basic studies of hereditary birth defect prevention and control, prenatal and preimplantation genetic diagnosis, human gametogenesis and male infertility. He has presided over 16 national and provincial-level research projects. As a core team member, he has received 4 awards, including the First Prize of the Ministry of Education Science and Technology Progress Award (2007) and the Second Prize of the National Science and Technology Progress Award (2009). He has edited 6 monographs and published more than 220 papers. Among them, over 110 papers have been published as the first or corresponding author (including co-authorship) in top SCI journals such as Science, The New England Journal of Medicine, Nature Communications, The American Journal of Human Genetics, Signal Transduction and Targeted Therapy, Autophagy and Cell Discovery.

(Alphabetical Order by Last Name) Speaker Profiles

Min Tang

Associate Professor, Shanghai University of Traditional Chinese Medicine
Chief Scientist, Cyberiad Biotechnology (Shanghai)

Tang' s research focuses on the biomanufacturing of human cell-based tissues and organ models. She has been selected for the Shanghai Science & Technology 35 Under 35 and the Shanghai Leading Overseas Talents Program, and has received the First Prize of the Shanghai Pharmaceutical Science and Technology Award, among other honors.
She has developed patented biomanufacturing instruments, including bioprinters and tissue-generation systems, as well as tissue-mimetic biomaterials. Her work has enabled the construction of in vitro tissue and disease models, together with computational algorithms for mechanistic studies, drug discovery, and precision medicine applications.
Tang received her training at Washington University in St. Louis, Columbia University, and the University of California, San Diego. Her research has been published in journals including Cell Research and Advanced Materials, with over 2500 citations. The bioprinted tumor-immune model developed by Tang was featured by Nature as a new tool for cancer research. She has also contributed to three textbooks on bioprinting and tissue engineering, and serves on the 3D Printing Technology Branch of the China Medical Biotechnology Association and other academic committees.

Huarong Tang

Chief Physician, Zhejiang Cancer Hospital

Huarong Tang, MD, Chief Physician of Department of Gynecologic Radiation Oncology, Medical Group Leader, and Specially Appointed Associate Researcher, Zhejiang Cancer Hospital; Member, Cervical Cancer Committee of the China Anti-Cancer Association (CACA); Member, Gynecologic Oncology Specialist Committee of the Zhejiang Medical Doctors Association; Member, Cervical Cancer Prevention and Control Group, Reproductive Health Specialist Committee of the Zhejiang Provincial Preventive Medicine Association. She specializes in radiation therapy for gynecological malignancies—including cervical cancer, endometrial cancer, vaginal cancer, and vulvar cancer—as well as comprehensive treatment strategies for advanced-stage gynecological tumors.

Woody Tang

Chief Medical Officer, Global R&D Center, Fosun Pharma

Woody Tang serves as Chief Medical Officer at Fosun Pharma Global R&D Center. He brings 15 years of professional experience in oncology drug development within the pharmaceutical industry.
Prior to joining Fosun Pharma, Dr. Tang held a series of key leadership positions at Novartis, including Medical Affairs Manager, Clinical Development Manager, Clinical Development Director, and Head of the Solid Tumor Therapeutic Area. He led the development and supported launch of several key products and pipeline assets in China, such as Jakavi, Afinitor, Kisqali, Piqray, and Pluvicto.
Dr. Tang earned his Doctor of Medicine degree from Peking Union Medical College and has many years of clinical practice experience as an internal medicine physician at Peking Union Medical College Hospital following graduation.

Speaker Profiles (Alphabetical Order by Last Name)

Chenji Wang

Associate Professor, School of Life Sciences, Fudan University

Dr. Wang is a Principal Investigator at the State Key Laboratory of Genetics and Development of Complex Phenotype, Fudan University, where he also serves as an Associate Professor. His research program is dedicated to deciphering the molecular mechanisms underlying neurodevelopmental disorders (NDDs), with a specific focus on genetic mutations within the ubiquitin-proteasome system, autophagy, vesicle trafficking, organelle interactions, and metabolite regulation. His lab actively explores precision therapeutic strategies for NDDs. To date, he has published over 40 papers, including Nature Medicine, Science Advances, Nature Communications, and the Journal of Clinical Investigation.

Cuijin Wang

Attending Physician, Department of Neurology, Shanghai Children's Medical Center, Shanghai Jiao Tong University School of Medicine

Cuijin Wang, Attending Physician, Master of Medicine. She has been engaged in clinical work at the Department of Neurology, Shanghai Children's Medical Center, Shanghai Jiao Tong University School of Medicine since July 2013.
She specializes in the diagnosis and treatment of general pediatric diseases and pediatric neurological disorders, with core research and clinical focus on rare diseases including spinal muscular atrophy (SMA) and Duchenne muscular dystrophy (DMD). She is proficient in pediatric general medical care, routine pediatric neurological disease management, pre-vaccination medical evaluation and pediatric electroencephalography (EEG) interpretation.
She is the Committee Member of the Patient Support Working Committee, Shanghai Anti-Epilepsy Association. She is the co-author of the book Rare Diseases published by Shanghai Jiao Tong University Press. She has participated in numerous clinical research projects targeting SMA and DMD. She has been awarded numerous honors including Shanghai Outstanding Resident Physician, Outstanding Supervising Physician, President's Award and Merit Citation. Her team has been honored as a Civilized Team.

(Alphabetical Order by Last Name) Speaker Profiles

Fudi Wang

Qiushi Distinguished Professor, Zhejiang University
Director of the Institute of Nutrition and Food Safety, Zhejiang University

Prof. Dr. Fudi Wang is a globally renowned pioneer in ferroptosis and metal metabolism, with landmark discoveries of novel genes, key mechanisms, and innovative therapeutic targets. He first proposed the concepts of "ferroptosis signaling" and the "FerroLipid" theory, founded the new discipline of Ferrology, and made foundational contributions to the emerging fields of Zincology and Cuprology.

He has published over 260 peer-reviewed papers in top-tier journals including Cell and Nature, with more than 28,000 citations and an H-index of 81. He is a Clarivate Highly Cited Researcher, recipient of numerous prestigious academic awards, and holds several national-level academic honorary titles in China. He also presides over a number of key national research programs, demonstrating his leading role in the field.

Prof. Wang is the Founding Editor-in-Chief of Element, serves on the editorial board of Cell Metabolism, and acts as Deputy Editor of Research. He previously served in senior academic leadership roles, including as President and Vice President at several universities in China.

Hongxing Wang

Chief Technology Officer, Euhearing Therapeutics

Dr. Hongxing Wang serves as the Chief Technology Officer at Euhearing Therapeutics, a biotech startup focused on developing innovative drugs for hereditary deafness and acquired hearing loss. He is a world-renowned AAV technology expert in the field of gene therapy. As a pioneer in AAV capsid engineering, his team has developed CNS-targeted novel capsids that have been licensed to major pharmaceutical companies such as Novartis, Pfizer and Neurocrine, generating billions of dollars in commercial value. With over a decade of industry experience, Dr. Wang worked in several global gene therapy companies including Voyager, Affinia, and NeuShen, with strong expertise in AAV vector development, process development and quality control for AAV vector manufacturing, and clinical translation as well. He has authored 25+ patents in the US and Europe, and successfully advanced multiple gene therapy programs to IND stage. Currently, he is leading Euhearing team in developing multiple first-in-class gene therapies for hereditary deafness, accelerating the clinical translation and commercial application of AAV genetic products.

Speaker Profiles (Alphabetical Order by Last Name)

Hongyan Wang

Dean of the Institute of Metabolism and Integrative Biology at Fudan University
Professor at the Obstetrics and Gynecology Hospital Affiliated with Fudan University

Hongyan Wang, National Outstanding Youth Scholar, Changjiang Scholar of the Ministry of Education, Leading Talent of the Ten Thousand Talents Program, Chief of the 973 Project. Long term commitment to genetic research on major birth defects, focusing on genetic etiology, molecular mechanisms, and metabolic regulation, developing new prevention and control strategies, and identifying genetic and metabolic fingerprints of folate resistant populations. Published over 80 papers as corresponding author (including co authors) in SCI journals such as NEJM and Circulation. Received funding from key, original, and basic science centers of the National Natural Science Foundation of China. Won the first prize of the Ministry of Education' s Natural Science Award . She has successively won the National March 8th Red Flag Bearer, the National May 1st Labor Medal, the Wu Yang Medical Award, the Tan Jiazhen Life Science Innovation Award, and the 7th China Young Female Scientist Award. She is currently the Chairman of the Women Scientists Branch of the Chinese Society of Genetics and the Vice Chairman of the Medical Genetics Branch of the Chinese Medical Association.

