



Sponsors

3rd CNE International Cystinosis Conference

Research Meeting, July 7, 2022

Pharmaceutical Companies



Mdeon visa number: 22/V2/13900/008177

Patient Organisations



3rd CNE International Cystinosis Conference

Remarks before we start

COVID

- Many people with cystinosis have had kidney transplants and will be immunosuppressed.
- While mask wearing is not mandatory in Belgium, out of respect to people in our community who may feel safer, **we welcome and recommend mask wearing** while indoors at conference sessions.
- If you feel unwell, we ask you to take an antigen/lateral flow test and to please not attend conference sessions if you get a positive result.

Red key cord

- If you need support, ask people from the Dutch-Flemish Cystinosis Group wearing a red key cord or ask at the registration desk.
- People with a red key cord will also guide you for lunch to the Jubilee Hall.

3rd CNE International Cystinosis Conference

Remarks before we start

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- 1) German, Deutsch
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- 5) Dutch, Nederlands
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- 7) Turkish, Türk

3rd CNE International Cystinosis Conference

Friday July 8 th , 2022			
Main Conference			
08:00	08:30	Registration and Welcome coffee	Patient team
Plenary session 0			
<i>Starting to know each other</i>		<i>Session Chair: Fons Sondag</i>	
		Subject	Speaker
08:30	08:45	Opening	Elena Levtchenko, Anne Marie O'Dowd, Claudia Sproedt
08:45	09:15	Kick-off: What do you know about cystinosis? Interactive quiz	Patient team

3rd CNE International Cystinosis Conference



Opening of the conference

1. Anne-Marie O'Dowd, Chair of Cystinosis Network Europe
2. Claudia Sproedt, Founder of Cystinosis Network Europe
3. Elena Levchenko, Scientific Chair of the 3rd CNE ICC

Chair : Fons Sondag, Dutch-Flemish Cystinosis Group & CNE



4th International Cystinosis Conference

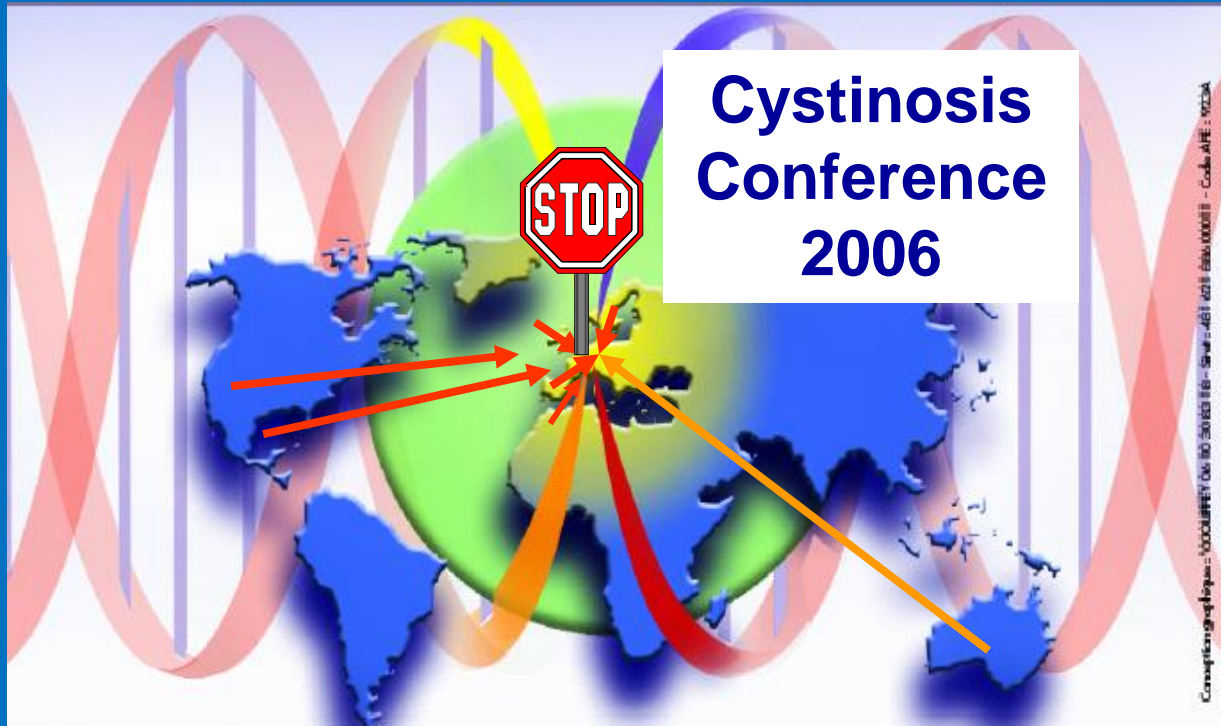
Working together for Cystinosis



Noordwijkerhout, June 30 - July 2, 2006

WHO ?

