

INTERNATIONAL SCHOOL ON RARE DISEASES

LIVE

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ACCREDITATION IS OPEN
TO ALL HEALTHCARE
PROFESSIONALS

2 NOV - 20 JAN
2026 - 2027

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DIRECTORS

DOMENICA TARUSCIO

KOS President
ICORD Board Member

CYNTHIA TIFFT

ICORD President
NIH, USA

SESSIONS

2 sessions per week
Monday and Wednesday
14:00–16:00 CET
32 total hours · 8 weeks

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SCHOOL OVERVIEW

The International School on Rare Diseases is an international educational initiative developed through the collaboration between the Study Centre KOS – Science, Art, Society (Rome, Italy) and ICORD.

The School offers a unique opportunity to explore the key challenges and emerging opportunities in the field of rare diseases through a global and multidisciplinary perspective. Bringing together internationally recognized experts, healthcare professionals, researchers, policymakers, patient advocates, and industry leaders, the program provides advanced knowledge, promotes the exchange of best practices, and encourages meaningful collaboration across disciplines, regions and geographical boundaries.

Through interactive learning and dialogue, participants gain valuable insights into scientific advances, healthcare innovation, policy developments, and patient-centered approaches that are shaping the future of rare disease care and research. By fostering international collaboration and strong professional networks, the School aims to contribute to better research, healthcare outcomes, and quality of life for people living with rare diseases worldwide.

MAIN GOAL

- Synthesize cross-sector collaboration strategies for preparedness and response in rare diseases.
- Explain core public-health concepts and how they govern population-level decision-making.
- Apply ethical and legal frameworks to genomics, data sharing, and advanced therapeutics.
- Integrate environmental, social, and governance dimensions using the One Health lens.
- Evaluate trial design and regulatory pathways for small populations and orphan drugs.
- Navigate rare-disease definitions, epidemiology, genetics, diagnostics, and care pathways.
- Build a systems-level understanding that connects public health foundations, rare-disease practice, and One Health approaches, using longitudinal case studies.



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MODULE 1: FOUNDATION OF PUBLIC HEALTH

MODULE LEADS: RUMEN STEFANOV, MANUEL POSADA

FACULTY: EVA BERMEJO SÁNCHEZ, JOSEPH GIULIANO, PABLO DURÁN

GENERAL PRINCIPLES

This module provides the conceptual foundations of public health as a discipline, an organised societal function, and a field of policy and governance. It introduces the population-level perspective required to define health needs, measure disease burden, identify inequalities, set priorities, organise services, and evaluate public-health interventions.

Rare diseases are used as the applied context throughout the module. Particular attention is given to the methodological and organisational challenges created by small populations, incomplete case ascertainment, fragmented data, diagnostic delay, unequal access, and the need for coordinated national and international responses.

The module combines lectures, guided discussion, data interpretation, and a final individual applied assignment. The assignment will allow participants to integrate the concepts introduced during the four sessions and apply them to the adaptation of a hypothetical national health system for rare diseases.

EXPECTED OUTCOMES

Upon completion of the module, participants are expected to:

- define the principal concepts, functions, and population orientation of public health;
- analyse determinants, inequalities, unmet needs, and barriers affecting rare-disease populations;
- interpret incidence, prevalence, case ascertainment, disease burden, and uncertainty in small populations;
- explain the purpose, design, strengths, and limitations of population-based rare-disease registries;
- explain the complementary use of the International Classification of Diseases and ORPHAcodes within health-information systems;
- understand how international rare-disease commitments can be translated into regional and national policies;
- evaluate health-system organisation, financing, governance, accessibility, equity, and sustainability in relation to rare diseases;



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- apply ethical and legal principles to public-health decisions concerning access, priority-setting, data use, and resource allocation;
- identify priority health-system gaps and propose feasible adaptations involving registries, coding, referral pathways, specialised services, professional networks, financing, governance, and accountability;
- formulate measurable indicators for monitoring implementation of rare-disease policy and health-system interventions.

