UNIVERSITÀ DEGLI STUDI DI BARI ALDO MORO

SIF
SOCIETÀ ITALIANA DI FARMACOLOGIA

RAREDISEASEDAY.ORG

Convegno Monotematico Congiunto
Società Italiana di Farmacologia (SIF)

SHAPING THE FUTURE OF PEDIATRIC HEALTH: INNOVATIVE RESEARCH APPROACHES FOR RARE AND METABOLIC DISEASES IN CHILDREN

organizzato dai Gruppi di Lavoro SIF
"Farmacologia Pediatrica" e "Malattie Rare e Farmaci Orfani – SIF4RARE"

23 - 24 FEBBRAIO 2026

Palazzo Ateneo | Bari
Aula Magna Aldo Cossu,
Aula Convegni Biblioteca

CON IL PATROCINIO DI

Dipartimento di
Farmacia – Scienze del Farmaco

UNIVERSITÀ
DEGLI STUDI
DI TRIESTE

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Universitario Clinico di Scienze
Mediche Chirurgiche e della Salute

Ordine dei Farmacisti
delle province di Bari
e Barletta-Andria-Trani

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Rare Diseases Italy

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European Paediatric Translational Research Infrastructure

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Pier Luigi Canonico (Novara)
Elisabetta Bigagli (Firenze)
Gabriele Stocco (Trieste)
Viviana Giannuzzi (Bari)

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Elena Conte
Michela De Bellis
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Antonella Liantonio
Paola Mantuano
Antonietta Mele
Sabata Pierno
Domenico Tricarico

Brief Scientific Overview

This meeting focuses on rare diseases and pediatric metabolic disorders, addressing key challenges in early diagnosis, drug safety, and innovative therapeutic strategies. The program integrates pharmacovigilance, real-world evidence, precision medicine, and biomarkers, highlighting the role of artificial intelligence and advanced research platforms in pediatric drug development. Special attention is given to drug repositioning and translational approaches for rare and ultra-rare diseases, promoting personalized and safe treatments through interdisciplinary collaboration.

Abstract book

https://www.sifweb.org/eventi/shaping-the-future-of-pediatric-health-innovative-research-approaches-for-rare-and-metabolic-diseases-in-children-2026-02-23

Invited Speakers and Chairpersons

Domenica ANCONA	Direttrice Dipartimento Farmaceutico ASL BAT Referente Centro Regionale Farmacovigilanza
Giuseppina ANNICCHIARICO	Coordinatrice CoReMaR – AReSS Puglia
Mariana BIANCO	Presidente Rete A.Ma.Re. Puglia
Roberto BELLOTTI	Magnifico Rettore Università degli Studi di Bari Aldo Moro
Elisabetta BIGAGLI	Ricercatrice Farmacologia Coordinatrice GdL SIF Farmacologia Pediatrica
Mariarosaria BUCCI	Professoressa Farmacologia Università degli Studi di Napoli Federico II
Pier Luigi CANONICO	Professore Emerito Farmacologia Università del Piemonte Orientale
Annalisa CAPUANO	Università della Campania Vanvitelli Coordinatrice Sezione Farmacologia Clinica SIF
Giuseppe CIRINO	Professore Farmacologia Università degli Studi di Napoli Federico II, Past-President SIF
Emilio CLEMENTI	Professore Farmacologia Università degli Studi di Milano Statale
Diana CONTE CAMERINO	Professoressa Farmacologia Università degli Studi di Bari Aldo Moro
Luigi D'AMBROSIO LETTIERI	Presidente Ordine dei Farmacisti Bari/BAT
Carola DE BEAUFORT	Consultant Pediatric Diabetes & Endocrinology Centre Hospitalier de Luxembourg
Annamaria DE LUCA	Professoressa Farmacologia Università degli Studi di Bari Aldo Moro - Coordinatrice GdL SIF4RARE
Nunzio DENORA	Professore Tecnologia Farmaceutica Università degli Studi di Bari Aldo Moro
Mattia GENTILE	Direttore Laboratorio di Genetica Medica Ospedale Di Venere, Bari

