



# 5th Alpha-1 Patient Congress

## Patient Congress Speakers

### Bruno Balbi

Divisione di Pneumologia Riabilitativa  
Veruno, Italy

### Mark Brantly

University of Florida  
Miami, FL, USA

### Nicola Brunetti-Pierri

Telethon Institute of Genetics and Medicine  
Napoli, Italy

### Ken Chapman

University of Toronto  
Toronto, Canada

### Joanna Chorostowska-Wynimko

National Institute of Tuberculosis and Lung Diseases  
Warsaw, Poland

### 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

### Guillermo Menga

Hospital de Rehabilitación Respiratoria,  
Buenos Aires, Argentina

### Johan Prevot

IPOPI (International Patient Organisation  
for Primary Immunodeficiencies)  
Estoril, Portugal

### Sandy 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 (All Attendees)

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: Shane Fitch

**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 Medicine Agency** • Moderator: Shane Fitch

■ **EMA Hearing Process & Timeline on Drug Availability** (20 min)

Laura Fregonese

■ **Case Study: UK Campaign — Advocacy and Government  
Relations** (20 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** • Jeffrey Teckman (20 min)

■ **Panel Discussion** (40 min)

12:30 pm – 2:00 pm

**Lunch** • La Veranda Restaurant • Global Village (*open after 1:15pm*)

2:00 pm – 3:00 pm

**Lung Disease: Current and Future Therapies**

Moderator: Shane Fitch

■ **Alpha-1 Lung Disease: Current and Future Therapeutic  
Solutions** • Sandy Sandhaus

■ **Efficacy and Safety of Inhaled Alpha-1 Antitrypsin**  
Matthias Griese

■ **Effect of Intravenous Alpha-1 Antitrypsin on Lung Structure**  
Jonathan Edelman

■ **Future of Gene and Stem Cell Therapy** • Jan Stolk

3:05 pm – 4:00 pm

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

■ **Research Registries: General Overview** (10 min) • Ken 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*

## SATURDAY, 11 APRIL

- 7:00 am – 8:45 am *Breakfast Buffet*
- 9:00 am – 9:20 am **Qigong Session — Optional and weather-permitting, on patio outside Conference Hall Shane Fitch** • Global Village  
(open after 8:45am)
- 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
  - **Other Partnerships and Networks** (15 min) • Frank Willersinn
- 10:10 am – 10:45am **Patient Advocacy** • Johan Prevot, IPOPI
- **Introduction** (15 min)
  - **Open 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 – 2:00pm *Lunch* • La Veranda Restaurant • Global Village (open after 1:15pm)
- 2:00 pm – 3:00 pm **Meet the Alpha-1 Experts** • Ken Chapman, Bruno Christ
- **An opportunity to have your medical and research questions answered** • Luciano Corda, Sandy Sandhaus,
- 3:00 pm – 3:45 pm **Group Networking By Region or Language** • 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/Dr. Frank Willersinn
- **Discussion Time**
- 4:45 pm – 5:00 pm **Closing Ceremony** • John Walsh
- 6:30 pm – 7:30 pm **Optional Tuscan Wine Tasting Class**
- 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)  
Chairman: 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) • Sandy Sandhaus
  - **Can we affect the Natural History of Alpha-1?** (15 min) • Ken Chapman
  - **Questions and Answers** (10 min)

## Scientific Program

### 2nd Biennial International Research Conference on Alpha-1 Antitrypsin

Novel Concepts in the Mechanisms,  
Monitoring and Treatment of COPD in  
Alpha-1 Antitrypsin Deficiency



#### THURSDAY, 9 APRIL, 2015

##### Welcome

2:00 pm – 2:05 pm J. Walsh (Alpha-1 Foundation)

##### Introduction

2:05 pm – 2:10 pm A. Wanner (University of Miami, USA)  
(Co-Chair)  
G. Lungarella (University of Siena,  
Italy) (Co-Chair)

##### Session 1: New Basic Mechanisms

2:10 pm – 2:30 pm **The Gene and its Variants**  
M. Brantly (University of Florida, USA)

2:30 pm – 2:50 pm **Discussion**

2:50 pm – 3:10 pm **Well and Less Well Known  
Functions of AAT**

S. Janciauskiene (Hannover Medical  
School, Germany)

3:10 pm – 3:30 pm **Discussion**

3:30 pm – 3:50 pm **New Concepts in AATD-related  
Disease Mechanisms**

S. Marciniak (University of Cambridge,  
United Kingdom)

3:50 pm – 4:10 pm **Discussion**

4:10 pm – 4:20 pm **Coffee Break**

4:20 pm – 4:40 pm **Neutrophil Function and Lung  
Injury in AATD**

N. McElvaney (Royal College of  
Surgeons in Ireland, Ireland)

4:40 pm – 5:00 pm **Discussion**

5:00 pm – 5:20 pm **Immune Inflammation in Alpha-1  
Antitrypsin Deficiency Emphysema  
and “Usual” COPD: Same or  
Different Diseases?**