DAY 1. MONDAY, NOVEMBER 2, 2026, 14.00-16.00 CET **Session 1: Public Health Foundations and Population Needs**

WELCOME AND MODULE OVERVIEW: 14.00-14.10

Directors of the School: Domenica Taruscio, Cynthia Tift

Module Co-Leads: Rumen Stefanov, Manuel Posada

LECTURE 1: 14.10-14.40

Public Health and Population Health: Definitions, Core Functions, and Historical Evolution

Rumen Stefanov, Bulgaria

LECTURE 2: 14.40-15.10

Population Health Needs and Inequalities: From Determinants and Disease Burden to Policy Priorities

Manuel Posada, Spain

LECTURE 3: 15.10-15.40

Prevention and Public Health Interventions in Rare Diseases: The Example of Congenital Malformations

Eva Bermejo Sánchez, Spain

DISCUSSION: 15.40-16.00

Rumen Stefanov, Manuel Posada, Eva Bermejo



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DAY 2. WEDNESDAY, NOVEMBER 4, 2026, 14.00-16.00 CET **Session 2: Rare-Disease Epidemiology, Registries and Coding**

WELCOME AND OVERVIEW: 14.00-14.10

Rumen Stefanov, Manuel Posada

LECTURE 1: 14.10-14.40

Epidemiological Reasoning for Rare Diseases: Case Definitions, Incidence, Prevalence, Case Ascertainment, Small Numbers, and Uncertainty

Manuel Posada, Spain

LECTURE 2: 14.40-15.10

Population-Based Rare-Disease Registries and Surveillance: Design, Coverage, Data Quality, Linkage, Governance, and FAIR (Findable, Accessible, Interoperable and Reusable) Data Principles

Joe Giuliano, USA

LECTURE 3: 15.10-15.40

Coding Rare Diseases in Health-Information Systems: Complementary Use of the International Classification of Diseases and ORPHAcodes

Rumen Stefanov, Bulgaria

GUIDED DATA EXERCISE AND DISCUSSION: 15.40-16.00

Participants will examine a simplified rare-disease data scenario and identify methodological and organisational problems related to case ascertainment, denominators, geographical coverage, duplicate records, missing data, diagnostic coding, and selection bias. The exercise will illustrate how population-based registries, routine health-information systems, and complementary use of ICD and ORPHAcodes can support epidemiological surveillance, health planning, and policy development.

Moderators: Rumen Stefanov, Manuel Posada, Joe Giuliano



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DAY 3. MONDAY, NOVEMBER 9, 2026, 14.00-16.00 CET **Session 3: From Global Rare-Disease Policy to Regional and National Action**

WELCOME AND OVERVIEW: 14.00-14.10

Rumen Stefanov, Manuel Posada, Pablo Durán

LECTURE 1: 14.10-14.40

PAHO/OPS: Rare diseases & public health strategic action plans

Pablo Durán, PAHO/OPS/WHO Regional Office for the Americas

LECTURE 2: 14.40-15.10

Rare Diseases WHO Resolution and how to develop it in the Americas (brief presentation of the Resolution followed by Q&A)

Pablo Durán, PAHO/OPS/WHO Regional Office for the Americas

LECTURE 3: 15.10-15.40

Strategies to harmonize health policies across Latin America and Caribbean countries

Pablo Durán, PAHO/OPS/WHO Regional Office for the Americas

Moderators: Rumen Stefanov, Manuel Posada, Pablo Durán

DAY 4. WEDNESDAY, NOVEMBER 11, 2026, 14.00-16.00 CET **Session 4: Health-System Adaptation and Public-Health Decision-Making**

WELCOME AND OVERVIEW: 14.00-14.10

Rumen Stefanov, Manuel Posada



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LECTURE 1: 14.10-14.40

Health Systems and Rare Diseases: Organisation, Financing, Governance, Universal Health Coverage, Equity, and Sustainability

Rumen Stefanov, Bulgaria

LECTURE 2: 14.40-15.10

Ethics and Law in Public-Health Decision-Making for Rare Diseases: Justice, Solidarity, Proportionality, Autonomy, Privacy, and Data Governance

Rumen Stefanov, Bulgaria

APPLIED CASE INTRODUCTION: 15.10-15.40

Adapting a National Health System for Rare Diseases

Rumen Stefanov, Manuel Posada

Participants will be introduced to a hypothetical national health-system scenario incorporating the principal issues discussed throughout Module 1, including:

- incomplete epidemiological information;
- limited use of rare-disease-specific coding
- diagnostic delay;
- geographical inequalities in access;
- fragmented referral and care pathways;
- limited availability of centres of expertise and professional networks;
- financing and reimbursement challenges;
- insufficient coordination between institutions;
- patient participation and accountability.