Invited Speakers and Chairpersons

Marcello GEMMATO	Sottosegretario di Stato alla Salute con delega alle Malattie Rare
Viviana GIANNUZZI	Fondazione per la Ricerca Farmacologica Gianni Benzi Onlus
Mariagrazia GRILLI	Professoressa Farmacologia Università del Piemonte Orientale
Paola IMBRICI	Professoressa Farmacologia Università degli Studi di Bari Aldo Moro
Nicola LAFORGIA	Direttore UOC Neonatologia e Terapia Intensiva Neonatale Policlinico di Bari, Università degli Studi di Bari Aldo Moro
Francesco LEONETTI	Direttore Dipartimento Farmacia-Scienze del Farmaco Università degli Studi di Bari Aldo Moro
Antonella LIANTONIO	Professoressa Farmacologia Università degli Studi di Bari Aldo Moro
Marianna LUCAFÒ	Ricercatrice Farmacologia Università degli Studi di Trieste
Sabata PIERNO	Professoressa Farmacologia Università degli Studi di Bari Aldo Moro
Paolo STELLA	Dirigente Servizio Farmaci, Dispositivi Medici e Assistenza Integrativa & Responsabile Regionale di Farmacovigilanza-Regione Puglia
Gabriele STOCCO	Professore Farmacologia, Università degli Studi di Trieste già Coordinatore GdL SIF Farmacologia Pediatrica
Maurizio TAGLIALATELA	Professore Farmacologia Università degli Studi di Napoli Federico II
Antonio TORSELLO	Professore Farmacologia Università degli Studi di Milano Bicocca
Domenico TRICARICO	Professore di Farmacologia Università degli Studi di Bari Aldo Moro
Clara VAN KARNEBEEK	Professor Personalized Medicine for Genetic Metabolic Disease, Amsterdam University Medical Center
Valentina VELLECCO	Professoressa Farmacologia Università degli Studi di Napoli Federico II

February 23rd

12:00-13:30 Registration of participants and light welcome lunch

13:30-13:50 **Welcome, greetings, and opening remarks**

Roberto Bellotti	Magnifico Rettore Università degli Studi di Bari Aldo Moro
Marcello Gemmato	Sottosegretario Ministero Salute con delega alle Malattie Rare
Giuseppe Cirino	Past-President Società Italiana Farmacologia - SIF
Annamaria De Luca	Coordinatrice GdL SIF4RARE
Elisabetta Bigagli, Gabriele Stocco	Coordinatori GdL SIF Farmacologia Pediatrica

13:50-14:00 **Greetings and Introduction to Session 1**

Partnership on Pharmacovigilance Puglia Region & Department of Pharmacy-Drug Sciences, University of Bari

Paolo Stella, Dirigente Servizio Farmaci, Dispositivi Medici e Assistenza Integrativa & Responsabile Regionale di Farmacovigilanza – Regione Puglia
Francesco Leonetti, Direttore Dipartimento Farmacia – Scienze del Farmaco
Luigi d'Ambrosio Lettieri, Presidente dell'Ordine dei Farmacisti Bari/BAT

SESSION 1 • Pharmacovigilance and RWE in pediatrics and rare disorders
Chairpersons: Domenica Ancona, Giuseppina Annicchiarico, Mariana Bianco Elisabetta Bigagli, Diana Conte Camerino, Annamaria De Luca

14:00-14:20 **INVITED TALK**
Integrative pharmacovigilance and AI-based framework for drug safety evaluation in paediatrics and rare diseases: the role of clinical pharmacologist
Annalisa Capuano

SELECTED ORAL COMMUNICATIONS

14:20-14:30 **Comparison of biologic use in pediatric patients for the treatment of immune-mediated diseases in pivotal clinical trials vs the real-world setting using the large-scale Italian VALORE project**
C. Bellitto, Y. Ingrasciotta, F. Soardo, A. Spini, L. L'Abbate, M. Carollo, O. Leoni, A. Mazzone, D. Ancona, P. Stella, [...], F.F. Bernardi, G. Trifirò (Verona)

14:30- 14:40 **Selumetinib for symptomatic plexiform neurofibromas in NF1: a monocentric real-world experience in pediatric and adult patients**
M. Lo Bianco, R. Leonardi, S. Rinella, G. Anastasio, G. Gozzo, [...], A. Polizzi, M. Ruggieri (Catania)