Lijie Wang

Head of Scientific Innovation, CorrectSequence Therapeutics

Dr. Wang received her Ph.D. from Shanghaitech University in Jan 2021 and joined CorrectSequence Therapeutics in the same year. As the principal inventor of the company' s proprietary base editor tBE, Dr. Wang leads the optimization and application of base editor, overseeing efforts to adapt and enhance tBE for diverse delivery modalities, as well as the development of proof-of-concept validation approaches. Currently, Dr. Wang is responsible for multiple R&D pipelines focused on hematologic and metabolic diseases, driving the preclinical development of in vivo and ex vivo gene-editing therapeutic candidates targeting a range of disease-associated genes. As the first-author or co-author, Dr. Wang has published these results in Nature , Nature Cell Biology, Cell Research and Cell Stem Cell.

Xiaochuan Wang

Director and Professor, Department of Allergy and Clinical Immunology, Children' s Hospital of Fudan University

Xiaochuan Wang, graduated from Hubei Medical College in 1986 with a bachelor' s degree, graduated from Shanghai Medical University in 1993 with a master' s degree, and graduated from Shanghai Medical University in 1996 with a doctoral degree. In 1997, as visiting scholar at the Nestlé Research Center in Lausanne, Switzerland. From 2000 to 2002, visiting professor in the Department of Immunology at the Children' s Hospital of the State University of New York at Buffalo. Engaged in pediatrics immunology/allergy, including clinical, research, and teaching work on various primary and secondary immunodeficiency diseases, immunodeficiency, allergic diseases, and diagnosis and treatment of adverse reactions to vaccination.

(Alphabetical Order by Last Name) Speaker Profiles

Xingyu Wang

Senior Researcher, National Human Genetic Resources Center
Senior Scientist, Beijing Hypertension League Institute

Professor Xingyu Wang earned his undergraduate degree from Lanzhou University, followed by a Ph.D. in Biochemistry from the University of Ottawa in Canada and postdoctoral training at Harvard Medical School. He currently serves as a Senior Researcher at the National Human Genetic Resources Center, Director of the Division of Population Genetics at the Beijing Hypertension League Institute, and Visiting Professor in the Laboratory of Statistical Genetics at Rockefeller University. He is a distinguished scientist specializing in cardiovascular and cerebrovascular genetic epidemiology, and the management and application of bioresources.

His research focuses on the impact of genetic factors—in conjunction with traditional risk factors—on the pathogenesis of cardiovascular and cerebrovascular diseases and pharmacogenetics. He has published over 100 articles in internationally renowned journals, and his research has received support from the Ministry of Science and Technology through both International Cooperation Program and its Human Genetic Resources Platform Program. Additionally, he is a member of several professional organizations dedicated to biobanking and bioresource management.

Yi Wang

Director, National Children' s Medical Center
President, Children' s Hospital of Fudan University

Yi Wang is a National Distinguished Physician and Shanghai Outstanding Academic Leader. She serves as Principal Investigator of the State Key Laboratory of Brain Function and Brain Diseases, Chief Scientist of a National Key R&D Program, Project Leader of both a National Clinical Key Specialty Construction Program and the International Human Phenome Project. She is also the Coordinator of the Shanghai Brain-Computer Interface (BCI) Collaborative Innovation Cluster, Deputy Director of the Shanghai Key Laboratory for Rare-Disease Gene Editing & Cell Therapy, Lead Scientist of the Shanghai Municipal AI-Driven Research Paradigm Reform & Discipline Advancement Program, and Co-Principal of the MIIT "BCI" Future-Industry Innovation Mission Project.

Her academic appointments include Chair of the Neurology Subgroup of the Chinese Pediatric Society, Member of the Epilepsy Genomics Task Force of the Genetics Commission of the International League Against Epilepsy (ILAE), Vice Chair of the Rare Diseases Committee of the Chinese Hospital Association, Director of the Shanghai Quality Control Center for Pediatrics, Pediatric Surgery & Childhood Autism, and Convenor of the Shanghai Pediatric Internal Medicine Alliance. She has led or participated in 16 multicenter clinical trials (including six gene-therapy trials) and has authored or co-authored more than 10 national/international guidelines. She is corresponding or co-corresponding author of over 120 SCI papers, and has served as editor or co-editor of more than 20 books and textbooks.

Speaker Profiles (Alphabetical Order by Last Name)

Yu Wang

Principal Investigator, Shenzhen Institute of Advanced Technology, Chinese Academy of Sciences

Dr. Yu Wang is a PI at the Shenzhen Institute of Advanced Technology, Chinese Academy of Sciences, and Director of the Shenzhen Key Laboratory of Genome Manipulation and Biosynthesis. He holds a B.S. from the University of Science and Technology of China and a Ph.D. from Harvard University, and has been selected for high-level talent programs of the Chinese Academy of Sciences, Guangdong Province, and Shenzhen. His main research focuses on foundational technologies for molecular and gene-based drug development and their translational applications. Recently, as the corresponding (last) author, he has published research papers in leading international journals such as Nature Biotechnology (highlighted by a Research Briefing), Science Translational Medicine, Advanced Science, Cancer Research, Nucleic Acids Research, and PNAS. He currently serves as a Standing Committee Member of the Gene Engineering and Cell Engineering Pharmaceutical Committee of the China Medicinal Biotech Association, a Committee Member of its Gene Editing Technology Branch, and a Young Editorial Board Member of Life Medicine. Some of his research outcomes have advanced to the translational stage, and among his multiple invention patents, one has been licensed to Roche-Genentech.

Zhiqiang Wang

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

Zhiqiang Wang, M.D. & Ph.D., is a chief physician, professor and doctoral supervisor in the Department of Neurology of the First Affiliated Hospital of Fujian Medical University. He is a member of the Myopathy Group of the Chinese Medical Association Neurology Society, a member of the Neuromuscular Disease and Electromyography Group of the Chinese Medical Doctor Association Neurology Branch, deputy director of the DMD/BMD Group of the China Alliance for Rare Disease, deputy director of the Youth Committee of the Fujian Medical Association Neurology Branch, deputy director of the Fujian Medical Association Genetics Branch, executive director of the Fujian Provincial Genetics Society, and a high-level talent of Fujian Province.

Dr. Wang specializes in the diagnosis and treatment of neuromuscular disorders, peripheral neuropathy and neurogenetic diseases, and has accumulated rich clinical experience in neuromuscular pathology and molecular diagnosis. His research focuses on the diagnosis, treatment and pathogenesis of muscular dystrophy, metabolic myopathy and inflammatory myopathy. He has hosted four research projects of the National Natural Science Foundation of China (NSFC). For the recent three years, Dr. Wang has published more than 20 original articles as corresponding author (or first author) in journals including Brain (three papers), Journal of Cachexia, Sarcopenia and Muscle, Neurology, Neurology: Genetics, Annals of Neurology, The Lancet Regional Health, Acta Neuropathologica, Journal of Medical Genetics, and European Journal of Neurology.

(Alphabetical Order by Last Name) # Speaker Profiles

Wensheng Wei

Professor at the School of Life Sciences, the Biomedical Pioneering Innovation Center and the Peking University-Tsinghua University Joint Center for Life Sciences (Peking University), Director of the Genome Editing Research Center, Peking University
Lead Scientist at Changping Laboratory

Dr. Wei's research group focuses on developing genome editing technologies, novel gene and cell therapies, and high-throughput functional genomics approaches. It also investigates the molecular mechanisms underlying major diseases such as cancer and infectious diseases, aiming to identify new therapeutic targets and strategies for more effective treatments.