The case will form the basis of the individual final applied assignment. Participants will complete the assignment individually after the module and submit it by e-mail within one week of the final session.

FINAL DISCUSSION, QUESTIONS, AND ASSIGNMENT INSTRUCTIONS: 15.40-16.00

Rumen Stefanov, Manuel Posada



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MODULE 2: RARE DISEASES: CONCEPTS & SYSTEMS

MODULE LEADS: CYNTHIA TIFFT, MARIA TERESA ACOSTA

FACULTY: KATHY FLYNN, STEVEN GROFT, SECUNDO MARIZ, CYNTHIA POWELL, DEBRA S. REGIER, HEIDI L. REHM, MARCO SALVATORE, MAURIZIO SCARPA, KERT VIELE

GENERAL PRINCIPLES

This module will focus on the core scientific, clinical, and systemic dimensions of rare diseases in the context of public health including how rare diseases are defined, diagnosed, managed and supported across health-care systems, with emphasis on genetics, clinical pathways, therapeutic development and patient experience.

EXPECTED OUTCOMES

Upon completion of the module, participants are expected to:

- Understand the scientific and clinical foundations of rare diseases
- Describe diagnostic and care pathways
- Understand the principles and challenges of therapeutic development
- Assess the impact of rare diseases on patients and families
- Evaluate the role of health-care systems and policies
- Integrate biological, clinical, social, and policy perspectives.

DAY 1. MONDAY, NOVEMBER 16, 2026, 14.00-16.00 CET

WELCOME AND OVERVIEW: 14.00-14.10

Cynthia Tift, Maria Acosta

LECTURE 1: 14.10-14.40

The rare disease landscape at the global level

Steven Groft, USA

LECTURE 2: 14.40-15.10

Overview of clinical genetics including Mendelian inheritance, penetrance & expressivity

Debra S. Regier, USA



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LECTURE 3: 15.10-15.40

Current and emerging genetic testing and diagnostics

Heidi L. Rehm, USA

DISCUSSION: 15.40-16.00

DAY 2. WEDNESDAY, NOVEMBER 18, 2026, 14.00-16.00 CET

WELCOME AND OVERVIEW: 14.00-14.10

Cynthia Tifft, Maria Acosta

LECTURE 1: 14.10-14.40

Access to care and management of rare diseases the importance of multidisciplinary teams

Maurizio Scarpa, Italy

LECTURE 2: 14.40-15.10

Therapeutic approaches to rare diseases: standard of care and emerging biologics

Cynthia J. Tifft, USA

LECTURE 3: 15.10-15.40

Clinical trial design for small populations including natural history comparators, Bayesian modeling and decentralized trials

Kert Viele, USA

DISCUSSION: 15.40-16.00

DAY 3. MONDAY, NOVEMBER 23, 2026, 14.00-16.00 CET

WELCOME AND OVERVIEW 14.00-14.10

Cynthia Tifft, Maria Acosta



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LECTURE 1: 14.10-14.40

The development of orphan drugs for rare diseases: regulatory frameworks and incentives

Secundo Mariz, EMA, The Netherlands

LECTURE 2: 14.40-15.10

Quality of Life Assessments in rare diseases- using functional burden, emotional impact and caregiver strain as measures of efficacy in clinical trials

Roberto Giugliani, Brazil

LECTURE 3: 15.10-15.40

The role of advocacy organizations in rare diseases: education, fundraising, research facilitation and policy impact

Kathy Flynn, USA

DISCUSSION: 15.40-16.00

DAY 4. WEDNESDAY, NOVEMBER 25, 2026, 14.00-16.00 CET

WELCOME AND OVERVIEW 14.00-14.10

Cynthia Tifft, Maria Acosta

LECTURE 1: 14.10-14.40

From data to better care and quality of life: the example of the cystic fibrosis registry.