14:40-14:50 **Pharmacovigilance of orphan drugs: a descriptive analysis of EudraVigilance spontaneous reports**
F. Calapai, I. Ammendolia, M. Currò, L. Cardia, E. Esposito (Messina)

February 23rd

14:50-15.00 **Safety monitoring of GLP-1 RA liraglutide in paediatric patient by using EudraVigilance pharmacovigilance database**
V. Liguori, A. Capuano

15:00-15:30 **Round table-like discussion** (all Session 1 participants)
Stakeholders' view on priorities and challenges on the topic

15:30-16:00 Coffee break

16:00-16:30 **KEYNOTE LECTURE**
Shift to early diagnosis of rare pediatric metabolic conditions: Impact on care, treatment and drug development
Carola de Beaufort
Chairpersons: Mattia Gentile, Viviana Giannuzzi

SESSION 2 • Pharmacokinetic and biomarkers in precision medicine
Chairpersons: Emilio Clementi, Mariagrazia Grilli

16:30-16:50 **INVITED TALK**
How to ensure drug exposure in special populations: the role of novel drug delivery formulations
Nunzio Denora

SELECTED ORAL COMMUNICATIONS

16:50-17:00 **Sex differences in vancomycin pharmacokinetics in pediatric patients: real-world data from a therapeutic drug monitoring registry**
P. Dalla Zuanna, D. Curci, G. Ponis, M. Franzin, R. Ruoso, R. Del Savio, P. Colomban, R. Franca, M. Lucafò, P. Elefante, A. Taddio, M. Rabusin, G. Decorti, R. Addobbati, A. Fabretto, G. Stocco (Trieste)

17:00-17:10 **Mitochondrial stress biomarkers in neonatal encephalopathy: from experimental models to clinical application**
S. Carloni, S. Benedetti, A. Casabianca, C. Orlandi, F. Luchetti, M.G. Nasoni, M. Weiss, W. Balduini (Urbino)

17:10-17:20 **Lysosomal acid lipase activity as a novel biomarker of histological progression in MASLD: a single-center prospective study**
R. Lombardi, C. Garavaglia, F. Cinque, A. Cespiati, C. Bertelli, G. Pisano, G. Oberti, J. Currà, E. Calzavara, F. Alletto, G. Cincotto, A.L. Fracanzani, M. Gomaraschi (Milano)

17:20-17:30 **Individualised treatments for rare and ultra-rare diseases: ethics and regulatory insights from a first ERDERA multi-stakeholders consensus meeting**
S. Torretta, A. Landi, A. Aartsma-Rus, M.L. Dalessandro, F. Bonifazi, V. Giannuzzi (Bari)

17:30-17:45 Discussion

February 23rd

17:45-19:30 **POSTER SESSION**

TOPIC 1 • Pharmacovigilance, Regulatory Science and Drug Safety
Chairpersons: Elisabetta Bigagli, Paola Imbrici

TOPIC 2 • Pharmacokinetics, Therapeutic Drug Monitoring, Biomarkers and Formulation Strategies for Therapy Optimization
Chairpersons: Domenico Tricarico, Valentina Vellecco

TOPIC 3 • New Drug Targets and Experimental Models for Drug Development
Chairpersons: Antonella Liantonio, Antonio Torsello

TOPIC 4 • Drug Repositioning, Translational and Clinical Pharmacology
Chairpersons: Sabata Pierno, Gabriele Stocco

20:30 Dinner SIF members

February 24th

SESSION 3 · Mechanisms and models for drug development in pediatrics and rare diseases
Chairpersons: Giuseppe Cirino, Nicola Laforgia

SELECTED ORAL COMMUNICATIONS

9:00-9:10 **Molecular basis for ligand-induced switching from activation to inhibition in Kv7.2 channels**
F. Miceli, G. Carleo, N. Iraci, F. De Rosa, P. Campiglia, A. Bertamino, Z.B. Gao, J. Guo, C. Ostacolo, M. Taglialatela (Napoli)

