

Thursday July 7 th , 2022			
Research Meeting			
(Closed session for medical professionals, researchers and CNE representatives)			
12:00	12:30	Registration of participants research meeting	
12:30	13:00	Walking lunch	
		Subject	Speaker
A. Novel disease models for cystinosis			
13:00	13:05	Welcome address	Martine Besouw & Koenraad Veys
13:05	13:25	The effects of Cysteamine and Everolimus treatment on cystinotic rats	Jennifer Hollywood
13:25	13:40	MFSD12 as a novel therapeutic target in cystinosis	Jennifer Hollywood
13:40	13:45	Q&A	
13:45	14:05	Advanced <i>in vitro</i> models for cystinosis	Manoe Janssen
14:05	14:25	Genistein improves renal disease in the mouse model of nephropathic cystinosis	Ester De Leo
14:25	14:35	Q&A	
14:35	14:50	Tea break & poster session	
B. Towards innovation in treatment of cystinosis			
Novel treatment strategies			
14:50	15:10	Gene repair in cystinosis	Manoe Janssen/ Elena Sendino Garvi
15:10	15:30	The potential of mRNA based therapy to treat nephropathic cystinosis	Tjessa Bondue
15:30	15:40	Q&A	
Novel therapeutic targets & promising drugs			
15:40	15:55	Prospects for translational read-through therapy of nonsense mutations in cystinosis	Paul Goodyer
15:55	16:10	Novel mechanisms and therapy for kidney injury in nephropathic cystinosis	Minnie Sarwal
16:10	16:20	Q&A	
Innovation in treatment of ocular cystinosis			
16:20	16:30	Development and testing of a controlled release cysteamine-eyedrop	Morgan Fedorchak
16:30	16:40	Biomaterial mediated transfer of CTNS for ocular cystinosis	Valeria Graceffa
16:40	16:50	Development of contact lenses for ocular complications of cystinosis	Anuj Chauhan
16:50	17:00	Q&A	
17:00	17:05	Final comments on research meeting	Martine Besouw & Koenraad Veys

Thursday July 7 th , 2022		
Paramedical Meeting (Closed session for paramedical professionals and CNE representatives)		
12:00	12:30	Registration of participants paramedical meeting
12:30	13:00	Walking lunch
		Round Table Discussion
13:00	17:00	How to fit the intake of cysteamine with the food & drinks schedule
		Transitioning from tube to oral feeding
		Therapy compliance
		Transition from paediatric to adult care
		Inputs from outside the organising committee
14:35	14:50	Tea break

Thursday July 7 th , 2022			
Main Conference			
17:30	19:30	Welcome reception & registration	Patient team

Friday July 8 th , 2022			
Main Conference			
08:00	08:30	Registration and Welcome coffee	Patient team
Starting to know each other		Session Chair: Fons Sondag	
		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
Plenary session 1		Session Chair: Franceso Emma	
Cystinosis: a global view			
		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
Plenary session 2:		Session Chair: David Cassiman	
How to monitor cysteamine treatment?			
11:45	12:00	Monitoring of cysteamine treatment: laboratory perspective	Sandrine Marie
12:00	12:30	Monitoring of cysteamine treatment: physician perspective	Marlies Cornelissen
12:30	12:45	Q&A Monitoring of cysteamine treatment	Presenters
12:45	13:45	Lunch	All
Plenary session 3:		Session Chair: Koenraad Veys	
How to improve current treatment?			
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
16:15	16:45	Short poster presentations	Prep. Koenraad Veys, Martine Besouw
16:45	17:15	Neurocognitive functioning in cystinosis patients	John J. Foxe
17:15	18:00	Poster Session 1	All
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	All

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
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
Plenary session 5:			
<i>Future perspectives in cystinosis: 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
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

Sunday July 10 th , 2022			
Patient & Family Workshops			
(Closed session for patients & parents)			
8:30	9:00	Welcome coffee	
		Subject	Responsible
Plenary session 6:			
Quality of life & Workshops		Session Chair: Elena Levtchenko	
09:00	09:45	Results quality of life survey, Leuven	Lore Willem
09:45	10:00	Introduction to the workshops	Fons Sondag
10:00	10:30	Coffee break	All
Parallel Sessions: Workshops			
<p><u>Parents</u> do not (both) have to attend the workshop belonging to the age group of their child. Attending a higher age group gives information on what they can expect. Except for the youngest age group no medical doctors will participate in the workshops.</p> <p>The best way for <u>adolescent</u> patient to exchange information is probably not a formal workshop. Instead a Graffiti tour through Leuven is offered. Be aware of the earlier starting time. Siblings are welcome too!</p>			
10:30	12:00	<u>Adult patient workshop:</u> How to cope with cystinosis: school, jobs, private life, friends, relations. What psycho-social support do patients need?	Patient team
10:15	12:00	<u>Adolescent patients & siblings 12-18:</u> Graffiti tour Leuven	Patient team
10:30	12:00	<u>Parents of adults:</u> Can we ever give up: parent adult child relation, loss of control, support autonomy, how to overcome setbacks	Patient team
10:30	12:00	<u>Parents adolescents 12-18:</u> What will the future bring: compliance, transfer to adult clinic, helping to accept the condition, cope with bullying, our own feelings and struggles as parents	Patient team
10:30	12:00	<u>Parents of young children 6-12:</u> Discuss day to day issues: taking medicines at school, get support at school, how to tell the other children in the group, playing and sleeping at a friend's home	Patient team
10:30	12:00	<u>Parents of young children 0-6:</u> When do we get rest from coping with: thirst, polyuria, vomiting, feeding problems/tubes, family support, paying attention to siblings	Patient team, Elena Levtchenko
Plenary session 6: Chair: Elena Levtchenko			
12:00	12:15	Conference closure Summary of conference by photos & drawings Farewell	Elena Levtchenko, Claudia Sproedt, Fons Sondag
12:15	13:00	Lunch	All