Marco Salvatore, Italy

LECTURE 2: 14.40-15.10

System-wide implications of rare diseases: cost effectiveness of diagnosis, reimbursement for high-cost treatments, and universal newborn screening.

Cynthia Powell, USA

LECTURE 3: 15.10-16.00

Case Presentation & Discussion summarizing Module 2 principles

Cynthia Tifft & Maria Acosta



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MODULE 3: UNDIAGNOSED DISEASES & APPROACH TO DIAGNOSIS

MODULE LEADS: WILLIAM GAHL, DAVID PEARCE, USA

FACULTY: DAVID ADAMS, THOMAS CASSINI, HELENE CEDERROTH, SESH COLE, PRIYA S. KISHNANI, KIM LEBLANC, MAY MALICDAN, DOMENICA TARUSCIO, LYNNE WOLFE, SHINYA YAMAMOTO

GENERAL PRINCIPLES

This module focuses on understanding undiagnosed diseases within clinical and public health contexts. Participants will explore the causes, challenges, diagnostic pathways, and global structures supporting the Undiagnosed Diseases Programs (UDPs). The module emphasizes phenotyping, sequencing, functional studies, data sharing, and the discovery of new diseases.

EXPECTED OUTCOMES

Upon completion of the module, participants are expected to:

- Understand the clinical and public-health dimensions of undiagnosed diseases
- Describe diagnostic pathways for undiagnosed diseases
- Understand the role of functional studies
- Recognize the importance of data sharing, matchmaking, and international collaboration
- Understand the role and organization of Undiagnosed Diseases Programs (UDPs) and global collaborative networks supporting diagnosis and research.
- Explore approaches to novel disease and gene discovery, integrating clinical, genomic, functional, and shared data.



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DAY 1. MONDAY, NOVEMBER 30, 2026

WELCOME AND OVERVIEW 14.00-14.10

William Gahl, David Pearce

LECTURE 1: 14.10-14.40

The NIH UDP Model: What is an Undiagnosed Disease? What defines a new disease discovery? Collaborative Networks and Matchmaking Platforms

Lynne Wolfe, David Adams, May Malicdan, NIH

LECTURE 2: 14.40-15.10

Global variation in diagnostic resources. How can we increase diagnostic capability in low and middle-income countries?

Domenica Taruscio, Rome

LECTURE 3: 15.10-15.40

Burden of not having a diagnosis: patients and caregivers perspective

Helene Cederroth, Wilhelm Foundation

DISCUSSION: 15.40-16.00

DAY 2. WEDNESDAY, DECEMBER 2, 2026

WELCOME AND OVERVIEW 14.00-14.10

William Gahl, David Pearce

LECTURE 1: 14.10-14.40

Burden of not having a diagnosis: Economic and healthcare-utilization impacts

Kim LeBlanc, UDN Coordinating Center, Harvard



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LECTURE 2: 14.40-15.10

What's in a Name? The value of a diagnosis for clinical management, prognosis and surveillance

Priya Kishnani, Duke

LECTURE 3: 15.10-15.40

What's in a Name? The value of a diagnosis for treatment, clinical trials and family planning

Rhiannon Edwards and/or Jamie Duckers
Cardiff, Wales

DISCUSSION: 15.40-16.00

DAY 3. MONDAY, DECEMBER 7, 2026

WELCOME AND OVERVIEW 14.00-14.10

William Gahl, David Pearce

LECTURE 1: 14.10-14.40

The Approach to Diagnosis: Deep phenotyping, imaging, next generation sequencing and sequence reanalysis

Thomas Cassini, Vanderbilt

LECTURE 2: 14.40-15.10

The Approach to Diagnosis: functional studies and model organisms

Shinya Yamamoto, Baylor

LECTURE 3: 15.10-15.40

Therapy in Newly Discovered Disorders: mechanism-based therapy, drug repurposing, gene therapies and personalized medicine

Sesh Cole, Washington University in St. Louis

DISCUSSION: 15.40-16.00



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DAY 4. WEDNESDAY, DECEMBER 9, 2026

WELCOME AND OVERVIEW 14.00-14.10

William Gahl, David Pearce

LECTURE 1: 14.10-14.40

Rare Diseases: Ethical, legal and policy considerations, data sharing, and the value of registries

Monica McClain Drum, RDI

LECTURE 2: 14.40-15.10

Communication Matters: How to talk about genetics and undiagnosed disease programs with your patients with lower health literacy

Emma Palmer, Sydney

CASE PRESENTATION & DISCUSSION SUMMARIZING MODULE 3 PRINCIPLES: 15.10-16.00.