9:10-9:20 **Genotype-phenotype-drug response correlation of newly identified SCN1A variants from the Italian Registry of Dravet Syndrome Residras**
G. Dinoi, C. Arigliano, D. Mei, I. Canfora, E.M. Rubino, E. Parrini, E. Conte, V. Sciruicchio, S. Balestrini, R. Guerrini, A. Liantonio, A. De Luca, P. Imbrici (Bari)

9:20-9:30 **ATM deficiency impairs calcium homeostasis in human neural progenitors: a cellular platform for drug repurposing strategies in pediatric Ataxia-Telangiectasia**
E. Pessolano, G. Boni, M. Grilli (Novara)

9:30-9:40 **Functional and pharmacological characterization of the p.L689F Nav1.4 sodium channel variant associated with Myotonia Permanens**
P. Laghetti, C. Altamura, I. Saltarella, D. Sternberg, S. Vicart, J.F. Desaphy (Bari)

9:40- 9:50 **AQP9 Aquaporin-9 has a role and relevance as drug target in Wolman disease**
P. Gena, D. Mentino, S. Garra, F. Liguigli, N. Zagaria, M. Mastrodonato, G. Calamita (Bari)

9:50 – 10:00 **Precision therapy for Aicardi-Goutières paediatric patients using patient-specific in vitro preclinical models based on induced pluripotent stem cells**
G. Zudeh, L. Pugnetti, D. Di Filippo, R.M. Ferraro, A. Tesser, A. Tommasini, S.C. Giliani, R. Franca, M. Lucafò, G. Stocco (Trieste)

10:00-10:10 **Disease-specific LKB1 dysregulation in Duchenne Muscular Dystrophy unveils a novel HDAC inhibitor mechanism**
B. Boccanegra, L. Tulimiero, R. Quarta, E. Conte, S.A. Licandro, A. Decio, R. Lenti, A. Ladisa, G. Dinoi, L. Claudione, S. Pierno, P. Mantuano, O. Cappellari, G. Fossati C. Steinkühler, A. De Luca (Bari)

10:10-10:30 Discussion

10:30-11:00 Coffee break

February 24th

11:00-11:30 **KEYNOTE LECTURE**

Accelerating drug repurposing for rare diseases: the SIMPATHIC approach
Clara van Karnebeek
Chairpersons: Pier Luigi Canonico, Maurizio Taglialatela

SESSION 4 • Drug repositioning in pediatrics and rare diseases
Chairpersons: Mariarosaria Bucci, Marianna Lucafò

SELECTED ORAL COMMUNICATIONS

11:30-11.40 **Targeting the ClC-5 Cl^-/H^+ antiporter in rare diseases: in silico drug repurposing as a first step toward the discovery of pharmacological modulators**
D. Trisciuzzi, A.V. Buono, M. Pusch, E. Conte, G. Dinoi, A. De Luca, P. Imbrici, O. Nicolotti, A. Liantonio (Bari)

11:40-11:50 **Drug repurposing using an integrated in silico/in vitro approach to identify therapies for KCNA1- and KCNA2-related neurological disorders**
A.G. Cerchiara, M. Marinelli, A.R. Tondo, D. Trisciuzzi, G. Dinoi, A.V. Buono, A. Mele, L. Siragusa, A. Liantonio, O. Nicolotti, A. De Luca, P. Imbrici (Bari)

11.50- 12.00 **Hydrogen sulfide restores PGC-1α and promotes slow-twitch muscle fibers in Duchenne Muscular Dystrophy**
V. Casale, M. Smimmo, D. D'Andrea, G. Persico, G. Cirino, M. Filipovic, M. Bucci, V. Vellecco (Napoli)

12:00-12:10 **Oxerutin and doxocycline improve quality of life and wound healing in patients with epidermolysis bullosa: a monocentrical pilot study**
M. Murciano, M.P. Ferrante, G. Cicco, L. Lospalluti, G. Annicchiarico (Bari)

12:10-12:20 **Off-label use of sirolimus in children with autoimmune lymphoproliferative syndrome**
G. Loreto, D. Cuzzubbo, M. Licciardello, G.L. Romano, C. Bucolo, G. Russo, A. Lazzara, F. Drago, L. Gozzo (Catania)

