

**World Conference on PCDH19: Scientific Research Status and Therapeutic Perspectives
Fifth Edition - Rome, 22-23 November 2019**

CONFERENCE, FRIDAY 22TH 9.30 AM/6 PM

REGISTRATION	9:00-9:20
Francesca Squillante - Onlus Insieme per la Ricerca PCDH19 - <i>Welcome Speech</i>	9:30-9:40
SESSION 1 – Clinical Overview	
Moderator Bernardo Dalla Bernardina - Università degli Studi di Verona	
Federico Vigeveno - IRCCS Ospedale Pediatrico Bambino Gesù <i>PCDH19: Update on Clinical Aspects</i>	9:40-10:00
SESSION 2 – PCDH19 Clinical Studies	
Moderator Jozef Gecz - University of Adelaide	
Carla Marini - Azienda Ospedaliero Universitaria Meyer <i>PCDH19-Related Disorder: Neuroimaging Study of a Cohort of Patients Evaluating Morphological Brain Abnormalities, Cortical Thickness and Gyration Index</i>	10:00-10:20
Alex Aimetti - Marinus Pharmaceuticals, Inc. Marinus Ltd <i>Biomarker-Stratified Phase 3 Study Evaluating Ganaxolone in Children with PCDH19-Related Epilepsy</i>	10:20-10:40
COFFEE BREAK	10:40-11.15
ROUND TABLE on PCDH19 Clinical Features and Treatments	
Moderator Nicola Specchio - IRCCS Ospedale Pediatrico Bambino Gesù	
Raffella Cusmai - IRCCS Ospedale Pediatrico Bambino Gesù/ Francesca Darra - Università degli Studi di Verona/ Francesca Ragona -Istituto Neurologico Carlo Besta / Marina Trivisano - IRCCS Ospedale Pediatrico Bambino Gesù and more ...	
LUNCH	12.45-14.00
SESSION 3 – Parents' Feedback	
Questions and Suggestions, Experiences and Frustrations from Parents	14.00–15.00
SESSION 4 – New Insights on PCDH19 Disease	
Moderator Patrizia D'Adamo IRCCS Ospedale San Raffaele	
Paul Thomas – University of Adelaide <i>Investigating the Pathology of PCDH19-GCE Using Mouse Models</i>	15:00-15:20
Jack Parent – University of Michigan <i>Combining Human Pluripotent Stem Cell and Rodent Models to Study PCDH19</i>	15:20-15:40
SESSION 4 – PCDH19 Preclinical Investigations and Genetic Approaches	
Moderator Renzo Guerrini - Azienda OspedalieroUniversitaria Meyer	
Silvia Bassani – Consiglio Nazionale delle Ricerche <i>The Epilepsy-Related Protein PCDH19 regulates Tonic Inhibition, GABAAR Kinetics and the Intrinsic Excitability of Hippocampal Neurons</i>	15:40-16:00
Elisa Frullanti –Università degli Studi di Siena <i>Gene Editing and PCDH19</i>	16:00-16:20
SESSION 5 – PCDH19 State of the Art	
Jozef Gecz – University of Adelaide <i>A Decade of Trials and Tribulations of PCDH19 Research to Make a Difference</i>	16:20-16:50
QUESTIONS & ANSWERS	16.50-17:50
SESSION 6 – What Do We Need Now?	
Alessandro Ricci - Onlus Insieme per la Ricerca PCDH 19: <i>What Kind of Research Should We Fund?</i>	17:50-18:00

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POSTER SESSION, SATURDAY 23TH 9.00 AM/12.30 AM

9:00-11:00

ORAL PRESENTATIONS OF SELECTED POSTERS

Moderators

**Bernardo Dalla Bernardina - Università degli Studi di Verona
Jozef Gecz— University of Adelaide**

Claudia Compagnucci – IRCCS Ospedale Pediatrico Bambino Gesù
Towards the In Vitro Understanding Of PCDH19-Epilepsy

Giorgia Giansante - Istituto di Neuroscienze CNR- Milano
Genetic Silencing of PCDH19 as Treatment for Early Infantile Epileptic Encephalopathy Type 9 (EIEE-9)

Francesca Darra - Università degli Studi di Verona
PCDH19 EPILEPSY: Preliminary Analysis of 55 Cases Included in RESIDRAS

Didi Lamers - Scuola Normale Superiore and Istituto Nanoscienze CNR di Pisa.
Cortical Excitability in a Conditional Model of PCDH19 Epilepsy

Isabel Martinez-Garay - Division of Neuroscience, School of Biosciences, Cardiff University
PCDH19: Proteolytic Processing and Gene Regulatory Functions

Duyen H Pham - University of Adelaide
Integrated in Silico and Experimental Assessment of Disease-Relevance of Protocadherin 19 (PCDH19) Missense Variants

Emilio Russo - Antonio Leo - Università di Catanzaro
Targeting the Gut Microbiota to Treat Epilepsy

11:00-12:30

ROUND TABLE ON GENE EDITING THERAPIES-