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MODULE 4: INTEGRATING RARE DISEASES INTO ONE HEALTH

MODULE LEADS: DOMENICA TARUSCIO, ALBERTO MANTOVANI

FACULTY: MARIA GRAZIA DENTE, ANGELA GENOVA, GREGORY WINSTON GILCREASE, BABAK KHOSHNOOD, PABLO MENENDEZ CARLOS GONÇALO DAS NEVES, SAVINO SCIASCIA, CELINA TURCHI

GENERAL PRINCIPLES

This module will focus on providing participants with an integrated understanding of how human, animal, and environmental health systems intersect with rare disease prevention, surveillance, etiology, and management. This module connects population level frameworks with interdisciplinary collaboration, policy structures, and applied case studies

EXPECTED OUTCOMES

Upon completion of the module, participants are expected to:

- Understand the One Health approach and its relevance to rare diseases across human, animal, and environmental health.
- Recognize the interconnections between human, animal, and environmental factors in rare disease etiology, prevention, surveillance, and management.
- Apply population-level and One Health perspectives to the identification and understanding of rare disease determinants and risks.
- Understand the role of integrated surveillance and data sharing across human, animal, and environmental health systems.
- Evaluate the importance of interdisciplinary and cross-sector collaboration in addressing complex rare disease challenges.
- Explore how One Health principles can inform policies and practical interventions, using applied case studies to translate integrated approaches into public-health action



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DAY 1. MONDAY JANUARY 11, 2027 14.00 – 16.00 CET

Session 1. One Health: principles, conceptual foundations, from science to policy

WELCOME AND MODULE OVERVIEW: 14.00-14.10

Module Co-Leads: Domenica Taruscio, Alberto Mantovani

LECTURE 1: 14.10 - 14.40

One Health Principles: conceptual foundations linking human, animal, and environmental health.

Carlos Gonalo das Neves, European Food Safety Authority

LECTURE 2: 14.40-15.10

Rationale for applying One Health to rare diseases, including shared risk factors, cross-species biological mechanisms, and ecological influences.

Domenica Taruscio, Italy

LECTURE 3: 15.10-15.40

From science to policy: the Technical Advisory Group for One Health of World Health Organization- European Region

Alberto Mantovani, Italy

DISCUSSION: 15.40-16.00

Alberto Mantovani, Carlos Gonalo das Neves, Domenica Taruscio

DAY 2. WEDNESDAY, JANUARY 13, 2027, 14.00-16.00 CET

Session 2. Environmental and Ecosystem Factors in the One Health perspective. One Health outlook on environmental determinants that influence the emergence, progression, detection, or distribution of rare diseases



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WELCOME AND OVERVIEW 14.00-14.10
Domenica Taruscio, Alberto Mantovani

LECTURE 1: 14.10-14.40
Environmental issues in One Health
Alberto Mantovani, Italy

LECTURE 2: 14.40-15.10
Registries of birth defects: the search for risk factors
Babak Khoshnood, France

LECTURE 3: 15.10-15.40
Environmental toxic exposures and rare diseases
Alberto Mantovani, Italy

DISCUSSION: 15.40-16.00
Moderators: Babak Khoshnood, Alberto Mantovani, Domenica Taruscio

DAY 3. MONDAY, JANUARY 18, 2027 14.00-16.00 CET
Session 3: Social and Governance aspects. One Health outlook on the social factors and governance issues that shape surveillance, prevention, outcomes, and vulnerability in rare disease populations

