



# 5th Alpha-1 Patient Congress

**Meeting Location: Salone Pascoli**

## Medical and Research Presentations:

### Mark Brantly

University of Florida  
Miami, FL, USA

### Kenneth Chapman

University of Toronto  
Toronto, Canada

### Bruno Christ

Universitätsklinikum Leipzig AöR  
Leipzig, Germany

### Luciano Corda

Centro Riferimento Deficit Alfa1-Antitripsina  
Brescia, Italy

### Jonathan Edelman

CSL Behring  
King of Prussia, PA, USA

### Ilaria Ferrarotti

Center for Diagnosis of Inherited Alpha-1  
Antitrypsin Deficiency  
Pavia, Italy

### Laura Fregonese

European Medicines Agency  
London, UK

### Matthias Griese

University of Munich  
Munich, Germany

### David Lomas

University College London  
London, UK

### Robert Sandhaus

National Jewish Health  
Denver, CO, USA

### Robert Stockley

Queen Elizabeth Hospital Birmingham  
Birmingham, UK

### Jan Stolk

Leiden University Medical Center  
Leiden, The Netherlands

### Charlie Strange

Medical University of South Carolina  
Charleston, SC, USA

### Jeffrey Teckman

Saint Louis University School of Medicine  
St. Louis, MO, USA

### Adam Wanner

Alpha-1 Foundation  
Miami, FL, USA

## THURSDAY, 9 APRIL

6:30 pm – 7:30 pm

**Welcome Reception — Homage to Dr. Maurizio Luisetti** • Hotel Lobby

7:30 pm – 9:00 pm

**Dinner** • La Veranda Restaurant (Patient Congress) • Sala Degli Artisti (Research Conference Participants)

## FRIDAY, 10 APRIL

7:00 am – 8:45 am

**Breakfast Buffet** • La Veranda Restaurant

9:00 am – 10:30 am

**Patient Congress Welcome**

Gonny Gutierrez

**Alpha-1 Global Steering Committee Introductions**

Steering Committee Members

**Alpha-1 Global Patient Community Updates**

Moderator: Gonny Gutierrez

**Australia & New Zealand** (12 min) • Steve Knowles/Marilyn Black

**Canada** (12 min) • Mimi McPhedran

**United States** (12 min) • Henry Moehring

**South America** (12 min) • Guillermo Menga

**Central Europe** (12 min) • Joanna Chorostowska

**Western Europe** (12 min) • Catarina Pyrrait

10:30 am – 10:50 am

**Break**

10:50 am – 11:30 am

**European Medicines Agency** • Moderator: Shane Fitch

■ **Scientific and Regulatory Pathways of Medicines for Rare Diseases** (25 min) • Laura Fregonese

■ **Case Study: UK Campaign — Advocacy and Government Relations** (15 min) • Karen North

11:30 am – 12:30 pm

**Liver Disease: Therapies on the Horizon**

Moderator: Karen Erickson

Panelists: Nicola Brunetti-Pierri (Genetics Investigator), Bruno Christ (Liver Research), Bruce Given (Arrowhead), Alice Margaça (Patient Representative), Jeffrey Teckman (Clinician)

■ **Liver Overview** (20 min) • Jeffrey Teckman

■ **Panel Discussion** (40 min)

12:30 pm – 2:00 pm

**Lunch Break** • La Veranda Restaurant

2:00 pm – 3:00 pm

**Lung Disease: Current and Future Therapies**

Moderator: Shane Fitch

■ **Alpha-1 Lung Disease: Current and Future Therapeutic Solutions** (15 min) • Robert Sandhaus

■ **Efficacy and Safety of Inhaled Alpha-1 Antitrypsin** (15 min) • Matthias Griese

■ **Effect of Intravenous Alpha-1 Antitrypsin on Lung Structure** (15 min) • Jonathan Edelman