200 participants: 4 continents



Australia
Belgium
Denmark
France
Germany
Israel
Italy
Ireland
Morocco
Mexico
Netherlands
Poland
Spain
Turkey
UK
USA

WHO ?

Organizers:

Cystinosis Foundation (J. Hotz)

Orphan Europe

Cystinosis Group Netherlands

AIRG

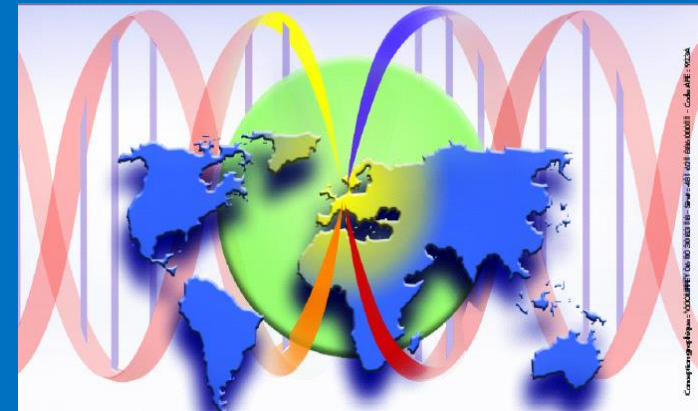
National Cystinosis

Organizations

(UK, Ireland, Germany)



Jean Hotz
1928 - 2020



Scientific committee:

E. Levtchenko

N. Knoers

H. Blom

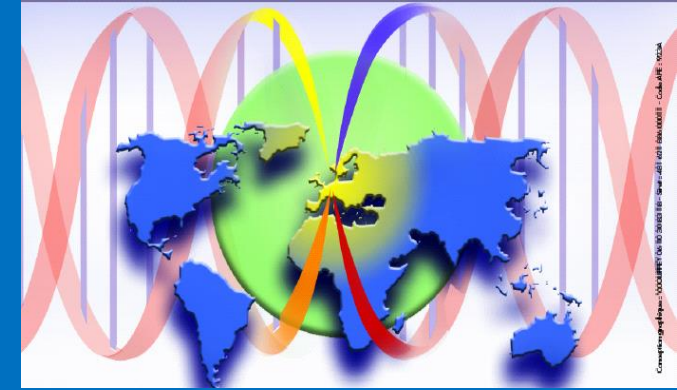
W. Gahl

L. Monnens

P. Niaudet

W. Van't Hoff

WHAT ?



Friday

2 state of art lectures

Professionals

- I. Study of disease mechanism
- II. Free communications/posters

Families

- I. Living with cystinosis
- II. Reports from around the world

Testimonies

Professor Rizzoni Award

Reception

Saturday

- Renal and extra-renal disease in cystinosis
- Growth
- Cognitive function
- Cystine measurement
- Clinical data base
- Treatment issues

2 workshops

Young children

Adolescents and adults

Sunday

- Role of physical exercise
- Working Out in Cystinosis
- State of art lecture

3rd CNE International Cystinosis Conference



16 years later...