M. Cosio (McGill University, Canada  
and Università di Padova, Italy)

5:20 pm – 5:40 pm **Discussion**

5:40 pm – 6:00 pm **Animal Models of Pulmonary  
Emphysema**

I. Petrache (Indiana University, USA)

6:00 pm – 6:20 pm **Discussion**

## FRIDAY, 10 APRIL, 2015

### Session 2: Diagnosis and Assessment

8:00 am – 8:20 am	<b>Detecting Alpha-1 Antitrypsin Deficiency</b> J. Stoller (Cleveland Clinic, USA)
8:20 am – 8:40 am	<b>Discussion</b>
8:40 am – 9:00 am	<b>An ECLIPSE View of AATD</b> D. Lomas (University College London, United Kingdom)
9:00 am – 9:20 am	<b>Discussion</b>
9:20 am – 9:40 am	<b>Monitoring Lung Function and QoL</b> R. Stockley (University Hospital Birmingham, United Kingdom)
9:40 am – 10:00 am	<b>Discussion</b>
10:00 am – 10:10 am	<i>Coffee Break</i>
10:10 am – 10:30 am	<b>Monitoring Biomarkers</b> G. Turino (Mount Sinai St. Luke's Hospital, USA)
10:30 am – 10:50 am	<b>Discussion</b>
10:50 am – 11:10 am	<b>Monitoring Lung Density</b> B. Stoel (Leiden University Medical Center, The Netherlands)
11:10 am – 11:30 am	<b>Discussion</b>
11:30 am – 11:50 am	<b>The Risk of Lung Disease in Heterozygotes</b> E. Silverman (Harvard Medical School, USA)
11:50 am – 12:10 pm	<b>Discussion</b>
12:10 pm – 1:00 pm	<i>Lunch</i>

### Session 3: Treatment

1:00 pm – 1:20 pm	<b>Delivery of AAT to Airways</b> M. Griese (University of Munich, Germany)
1:20 pm – 1:40 pm	<b>Discussion</b>
1:40 pm – 2:00 pm	<b>Cell-based Therapies</b> J. Stolk (Leiden University Medical Center, The Netherlands)
2:00 pm – 2:20 pm	<b>Discussion</b>
2:20 pm – 2:40 pm	<b>Gene Therapy for AATD-related Lung Disease</b> R. Crystal (Weill Cornell Medical College, USA)
2:40 pm – 3:00 pm	<b>Discussion</b>
3:00 pm – 3:10 pm	<i>Coffee Break</i>
3:10 pm – 3:30 pm	<b>Review of RCTs with AAT</b> R. Sandhaus (National Jewish Health, USA)
3:30 pm – 3:50 pm	<b>Discussion</b>
3:50 pm – 4:10 pm	<b>Trials and Tribulations of Registering Plasma AAT and Its Dose</b> M. Forshag (GlaxoSmithKline, USA)
4:10 pm – 4:30 pm	<b>Discussion</b>
4:30 pm – 5:30 pm	<b>Joint Session — Scientists and Patients</b>

## In Memoriam

Prominent Alpha-1 researcher **Maurizio Luisetti, MD**, of Pavia, Italy, passed away last October 20, 2014. He was a pioneer in Alpha-1 research and was considered the “father” of the Italian Alpha-1 patient and research community. The 5th Alpha-1 Congress’ location was chosen because of Maurizio, and he was scheduled to be the Alpha-1 Research Conference’s Chairman this year.

Maurizio Luisetti was medical director at the Clinic of Respiratory Diseases, IRCCS Policlinico San Matteo for 20 years, and head of the clinic’s Laboratory of Biochemistry and Genetics for nearly 30 years in Pavia, where he coordinated an Alpha-1 research registry, testing program and research network. He was an adjunct professor at the University of Pavia’s School of Respiratory Diseases.

Fellow researcher Francesco Bonella, MD, said, “Besides his outstanding competence and high international profile, Maurizio was a person with extraordinary humanity and commitment towards his patients and coworkers. He also believed that progress in research depends on cooperation and networking. “He will be sadly missed,” said Bonella, “We have to continue our work with passion and determination in his memory. Thank you, Maurizio, for all you have done and all you gave us in these years.”