WELCOME AND OVERVIEW: 14.00-14.10
Domenica Taruscio, Alberto Mantovani

LECTURE 1: 14.10-14.40
Governance of cross-sectional collaboration
Maria Grazia Dente, Italy

LECTURE 2: 14.40-15.10
Social determinants and cultural influences, ethical and equity in the context of rare diseases.
Angela Genova, Italy



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LECTURE 3: 15.10-15.40

Sustainable development and health governance: the standpoint of rare diseases

Gregory. Winston Gilcrease, Savino Sciascia, Italy

DISCUSSION: 15.40-16.00

Moderators: Maria Grazia Dente, Angela Genova, Gregory, Winston Gilcrease, Alberto Mantovani, Savino Sciascia, Domenica Taruscio

DAY 4. WEDNESDAY JANUARY 20, 2027, 14.00-16.00 CET
Session 4. One Health Case Studies on multifactorial rare diseases.
Examples to illustrate how rare diseases arise from complex interactions across domains: application of One Health frameworks to multifactorial rare disease systems.

WELCOME AND OVERVIEW: 14.00-14.10

Domenica Taruscio, Alberto Mantovani

LECTURE 1: 14.10-14.40

Zika virus

Celina Turchi, Brazil

LECTURE 2: 14.40-15.10

Childhood leukemia

Pablo Menendez, Spain

LECTURE 3: 15.10-15.40

Hereditary angioedema

Giuseppe Novelli, Italy

DISCUSSION: 15.40-16.00

Moderators: Alberto Mantovani, Pablo Menendez, Giuseppe Novelli, Domenica Taruscio, Celina Turchi

CONCLUSIONS OF THE INTERNATIONAL SCHOOL: 16.00 – 16-30

Domenica Taruscio, Cyndi Tifft



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AFFILIATIONS

- Maria Teresa Acosta.** ICORD Board Member. Pediatric Neurologist. Gene Therapy Program GM1. National Human Genome Research Institute. Undiagnosed Disease Program, National Institutes of Health, USA
- David R. Adams.** Co-Director, NIH Undiagnosed Diseases Program, NHGRI/NIH, USA
- Eva Bermejo Sánchez.** Director, Scientific Researcher, Institute for Rare Diseases Research (IIER), Instituto de Salud Carlos III (ISCIII), Madrid, Spain
- Thomas Cassini.** Associate Director, Potocsnak Center for Undiagnosed and Rare Disorders, Vanderbilt University Medical Center, USA
- Helene Cederroth.** Founder and Chair, Wilhelm Foundation; Co-founder and Board Member, UDNI, Sweden
- Sesh Cole.** Professor of Pediatrics, Department of Pediatrics, Washington University in St. Louis School of Medicine; Undiagnosed Diseases Network (UDN), USA
- Carlos Gonçalo das Neves.** Chief scientific officer of European Food Safety Authority, co-chair of the One Health High-Level Expert Panel (OHHLEP)
- Maria Grazia Dente.** Senior Scientist, National Center for Global Health, Istituto Superiore di Sanità, Roma, Italy
- Jamie Duckers.** National Clinical Lead for Rare Disease, Wales; Cardiff and Vale University Health Board, UK
- Pablo Durán.** Regional Advisor in Perinatal Health Life Course, Women's and Children's Health Unit Health Systems and Services Department, PAHO/OPS/WHO Regional Office for the Americas
- Rhiannon Edwards.** Rare Disease Consultant Nurse, Cardiff and Vale University Health Board, Wales, UK
- Kathy Flynn.** Chief Executive Officer, National Tay-Sachs & Allied Diseases Association (NTSAD), USA
- William Gahl.** NIH, USA
- Angela Genova.** Professor in Sociology, Urbino University Carlo Bo, Italy
- Gregory Winston Gilcrease.** Sustainability in Health Master's program, University of Turin, Italy
- Roberto Giugliani.** UFRGS and Casa dos Raros, UDNI Board Member, Porto Alegre, Brazil
- Joseph Giuliano.** Founder & Managing Director @ Rare Medical Affairs LLC, Princeton, New Jersey, USA
- Stephen C. Groft.** Former Director, Office of Rare Diseases Research, NIH; ICORD Board Member, USA
- Babak Khoshnood.** The French National Institute of Health and Medical Research (INSERM). INSERM U1153, Equipe OPPaLE Paris, France
- Priya S. Kishnani.** Chief, Division of Medical Genetics, Duke University School of Medicine, USA
- Kimberly LeBlanc.** Senior Director, UDN Coordinating Center, Harvard Medical School, USA
- May Christine V. Malicdan.** Director of Translational Research, NIH Undiagnosed Diseases Program, NHGRI/NIH, USA
- Alberto Mantovani.** Vice-President of the Study Centre KOS, Roma, Italy. Member of Risk Assessment Committee – European Chemical Agency, past Member of Technical Advisory Group for One Health- WHO Europe
- Segundo Mariz.** Orphan Medicines Office, European Medicines Agency (EMA), The Netherlands
- Monica McClain Drum.** Senior Global Programme Manager, Rare Diseases International (RDI), France



