

Small Conference Report for International Society for Neurochemistry

a. Basic information (title of the meeting, dates, organizer, venue, etc.)

16th International Conference on Neuronal Ceroid Lipofuscinosis (Batten Disease)

12-16 September 2018, Royal Holloway University of London, Egham, London UK



Scientific Organising Committee (SOC):

Sara Mole	University College London, London, UK, Chair
Susan Cotman	Harvard Medical School, Boston, USA
Jonathan Cooper	Harbor UCLA Medical Center, Los Angeles, USA
Angela Schultz	University Medical Center Hamburg-Eppendorf, Hamburg, Germany
Alexander Smith	University College London, London, UK
Heather Band	Batten Disease Family Association, Farnborough, UK
Ruth Williams	Guy's and St Thomas' Hospital, London, UK
Emyr Lloyd-Evans	Cardiff University, Cardiff, UK
Jill Weimer	Sanford Research, Sioux Falls, USA
Juan Bolanos	University of Salamanca, Salamanca, Spain
Marco Sardiello	Baylor College of Medicine, Houston, USA
Margie Frazier	Batten Disease Support and Research Association, USA
Miriam Nickel	University Medical Center Hamburg-Eppendorf, Hamburg, Germany
Stephanie Hughes	University of Otago, Dunedin, New Zealand
Steve Gray	University of Texas Southwestern Medical Center, Dallas, USA
Thomas Wishart	University of Edinburgh, Edinburgh, UK
Wendy Heywood	UCL Great Ormond Street Institute of Child Health, London, UK

Logistics Planning Group (LPG):

Prof Sara Mole	University College London, London UK, Chair
Dr Claire Russell	Royal Veterinary College, London, UK
Heather Band	Batten Disease Family Association, Farnborough, UK
Dr Ruth Williams	Guy's and St Thomas' Hospital, London, UK
Dr Alexander Smith	University College London, London, UK
Dr Ahad Rahim	University College London, London, UK
Dr Brenda Williams	King's College London, London, UK

Administrative support:

Bioscientifica	Bristol, UK
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b. Registration and registration fees

Registration fees (professional)

	Early Bird Registration Up to 02 July 2018	Standard Registration from 03 July 2018 until 11 September 2018	Onsite Registration from 12 September 2018
Standard Registration (incl. Accommodation)	£710	£765	£885
Student Registration (incl. Accommodation)	£625	£685	£725
One Day Registration (no Accommodation)	£125	£165	£205

Family representative registration fees

Family Representative Registration	Fees
Family representative Full Conference (incl. Accommodation)	£500
Family representative Friday-Saturday (incl. Accommodation)	£160
Family representative Friday-Sunday (incl. Accommodation)	£270
One Day only family representative (no Accommodation)	£20

c. Program

Five scientific sessions comprising 46 oral presentations spread across 3 days:

- Genetics & biology of the NCLs
- 'Omic approaches
- Disease models & mechanisms
- Translational research – preclinical
- Translational research – clinical

Underpinning principles:

- Excellent research – all talks chosen from submitted abstracts; short and focused;
- Future succession, new researchers, gender equality, diversity – LPG, SOC, Chairs of sessions
- Lay summaries at talks & posters to engage family representatives

Use of *slido* for interactive Qs and polls to widen engagement

Poster sessions. There were a total of 75 posters displayed in all five poster sessions – with four sessions allocated, 25% of posters were attended by the presenter in each of the first four sessions. The final session invited all presenting authors to be present to enable those attending just for the weekend to interact. Four poster prizes were given, and four posters selected for platform presentations.

Market Place. This consisted of 20 stalls located in 2 rooms, and ran for 2 hours, with movement between stalls encouraged every 10mins. The Market Place was set up to encourage family representatives to engage one-to-one with researchers and clinicians. It also proved to be a useful opportunity for scientists to discuss their work and potential collaborative efforts.