Maria-Theresiacollege, Leuven, Belgium

July 7-10, 2022



Devoted to



Dr. Jerry Allan Schneider
1937 – 2021

Editorial

A Personal History of Cystinosis by Dr. Jerry Schneider

Cells **2022**, *11*, 945. <https://doi.org/10.3390/cells11060945>

<https://www.mdpi.com/journal/cells>

55 years ago (1967) Jerry Schneider unraveled
the cellular basis of cystinosis



cells

Cellular and Molecular Mechanisms of Nephropathic Cystinosis

Edited by

Elena N. Levchenko

Printed Edition of the Special Issue Published in *Cells*

www.mdpi.com/journal/cells



3rd CNE International Cystinosis Conference

Leuven, Belgium
7-10 July 2022



Fons Sondag

Marjolein Bos

3rd CNE International Cystinosis Conference

Leuven, Belgium
7-10 July 2022



Koenraad Veys



Martine Besouw

3rd CNE International Cystinosis Conference

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- AIRG-España
- Asociación Cistinosi España – www.grupocistinosi.org
- Associazione Cistinosi, Italy – www.cistinosi.it
- Cystinosis Foundation UK – www.cystinosis.org.uk
- Cystinosis Ireland – www.cystinosis.ie
- Cystinose-Selbsthilfe e.V., Germany – www.cystinose-selbsthilfe.de
- Dutch and Flemish Cystinosis Group – www.cystinose.nl
- Vaincre les Maladies Lysosomale (VML-France) – www.vml-asso.org
- Cystinosis Research Network – www.cystinosis.org
- Cystinosis support group Turkey
- Cystinosis support group Russia
- Cystinosis support group Australia
- Cystinosis support group South Africa
- Mexican Association of Cystinosis – www.cistinosi.org.mx



Claudia Sproedt



Anne-Marie O'Down



Denise Dunne

3rd CNE International Cystinosis Conference

Leuven, Belgium

7-10 July 2022

Scientific committee

Elena Levtchenko , David Cassiman, Mirian Janssen, Marlies Cornelissen, Francesco Emma, Katharina Hohenfellner, Aude Servais , Gema Ariceta, Rezan Topaloglu, Anna Bjerra, Graham Lipkin, Detlef Bockenhauer, Atif Awan, Ewa Elenberg, Jun Oh, Dieter Haffner, Lore Willem

CNE Board of directors

Anne-Marie O'Dowd, Claudia Sproedt, Marjolein Bos, Niamh O'Brien

Patient team of the Dutch Flemish Cystinosis Group

All team members: social activities & workshops

Organising committee:

Elena Levtchenko, Fons Sondag, Marjolein Bos/Claudia Sproedt
+Denise Dunne

The logo of KU Leuven, consisting of the text "KU LEUVEN" in white capital letters on a dark blue rectangular background.

KU LEUVEN

Conference Organization:
Ann Moerenhout

3rd CNE International Cystinosis Conference

Friday July 8 th , 2022			
Main Conference			
Plenary session 1			
<i>Cystinosis: a global view</i>			<i>Session Chair: Francesco Emma</i>
		Subject	Speaker
09:15	09:45	Overview of cystinosis treatment in 2022	Elena Levtchenko
9:45	10:15	Availability of treatment for cystinosis patients around the globe	Aurélia Bertholet Thomas
10:15	11:15	Bring good treatment for cystinosis patients around the globe (Pitches of 5 minutes & panel discussion)	Moderator Rezan Topaloglu MD's and patients
11:15	11:45	Coffee break	All

3rd CNE International Cystinosis Conference

Friday July 8th, 2022, 10:15-11:15

Bring good treatment for cystinosis patients around the globe

Short stories

Country	Organisation	Speaker
Turkey	Department of Pediatric Nephrology, School of Medicine, Hacettepe University, Ankara	Rezan Topaloglu
Germany	LebenEben, Cystinose Selbsthilfe	Soenke Oetjen & Markus Schmitt
Mexico	President of the Asociación Mexicana de Cistinosis	Victor Gomez
France	Centre de Référence des Maladies Rénales Rares, Bron	Aurélia Bertholet Thomas & Aude Servais
South Africa	Chairperson Cystinosis Foundation South Africa	Gail Daniels
USA	President & VP Development Cystinosis Research Network	Jonathan Dicks
Russia	Department of Nephrology, National Medical and Research Center for Children's Health, Moscow	Alexey Tsygin
Spain	Cistinosis Asociacion España (ACE)	Irati Vilariño



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Patient Organisations



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Main Conference			
Plenary session 2:			
<i>How to monitor cysteamine treatment?</i>			<i>Session Chair: David Cassiman</i>
11:45	12:00	Monitoring of cysteamine treatment: <u>laboratory</u> perspective	Sandrine Marie
12:00	12:30	Monitoring of cysteamine treatment: <u>physician</u> perspective	Marlies Cornelissen
12:30	12:45	Q&A Monitoring of cysteamine treatment	Presenters
12:45	13:45	Lunch	All