12:20-12:30 **Trehalose partially restores autophagy markers and membrane trafficking in a CLN1 C451T cellular model of Infantile Neuronal Ceroid Lipofuscinosis (INCL)**
G. Carbone, V. Canfora, G.M. Camerino (Bari)

12:30-12:50 Discussion

12.50-13.30 **SIF Best Oral Communication and Best Poster Award Ceremony to "Early Career Pharmacologists"**
Award presentation: Mariarosaria Bucci, Giuseppe Cirino

Conclusion of proceedings and farewells
Annamaria De Luca, Elisabetta Bigagli, Gabriele Stocco, Giuseppe Cirino

POSTER

TOPIC 1

Pharmacovigilance, Regulatory Science and Real-World Drug Safety

Chairpersons: Elisabetta Bigagli, Paola Imbrici

1. Real-World Safety of Tisagenlecleucel in Paediatric Acute Lymphoblastic Leukaemia: Evidence from the EudraVigilance Database
Cagnotta C., Capuano A

2. An easy-to-use flow chart to classify your clinical study
Dalessandro M.L., Sblano S., Torretta S., Conte R., Landi A., Bonifazi F., Giannuzzi G.

3. Desensitization enables resumption of Enzyme Replacement Therapy in two children with Rare Diseases after Hypersensitivity Reactions
Spataro F., Solimando A.G., de Martino G., Ria R., Vacca A., Montagnani M., Desantis V.

4. Off-label drug use in children: a ten-year experience of monitoring
Gozzo L., Loreto G., Romano G.L., Bucolo C., Lo Bianco M., Leonardi R., Polizzi A., Ruggieri M., Lazzara A., Drago F.

5. Post-marketing evaluation of fenfluramine safety and appropriate use in children under two years: analysis of EudraVigilance data
Dinoi G., Morleo G., Liantonio A., De Luca A., Imbrici P.

6. Defending paediatric medicines in Europe: EPTRI's strategic contribution and actions in shaping the new pharmaceutical legislation
Ruggieri L., Sblano S., Giannuzzi V., Sarracco E., Bonifazi D., Bonifazi F. (Duccio), Ceci A.

7. Pediatric safety profile of off-label anti-myotonic drugs: real-world evidence from EudraVigilance database
Saltarella I., Laghetti P., Barbaro S., Dell'Atti S., Desaphy J-F., Altamura C.

8. Real-World Drug Safety in Pediatric Oncohematology: An Observational Pharmacovigilance Study in Two Sicilian Hospitals
Vitale D.C., Longo L., Spoto S., Scorciapino M., Russo G., Affronti S., D'Angelo P., Lazzara A., Drago F.

POSTER

TOPIC 2
Pharmacokinetics, Therapeutic Drug Monitoring, Biomarkers and Formulation Strategies for Therapy Optimization

Chairpersons: Domenico Tricarico, Valentina Vellecco

9. Physicochemical and Thermal Characterization of Butyric Acid–Based DESs for Use in Self-Emulsifying Drug Delivery
Andriani E., Laquintana V., Cutrignelli A., Denora N.

10. High resolution mass spectrometry for typing and quantification of transthyretin and immunoglobulin light chains in cardiac amyloidosis
Bellich B., Porcari A., Bussani R., Merlo M., Stocco G., Franca R., Fabretto A., Sinagra G.

11. Innovative Microencapsulation Strategy for Age-Appropriate Oral Delivery of Amphotericin B in Paediatric Cystic Fibrosis
D'Amico V., Lopedota A.A., Lopalco A., Denora N.

12. 3D Bioprinting of Alginate–Gelatine Hydrogels with Lipid Nanoparticles for Localized Drug Delivery in pediatric Glioblastoma Therapy
Eljahesh A., Arduino I., Racaniello G.F., Caponio A., Di Molfetta D., Ahmed A., Iacobazzi R.M., Fiermonte G., Palmieri L., Denora N.

13. Immune alterations in genetic lysosomal acid lipase deficiency: data from naïve and ERT-treated patients
Garavaglia C., Cetti F., Bonacina F., Bellini R., Lucchi T., Madeo A., Grigore L., Tummolo A., Nascimbeni F., Romeo S., D'Erasmo L., Indolfi, Mangia G., Bruno A., Palano M.T., Norata G.D., Gomaraschi M.