Stalls:

- 1 Universitätsklinikum Hamburg-Eppendorf (UKE): Eva Wibbeler, Angela Schulz, Christoph Schwering, Miriam Nickel
- 2 UCL Batten disease Centre: Wendy Heywood, Elisa Tinelli
- 3 University of Rochester Medical Center / School of Medicine and Dentistry: Jonathan Mink, Amy Vierhile,
- 4 University of Missouri School of Medicine: Martin Katz, Grace Robinson, Rebecca Whiting
- 5 Genomics Research Centre (Massachusetts General Hospital) and LMU München: Susan Cottman, Madeleine Klein, Elizabeth Butz
- 6 Large animal models: David Palmer, Nadia Mitchell, Samantha Murray
- 7 Surprising models: Christopher Minnis, Claire Russell, Tom Wishart



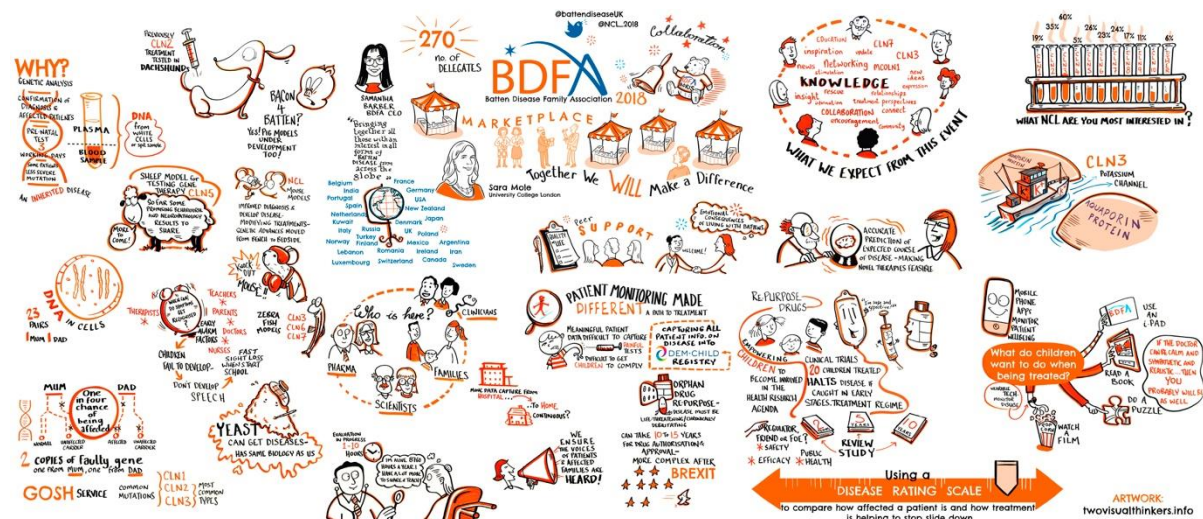
- 8 Cellular models: Gemma Gomez-Giro, Favio Pesaola, Stephane Lefrancois
- 9 UCL Gene Therapy Group: Sophia kleine Holthaus, Ahad Rahim, Sander Smith, Mikel Aristorena
- 10 University North Carolina Gene Therapy Group: Steven Grey, Alejandra Rozenberg, Elena Batracova
- 11 More than the brain: Jon Cooper, Hemanth Nelvagal
- 12 Drug Therapies: Rafael Badell Grau, Katie Shipley, Paul Trippier, Ritva Tikkanen, Helen Waller-Evans
- 13 BATCure Consortium: Emyr Lloyd-Evans, Juan Bolanos, Diego Medina
- 14 BATCure Patient Organisation Involvement: Heather Band, Evghenia Scripnic
- 15 JNCL and Education: Anne-Grethe Tøssebro, Barbara Cole, Heather Adams
- 16 JNCL and Adult living: Svein Rokne, Merete Staureby, Bengt Elmerskog
- 17 Clinical Nurse specialists/GP: Laura Lee, Christine, Caren
- 18 Family support: Harriet Lunnemann, Noreen Murphy
- 19 Batten disease Clinical Experts: John Ostergaard, Ruth Williams, Alexandro Simonati
- 20 Laboratory diagnosis: Ines Hoher de Halac, Marina Trivisano

Satellite meetings

Six independent satellite meetings took place before, during or after NCL2018, taking advantage of experts being together.

