

PROGRAMME

CME Credits Applied

Conference Chair: Dr William van't Hoff, UK

THURSDAY 26th JUNE

6.00 p.m. Conference Registration
Welcome Reception

FRIDAY 27th JUNE

8.00 – 8.45 am Conference Registration

9.00 – 9.15 Welcome and Introduction

9.15 – 9.30 **Formal Conference Opening**
Irish Minister for Health and Children –
Mary Harney TD

9.30 – 10.00 **Keynote Speaker**
Dr Jerry Schneider, USA
Title: 1000 papers on Cystinosis

To date, over 1000 research papers on cystinosis have been published on PubMed, the world-renowned medical and scientific research site. This is an incredible achievement for a condition that is in the 'ultra rare' category. Cystinosis research and treatment has come a long way since that first paper was published. Dr Schneider has made enormous contributions to the field and has set the tone for progress through collaboration. We are honoured that he has agreed to give the opening keynote presentation for the 5th International Conference – From Dreams to Reality - Cystinosis is 2008

10.00 – 10.30 Questions and Answers

10.30 – 10.45 **Conference Break**

10.45 – 12.30 pm **Parallel Session for Professionals**

Chair: TBC

The Basis of Cystinosis

This high-level scientific session is designed to update professionals on the current research. Its theme is The Basis of Cystinosis. World experts in the field will present.

Molecular Genetics	Dr Francesco Emma, Italy
Biochemical Model	Dr Elena Levtchenko, Belgium
A Disorder of Apoptosis	Dr Margaret Park, USA
Animal Models in Fanconi Syndrome and Cystinosis	TBC

10.45 – 12.30 **Parallel Session of Families**

Chair: Anne Marie O'Dowd, Ireland

Living with Cystinosis Panel TBC

This session for families is designed to discuss the practical issues facing families and patients living with cystinosis. The plan is for the session to be interactive with emphasis on ways families can help patients in areas such as education, physical development, eating issues, transition to adult care, for example. Issues faced by young people and adults will be discussed. Following on from previous conferences, Mack Maxwell will give an exercise demonstration

12.30 – 2.00 **Buffet Lunch (set up posters)**

2.00 – 3.30 **Plenary Session**

Chair: Dr Jerry Schneider, USA

Session for Original Research

The latest, cutting-edge research in cystinosis will be presented. Much of this research is funded by voluntary cystinosis support organisations from around the world. Presenters are requested to include one summary slide that is layperson- friendly.

3.30 – 3.45 **Conference Break**

3.45 – 5.30

Poster and Round Table Session

This formal poster session will see the conference hall transformed to enable attendees to view the posters and displays from researchers, cystinosis support groups, industry groups and others. Poster authors will be present to explain and discuss their work.

Along side this, professionals will sit at tables and families/patients will be able to discuss issues with them on an individual and informal basis.

SATURDAY 28TH JUNE

8.45 – 9.00 am Introduction

9.00 – 9.30 **The Family Perspective**

Sue Maguire, Ireland
Anne-Claire Panisset, France

This session will enable professionals understand the family perspective on the issues and challenges faced by patients and their families.

This session is designed to provide an opportunity for professionals to learn from the experiences of two people who live with cystinosis. Sue is mother to two children with cystinosis, one of whom recently had a transplant. Anne-Claire is an adult living successfully with cystinosis.

This session will be of interest to families and patients also.

9.30 – 10.00 Q&A

10.00 – 10.15 **Conference Break**

10.15 – 12.30 pm **Parallel Session for Professionals**

Chair: Dr Robert Kleta, UK

The Clinical Issues

Early Childhood

Dr Patrick Niaudet, France

The medical features of cystinosis in early childhood will be discussed. This will be an important presentation for families and of particular interest to families of recently-diagnosed children. The session is timed to enable parents to attend the children's session later in the morning.

School Life & Learning

Prof Doris Trauner, USA

This presentation describes research in relation to brain development, neurological, cognitive and academic functioning in children and adults with cystinosis.

Renal Complications of
Cystinosis

Dr Elena Levchenko, Belgium

The specific kidney problems of patients with cystinosis will be discussed.

Dialysis & Transplant

Dr Minnie Sarwal, USA

The features of dialysis and renal transplant will be addressed

Long Term Features

Dr William Gahl, USA

The long-term medical problems of cystinosis in young people and adults will be presented.

10.15 – 12.30 **Parallel Session for Families**

10.15 – 11.15

Young People **Only** Session

Dr William Gahl, USA

(Approx ages 13/14 to early 20's)

This session is for young people between the ages of 13/14 and early 20's. No parents here! Dr Gahl will be on hand to answer questions and facilitate discussion on issues that you want to raise. Hand in questions, anonymously if you wish, to the conference organisers in advance or ask questions on the day. You'll get to know everyone during the previous day's social event (see www.cystinosis.ie – under Young People).

11.15 – 12.30

Children with Parents Present Dr William van't Hoff, UK
(Approx age 6/7 to 12/13 years)

Children aged between 6/7 and 12/13 years take the lead facilitated by Consultant Paediatric Nephrologist Great Ormonde Street Children's Hospital Dr William van't Hoff. This event takes place in the playroom with the parents present. It will be an interactive, informal session where children get an opportunity to ask the questions.

12.30 – 1.30

Buffet Lunch

1.30 – 3.30

Plenary Session

Chair: Dr Craig Langman, USA

The Treatment

Medical Therapy Dr Paul Goodyer, Canada

This presentation involves the range of medications required for kidney problems (Fanconi Syndrome) and other issues in cystinosis.

Cysteamine Treatment Dr Ranjan Dohill, USA

The presentation involves Cystagon™ and its importance in the treatment of cystinosis. New cysteamine treatment (slow release therapy) will also be discussed

Monitoring Therapy Dr Bruce Barshop, USA

This presentation involves the testing of cystine levels in the body and related issues

Adverse Events on
Cysteamine Treatment Dr Atif Awan, Ireland

This presentation discusses the issue of adverse events such as skin lesions and other effects that may occur on cysteamine treatment

Transition & Adult Care Dr. Graham Lipkin, UK

This presentation will be of interest to Adult Nephrologists and patients facing into the challenges of transition to adult care

Progress on Novel Treatments
Around the World Dr Don Cairns, Scotland

Research is on-going around the world into new treatments for cystinosis. This presentation brings us up to date on progress

3.30 – 3.45

Conference Break

3.45 - 4.45

Full Conference

Q & A

Expert Panel

*The experts form a panel to take questions from the attendees.
Questions can be given in advance to conference organisers*

4.45 – 5.15

Keynote Speaker

Dr William van't Hoff, UK

5.15 – 5.30

Conference Wrap and end