

Monday, October 2nd

International Kleefstra Syndrome Family + Scientific Conference

Opening by IDefine CSO, Eric Scheeff
10:30 AM - 11:00 AM EST

Getting to a treatment for Kleefstra
Syndrome: a Community Project

Nael Nadif Kasri, Radboud University
11:00 AM - 11:30 AM EST

Update on the BRAINmodel project
involving Kleefstra Syndrome

Scott Dindot, Texas A+M/Ultragenyx
11:30 AM - 12:00 PM EST

Preclinical Development of an ASO
Therapy for Angelman syndrome

BREAK 12:00PM - 12:15PM EST

Melissa Keenan, La Jolla Labs
12:15 PM - 12:45 PM EST

An overview of the rare disease
landscape and drug development

Yael Weiss, Mahzi
12:45 PM - 1:15 PM EST

Developing gene-based therapies for
genetic disorders: A joint effort between
biotech, academia and patient groups

Carl Ernst, McGill University
1:15 PM - 1:45 PM EST

Updates on cell modeling of EHMT1
syndrome in human neural progenitor
cells

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TODAY!**



Tuesday, October 3rd

International Kleefstra Syndrome Family + Scientific Conference

Virginie McNamar, Ciitizen
10:30 AM - 11:00 AM EST

Launching a new initiative
with Ciitizen Rare Patient
Network

Tjitske Kleefstra, Erasmus University
11:00 AM - 11:30 AM EST

Updates on the Kleefstra
Syndrome community and
projects

Charlotte Gaasterland, Knowledge Institute of
the Dutch Federation of Medical Specialists
11:30 AM - 12:00 PM EST

Update on the Kleefstra
Syndrome Guideline Project

BREAK 12:00PM - 12:15PM EST

Terry Jo Bichell, CombinedBrain
12:15 PM - 1:00 PM EST

COMBINEDBrain: Working together
to develop resources for rare genetic
neurodevelopmental disorders

Lottie Morison, Murdoch Children's
Institute
1:00 PM - 1:30 PM EST

Speech and language abilities in Kleefstra
Syndrome

Kristen Connors, Boston University
1:30 PM - 2:00 PM EST

Disease Concept Modeling for
Kleefstra Syndrome

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Wednesday, October 4th

International Kleefstra Syndrome Family + Scientific Conference

Igor Ulitsky, Weizmann Institute of
Science

10:00 AM - 10:30 AM EST

Antisense oligonucleotide (ASO)
based approaches for up-
regulating EHMT1

Hans van Bokhoven, Radboud University

10:30 AM - 11:00 AM EST

IPSC-derived neural cells and
strategies for upregulating the
wildtype allele in patient cells

Zoe Frazier, Boston Children's Hospital

11:00 AM - 11:30 AM EST

Update on the Boston
Children's Hospital Kleefstra
Clinic

BREAK 11:30 AM - 11:45 AM EST

Siddharth Srivastava + Kimberly
Wiltrout, Boston Children's Hospital

11:45 AM - 12:15 PM EST

Overview of Seizures and
Epilepsy Features in Kleefstra
Syndrome

Jonathan Lipton, Boston Children's
Hospital

12:15 PM - 12:45 PM EST

The Intersection of Kleefstra Syndrome
with the Circadian Clock

Closing, Geoff Rhyne, IDefine

12:45 PM - 1:00 PM EST

Driving Progress Forward

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