Cartoonists

2 visual thinkers – to capture the meeting from a lay perspective.



d. Speakers

Forty-six oral presentations in 5 scientific sessions:

Genetics and Biology (Chairs: Marco Sardiello, Thomas Wishart)

Nicole Miller, Emyr Lloyd-Evans, Alberto di Ronza

'Omics approaches (Chairs: Marco Sardiello, Thomas Wishart)

Stephano Doccini, Monther Abu-Remaileh, Wendy Heywood, Rachel Kline, Hannah Best, Stephan Storch, Torbjörn Lundstedt

Disease Models and Mechanisms (Chairs: Jon Cooper, Jill Weimer)

Rebecca Ahrens-Nicklas, Meaghan McLaren, Jonathan Cooper, Katie Shipley, Rafaella Magnoni, Madeleine Klein, Emyr Lloyd-Evans, Christopher Minnis, Kevin Koster, Juan Bolanos

Translational Research – Preclinical (Chairs: Adriana Cismondi, Alexander Smith, Steve Gray, Stephanie Hughes)

Marco Sardiello, Elena Batracova, Luis Tecedor, Nadia Mitchell, Alejandra Rozenberg, André Marques, Tyler Johnson, Shibi Likhite, Kathrin Meyer, Marco Peviani, Stephanie Hughes, Mikel Aristorena, Saul Herranz-Martin, Sophia kleine Holthaus

Translational Research – Clinical (Chairs: Alessandro Simonati, Miriam Nickel, Jonathan Mink, Ruth Williams)

Heather Adams, Tufikameni Brima, Angela Schultz, Charlotte Camp, Christoph Schwering, Simon Dutz, Jonathan Mink, Eva Wibbeler, Emily de los Reyes, Kristin Page



e. Plenary Speakers

Five keynote speakers

Alfried Kohlschütter (University Medical Center Hamburg-Eppendorf, Germany)
Long clinical experience of the NCLs - looking back to step forward

Susan Cotman (Massachusetts General Hospital, USA)
Genetics and Biology of the NCLs

Ahad Rahim (University College London, UK)
Gene therapy for rare childhood neurodegenerative diseases

Fran Platt (University of Oxford, UK)
A success story: drug development for a lysosomal disease

Elin Haf Davies (Aparito, UK)
A Path to Treatment

f. Highlight of the meeting

Rather than a particular moment, the highlight of the meeting was the depth and breadth of the research – especially the translational research – being undertaken. While until a few years ago almost all the research focus went into the two most common forms of the condition (CLN2 and CLN3), the preclinical translational research session this year comprised talks that highlighted the progress being made in the development of therapies for CLN1, CLN2, CLN3, CLN5, CLN6, CLN7, CLN8 and CLN10.

g. Participants

270 registered, including Family representatives - represented throughout, and some arriving for the final day of presentations.

Family representatives are those that know someone affected by NCL, the disease that was the topic of the conference. The involvement of patients and their families in research is crucial for researchers to maintain a relevant focus, understand the needs of the end-users, and develop therapeutic strategies, clinical trials and professional support that are acceptable to the patients and families. Family representatives raise significant research funding as government and pharmaceutical funding is minimal for this type of rare disease and their involvement is absolutely imperative for clinical trials. They are therefore key stakeholders in the research and attending part of the conference enables them to see what that funding leads to and receive and feedback on information about existing, new and future research and clinical trials. Interactions between family representatives and researchers also motivates researchers and increases fundraising efforts by family representatives. In addition, several family representatives are involved in related charities and have carried out studies, some of which were presented as posters, and one of which won a poster prize.