14. Urinary microRNA profiling predicts hemodynamically significant patent ductus arteriosus and ibuprofen response in preterm infants: a prospective study
Gori M., Poggi C., Dani C., Luceri C., Bigagli E.

15. Microfluidic Formulation of Precision Lipid Nanoparticles for Gene Delivery to Muscle Cells
Iacobazzi R.M., Arduino I., Quarta R., Cerchiara A., Lopalco A.G., Lopedota A.A., Cappellari O., De Luca A., Denora N.

16. Ketamine adverse reactions: mechanistic investigation and biomarker identification
Messineo L., Frau R., de Gennaro A., Concas L., Cozzi G., Barbi E., d'Adamo P., Stocco G., Lucafò M.

17. Role of dyslipidemia in Alagille Syndrome on cardiovascular and renal outcomes
Ossoli A., Masenello V., Pavanello C., Gomaraschi M., Vidal E., Ranucci G., Calabresi L., Cananzi M.

18. Uncovering immune and inflammatory involvement in Wolfram syndrome
Panfili E., Chimienti R., Gargaro M., Frontino G., Tognoloni A., Siracusano G., Tomei D'Orazio M., Pallotta M.T.

19. Preliminary Comparison of Two AUC_{0-12} Estimation Approaches for Mycophenolate in Paediatrics: A Retrospective Cohort Study
Ponis G., Dalla Zuanna P., Curci D., Franzin M., Tommasini A., Pastore S., Ruoso R., Del Savio R., Colomban P., Addobbati R., Fabretto A., Decorti G., Franca R., Stocco G.

POSTER

TOPIC 3
New Drug Targets and Experimental Models for Drug Development
Chairpersons: Antonella Liantonio, Antonio Torsello

20. A novel cellular model for studying Ataxia-Telangiectasia pathophysiology
Boni G., Pessolano E., Ghignone A., Robotti E., Grilli M.

21. Mitsugumin 29 as a novel modulator of SOCE and potential therapeutic target in Tubular Aggregate Myopathy
Buono A.V., Dinoi G., Lanza M., De Luca A., Imbrici P., Liantonio A., Conte E.

22. Patient-derived 3D muscle-on-chip platform for patient-oriented preclinical studies in Duchenne muscular dystrophy
Quarta R., Cristiano E., Han M., Marinelli M., Gaio N., Mouly V., De Luca A., Cappellari O.

23. Defining the cellular and metabolic signatures of SEPN1-Related Myopathy using patient- derived skeletal muscle cells for drug target identification
Conte E., Dinoi G., Imbrici P., De Castro F., Lanza M., Gibertini S., Maggi L., Zito E., Buono A.V., de Candia M., Altomare C.D., De Luca A., Fanizzi F.P., Liantonio A.

24. Dysregulation of the HGF/c-MET signaling axis in Duchenne muscular dystrophy: a new potential therapeutic target
Cristiano E., Lenti R., Quarta R., Boccanegra B., Mantuano P., Cappellari O., De Luca A.

25. AQP3 and AQP9 are pivotal in human leukocyte motility and show pharmacological relevance as drug targets in immune disorders
Garra S., Mejlstrup Hymøller C., Zagaria N., Gena P., Liguigli F., Di Molfetta D., Cardone R.A., Rützler M., Birkelund S., Calamita G.

26. Genotype-dependent mitochondrial and myogenic defects in 2D cellular models of Duchenne Muscular Dystrophy
Ladisa A., Marinelli M., Cristiano E., Quarta R., Boccanegra B., Cerchiara A.G., Barile S., Mouly V., Lasorsa M., Imbrici P., Cappellari O., De Luca A.

27. Anti-atrophic effects of JMV2894, a growth hormone secretagogue, in Duchenne muscular dystrophy: novel insights from a preclinical study in D2-mdx mice
Mantuano P., Boccanegra B., Marinelli M., Cristiano E., Lenti R., Tulimiero L., De Bellis M., Fehrentz J.A., Denoyelle S., Bresciani E., Torsello A., Mele A., Liantonio A., Cappellari O., De Luca A.

