

16th International Collaborative Forum of Human Gene Therapy for Genetic Disease

Generate Innovation with Young Power

Date

Venue

President

January 23, 2026 (Fri.)

The Jikei University School of Medicine, Building 1 Hall

Prof. Hitoshi Osaka (Department of Pediatrics, Jichi Medical University)

8:20-8:30	Opening remarks	Hitoshi Osaka (President of the 16th GT Forum) Masafumi Onodera (President of the GT-Forum)
8:30-9:20	Morning Seminar Gene Therapy for Duchenne Muscular Dystrophy Duchenne Muscular Dystrophy: Evolving Landscape of Gene Therapy and Clinical Insights Yuko Shimizu-Motohashi (National Center of Neurology and Psychiatry)	Chair : Kazuhiro Muramatsu (Jichi Medical University)
		sponsored by Chugai Pharmaceutical Co., LTD
9:30-11:00	Session1 Gene Therapy for Metabolic Disease	Chairs : Toya Ohashi (The Jikei University School of Nursing) Hiroshi Kobayashi (The Jikei University School of Medicine)
	1) Treatment with UX111 Reduced Cerebrospinal Fluid (CSF) Heparan Sulfate (HS) Exposure and Stabilized or Improved Functioning across Dose, Age, and Stage of MPS IIIA Heather A. Lau (Ultragenyx Pharmaceutical)	
	2) Results from a pivotal phase 3 double-blind placebo-controlled trial of DTX401 for the treatment of individuals with glycogen storage disease type Ia (GSDIa) Richard Collis (Ultragenyx Pharmaceutical Inc.)	
	3) AT845 gene replacement therapy for late-onset Pompe disease: an update on safety and preliminary efficacy data from FORTIS, a phase 1/2 open-label clinical study Chieri Hayashi (Astellas Pharma Inc.)	
11:10-12:00	Session2 Gene Therapy for Sensory Organs	Chairs : Koji Nishiguchi (Nagoya University) Masafumi Onodera (Osaka University)
	1) Gene-Specific and Gene-Agnostic Therapies for Inherited Retinal Diseases Yusuke Murakami (Kyushu University)	
	2) Preliminary Safety and Efficacy of AK-OTOF Gene Therapy for Otoferlin Gene (OTOF) -mediated Hearing Loss Katie Wachtel (Eli Lilly and Company)	
12:10-13:00	Luncheon Seminar Testing service for evaluation of viral safety at Takara Bio Yuko Kato-Mori (Takara Bio Inc.)	Chair : Hideto Chono (Takara Bio Inc.)
		sponsored by Takara Bio Inc.
13:15-14:00	Session3 Various Modalities for Genetic Diseases (ex vivo gene therapy • Nucleic Acid Therapy)	Chairs : Torayuki Okuyama (Saitama Medical University) Toru Uchiyama (National Center for Child Health and Development)
	1) Clinical Development of Atidarsagene Autotemcel, "arsa-cel," Autologous Hematopoietic Stem Cell Gene Therapy (HSC-GT) for Treatment of Early-Onset Metachromatic Leukodystrophy (MLD) , and Implications for MLD Newborn Screening (NBS) Kent Christopherson (Global Medical Affairs, Orchard Therapeutics)	
	2) Challenges toward personalized nucleic acid drug development for rare genetic disorders Hiroya Kuwahara (NucleoTIDE and PepTIDE Drug Discovery Center, Institute of Science Tokyo)	
14:10-15:00	Sponsored Seminar Various Modalities: Enzyme Replacement ERT, as a treatment approaches for genetic disorders Hiroshi Kobayashi (The Jikei University School of Medicine)	Chairs : Karin Kojima (Jichi Medical University) Motomichi Kosuga (National Center for Child Health and Development)
		sponsored by Sanofi
15:10-16:00	Session4 Empowering Young Leaders to Drive Genetic Medicine from Japan to the World Generate Innovation with Young Power : The Future of Gene Therapy Driven by the Next Generation Takafumi Nakamura (Tottori University) Yoshiyuki Saito (The University of Tokyo) Haruka Mizuno (Meijo University) Ayaka Kikuchi (Meiji Pharmaceutical University)	Chair : Hitoshi Osaka (Jichi Medical University)
16:10-17:00	Sweets Seminar Accelerating Gene Therapy Development with VectorBuilder Miho Matakatsu (VectorBuilder Inc.)	Chairs : Takashi Okada (The Institute of Medical Science, The University of Tokyo) Takanori Yamagata (Tochigi Rehabilitation Center)
		sponsored by VectorBuilder Inc.
17:10-18:00	Session5 Special Lecture ExpEditing AAV gene therapy Alberto Auricchio President, European Society of Gene & Cell Therapy (ESGCT)	Chair : Yoshikatsu Eto (Advanced Clinical Research Center, Southern Tohoku Institute for Neuroscience)
18:00-18:10	Closing remarks	Hironori Nakagami (Vice President, Japan Society of Gene and Cell Therapy/Department of Health and Medicine) Motomichi Kosuga (President of the 17th GT Forum) Hitoshi Osaka (President of the 16th GT Forum)