Participants

Total number registrants	270
Number of student registrants	26
Family representative registrants	28
Exhibitors	7
Unique institutions	110
Unique countries	19



h. Material distribution

As well as Delegate badges, conference bags were provided with abstract books and pamphlets from some of the sponsors. Abstract books were comprehensive including sponsor information and recipients of ISN sponsored attendees (covering registration costs).

i. Social events

Social networking – all meals together either in reserved cafeteria (breakfast, evening meal) or buffet in conference venue (lunch)

Evening receptions before evening meal on 1st, 3rd, 4th days

One ‘pub’ social in reserved bar on site, including ping pong tournament with 14 teams competing (2nd day)

One banquet and short international table quiz (4th day)

j. Travel awards

As a conference NCL 2018 did not offer any travel grants, but did reimburse the expenses of some keynote speakers, covering their travel, registration fees and the purchase of some conference equipment. This reimbursement was offered upon request.

Travel subsidies for Invited Speakers/Plenary Speakers

Susan Cotman	£1534.25
Elin Haf Davies	£28.80 (travel)
Alfried Kohlschütter	€127.94 (£114.13)
Frances Platt	None required
Ahad Rahim	None requested

k. Sponsorship

Total commercial	£23,000.00
Total charity and grants	£66,524.00
Overall total	£89,524.00

l. General budget; detailed ISN budget, how the ISN funds were utilized

(The budget used for each speaker or awarded person must be specified separately)

Total amount received	£10,500
Allocated to young researchers	£6492.71
Allocated to speakers	£1677.18
Allocated to family representative registrations	£2330.11
Total amount allocated	£10,500

Grant awardees

Name	Nationality	Amount
Jamal Al Ali	Lebanon	£625
Stefano Doccini	Italy	£625
Ivana Jedlickova	Czech Republic	£625
Emily Kirkham	UK	£625
Rachel Kline	UK	£625
Meagan McLaren	Canada	£625
Katie Shipley	UK	£625
Niklas Thießen	Germany	£625
Seda Yaşa	Canada	\$207.00 (£157.71)
Christopher Minnis	UK	£312.50
Charlott Repschlager	UK	£312.50
Samantha Murray	New Zealand	£710
Total		£6492.71

m. List of participants

This list includes all delegates who gave permission for their names to be included on a delegate list

First Name	Surname	City	Country
Monther	Abu Remaileh	Cambridge	USA
Heather	Adams	Rochester New York	USA
Rebecca	Ahrens-Nicklas	Philadelphia	USA
Abdullah	Alshehri	Cardiff	UK
Brian N.	Andersen	Århus N	Denmark
Mikel	Aristorena	London	UK
Ana	Assis	Torrance	USA
Rafael	Badell Grau	Museum Ave	UK
Alex	Bailey	Suite 210	USA
Lakshya	Bajaj	HOUSTON	USA
Heather	Band	Farnborough	UK
Samantha	Barber	Farnborough	UK
Elena	Batrakova	Chapel Hill	USA
Christian	Behnke	Hellerup	Denmark
Mette Olin	Behnke	Hellerup	Denmark
Jane	Bell	Exeter	UK
Craig	Benson	Austin	USA
Michelle	Berg	Cleveland	USA
Hannah	Best	Dunedin	New Zealand
Kevin	Bohn	Hamburg	Germany
Daniela	Borchert	Hamburg	Germany
Tufikameni	Brima	Rochester	USA
Katarzyna	Brooks	Tadley	UK
Leyla	Bulut	RUEIL MALMAISON	France
Nicholas	Buss	Rockville	USA
Elisabeth	Butz	Munich	Germany
Jacob	Cain	Sioux Falls	USA
Charlotte	Camp	London	UK
Christine	Caren	Newbury	UK
Jeffrey	Castelli	Cranbury	USA
Jessica	Centa	North Chicago	USA
Zhe	Chen	Okinawa-ken	Japan
Holly	Chivers	Tadley	UK
Dorna	Chu	Redwood City	USA
Ines Adriana	Cismondi	Córdoba	Argentina
Gary	Clark	Houston	USA