Qiang Wen

Deputy Director (Acting) of the Pediatric Oncology Department and Associate Chief Physician at Zhejiang Cancer Hospital

Qiang Wen, Deputy Chief Physician, graduated from the Department of Clinical Medicine at Zhejiang University in 2001 and holds a Master's degree in Obstetrics and Gynecology from Zhejiang University. He started working at Zhejiang Cancer Hospital in 2001, specializing in Gynecological Oncology. In February 2025, he switched to Pediatric Oncology and became the head of Pediatric Oncology department at Zhejiang Cancer Hospital. He studied at University of Miami, Miller School of Medicine in 2014 as a visitor. Proficient in the diagnosis and treatment of various gynecological tumors and solid tumors in children, as well as minimally invasive surgeries such as laparoscopic and robotic surgeries. As the chief translator or deputy editor, he has published 3 books and over 20 academic papers, including 6 SCI articles. Hosted multiple research projects and holds 3 invention patents. Has won multiple awards (with the highest award being the first prize) in national and provincial surgical video competitions. His main research directions are surgical navigation fluorescent probes for malignant tumors, nerve and lymphatic vessel imaging, early screening self-test kits for cervical cancer, and the impact of chemotherapy on ovarian function in young patients.

Janet Woodcock

Former Acting Commissioner, U.S. Food and Drug Administration (FDA)
Former Director, FDA's Center for Drug Evaluation and Research

Janet Woodcock began her FDA career in 1986 at CBER, directing the Division of Biological Investigational New Drugs and the Office of Therapeutics Research and Review, where the first biotech treatments for multiple sclerosis and cystic fibrosis were approved. As CDER Director from 1994, she established global drug approval standards, introduced risk management, launched the Critical Path Initiative and Sentinel Network, and advanced patient-focused drug development.
She later held roles including Deputy Commissioner and Chief Medical Officer, and COO, returning as CDER Director (2007–2020). In 2020 she led therapeutics for Operation Warp Speed, accelerating COVID-19 monoclonal antibodies and antivirals. She served as FDA Acting Commissioner and subsequently as Principal Deputy Commissioner until her retirement in 2024. In 2025, she joined the Scientific Advisory Board of the Hope for Rare Foundation, contributing to the advancement of therapies for rare diseases.
Dr. Woodcock earned a B.S. from Bucknell University and an M.D. from Northwestern, and taught at Penn State and UCSF. Her many honors include the AMA Nathan Davis Award, the Garry Neil Prize, a Lifetime Achievement Award from the Institute for Safe Medication Practices, and the 2019 Biotechnology Heritage Award.

Speaker Profiles (Alphabetical Order by Last Name)

Yuxuan Wu

Co-Founder and CEO, YolTech Therapeutics

Dr. Yuxuan Wu is the co-founder and CEO of YolTech Therapeutics, where he drives the development of next-generation in vivo gene-editing therapies using LNP-mRNA delivery. To date, YolTech has advanced multiple therapeutic programs into clinical stage, with a diversified pipeline spanning genetic, metabolic, cardiovascular diseases.
Prior to YolTech, Dr. Wu developed BRL-101, a CRISPR-based therapy for patients with transfusion-dependent β-thalassemia, which achieved excellent efficacy (Nature Medicine, 2022). From 2015 to 2018, he worked at Harvard Medical School, where he contributed to the development of a CRISPR/Cas9-based editing system for hematopoietic stem cells to treat hemoglobin disorders by reactivating fetal hemoglobin expression (Nature Medicine, 2019, 2020).
Dr. Wu holds a Ph.D. in Biology from Wuhan University.

Zhi-Ying Wu

Qiushi Distinguished Professor, Zhejiang University
Director, Department of Medical Genetics and Center for Rare Diseases, Second Affiliated Hospital, Zhejiang University School of Medicine
Director, Zhejiang Provincial Key Laboratory of Precision Diagnosis and Treatment and Clinical Translation of Rare Diseases

Dr. Zhi-Ying Wu received her M.D. degree in Medicine from Fujian Medical University in 1990 and Ph.D. degree in Neurology from Tongji Medical University in 1999. From February 2001 to December 2003, she obtained her postdoctoral training at Columbia University in New York. Since 1990 to 2007, Dr. Wu had worked as a resident, attending doctor and chief physician in sequence in Department of Neurology, First Affiliated Hospital, Fujian Medical University. She was introduced as a distinguished professor to Huashan Hospital, Fudan University in 2007. In 2015, she was introduced to Zhejiang University School of Medicine as a Qiushi distinguished professor. Currently, Dr. Wu is a Chief physician, professor and tutor of Ph.D. students in Department of Medical Genetics and Center for Rare Diseases, Second Affiliated Hospital, Zhejiang University School of Medicine. She has long been committed to the screening of causative genes and the study of precision medicine in hereditary rare diseases, pioneering in China's rare disease research. She has achieved prominent accomplishments in the precise diagnosis and treatment of paroxysmal kinesigenic dyskinesia (PKD) and Wilson's disease (WD), and is internationally recognized for identifying three causative genes including PRRT2, TMEM151A and COX20. She is the first to report carbamazepine as a specific drug for PKD and demonstrate the efficacy of zinc therapy for pre-symptomatic WD individuals. Serving as corresponding author, she has published over 200 SCI papers in esteemed international journals, such as Nature Genetics, The Innovation, Brain, Molecular Neurodegeneration, Cell Reports,Neurology, Movement Disorders, Archive of Neurology and others. She has been invited to give keynote or special reports at international academic conferences over 20 times. She has been honored with various awards, including the 10th "China Youth Science and Technology Award" and the 9th "China Young Female Scientist Award".

(Alphabetical Order by Last Name) Speaker Profiles

Yunhong Wu

Chief Physician and Director of the Department of Neurology, Shanxi Children' s Hospital

Yunhong Wu, Chief Physician and Director of the Department of Neurology, and Master' s Supervisor at Shanxi Children' s Hospital. She concurrently holds the following positions: Member of the Neurology Group, the 19th Committee of the Chinese Pediatric Society, Chinese Medical Association; Member of the Pharmacotherapy Professional Committee and the Ketogenic Diet Professional Committee, China Association Against Epilepsy; Vice Chair of the Epilepsy and Neuroelectrophysiology Branch, Shanxi Medical Association; Vice Chair of the Vaccine and Immunization Professional Committee, Shanxi Preventive Medicine Association; Vice Chair of the Epilepsy and Neuroelectrophysiology Professional Committee, Shanxi Medical Doctor Association; Chair of the Neurological Disease Prevention and Treatment Professional Committee, Shanxi Maternal and Child Health Care Association; Vice President of the Shanxi Association Against Epilepsy, Chair of its Ketogenic Diet Professional Committee, and Vice Chair of its Pediatric Professional Committee; Vice Chair of the Rare Disease Professional Committee, Shanxi Health Association; and Deputy Director of the Shanxi Children' s Rare Disease Quality Control Center.

Weidong Xiao

Professor of Pediatrics, Grzegorz Nalepa Scholar for Molecular Therapy, and Associate Director of the Gene and Cell Therapy Program at the Herman B Wells Center for Pediatric Research, Indiana University

Weidong Xiao, PhD, is a Professor of Pediatrics, the Grzegorz Nalepa Scholar for Molecular Therapy, and Associate Director of the Gene and Cell Therapy Program at Indiana University' s Herman B Wells Center for Pediatric Research. Dr. Xiao earned his PhD from UNC-Chapel Hill, where he began his pioneering work on adeno-associated viral (AAV) vectors. Before joining Indiana University, he held faculty positions at the University of Pennsylvania and Temple University. His current research focuses on molecular virology, gene therapy vector development, and treatments for hemophilia.

Xiao Xiao

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

Dr. Xiao Xiao received his Ph.D. degree from the University of Pittsburgh in the US in 1992. His research mainly focuses on innovation of gene delivery vectors and gene therapy for diseases including Duchenne muscular dystrophy, hemophilia B and A, lysosomal storage diseases, autoimmune, neurodegenerative and age-related diseases, etc. He has published more than 230 academic papers on international journals with over 20,000 citations. The gene therapy drug for hemophilia B developed by Belief BioMed has received marketing approval from China NMPA. It is the first gene therapy drug for genetic diseases approved in China.