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Pablo Menendez. Josep Carreras Leukemia Research Institute, Barcelona, Spain; Red Española de Terapias Avanzadas (TERAV) - Instituto de Salud Carlos III (ISCIII), Madrid, Spain; Department of Biomedicine, School of Medicine, University of Barcelona, Barcelona, Spain

Giuseppe Novelli. Full Professor of Medical Genetics, Department of Biomedicine and Prevention, Genetics Section, University of Rome Tor Vergata, Italy, UDNI Member

Elizabeth Emma Palmer. Associate Clinical Professor at UNSW Sydney and a Clinical Geneticist at Sydney Children's Hospitals Network

David A. Pearce. Chair, IRDiRC, WF UDNI; Sanford Research, USA

Manuel Posada. Full Research Professor, MD, PhD. Coordinator of the Iberoamerican Network of Health Expert on Rare Diseases (RIBERSER), Foundation CSAI, Ministry of Health, Spain. ICORD Auditor.

Cynthia M. Powell. Professor, Division of Genetics and Metabolism, Department of Pediatrics, University of North Carolina at Chapel Hill School of Medicine, USA

Debra S. Regier. Chief of Genetics and Metabolism, Children's National Hospital; Professor of Pediatrics, George Washington University School of Medicine and Health Sciences, USA

Heidi L. Rehm. Chief Genomics Officer, Massachusetts General Hospital; Broad Institute of MIT and Harvard; Harvard Medical School, USA

Marco Salvatore. Senior Researcher, Head, Interdepartmental Undiagnosed Rare Diseases Unit, National Centre for Rare Diseases, Istituto Superiore di Sanità, Italy

Maurizio Scarpa. Full Professor, Director Rare Disease Program IRCCS San Gerardo dei Tintori Foundation Monza, Italy; Coordinator, MetabERN, Italy

Savino Sciascia. Professor, Department of Clinical and Biological Sciences, University of Turin; Sustainability in Health Master's Programme; UDNI, Italy

Rumen Stefanov. ICORD Past President; Chair, Department of Social Medicine and Public Health, Medical University of Plovdiv, Bulgaria. Director, Institute for Rare Diseases, Bulgaria

Domenica Taruscio. ICORD Board Member, President of the Study Centre KOS, Roma, Italy. UDNI Co-founder and Board Member. Former Director National Centre Rare Diseases, Istituto Superiore di Sanità, Roma, Italy

Cynthia Tift. ICORD President, NIH, USA

Celina Turchi. Federal University of Pernambuco, Recife, Brazil

Kert Viele. Senior Statistical Scientist, Berry Consultants, LLC, USA

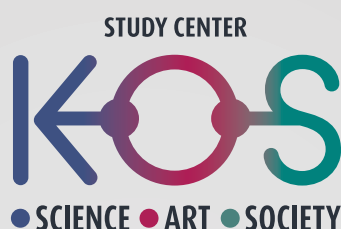
Lynne Wolfe. NIH Undiagnosed Diseases Program, National Human Genome Research Institute (NHGRI), National Institutes of Health (NIH), Bethesda, MD, USA

Shinya Yamamoto. Co-Director UDN Model Organisms Screening Center Baylor College of Medicine, USA



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We inspire global collaboration
for all rare disease stakeholders



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