

Sponsors

3rd CNE International Cystinosis Conference Family Conference July 8-10, 2022

Pharmaceutical Companies Patient Organisations



3rd CNE International Cystinosis Conference

Remarks before we start

COVID

- Many people with cystinosis have had kidney transplants and will be immunosuppressed.
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3rd CNE International Cystinosis Conference

Friday July 8th, 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

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Opening of the conference

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

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



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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

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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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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 8th, 2022

Main Conference

Plenary session 3:

How to improve current treatment?

Session Chair: Koenraad Veys

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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Friday July 8 th , 2022			
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

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

Short Poster Presentations

Subject	Speaker
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 Berlingerio
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
Employing isogenic cell models to study the underlying mechanism of cystinosis myopathy	Louise Medaer
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

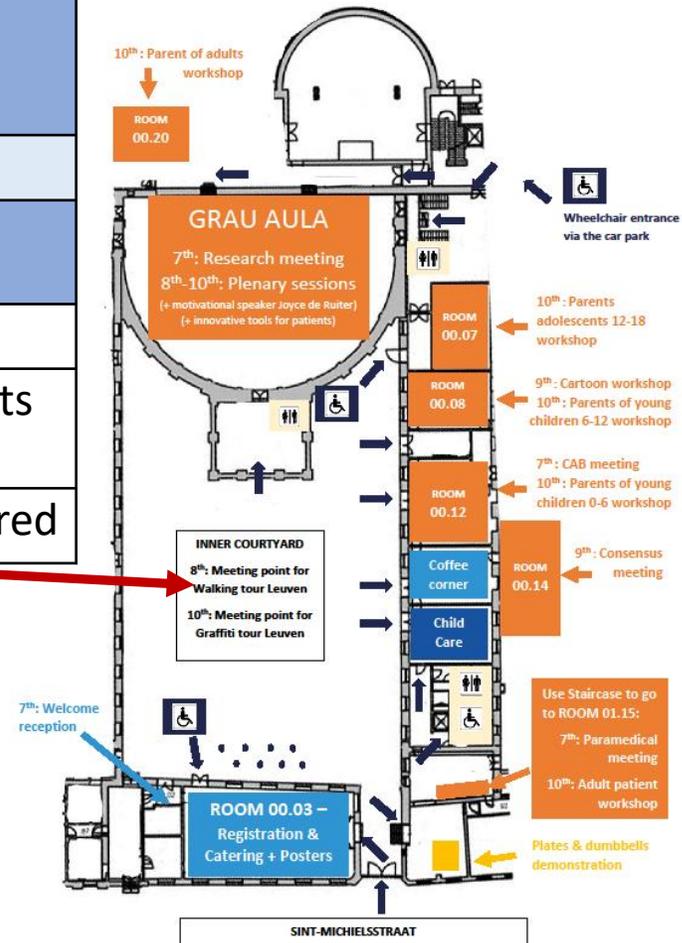
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Friday July 8th, 2022

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



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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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3rd CNE International Cystinosis Conference

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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Saturday July 9 th , 2022			
Main Conference			
		Subject	Speaker
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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Saturday July 9 th , 2022			
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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