Dr. Xiao Xiao served as a Distinguished Professor of Gene Therapy at the Eshelman School of Pharmacy, University of North Carolina at Chapel Hill; an adjunct professor at the School of Pharmacy and School of Bioengineering, East China University of Science and Technology; Dr. Xiao also served as a member of the Board of Directors of the American Society of Gene and Cell Therapy; a lifetime member of the Chinese Society of Biologists; an editorial board member of many international journals in the field of gene therapy and a reviewer for the U.S. National Institutes of Health (NIH) grants; and a co-founder of Hope for Rare Foundation for rare disease scientific research and translational medicine. Dr. Xiao is also a co-founder of gene therapy companies including AskBio and Bamboo in the United States; and a co-founder of Belief Biomed in China.

Speaker Profiles (Alphabetical Order by Last Name)

Xuehui Xiao

Parent of a Child with Duchenne Muscular Dystrophy (DMD)
Member of the Committee, China DUCHENNE Family Network

Xuehui Xiao, parent of a child with Duchenne Muscular Dystrophy (DMD), expert patient, member of the Committee of the China DUCHENNE Family Network, and Associate Chief Physician in Ophthalmology.
The China DUCHENNE Family Network is a nationwide, nonprofit, and mutual-aid patient community voluntarily joined by families of DMD patients. Its vision is to help DMD patients grow up healthily, and its mission is to unite the strength of DMD patient families, advance diagnosis, treatment, and drug development, and ultimately improve the quality of treatment and quality of life for patients.

Yi Xing

Francis West Lewis Chair, Children's Hospital of Philadelphia
Associate Chief Scientific Officer for Omics, Technology & Engineering, Children's Hospital of Philadelphia
Professor, Department of Pathology and Laboratory Medicine, University of Pennsylvania

Yi Xing, Ph.D. is Associate Chief Scientific Officer for Omics, Technology & Engineering at the Children's Hospital of Philadelphia (CHOP), where he also serves as Executive Director of the Department of Biomedical and Health Informatics, Founding Director of the Center for Computational and Genomic Medicine, and the Francis West Lewis Chair in Computational and Genomic Medicine—an endowed position named after the physician who founded CHOP as the first children's hospital in North America in 1855. He is also a Professor in the Department of Pathology and Laboratory Medicine at the University of Pennsylvania.
Dr. Xing is internationally recognized for his research at the interface of computational biology, genomics, and precision medicine, with a focus on transcriptomics, RNA regulation, rare diseases, and cancer immunotherapy. His lab develops innovative computational and experimental technologies to decipher transcriptomic variation and translate RNA-level insights into clinical applications.

(Alphabetical Order by Last Name) **Speaker Profiles**

Cong Xu

Co-Founder and Chief Operating Officer, Drug Farm

Tony Xu, Co-Founder and Chief Operating Officer (COO) of Drug Farm. He also serves as Legal Representative and Chairman of Shanghai Dosfarm Biotech Co., Ltd. He graduated with a bachelor's degree in Biomedical Science from Fudan University. In 2012, he received his Ph.D. in Genetics from Harvard University, where he conducted research on image-based high-throughput drug screening. During his doctoral studies, he discovered the world' s first muscle stem cell-related drug, and the work was published as a cover article in Cell (first author).

After earning his Ph.D., he joined L.E.K. Consulting as a Management Consulting Project Manager and Senior Life Sciences Specialist. Over three years, he led more than 20 management consulting projects spanning biopharmaceuticals and private equity funds. In 2015, together with renowned geneticist Professor Tian Xu, he co-founded Drug Farm. He leads the company in applying artificial intelligence to drug discovery, focusing on identifying novel targets and developing first-in-class (FIC) innovative drugs for hepatitis B, oncology, autoimmune diseases, and other areas. The company' s proprietary DF-006 is the first case in China that advanced a completely novel target (ALPK1) into international clinical trials. Xu Cong received the "Jiaxing Talent Day" Golden Name Card Talent Award in 2021, and was recognized as a "Leading Pioneer" by the Shanghai Municipal Government in 2025.

Hong Xu

Professor and Discipline Leader of Nephrology, Renal Transplantation, and Rheumatology, Children' s Hospital of Fudan University

Hong Xu, Professor, Chief Physician and Doctoral Supervisor at Children' s Hospital of Fudan University. Professor Xu currently serves as the Discipline Leader of the Department of Nephrology and Rheumatology, and Director of the Shanghai Research Center for Renal Development and Pediatric Nephrology.

Professor Xu also holds concurrent posts as President of the Asian Society of Pediatric Nephrology, Director of the Nephrology Specialty Alliance of the National Children' s Medical Center, Principal Investigator (PI) of the State Key Laboratory of Kidney Diseases, Chairman of the Nephrology Special Committee of the Pediatric Physicians Branch of the Chinese Medical Doctor Association, President of the Rare Disease Committee of Shanghai Medical Doctor Association, and President of the Rare Disease Branch of Shanghai Association of Traditional Chinese Medicine.

Professor Xu has long dedicated herself to the clinical practice and scientific research of pediatric hereditary rare kidney diseases. She led the establishment of China' s first Pediatric Hereditary Nephropathy Database, and pioneered the application of automated peritoneal dialysis and specialized pediatric kidney transplantation in China.

Professor Xu has undertaken over 20 national and provincial research projects, published 185 papers, and led the formulation of 8 clinical guidelines and expert consensus. She has been awarded the First Prize of Shanghai Medical Science and Technology Award, the Second Prize of Shanghai Science and Technology Progress Award, the Soong Ching-ling Pediatric Medicine Award.

Speaker Profiles (Alphabetical Order by Last Name)

Hui Yang

Principal Investigator and Director of the Gene Therapy Center at the Shanghai Institute of Materia Medica, Chinese Academy of Sciences

Hui Yang, Principal Investigator and Director of the Gene Therapy Center at the Shanghai Institute of Materia Medica, Chinese Academy of Sciences. He has long been dedicated to the development of gene editing technologies, safety evaluation, and clinical translation for genetic disease therapy. He pioneered the highly sensitive gene editing off-target detection technology GOTI, developed a series of compact RNA/DNA editing tools with independent intellectual property (e.g., Cas13X/Y, hfCas12Max), and created a deaminase-independent glycosylase-based base editing system. He has published nearly 70 corresponding-author papers in top-tier journals such as Science, Nature, and Cell. His research was selected as one of China's "Top 10 Advances in Life Sciences in 2019" and has successfully advanced multiple gene editing therapeutics into cutting-edge global clinical research stages.

Richard Yang

Rare Disease Patient
Founder and Chairman, Reflection Biotechnologies

Richard Yang is a rare disease patient-turned-researcher. He suffers from Bietti Crystalline Dystrophy (BCD), a rare retinal degeneration. He is the first named inventor on the world's first BCD gene therapy patent. In the process of progressive vision loss, he initiated and drove the first proof-of-concept study of BCD gene therapy, achieving the 0-to-1 breakthrough in BCD gene therapy research, and obtaining orphan drug designation from the U.S. FDA. He has served as a member of the Orphan Drug Development Guidebook (ODDG) Task Force of the International Rare Diseases Research Consortium (IRDiRC).
He has published in international academic journals as sole author, co-first author, or co-corresponding author covering transparent science in rare disease research, BCD gene therapy, and global genetic prevalence of BCD. He founded Reflection Biotechnologies (ReflectionBio) which applies the By Patients, For Patients™ philosophy in orphan drug development: With relentless efforts and the right partners, we, as patients, can make a difference in driving R&D to help ourselves and others. He earned a B.S. degree from Peking University and a J.D. degree from the University of Michigan.

Yang Yang

Professor, State Key Laboratory of Biotherapy, Sichuan University

Yang Yang, Ph.D., is a Professor and Ph.D. Supervisor specializing in gene therapy for genetic diseases. His research focuses on AAV-mediated gene delivery and gene editing, and the clinical translation of gene therapy products.
Dr. Yang has published over 60 papers as first or corresponding author in leading journals, including The New England Journal of Medicine, Nature Biotechnology, Nature Communications, Blood, and Science Advances. He holds 15 invention patents, of which 7 have been granted.
He has led multiple national-level research programs, including grants from the National Natural Science Foundation of China and the National Major New Drug Development Program. Several gene therapy candidates developed by his team have advanced into investigator-initiated trials, with three currently in Phase II clinical studies.