Christina	Clark	Columbus	USA
Sean	Clark	Cranbury	USA
Barbara	Cole	Addlestone	UK
Emily	Coode	Milton Keynes	UK
Jonathan	Cooper	St Louis	USA
Susan	Cotman	Boston	USA
Barbara	Csanyi	London	UK
An	Dang Do	Bethesda	USA
Beverly	Davidson	Philadelphia	USA
Elin Haf	Davies	Wrexham	UK
Glyn	Dawson	Chicago	USA
Emily	De Los Reyes	Columbus	USA
Shannon	Dean	Rochester	USA
Joshua	Dearborn	St. Louis	USA
Noel	Dela Paz	Rockville MD	USA
Alberto	di Ronza	Houston	USA
STEFANO	DOCCINI	Calambrone - Pisa	Italy
Sarah	DODKIN	York road	UK
Judy	Doyle	Cleveland	USA
Simon	Dulz	Hamburg	Germany
Samantha	Eaton	Roslin	UK
Kasper	Elmegaard Olesen	Middelfart	Denmark
Bengt	Elmerskog	Trondheim	Norway
Ashley	Falke	Columbus	USA
Jean-Marie	Favreau	Clermont-Ferrand	France
Vivian	Fernandez	suite 210	USA
Alan	Finglas	Tyrrelstown	Ireland
Robert	Fiorentino	Rockville MD	USA
Joseph	Fire	Rockville	USA
Lorna	FitzPatrick	Manchester	UK
Nicholas	France	New York	USA
Emily	Gardner	London	UK
Rob	Geer	Wyoming	USA
Hans	Goebel	Mommenheim	Germany
GEMMA	GOMEZ GIRO	Belvaux	Luxembourg
Russell	Gotschall	Cranbury	USA
Steven	Gray	Dallas	USA
Patrizia	Guarneri	Palermo	Italy
Nikolaj	H. T. Petersen	Copenhagen	Denmark
Ingrid Bergliot	Helland	Oslo	Norway
Saul	Herranz-Martin	London	UK
Wendy	Heywood	London	Guinea-Bissau
Masakuni	Horiguchi	Osaka	Japan
Lilli Beate	Hossner	Hamburg	Germany
Stephanie	Hughes	Dunedin	New Zealand
Emanuela	Izzo	Milan	Italy
Ronald	Jansen	Beuningen Gld	Netherlands
Ivana	Jedličková	Prague	Czech Republic
Tyler	Johnson	Sioux Falls South Dakota	USA
Karen	Kahn	Scottsdale	USA
Martin	Katz	Columbia	USA
Sarah	Kenrick	Tadley Hampshire	UK
Danielle	Kerkovich	Austin	USA
Keunpyo	Kim	Rockville	USA
Tracy	Kirby	Columbus	USA
Emily	Kirkham	Cardiff	UK
Priya	Kishnani	Durham	USA
Sophia	kleine Holthaus	London	UK
Rachel	Kline	Roslin	UK
Alfried	Kohlschütter	Hamburg	Germany
Kevin	Koster	Chicago	USA
Attila	Kovács	Sioux Falls	USA
Einar Kleinhans	Krogsaeter	Munich	Germany
Heather	Lambert	Tadley	UK
Heather	Lambert	Tadley	UK