28. Targeting intestinal epithelial homeostasis in paediatric ulcerative colitis: an organoid-based drug discovery approach
Muzzo A., Fratestefano F., Silvani A., Grazioso G., Passarella D., Fornasiero E.F., Aloisi F., Secco L., Sgarra R., Bramuzzo M., Decorti G., Stocco G., Lucafò M.

29. Five variants in the voltage sensing domain of kv7.2 potassium channels cause developmental and epileptic encephalopathy by gain-of-function effects
Priore A., Schipani G., Weckhuysen S., Syrbe S., McTague A., Barrese V., Taglialatela M., Miceli F.

POSTER

TOPIC 4
Drug Repositioning, Translational and Clinical Pharmacology
Chairpersons: Sabata Pierno, Gabriele Stocco

30. Preclinical evaluation of conjugated linoleic acid dietary supplementation on antioxidant pathway restoration in SOD1-G93A mouse model of Amyotrophic Lateral Sclerosis
Claudione L., Boccanegra B.A., Mantuano P.A., De Bellis M.A., Bacchetti F.B., Milanese M.B., Bergamo P.C., De Luca A., Pierno S.

31. Drug repurposing of SGLT2 inhibitors for Lafora Disease: bridging preclinical research and clinical evidence
Dinoi G., d'Orsi G., Lolli G., Conte E., Ciccone F., Calò E., Perrone E., Cazzanelli G., Altomare C.D., De Luca A., Carella M., Imbrici P., Liantonio A.

32. In vitro cytotoxicity effects of Tyrosine Kinase Inhibitors (TKIs) on Diffuse Intrinsic Pontine Glioma
Di Turi A., Denora M., Tricarico D.

33. Early renal fibrotic alterations in D2 mdx mouse model of Duchenne muscular dystrophy and potential protective effects of the growth hormone secretagogue JMV2894
Lenti R., De Bellis M., Denoyelle S., Liantonio A., De Luca A.

34. Metachromatic Leukodystrophy with Dystonia - Successful Management with Cannabidiol
Lo Bianco M., Leonardi R., Rinella S., Anastasio G., Gozzo L., Bucolo C., Romano G.L., Lazzara A., Drago F., Ruggieri M., Polizzi A.

35. Beneficial effects of SS-31 peptide on cardiac mitochondrial morphology and dysfunction in a murine model of Barth Syndrome
Lobasso S., Russo S., De Rasmo D., Signorile A.

36. From the First FDA-Approved Therapy to Next-Generation ClpP Activators: Deconstructing Dordaviprone (ONC201) for Pediatric H3K27-Altered Diffuse Midline Glioma
Miciaccia M., Armenise D., Baldelli O.M., Liturri A., Bruni F., Loguercio Polosa P., Ferorelli S., Perrone M.G., Scilimati A.

37. Targeting inflammation and redox imbalance in Duchenne muscular dystrophy using a corticosteroid–H_2S hybrid
Smimmo M., Casale V., Sparaco R., Persico G., Frecentese F., Vellecco V., Bucci M.

38. Protective Role of FHR-2 in the Glomerular Microenvironment: Regulation of the Terminal Complement Pathway and Its Pathogenetic Implications in Pediatric aHUS.
Stea E.D., Skerka C., Zipfel P.F., Rampino T., Gregorini M., De Amici M., Testa G., Torre C., Pontrelli P., Gesualdo L..

39. L-Citrulline exerts diaphragm-specific effects in mdx mice alone or in combination with gold standard steroids: insight into its potential as adjuvant therapy in Duchenne muscular dystrophy
Tulimiero L., Boccanegra B., Mantuano P., Mele A., De Bellis M., Lenti R., Sanarica F., Cirmi S., Conte E., Cappellari O., Sherrard A.E., Green A., Srinivasa M., Fiorotto M.L., De Luca A.

INFO

SEDE
Aula Magna "Aldo Cossu"
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70122 Bari

ISCRIZIONE
L'iscrizione è gratuita ed include: partecipazione alle sessioni scientifiche, coffee break e attestato di partecipazione.

ATTESTATI DI PARTECIPAZIONE
Al termine del congresso verrà rilasciato a tutti i partecipanti regolarmente iscritti l'attestato di partecipazione via e-mail. La consegna degli attestati avverrà solo e soltanto al termine dei lavori.

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