

# 4<sup>th</sup> ES AIHF PO

SYNERGY  
CERTIFICATION  
COURSE

Rare diseases  
and orphan  
medicines  
as an integral  
part  
of European  
healthcare  
setting



11-12 May 2023

Acquario romano Rome  
Rome, Italy

Rare diseases are defined by the European Union as life-threatening or chronically debilitating conditions with a prevalence lower than 4 per 10 000 inhabitants. Around 6000 diseases are described, affecting between 6% and 8% of the European population. Due to the severity and the distribution of rare diseases across Europe, its multifaceted nature and accompanying economic challenges, it is important to improve the knowledge and the awareness of rare diseases among healthcare professionals and patients. Moreover, since a multidisciplinary healthcare professionals' approach is needed to manage rare diseases, it is crucial to improve their collaboration and perception of various aspects and specificity of their management.

During this EAHP Synergy Certification Course the attendees will be able to acquire more knowledge about:

- European policies on rare diseases and health insurance funds management.
- Access to and approval of new medicines according to the EMA perspective.
- The management and the therapeutic pathways for rare diseases; the relevance of the rare disease's networks and the role of genetics in rare diseases.
- The patient perspectives and the available patient support programmes in Europe related to rare diseases.
- The role of the Real World Evidence (RWE) in the evaluation of rare diseases.
- The sustainability of innovative treatments for rare diseases in the context of overall public health care system.
- The complexity of dispensing orphan medicines or medical devices for patients affected by rare diseases according to the experience of the hospital pharmacist.
- The effective communication on rare diseases across healthcare sectors and with patients.
- Available databases, observatories and websites with useful information on rare diseases and orphan medicines.

An interactive panel discussion will facilitate exchange and interaction among synergy certification course participants and the speakers coming across Europe.

# Program “The European Regulatory Framework”

## Day 1, May 11<sup>th</sup> 2023

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- 15.00-15.10** Invitational speech by the organizers, Italian Society of Hospital Pharmacists (SIFO) and European Association of Hospital Pharmacists (EAHP)  
**Arturo Cavaliere, Andras Sule**
- 15.10-15.25** Welcome - needs and objectives of the 4<sup>th</sup> EAHP Synergy Certification Course-Day 1  
**Piera Polidori**
- 15.25-15.30** *Moderators: **Marcello Pani and Maria Ernestina Faggiano***
- 15.30-16.00** European policies on rare diseases and health insurance funds managements  
**Istvan Ujhelyi, Tomislav Sokol - remote connection**
- 16.00-16.40** Management and therapeutic pathways for rare disease: comparison between regions  
**Giuseppe Limongelli, Maurizio Scarpa**
- 16.40-16.55** *Coffee break*
- 16.55-17.10** European patients’ perspectives on rare diseases  
**Simone Boselli**
- 17.10-17.25** Access to orphan drugs  
**Armando Magrelli**
- 17.25-17.45** Example of rare diseases the experience of the Gemelli hospital - Rome  
**Stanislao Rizzo**
- 17.45-18.00** Interactive panel discussion  
**Session Speakers**
- 18.00-18.15** Summary - End of Day 1  
**Piera Polidori**

**18.15 COCKTAIL**

# Program “Organization of healthcare teams for the treatment of rare diseases”

## Day 2, May 12<sup>th</sup> 2023

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*Moderators: **Alessandro D’Arpino and Carlo Polidori***

- 09.00-09.15** Welcome - needs and objectives of the 4<sup>th</sup> EAHP Synergy Certification Course- Day 2  
**Alessandro D’Arpino, Carlo Polidori**

- 09.15-09.35** Effective communication in Rare Diseases  
**Daniela Scala**
- 09.35-10.05** Patient support programs in Europe  
**Jacopo Koch**
- 10.05-11.05** Genetics in rare diseases  
**Maria Piccione, Emanuela Salzano**
- 11.05-11.20** *Coffee break*
- 11.20-11.45** The role of the RWE in the evaluation of rare diseases  
**Gianluca Trifirò**
- 11.45-12.10** The sustainability of innovation for rare diseases in the context of public health  
**Federico Spandonaro**
- 12.10-12.40** Interactive panel discussion  
**Session Speakers**
- 12.40-14.00** *Lunch*

## Program “From theory to practice” Day 2, May 12<sup>th</sup> 2023

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*Moderators:* **Barbara Meini and Francesco Cattel**

- 14.00-14.15** The Key role of Patient’s Associations in early access and evaluations of therapies  
**Annalisa Scopinaro**
- 14.15-14.45** Complexity of dispensing of orphan drugs or medical devices for patients affected by rare diseases  
**Maurizio Pastorello**
- 14.45-15.00** Omar: communication at the service of rare disease  
**Ilaria Ciancaleoni Bartoli**
- 15.00-15.15** Orphanet: a database at the service of rare diseases  
**Michele Nutini**
- 15.15-15.30** Take home message- illustration of survey data and take-home messages  
**Piera Polidori**
- 15.30** Learning Test CME