(Alphabetical Order by Last Name) Speaker Profiles

Yue Yang

Principal Investigator and Director of Center of Excellence for Translational and Regulatory Science, School of Pharmacy, Tsinghua University

Dr. Yue Yang is a Principal Investigator and doctoral supervisor at the School of Pharmaceutical Sciences, Tsinghua University, where she also serves as Director of the Center of Excellence for Translational and Regulatory Sciences. Her research interests cover drug regulatory science, pharmaceutical policy, health technology assessment, and healthcare insurance access. She is a member of the WHO Expert Committee on Specifications for Pharmaceutical Preparations (WHO ECSPP), an expert in the Expert Group on Pharmaceutical Pricing and Bidding Procurement of the National Healthcare Security Administration, and a board member of the China Association for Drug Administration Research. Continuously deepening her expertise in drug regulatory science, drug policy and legislation, innovative drug value assessment, and medical insurance access, she participated as a core expert in the formulation and revision of the Drug Administration Law and the Vaccine Administration Law. She is also the chief editor of the representative work Drug Policy for Rare Diseases, which focuses on rare disease policy.

Chengqi Yi

Boya Professor, Peking University

Chengqi Yi is a Boya Professor at School of Life Sciences at Peking University. He is also an Investigator at Peking-Tsinghua Center for Life Sciences, Investigator and vice dean of Beijing Advanced Center of RNA Biology (BEACON), and holds a joint professorship at College of Chemistry and Molecular Engineering at PKU.

His lab studies RNA modifications and their roles in gene expression regulation. In particular, we focus on the regulation and functions of non-m6A RNA modifications, including pseudouridine (Ψ), N1-methyladenosine (m1A) and N6,2-O' -dimethyladenosine (m6Am). In the past 10 years, Yi Lab has characterized their modification enzymes, developed quantitative tools to profile the modification pattern in the transcriptome, and investigated their roles in RNA metabolism and function. By manipulating modifications, his lab developed RNA editing tools to suppress disease-causing nonsense and missense mutations, as well as to expand the genetic code at the RNA level. Overall, these findings add to the expanding repertoire of mRNA modifications and open up new directions of post-transcriptional modifications and epitranscriptomics.

Speaker Profiles (Alphabetical Order by Last Name)

Hao Yin

Hongyi Distinguished Professor, Wuhan University

Hao Yin is a Hongyi Distinguished Professor at Wuhan University. In 2024, he was awarded funding from the National Science Fund for Distinguished Young Scholars and currently leads a National Key R&D Program project. He received his Bachelor's degree from Nanjing University and his Ph.D. from the University of Colorado. He completed his postdoctoral training in Robert Langer's laboratory at the Massachusetts Institute of Technology. In February 2018, he established his research group at Wuhan University.

He has published or had accepted over 50 papers in international journals, with approximately 15,000 citations. Twelve of his publications have been recognized as ESI Highly Cited Papers. Many of his research findings have been reported by mainstream international media and highlighted in commentary articles in Nature-branded journals. Hao Yin has long focused on gene editing and gene therapy. As corresponding author, he has published or had accepted multiple papers in leading journals, including Cell (2024), Nature (2026), Nature Biotechnology (2025), Nature Chemical Biology (2024, 2026), Nature Biomedical Engineering (2020, 2022, 2024, 2026), and Nature Methods (2022). He has filed several PCT international patents based on his work at Wuhan University, some of which have been licensed or commercialized.

Fengwei Yu

Distinguished Investigator, Temasek Life Sciences Laboratory, National University of Singapore

Dr. Fengwei Yu is a Distinguished Investigator (Professor level) at Temasek Life Sciences Laboratory (TLL), Singapore. He has established his own research lab for 23 years (since 2003). Dr. Yu was elected as the President of Society for Neuroscience Singapore (2015-2018) and currently is the executive committee member in Stem Cell Society Singapore (2025-2028) and Singapore Society for Developmental Biology (2018-present). He was awarded Singapore National Young Scientist Award (2007) and is currently serving as a Section Editor in the PLOS Genetics journal. His research has focused on the mechanisms of stem cell biology as well as neuronal development/remodelling in Drosophila in the past 30 years. As a corresponding author, he has published his important findings in Nature Cell Biology, Nature Neuroscience, Neuron, Developmental Cell, Genes & Development, EMBO Journal, PLOS Biology, Cell Reports and PNAS. Since 2023, his lab has been also using human cerebral organoid model to understand the mechanisms of neural stem cell proliferation, neuronal differentiation/remodelling and their implications in neurodevelopmental disorders, especially autism spectrum disorders.

(Alphabetical Order by Last Name) Speaker Profiles

Wei Zhang

Associate Director of ASO Core Research, n-Lorem Foundation

Dr. Wei Zhang is Associate Director of ASO Core Research at n-Lorem Foundation, specializing in antisense oligonucleotide (ASO) drug development, translational research, and precision medicine for rare diseases. With more than 15 years of research and team leadership experience, he has contributed to over 50 patient-focused ASO programs, advanced more than 20 projects into clinical stages, filed 5+ ASO patents, and collaborated with Ionis, St. Jude, and GondolaBio on ASO development programs. He offers strong capabilities in platform building, translational execution, and scientific innovation.
Experience: Currently serves as Associate Director of ASO Core Research at n-Lorem Foundation. Previously held roles as Professional Researcher at Cleveland Clinic/Case Western Reserve University, Adjunct Assistant Professor at CCLCM, and Postdoctoral Researcher at the University of Wisconsin–Madison.
Education: Ph.D., National University of Singapore; M.S., Georgia Institute of Technology; B.S., Zhejiang University; MPA, Macau University of Science and Technology.

Chuanling Zhang

Associate Researcher, School of Pharmacy, Peking University

Chuanling Zhang graduated from Peking Union Medical College in July 2010 with a Ph.D. in Genetics. In the same year, he joined the School of Pharmaceutical Sciences, Peking University as an Assistant Researcher. From November 2018 to November 2019, he conducted visiting research at Stanford University School of Medicine, and in August 2020 he was promoted to Associate Researcher. He was selected for the "Young Talent Support Program for Professional and Technical Positions at Peking University".
His long-term research focuses on new technologies for the precise labeling and modification of viruses, with an emphasis on viral structural modification and functional regulation, and virus–host interactions. Through years of research, he has achieved the following outcomes: 1. Established novel methods for the precise labeling and modification of viruses with unnatural amino acids/sugars, providing new tools for studying viral infection pathways, controlled replication, and virus–host interactions; 2. Constructed a series of novel viral vectors with organ-targeting capability and low immunogenicity, providing safe and efficient gene delivery vehicles for disease treatment. He has successively led multiple projects funded by the National Natural Science Foundation of China, the Ministry of Education, and the Beijing Natural Science Foundation. As first or corresponding author, he has published more than 20 research papers in journals such as Nucleic Acids Research, Biomaterials, and Journal of Controlled Release, and has been granted multiple domestic and international patents. His research achievements were selected for the "Selected Outstanding Achievements of the Beijing Natural Science Foundation during the 12th Five-Year Plan Period".

Speaker Profiles (Alphabetical Order by Last Name)

Feng Zhang

Professor, Institute of Medical Genetics and Genomics, Fudan University
Academic Vice President, Children's Hospital of Fudan University

Feng Zhang is a Professor at the Institute of Medical Genetics and Genomics, Fudan University, and serves as the Academic Vice President of the Children's Hospital of Fudan University. He has been supported by numerous prestigious national programs, including the Changjiang Scholar Distinguished Professorship, the National Science Fund for Distinguished Young Scholars, the National Ten Thousand Talents Program (Leading Talent in Scientific and Technological Innovation), and the National Key Research and Development Program. He also serves as an Executive Director of the Genetics Society of China and Chair of its Youth Working Committee, as well as Vice Chair of the Reproductive Health Committee of the Chinese Association of Rehabilitation Medicine. His honors include the 15th China Youth Science and Technology Award, China's 21st Century Important Medical Achievements, the First Prize of the Natural Science Award of the Ministry of Education, the WuXi AppTec Outstanding Achievement Award in Life Chemistry, and China's Important Medical Progress of 2023. Dr. Zhang is dedicated to basic and translational research on human genetic variations that lead to infertility, birth defects, and other diseases. He has published over 200 papers in SCI-indexed journals such as The New England Journal of Medicine, Nature Medicine, Nature Genetics, The American Journal of Human Genetics, Cell Research, The Journal of Clinical Investigation, Nature Communications, and Science Advances.

