



★ The Cystinosis Foundation

6th International Cystinosis Conference September 23 – 26, 2010

Congress Village Ge.Tur.
Viale Centrale, 29
33054 Lignano Sabbiadoro (UD) ITALY
<http://www.getur.com>
midway (100 km) between Venice and Trieste

PRELIMINARY PROGRAMME

THURSDAY, SEPTEMBER 23, 2010

17.00 - 20.00 Registration and Welcome
19.30 DINNER

FRIDAY, SEPTEMBER 24, 2010

07.00 - 08.00	CONFERENCE REGISTRATION	
08.00 - 08.15	WELCOME & INTRODUCTION	F. EMMA, M. MAZZINA, J. HOBBS - HOTZ
08.15 - 08.45	50 years of cystinosis research and treatment	J. SCHNEIDER
08.45 - 10.25	GENETICS AND REGISTRIES	
08.45 - 09.05	Genetics of cystinosis and prenatal counselling	C. ANTIGNAC
09.05 - 09.25	The new European Registry	P. NIAUDET
09.25 - 09.55	Cystinosis in emerging countries	R. TOPALOGLU, A. TSYGIN
09.55 - 10.15	Time for questions	
10.15 - 10.30	COFFEE BREAK	
10.30 - 12.00	LONG-TERM OUTCOME AND NEW TREATMENTS	
10.30 - 10.50	Long-term outcome in cystinosis	W. GAHL
10.50 - 11.10	Eye problems and new cysteamine collyriums	M. DEL MONTE
11.10 - 11.30	Muscle wasting in nephropathic cystinosis	D. TRAUNER
11.30 - 11.50	Update on cysteamine treatment and adverse events	E. LEVTCHENKO
11.50 - 12.10	Long-acting cysteamine	R. DOHIL
12.10 - 12.30	Time for questions	



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12.30 - 13.30 LUNCH

13.30 - 14.30 WORKSHOP FOR CHILDREN (6-12) WITH PARENTS PRESENT F. EMMA
 13.30 - 14.30 WORKSHOP FOR ADOLESCENTS AND YOUNG ADULTS W. GAHL, M. OSTERMANN

14.30 - 14.50 Cognitive performance in cystinosis D. TRAUNER
 14.50 - 15.10 Psychological aspects and medication compliance M. OSTERMANN
 15.10 - 15.30 Time for questions

15.30 – 15.45 COFFEE BREAK

15.45 - 16.45 Adult patients and Parents Informal Discussion M. BOS
 16.45 - 17.05 Physical Activity Regime M. MAXWELL
 Parents retrieve children and everyone meet at pool
 17:45 - 18:30 Water Aerobics M. MAXWELL

Session for Professionals

LESSONS FROM OTHER DISEASES (ELENA LEVTCHENKO)

15.45 - 16.05 Molecular biology of Lowe disease A. DE MATTEIS
 16.05 - 16.25 Murine models of proximal tubular defects E. CHRISTENSEN
 16.25 - 16.45 Altered endocytosis in renal Fanconi syndromes P. COURTOY

16.45 – 17.00 BEVERAGE BREAK

Session for Professionals

NEW FRONTIERS (CORINNE ANTIGNAC)

17.00 - 17.20 Bone marrow transplantation S. CHERQUI
 17.20 - 17.40 Gene therapy for metabolic diseases A. AIUTI
 17.40 - 18.00 Mesenchymal stem cells P. GOODYER
 18.00 - 18.30 Zinc finger nucleases for homology directed repair in cystinosis P. HARRISON

18.30 Child care closes

19.30 Dinner

SATURDAY, SEPTEMBER 25, 2010

Families & Professionals

08.00 - 08.10 Opening Remarks
 08.10 - 08.30 Children's information on cystinosis W. VAN' HOFF
 08.30 - 08.50 Lay presentation on bone marrow transplantation
 for families S. CHERQUI
 08.50 - 09.10 Lay presentation on gene therapy for families S. CHERQUI
 09.10 - 09.30 Time for questions



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KIDNEY, GASTROINTESTINAL PROBLEMS AND GROWTH

09.30 - 09.50 Treatment of Fanconi Syndrome P. GOODYER

09.50 – 10.10 COFFEE BREAK

10.10 - 10.30 Kidney transplantation – steroid avoidance protocols P. NIAUDET

10.30 - 10.50 Growth, puberty and sexual development M. GRECO

10.50 - 11.10 Gastro-intestinal problems related to cystinosis R. DOHIL

11.10 - 11.45 Time for questions

11.45 - 13.15 LUNCH

13.15 - 14.30 WORKSHOP FOR ADULT PATIENTS

W. VAN' HOFF, M. OSTERMANN

13.15 - 15.00 POSTER SESSION / ROUND TABLE

Posters and displays from researchers, industry groups and others. Authors are present to discuss their work. In addition, professionals will sit at tables and Patients/families will be able to discuss issues with them on an individual basis.

15.00 – 15.15 COFFEE BREAK

Q&A SESSIONS W/ PANELS OF EXPERTS + 2 PARENTS/PATIENTS

15.15 - 16.15 Preserving renal function

R. TOPALOGLU, E. LEVTCHENKO, P. NIAUDET

16.15 - 17.15 Gastrointestinal/pancreas/feeding/growth problems

R. DOHIL, M. GRECO, P. GOODYER

17.15 - 18.15 Neurological/psychological aspects

M. OSTERMANN, D. TRAUNER, W. VAN'T HOFF

18.15 Child care closes

19.30 DINNER

SUNDAY, SEPTEMBER 26, 2010

09.00 - 10.00 LIVING WITH CYSTINOSIS:

A discussion on living with cystinosis, focusing on education, issues related to overprotection, transition to adult life and coping.

10.00 - 10.30 Presentation of W.L. Hobbs Award.

10.30 - 11.00 Closing remarks and conclusion of conference.

F. EMMA, M. MAZZINA, J. HOBBS-HOTZ