

# 16<sup>th</sup> International Collaborative Forum of Human Gene Therapy for Genetic Disease

## Generate Innovation with Young Power

Date	January 23, 2026 (Fri.)
Venue	The Jikei University School of Medicine, Building 1 Hall
President	Prof. Hitoshi Osaka (Department of Pediatrics, Jichi Medical University)

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