Shuning Zhang

Senior Vice President, Medical and Clinical Affairs, Zhejiang Hopstem Bioengineering Company Limited

Shuning Zhang earned a Master of Medicine from Peking University Health Science Center, a Ph.D. from Rensselaer Polytechnic Institute (USA), and completed postdoctoral training at Albany Medical Center (USA). She subsequently served as an Instructor and later Associate Research Professor at the University of Washington. She has been recognized as an Overseas High-Level Talent of Zhejiang Hangzhou Future Sci-Tech City, a High-Level Talent of Qiantang District and Hangzhou Municipality, a Young Science and Technology Elite at the Hangzhou Academician Expert Workstation, a Core Member of the Zhejiang Provincial Leading Innovation Team Award, Head of Excellent Team at Huadong Medicine, and Management Star at Hopstem Biotechnology. Previously, she held the positions of Medical Director at Zhejiang Adamerck Pharmaceutical and Director of the Clinical Medicine Department at Huadong Medicine Global New Drug R&D Center, where she was responsible for the initiation and clinical development of multiple Class 1.1 innovative drug projects, including several international collaboration projects.

Xin Zhang

Chief Physician, Children's Hospital of Zhejiang University School of Medicine

Xin Zhang, Chief Physician in the Department of Neurology, Children's Hospital, Zhejiang University School of Medicine. He presided over one project each under the "Jianbing Lingyan + X" Program, Zhejiang Provincial Basic Public Welfare Research Program, and Zhejiang Provincial Traditional Chinese Medicine Science and Technology Program, and published eight papers as first author or corresponding author.

(Alphabetical Order by Last Name) Speaker Profiles

Yue Zhang

Principal Investigator of the Hundred Talents Program, Liangzhu Laboratory, Zhejiang University
Member of the Gene Editing Technology Branch, China Association of Medical Biotechnology
Deputy Director of the Cell and Gene Therapy Platform, Liangzhu Laboratory

Dr. Yue Zhang is a Principal Investigator of the Hundred Talents Program at Liangzhu Laboratory, Zhejiang University, Doctoral Supervisor, and Adjunct Researcher at the Children' s Hospital Affiliated to Zhejiang University School of Medicine. He is a recipient of the Zhejiang Provincial Long-Term Program for Overseas High-Level Talents Innovation, and serves as a committee member of the Gene Editing Technology Branch of the China Association of Pharmaceutical Biotechnology.
Dr. Zhang has innovatively developed a novel gene therapy strategy that uses AAV to deliver inhibitory tRNA for nonsense mutation readthrough. His related findings have been published in Nature and highlighted by Nature Reviews Drug Discovery.
His research group focuses on the research and clinical translation of gene therapy for genetic and rare diseases, devoting to the development of delivery vectors with high transduction efficiency and high targeting specificity, so as to promote the clinical and industrial translation of gene therapy for rare diseases.

Ling Zhao

Founder of Volunteers Group for Chinese Dravet Syndrome Patients

Ling Zhao, founder of Volunteers Group for Chinese Dravet Syndrome Patients. Together with various partners, the Volunteers Group supports families of children with Dravet syndrome, facilitates research collaboration, and advocates for social support.

Changyang Zhou

Principal Investigator, Center for Excellence in Brain Science and Intelligence Technology / Institute of Neuroscience, Chinese Academy of Sciences

Dr. Changyang Zhou is a Principal Investigator and PhD supervisor at the Center for Excellence in Brain Science and Intelligence Technology (Institute of Neuroscience), Chinese Academy of Sciences, and founder of EpiGene Therapeutics. His research focuses on developing novel gene editing and epigenetic regulation tools. He published a landmark study on off-target effects of DNA base editors as first author in Nature, and reported a new epigenetic tool achieving durable gene silencing in non-human primates as corresponding author in Nature Biotechnology. He has authored over 20 high-impact papers (19 as first/corresponding author), with 2,000+ citations and an H-index of 15. As founder of EpiGene, he has advanced multiple gene and epigenetic editing therapies into clinical stages, making it the world's first epigenetic editing company to enter the clinic, securing over $100 million in funding. His honors include the Top 10 Advances in Chinese Life Sciences and the Ray Wu Prize.

Speaker Profiles (Alphabetical Order by Last Name)

Bo Zhu

Senior Advisor, Chinese Organization for Rare Disorders

Bo Zhu holds a dual academic background in Pharmacy and Business Administration. He began his career with seven years of service in the pharmacy department of a tertiary public hospital before joining China's largest pharmaceutical bidding agency, where he played a key role in the development and operation of a third-party e-transaction platform for centralized procurement of drugs and medical consumables. Over the following two decades, he worked in market access and policy research for both European and American multinational pharmaceutical companies, as well as for RDPAC (the Regulatory Affairs and Drug Development Committee of the China Association of Enterprises with Foreign Investment). This experience has given him deep insights into the challenges and opportunities confronting the R&D-based pharmaceutical industry, along with a comprehensive understanding of China's healthcare system reforms. He has led more than twenty industry research initiatives and possesses extensive expertise in pharmaceutical regulation and market access policy analysis.

Wencheng Zhu

Junior Principal Investigator, Institute of Molecular Physiology, Shenzhen Bay Laboratory

Dr. Wencheng Zhu is a Junior Principal Investigator and PhD Supervisor at the Institute of Molecular Physiology, Shenzhen Bay Laboratory. His laboratory focuses on human oocyte maturation and early embryonic development, aiming to elucidate the underlying molecular mechanisms and to explore non-genetic intervention strategies for improving the quality of aged oocytes and rescuing developmental failure in poor-quality embryos. As first or corresponding author (including co-first/co-corresponding author), Dr. Zhu has published multiple research articles in journals including Cell, Nature, Cell Res, Cell Discov, Genome Biol, and Cell Rep. He has led multiple research projects funded by the National Natural Science Foundation of China and the Shanghai Municipal Science and Technology Commission, among others. Dr. Zhu has received awards from organizations including the International Society for Stem Cell Research, Cold Spring Harbor Asia, and the Chinese Society for Biochemistry and Molecular Biology. He was also selected for the National Young Talents Program, the Shanghai Science & Technology 35 Under 35 Program, and the Shanghai Eastern Talent Plan.

(Alphabetical Order by Last Name) # Speaker Profiles

Xuefeng Zhu

Dean and Professor of the School of Basic Medical Sciences, North China University of Science and Technology

Xuefeng Zhu is a Professor and Dean of the School of Basic Medical Sciences at North China University of Science and Technology. After earning his Ph.D. from the Chinese Academy of Agricultural Sciences, he gained extensive international experience at the Karolinska Institute and the University of Gothenburg.

His research focuses on mitochondrial genomics and the mechanistic study of mitochondrial diseases. By integrating biochemical, cellular, and structural biology approaches, he aims to bridge the gap between fundamental mitochondrial biology and clinical application, identifying new therapeutic targets and intervention mechanisms for rare diseases. His work has been featured in leading journals including Cell, Molecular Cell, Nature Communications and Science Advance. He is also the inventor of three international patents.

Yongchuan Zhu

Investigator, Shanghai Mental Health Center, Shanghai Jiao Tong University School of Medicine

Dr. Zhu earned his Ph.D. at the Institute of Neuroscience, Chinese Academy of Sciences, and conducted postdoctoral research at the Johns Hopkins University School of Medicine and the Scripps Research Institute. His research centers on the molecular basis of neurodevelopment and cognitive dysfunction, the molecular and circuit mechanisms of learning and memory, as well as the pathogenesis of related psychiatric disorders. His work has appeared in high-impact international journals, including Science, Nature Communications, PNAS, and Cell Reports. Dr. Zhu is a recipient of the Shanghai Overseas High-Level Talent Program and the Shanghai Pujiang Talent Program, and has led multiple national research grants in China.