■ **Future of Gene and Stem Cell Therapy** (15 min) • Jan Stolk

3:05 pm – 4:00 pm

**Research Registry Updates** • Moderator: Frank Willersinn

■ **Research Registries: Overview** (10 min) • Kenneth Chapman

■ **Italian Registry Update** (10 min) • Ilaria Ferrarotti

■ **A.I.R. Registry Update** (10 min) • Robert Stockley

■ **US Registry Update** (10 min) • Charlie Strange

■ **Panel Discussion** (15 min) • Kenneth Chapman, Ilaria Ferrarotti, Robert Stockley, Charlie Strange

- 4:00 pm – 4:30 pm *Break* (Researchers Join the Patient Congress)
- 4:30 pm – 5:30 pm **Combined Session with Researchers and Patients**
- **Summary and Outcomes of Research Conference** (20 min) • Adam Wanner
  - **Can Small Molecules Fix Liver Disease?** (20 min) • David Lomas
  - **Can Gene Therapy Fix Lung Disease?** (20 min) • Mark Brantly
- 7:30 pm *Dinner* • La Veranda Restaurant

## SATURDAY, 11 APRIL

- 7:00 am – 8:45 am *Breakfast Buffet* • La Veranda Restaurant
- 9:00 am – 9:20 am **Qigong Session — Optional and weather-permitting, on patio outside Conference Hall** • Shane Fitch
- Move inside*
- 9:30 am **Daily Introductions** • Gonny Gutierrez
- 9:35 am – 10:10 am **Partnership and Networks to Strengthen the Alpha-1 Message**
- **PLUS (Plasma Users Platform) Collaboration** (20 min) • Johan Prevot, IPOPI (International Patient Organisation for Primary Immunodeficiencies)
  - **Other Partnerships and Networks** (15 min) • Frank Willersinn
- 10:10 am – 10:45 am **Patient Advocacy** • Johan Prevot, IPOPI
- **Introduction** (15 min)
  - **Discussion Time** (20 min)
- 10:45 am – 11:10 am *Break*
- 11:10 am – 11:55 am **Global Patient Community: Building a Sustainable Organisation**  
Johan Prevot, IPOPI
- **Introduction** (25 min)
  - **Question and Answer Time** (20 min)
- 11:55 am – 12:15 pm **Global Communication, Campaigns and Resource Sharing**  
Shane Fitch
- **Awareness, Detection and Access to Care**
- 12:15 pm – 12:30 pm **Morning Session Closing Discussion**
- 12:30 pm – 2:00 pm *Lunch Break* • La Veranda Restaurant
- 2:00 pm – 3:00 pm **Meet the Alpha-1 Experts** • Kenneth Chapman, Bruno Christ, Luciano Corda, Robert Sandhaus
- **An opportunity to have your medical and research questions answered**
- 3:00 pm – 3:45 pm **Group Networking** • Set-Up TBD
- 3:45 pm – 4:00 pm *Break*
- 4:00 pm – 4:45 pm **Next Steps for the Alpha-1 Global Community**  
Gonny Gutierrez, Frank Willersinn
- **Presentation of Plans and Discussion Time**
- 4:45 pm – 5:00 pm **Closing Ceremony** • John Walsh
- 6:30 pm – 7:30 pm **Tuscan Wine Tasting** • Hotel Lobby
- 7:30 pm – 9:00 pm *Dinner* • La Veranda Restaurant

## CLINICIANS BREAKOUT SESSION • Sala Maidai

- 9:30 am – 11:00 am
- Alpha-1 Antitrypsin Deficiency: a World View**  
Chairman: Bruno Balbi
- **Introductions** (5 min)  
Bruno Balbi
  - **Testing for Alpha-1 in Italy** (15 min) • Luciano Corda
  - **Stem Cells: A Promising Experiment for Treatment of Alpha-1 Liver Disease?** (15 min) • Bruno Christ
  - **The Natural History of Alpha-1** (15 min) • Robert Sandhaus
  - **Can we affect the Natural History of Alpha-1?** (15 min) • Kenneth Chapman
  - **Questions and Answers** (25 min)