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Friday July 8 th , 2022			
Main Conference			
Plenary session 3:			
<i>How to improve current treatment?</i>			<i>Session Chair: Koenraad Veys</i>
13:45	14:15	Overview of cystinosis research	Francesco Emma
14:15	14:45	New and improved medication for cystinosis: cysteamine prodrug	Herbie Newell
14:45	15:15	Long-term follow-up of nephropathic cystinosis: importance of international registries	Aude Servais
15:15	15:45	Medications after kidney transplantation and improved treatment of infections	Paul Grimm
15:45	16:15	Tea break	All



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Main Conference			
Plenary session 3: <i>How to improve current treatment?</i>			
			<i>Session Chair: Koenraad Veys</i>
16:15	16:45	Short poster presentations	Prep. Koenraad Veys, Martine Besouw
16:45	17:15	From session 4 <i>Cystinosis as a multi organ disease:</i> Neurocognitive functioning in cystinosis patients	John J. Foxe
17:15	18:00	Poster Session 1	All

3rd CNE International Cystinosis Conference

Friday July 8th, 2022, 16:45-17:15

Short Poster Presentations

Subject	Speaker
Employing isogenic cell models to study the underlying mechanism of cystinosis myopathy	Louise Medaer
Cystinosis Bone Metabolic Disease: towards a local bone inflammation	Candide Alioli
The Cystinosis Patient Central: A Survey to gain insight into the medication use and the perspective for treatment improvement from Dutch Cystinosis Patients	Cecile Berends
The role of mitochondrial oxidative stress on cell adhesion in cystinosis podocytes: a new possible therapeutic strategy?	Sante Princiero Berlingiero
The potential of <i>CTNS</i> -mRNA based gene replacement therapy to treat nephropathic cystinosis	Tjessa Bondue
Spectral domain optical coherence tomography-based retinochoroidal cystine crystal score (RCCCS): a window into infantile nephropathic cystinosis	Leonie F. Keidel
Outcome of infantile nephropathic cystinosis depends on age at initiation cysteamine and not on <i>CTNS</i> genotype: a multicenter sibling cohort study	Koenraad Veys

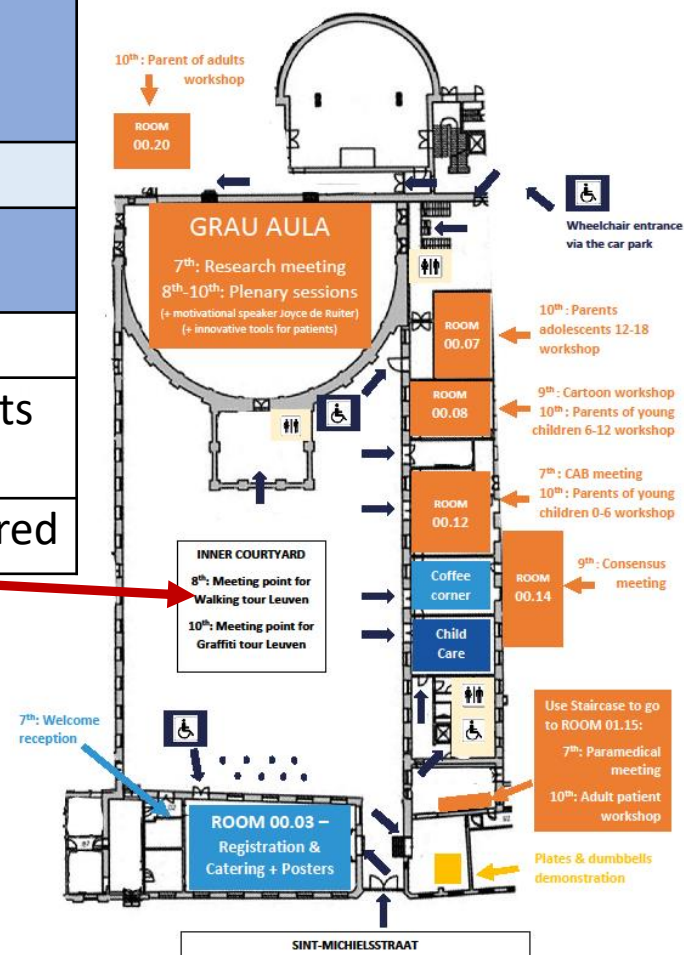
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Main Conference