Scientific Innovation Alliance for Rare Diseases

瑞鸥科学创新联盟

由瑞鸥公益基金会发起，本着交流、创新、共益的基本原则，联盟聚集了正在从事罕见病科研的研究机构、医疗机构、转化医学中心、高校和企业，搭建罕见病科研领域的交流平台。平台以行业信息交流、行业经验共享、行业资源互助为主要形式，促进领域内产、学、研、医、患的共同发展。

2022年至今，科创联盟平台吸引了81个成员加入，包括55个研究团队成员、26家企业成员，为多位联盟成员提供了项目咨询、寻找合作方、深度采访、管线咨询等服务。

The Hope for Rare Foundation has set up the Alliance for Scientific Innovation, comprised of representatives from industry, academia, research institutes, and patient advocacy groups, to promote collaboration and effective resource utilization.

Since 2022, the Alliance has attracted 81 members, including 55 research teams and 26 corporate members. The platform has provided services to many alliance members, including project consulting, partner matchmaking, in-depth interviews, and pipeline advisory support.

- 从事罕见病相关基础研究的高校、学术团体及研究者 (PI)

  Universities, Academic Organizations, and Principal Investigators (PIs) engaged in basic research on rare diseases.

- 从事罕见病研究和诊疗的高校、研究机构、医疗机构

  Universities, Research Institutes, and Medical Institutions engaged in rare diseases research and treatment.

- 开展罕见病药物研发的生物医药企业，测序类、动物造模、CRO、CMO/CDMO 企业

  Biopharmaceutical companies engaged in rare disease drug research and development, as well as Genetic Sequencing, Animal Modeling, CRO & CMO/CDMO companies.

- 关注罕见病领域成果转化的创新发展及转化中心、产业集群

  Innovative Development & Translational Centers and Industrial Clusters focused on the commercialization of achievements in the field of rare diseases.

- 关注罕见病相关产业的投资机构

  Investors focused on industries related to rare diseases.

www.hope4rare.org.cn

合作请联系/ For collaboration, please contact:
张女士/ Ms. Zhang: wenjun.zhang@hope4rare.org.cn
耿女士/ Ms. Geng: wenhan.geng@hope4rare.org.cn
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罕见病药物研发全球领导者

阿斯利康是一家科学至上的全球生物制药企业，专注于肿瘤、罕见病及涵盖心血管、肾脏及代谢、呼吸及免疫等生物制药领域的处方药研发及商业化。

AstraZeneca is a global, science-led biopharmaceutical company that focuses on the discovery, development, and commercialisation of prescription medicines in Oncology, Rare Diseases, and BioPharmaceuticals, including Cardiovascular, Renal & Metabolism, and Respiratory & Immunology.

作为阿斯利康罕见病业务子公司，瑞颂制药致力于为罕见病患者及其家庭研发并提供改变生命的药物。三十多年前，瑞颂制药首次引领将补体系统的复杂生物学转化为变革性药物，并在具有重大未满足需求的疾病领域，持续开展丰富的研发创新。依托阿斯利康的全球资源，瑞颂制药不断扩展业务版图，为全球更多罕见病患者提供服务。

Alexion, AstraZeneca Rare Disease, is focused on serving patients and families affected by rare diseases and devastating conditions through the discovery, development and delivery of life-changing medicines. A pioneering leader in rare disease for more than three decades, Alexion was the first to translate the complex biology of the complement system into transformative medicines, and today it continues to build a diversified pipeline across disease areas with significant unmet need, using an array of innovative modalities. As part of AstraZeneca, Alexion is continually expanding its global geographic footprint to serve more rare disease patients around the world.

目前，阿斯利康在中国已开展了超过了20个罕见病临床研究，重点关注肾脏、肾脏移植、神经科、血液等补体系统相关的疾病领域，并致力于拓展非补体生物学机制的疾病领域，如心肌淀粉样变性、代谢性疾病、罕见肿瘤等。

In China, AstraZeneca is currently conducting more than 20 clinical studies in rare diseases, primarily focused on complement-mediated conditions across nephrology, kidney transplantation, neurology, and hematology. In parallel, AstraZeneca continues to broaden its rare disease pipeline beyond complement biology into additional areas of high unmet need, including cardiac amyloidosis, metabolic disorders, and rare tumours.

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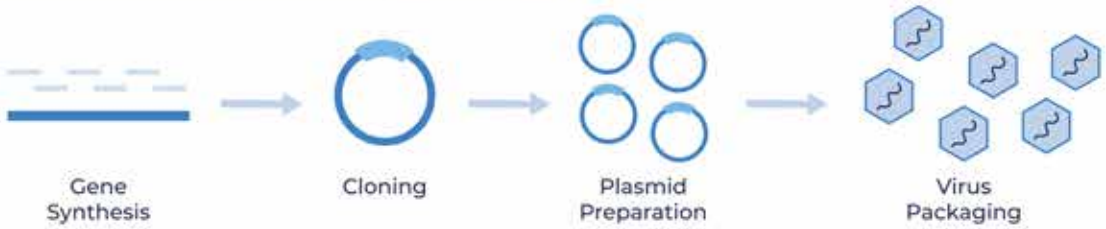

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智因团队深耕精准诊断行业十余年，构建了全球最大的中国人种基因-表型数据库，并以卓越的数据采集技术、全自动精准诊断AI云平台、大数据科研素材自动挖掘平台等硬核科技，以及覆盖多学科多领域的精准诊断产品和服务体系，成为了业内领先的一站式NGS精准诊断实验室及大数据整体解决方案提供商。

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遗传病的精准诊断一定程度上依赖数据库，比如排除良性变异可能要借助**普通人数据库**的变异分布频率（MAF），对变异进行致病性变异的升级需要借助**患者数据库**。智因东方利用“大数据+机器学习”基因数据训练系统进一步**提升约1/3遗传病诊断率**，**积累40万+的患者WES/WGS基因型-表型大数据**，同时兼具20万+人级的中国人种正常人对照数据库。拥有**强劲的科研转化能力，近2年智因大数据助力发现人类新致病基因23个。**

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智因大数据	√	√	纯中国	WES/WGS	20万+患者 20万+正常	大+中+小
dbSNP	√		欧美	文献		小
gnomAD(ExAC)	√		欧美	WES	万人级	小
千人基因组	√		亚洲	WGS	汉族200+	小
DGV	√		欧美	文献		大
HGMD		√	欧美	文献		小
Clinvar	√	√	欧美	文献		小
OMIM		√		文献		小
Decipher	√	√		arryCGH	万人级	大

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智能硬件 Intelligent hardware

- 耗材智能柜 consumables AI cabinet
- 智能货架 AI shelf
- 智能试剂柜 AI reagent cabinet
- 手持移动终端 handheld mobile terminal

SPD软件 SPD software

- B2B供应链协同平台 B2B supply chain collaboration
- 精细化管理平台 hospital fine management platform
- 智能看板 AI center screen
- 冷链监控平台 cold chain monitoring platform

运营团队 operations team

- 项目实施运营 implementation and operation
- 软硬件维护 software / hardware maintenance
- 手术跟台服务 intra-operative technical support

精细化管理流程 Refined management process

01寄售类高值耗材
01 Consignment high-value consumables

- ·单件贴码管理 ·Single piece code management
- ·全流程条码化管理 ·Full process barcode management
- ·智能柜 管理用后结算 ·Smart cabinet management post settlement

02跟台类高值耗材
02 High value consumables for Taiwan

- ·厂家出厂码全程管理 ·Full process management of manufacturer's factory code
- ·骨科智能AI识别系统全流程追溯 ·Orthopedic intelligent AI recognition system full process traceability
- ·预扫码、虚拟入库 ·Pre scan code, virtual storage
- ·备案用后结算 ·Post filing settlement

03低值耗材
03 Low value consumables

- ·定数管理 ·Fixed quantity management
- ·全条码化管理 ·Full barcode management
- ·二级库智能化管理 ·Intelligent management of secondary libraries

04检验试剂
04 Testing reagents

- ·定数管理 ·Fixed quantity management
- ·全条码化管理 ·Full barcode management
- ·消耗结算 ·Consumption settlement
- ·冷库智能化管理 ·Intelligent management of cold storage

BYX 保钰鑫 项目案例 SERVICE ITEMS

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BELIEF BIOMED

公司概况Company Profile

信念医药集团(Belief BioMed Inc.,BBM)是一家全球化的集基因治疗产品研发、生产和临床应用为一体的高科技企业,致力于通过安全高效的病毒载体技术为严重遗传疾病和慢性疾病提供更加有效的创新性基因疗法。

Belief BioMed Inc. (BBM) is a globally innovative biotech company that integrates the development, manufacturing and clinical application of gene therapy products. The company is committed to providing innovative and more effective gene therapies for severe genetic and chronic diseases through safe and efficient viral vector technology.