# Chief Scientific Project and Scientific Committee

## **Dott.ssa P. Polidori**

SIFO Head of Delegation EAHP - SIFO Board of Auditors (President) - EAHP Board Member

## **Dott. N. Miljkovic**

Director of Finances at EAHP

## **Dott.ssa M.E. Faggiano**

SIFO Board of Directors (Treasurer) - National University Referent

## Faculty

### **BOSELLI Simone**

Director of Public Affairs Director of Public Affairs EURORDIS

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SIFO HTA Laboratory Coordinator Hospital Pharmacy Director - AOU City of Health and Science of Turin

### **CAVALIERE Arturo**

President of SIFO Director of Pharmacy multispecialty department, ASL Viterbo Lazio Region

### **CIANCALEONI BARTOLI Ilaria**

Director in charge - Journalist registered on the Lazio OdG - O.Ma.R - Rare Disease Observatory

### **D'ARPINO Alessandro**

Vice President SIFO Pharmacist Director - Hospital Pharmacy, University Hospital of Perugia

### **FAGGIANO Maria Ernestina**

SIFO Board of Directors (Treasurer) - National University Referent Executive Pharmacist, Pharmacy Service, AOU Consortium Polyclinic of Bari

### **KOCH Jacobs**

CEO Healthcare Network Partners Italy Spa

### **LIMONGELLI Giuseppe**

Associate Professor Department of Translational Medical Sciences - U.O. Rare Cardiovascular Diseases - Monaldi Hospital - AORN Colli

### **MAGRELLI Armando**

Vice-Chair of Committee Orphan Medicinal Products, European Medicine Agency

### **MEINI Barbara**

Director of the Grosseto Territorial Pharmaceutical Complex Operating Unit, Local Health Authority Toscana Sud Est; Scientific Coordinator of SIFO Publishing, President of the SIFO Arbitration Board

### **NUTINI Michele**

Senior Information Scientist - Orphanet Italy

### **PANI Marcello**

SIFO Board of Directors (SIFO National Secretary) Director of Pharmacy multispecialty department A. Gemelli University Hospital

### **PASTORELLO Maurizio**

Director of the Pharmacy multispecialty department; Director of the Pharmaceutical Department

### **PICCIONE Maria**

Specialist center coordinator - Orphanet

### **POLIDORI Carlo**

Member of the Single Scientific Committee for Research and Training SIFO Professor in Pharmacology School of Pharmaceutical Sciences and Health Products, University of Pharmacy of Camerino (MC)

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### **RIZZO Stanislao**

Full Professor MED/30 OPHTHALMOLOGY Sacro Cuore Catholic University - Rome

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Pediatric Geneticist at Regional Referral Centre for Rare Genetic and Chromosomal Diseases

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Coordinator ASC SIFO Scientific Information, Counseling and Narrative Pharmacy Pharmacist Manager I level UO SD Nuclear Medicine AORN "A. Cardarelli" Napoli

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Director SOC Regional Reference Center for Rare Diseases, Udine University Hospital, Udine, Italy

### **SCOPINARO Annalisa**

President of UNIAMO - Italian Federation of Rare Diseases

### **SOKOL Tomislav**

MEP - European parliament

### **SPANDONARO Federico**

Department of Economy - Telematic University San Raffaele Rome President of the Scientific Committee of C.R.E.A. Sanità (Centre for Applied Economic Research in Healthcare)

### **SULE Andras**

President EAHP - Chief Pharmacist of Péterfy Hospital and Trauma Center e Chief Pharmacy Advisor for Duna Medical Center

### **TRIFIRÒ Gianluca**

Professor Department of Diagnostics and Public Health, Section of Pharmacology, University of Verona, Italy

### **UJHELYI Istvan**

Members of the European Parliament, Group of the Progressive Alliance of Socialists and Democrats in the European Parliament

## CONGRESS VENUE

Acquario Romano

Piazza Manfredo Fanti, 47 - Rome

## TARGET

n. 150 Pharmacist

## CME

The event is included in the SIFO Annual Plan 2023 for the profession of Pharmacist.

SIFO (Italian Provider ID 313) has assigned event n. 5,6 credits CME (Continuing Medical Education).

Please note that the release of the credit certification is subject to the participation of at least 90% of the duration of the work, the passing of the learning test and the completion of the evaluation form of the event.

## NATIONAL OBJECTIVE

no. 18 - technical-professional contents (knowledge and skills) specific to each profession, of each specialization and each activity ultra-specialist, including rare diseases and gender medicine

## TRAINING DURATION

8 hours

## REGISTRATION FEES

- SIFO member and EAHP member states pharmacists in good standing (membership fee for 2023 paid):  
**183,00 €** (150€ + applicable VAT 22%)
- Pharmacists non member:  
**305,00 €** (250€ + applicable VAT 22%)

## SIFO SCIENTIFIC SECRETARIAT

(Provider Standard Nazionale ID n. 313)

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## ORGANIZING SECRETARIAT

### PLANNING

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Con il contributo non condizionato di



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