Parallel sessions: *social events*

18:00	20:00	Dinner at own cost	
19:00	22:00	Social Event Adult Patients: Cooking workshop	Adult patients only
20:00	21:30	Walking tour Leuven	Who registered



3rd CNE International Cystinosis Conference

Cooking workshop for adult patients

Taxi to Cooking Workshop leaves at:

- 17h50 Hotel Ibis Budget
- 18h05 MTC - Sint Michielsstraat
- 18h15 Pentahotel
- 18h30 Hotel Ibis centrum
- 18h50 Arrival at Dishcover

3rd CNE International Cystinosis Conference

Walking tour Leuven

Meeting point:

- 19h45 MTC - Inner Courtyard

Afterwards, you can enjoy the music performances at Leuven's most beautiful squares during the free city festival Beleuvenissen.



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Saturday July 9 th , 2022			
Main Conference			
8:00	8:30	Welcome coffee	
		Subject	Speaker
Plenary session 4:			
<i>Cystinosis as a multi organ disease</i>		<i>Session chair: Katharina Hohenfellner</i>	
08:30	09:00	Bone disease in cystinosis including surgery	Dieter Haffner
09:00	09:30	Muscle disease in cystinosis	Pascal Laforêt
09:30	09:45	How to improve muscle function in cystinosis: IMPACT study	Christian Koepl
09:45	10:15	Eye complications in cystinosis & status of eye treatment	Hong Liang
10:15	10:45	Panel discussion on use of eyedrops	Ingele Casteels
10:45	11:15	Coffee break	All



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Plenary session 4:			
		<i>Cystinosis as a multi organ disease</i>	<i>Session chair: Katharina Hohenfellner</i>
11:15	11:45	Pregnancy and cystinosis	Graham Lipkin
11:45	12:15	Male fertility and cystinosis	Koenraad Veys
12:15	13:45	Lunch	Patients & Families
Parallel sessions:			
09:00	12:15	Cartoon workshop	Patients & Siblings age 13-18 only
13:45	14:45	Motivational speaker Joyce de Ruiter	Patients & families
12:15	14:45	Consensus treatment goals in cystinosis (incl. lunch)	MD's & researchers Chairs: Martine Besouw & Koenraad Veys



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Motivational Speaker

Motivational speaker: Joyce de Ruiter

Theme: change & agility:

- Parents giving birth to a child with an inborn disease unexpectedly must cope with the change in their lives and need to be agile.
- Patients with an inborn disease must be agile all the time. Cope with all the ups and downs of their disease. Not always be able to do what they want.

Book by Joyce the Ruiter:



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Main Conference			
		Subject	Speaker
Plenary session 5: <i>Future perspectives in cystinosis</i> <i>From newborn screening to gene therapy</i> <i>Session Chair: Martine Besouw</i>			
14:45	15:15	New-born screening for cystinosis & the effect of pre-symptomatic start of cysteamine treatment	Katharina Hohenfellner
15:15	15:45	Status of autologous HSC transplantation in cystinosis	Stephanie Cherqui
15:45	16:00	Stem cell transplant followed by living donor kidney transplant leaving the patient free of immunosuppression	Paul Grimm
16:00	16:30	Q&A gene therapy	All
16:30	17:00	Tea break	All

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Saturday July 9 th , 2022			
Main Conference			
		Subject	Speaker
Parallel sessions:			
17:00	18:00	Poster Session 2	All
17:00	17:30	Innovative tools for patients: <ul style="list-style-type: none">• Patients' empowerment tool• Cystinosis & Me App	Elena Levtchenko
Plenary session:			
19:00	21:00	Conference dinner	All
20:15	20:30	Best research poster & presentation awards	Koenraad Veys, Martine Besouw, Fons Sondag

Dinner only available for people who registered for the dinner!

Continue with next slide

3rd CNE International Cystinosis Conference



Presenters & others:

- Thank you for presenting on this conference
- Thank you for joining us
- Thank you for supporting us
- Please enjoy the dinner, but we wish you already a good trip back home

Patients and families:

- See you on Sunday
- We will have a program on Quality of Life and workshops



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