技术与平台Technology & Platform

工程化载体 Vector System

- **工程化AAV衣壳**
  Engineered AAV vector
- **更高的递送效率**
  Highly efficient delivery
- **更强的组织靶向性**
  Tissue-specific targeting
- **更低的免疫原性**
  Lower immunogenicity

多元的产品管线 Pipeline

- **罕见遗传疾病**
  Rare genetic diseases
- **重大常见疾病**
  Major common diseases
- **通过中国的研究者发起临床研究(IIT)加速产品的临床开发速度**
  Accelerated clinical development speed via investigator-initiated-trial pathway in China (IIT)

自主的生产平台 In-house Manufacturing

- **灵活的商业化交付能力(最高达2,000L)**
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- 全球首个获批上市使用工程化衣壳的AAV基因治疗产品

Synjunex™ (Dalnacogene Ponparvovec Injection)

- For the treatment of adult patients with moderate to severe hemophilia B (congenital coagulation factor IX deficiency)
- The clinical study results have been published on *The Lancet Haematology*, *The New England Journal of Medicine* and *Nature Medicine*
- The world's first approved AAV gene therapy product utilizing an engineered capsid

Marketed(已上市) BTD/PR | Marketed (已上市) | ODD/RPDD | ATMP | ODD

备注:
1. BTD: 突破性治疗品种; ODD: 孤儿药资格认定; RPDD: 儿科罕见病资格认定; ATMP: 先进治疗药物分类认证
2. 以上信息仅供介绍,非用药指导。医疗决策请咨询专业人士。

1.BTD: Break-through Designation; ODD: Orphan Drug Designation; RPDD: Rare Pediatric Disease Designation; ATMP: Advanced Therapy Medicinal Product
2.The above is descriptive information only, not a substitute for professional medical advice.

信念医药
Belief BioMed Inc.

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WuXi AppTec

WuXi Biology

Rare Disease Platform

- From the generation and characterization of rare disease animal model to colony management and pre-clinical pharmacology research
- Focus on a wide range of disease areas, including rare hematological, metabolic, neurological, muscular, and immunological disorders

From Model Generation to Pharmacology

Model generation & characterization (6-12 months)

- Broad range of customized animal models
- Zygotes injection & embryo transfer
- Model characterization

Colony management (0-12 months)

- Lineage purification
- Conventional breeding
- *In vitro* fertilization (IVF)
- Embryo & sperm cryopreservation

Efficacy, toxicity PK/PD (0-6 months)

- Featured assay: behavior test, metabolite detection, MRI, micro-CT, EEG, electron microscope
- Multiple modalities: ERT, RNA, AAV, ASO, HSC, small molecule

In House Collection of Mouse & Rat Rare Disease Models

Metabolic

- Gaucher
- Pompe disease
- Fabry
- MPS I
- Methylmalonic acidemia
- MPS II
- Propionic acidemia
- Citrullinemia
- Glutaric aciduria type 1
- Hyperuricemia
- Hypertyrosinemia
- Arginase-1 deficiency
- Primary hyperoxaluria
- Hyperhomocysteinemia
- Wilson's disease
- Phenylketonuria
- Ornithine transcarbamylase deficiency

Hematological

- Hemophilia A
- Hemophilia B
- B-thalassemia
- Sickle cell disease
- Pyruvate kinase deficiency
- Polycythemia vera
- Myelofibrosis
- Aplastic anemia
- Porphyria

Immunological

- Myasthenia gravis
- Immune hemolysis
- Immune thrombocytopenia
- Autoimmune hemolytic anemia
- Ankylosing spondylitis
- Cryopyrin-associated periodic syndromes

Neurological

- Spinal muscular atrophy
- Tourette's syndrome
- GBA-associated Parkinson
- Huntington's disease
- Amyotrophic lateral sclerosis
- Rett syndrome
- Infantile spasms
- Spinocerebellar ataxia
- Angelman syndrome
- Dravet syndrome

Others

- Duchenne muscular dystrophy
- Hereditary angioedema
- Alport syndrome
- Hypophosphatasia

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8
项IND/IIT合作
8 IND/IIT Collaborations

15
项AAV技术平台药物研发
15 AAV Drug Development Projects

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ASCC 中国SMA诊治中心联盟 Alliance for SMA Centers in China　SMA 中国SMA学院 China SMA Academy　Biogen　SPINRAZA (nusinersen)

NURTURE研究8年随访数据显示：诺西那生治疗的症状前SMA患儿，能够实现与正常儿童相似的运动功能发育

NURTURE study: long-term benefits of nusinersen in presymptomatic SMA over 8 years of follow-up

Kuntz NL,[1] De Vivo DC,[2] Crawford TO,[3] Kirschner J,[4] Finkel RS,[5] Bertini E,[6] Parsons JA,[7] Hwu W-L,[8] Pechmann A,[4] Butterfield, RJ,[9] Foster R,[10] Littauer R,[11] Tichler B,[12] Paradis A[11], Fradette S[11] on behalf of NURTURE study group

▶NURTURE研究纳入25例临床症状前启动诺西那生治疗的SMA患儿，入组最有可能发展为1型或2型SMA的婴儿；
其中，15例患者携带2个*SMN2*基因拷贝，10例患者携带3个*SMN2*基因拷贝*，首次给药时年龄≤6周，随访约8年[1]。

主要终点：所有患儿均存活，且无需永久性通气[1]

100%

- 患儿均存活
- 无需永久性通气#
- 未行气管造口术

▶诺西那生治疗的症状前SMA患儿，正在实现与正常儿童相似的运动功能发育[1]

在8年左右的随访中:100%患者存活且达到无支撑独坐，96%实现了独站，92%实现了独走

最有可能发展成为1型SMA的携带2个*SMN2*拷贝的患儿，100%存活，100%独坐，93%独站，87%独走

	2个*SMN2*基因拷贝* (n=15)	2拷贝且CMAP≥2mV** (n=8)	3个*SMN2*基因拷贝* (n=10)	
无支撑坐[1]	100%	100%	100%	达到里程碑
	73%	88%	100%	在正常发育的时间窗内[3]
独站[1]	93%	93%	100%	达到里程碑
	27%	50%	100%	在正常发育的时间窗内[3]
独立行走[1]	87%	87%	100%	达到里程碑
	40%	75%	100%	在正常发育的时间窗内[3]

在SMA自然病史中，1&2型SMA患者始终不能独立行走，1型SMA患者始终不能无支撑坐[2]

*携带2个*SMN2*基因拷贝的患者有可能出现1型SMA，携带3个*SMN2*基因拷贝的患者有可能出现2型SMA。**拷贝数为2的亚组患者兼顾*SMN2*基因拷贝数为2，且基线期尺神经CMAP波幅≥2 mV，并可检测到腱反射。
#永久性通气定义为：通气支持≥16小时/天且持续>21天未发生急性可逆性事件，或行气管造口术。
SMA：脊髓性肌萎缩症；SMN2：运动神经元存活基因2；CMAP：复合肌肉动作电位

1. Kuntz NL, et al. Presented at Cure SMA Meeting.
2. Govoni A, et al. Mol Neurobiol. 2018;55(8):6307-18.
3. WHO Multicentre Growth Reference Study Group. Acta Paediatr Suppl. 2006;450:86-95.

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Children's Hospital of Zhejiang University School of Medicine

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