Heather	Lambert	Tadley	UK
Logan	Langin	Sioux Falls	USA
Fernanda	Leal	Novato CA	USA
Stephane	Lefrancois	Laval	Canada
Stephen	Lehrman	Chapel Hill	USA
Tom	Lester	San Rafael	USA
Shibi	Likhite	Columbus	USA
Andrew	Lim	San Diego	USA
Emyr	Lloyd-Evans	Cardiff	UK
Pamela	Luijk	Nederhorst den Berg	Netherlands
Christian	Lundin	Gothenburg	Sweden
Olga	Lundin	Gothenburg	Sweden
Torbjörn	Lundstedt	Umeå	Sweden
Harriet	Lunnemann	Farnborough	UK
Yaxuan	Lyu	London	UK
Raffaella	Magnoni	Copenhagen	Denmark
Anette	Maischnack	Målev	Denmark
Andre	Marques	Kiel	Germany
Stefan	Mattheeuws	Mol	Belgium
Tristan	McKay	Manchester	UK
Meagan	McLaren	Peterborough	Canada
Rachel	McMinn	New York	USA
D. Elizabeth	McNeil	Cambridge MA	USA
Diego Luis	Medina Sanabria	Pozzuoli	Italy
Kathrin	Meyer	Columbus	USA
Nicole	Miller	Novato	USA
Timothy	Miller	Cleveland	USA
Jonathan	Mink	Rochester NY	USA
Christopher	Minnis	London	UK
Myriam	Mirza	Obertraubling	Germany
Nadia	Mitchell	Christchurch	New Zealand
Sara	Mole	London	UK
Clinton	Monfries	London	UK
Jill	Morris	Rockville	USA
christelle	Moufawad El Achkar	Boston	USA
Carsten	Munkholm	Målev	Denmark
Noreen	Murphy	Columbus	USA
Samantha	Murray	Christchurch	New Zealand
Hemanth Ramesh	Nelvagal	Torrance	USA
Angela Irena	Newcombe	Teignmouth	UK
Eric	Nicholas	Rochester	USA
Miriam	Nickel	Hamburg	Germany
Anna	Nicolau - Torrent	Tadley Hampshire	UK
Steffen Hartvig	Nielsen	Skoerping	Denmark
Maria	Nienhaus-Schuster	Bottrop	Germany
Anahita	Nikzad	Rockville	USA
Ines	Noher de Halac	Cordoba	Argentina
Sandra	Nusinoff Lehrman	Chapel Hill	USA
Andrew	Olaye	London	UK
Charles	O'Neill	Novato	USA
John R.	Østergaard	Århus N	Denmark
Kristin	Page	Durham	USA
Dilan	PALALI	RUEIL MALMAISON	France
David	Palmer	Lincoln	New Zealand
Salvatore	Papasergi	Palermo	Italy
Trine	Paus	Oslo	Norway
David	Pearce	Sioux Falls	USA
Marco	Peviani	BOSTON	USA
Francesco	Pezzini	Verona	Italy
JULIE	Pickering	Kingston upon Thames	UK
Nelleke	Plagman	Nederhorst den Berg	Netherlands
Fran	Platt	Oxford	UK
Forbes	Porter	Bethesda	USA
Andrew	Quigley	Didcot	UK
Ahad	Rahim	London	UK



Denia	Ramirez	Knoxville	USA
Allen	Reha	Cranbury	USA
Charlott	Repschlagel	London	UK
Katja	Rietdorf	Milton Keynes	UK
Greg	Robertson	London	UK
Grace	Robinson	Columbia	USA
Svein	Rokne	Asker	Norway
Alejandra	Rozenberg	Chapel Hill	USA
Annegret	Ruprecht	Hamburg	Germany
Claire	Russell	London	UK
Patrizia	Saladino	Palermo	Italy
Filippo M.	Santorelli	Calambrone-PISA	Italy
Marco	Sardiello	Houston	USA
Elmar	Schäfer	Bonn	Germany
Mathias	Schmidt	Calabasas	USA
Angela	Schulz	Hamburg	Germany
Christoph Fabian	Schwering	Hamburg	Germany
Issa	Seiler	Dortmund	Germany
Renee	Shediac	Novato	USA
Jordan	Sheets	Sioux Falls	USA
Katie	Shipley	Cardiff	UK
Alessandro	Simonati	Verona	Italy
Ole Christian	Slotten	Oslo	Norway
Sander	Smith	London	UK
Chiara	Soldati	Pozzuoli	Italy
Camilla	Spagner	Skoerping	Denmark
John	Spaltro	Cranbury	USA
Merete	Staureby	Virum	Denmark
Frank	Stehr	Hamburg	Germany
Stephan	Storch	Hamburg	Germany
Seiji	Sugiman-Marangos	Toronto	Canada
Luis	Tecedor	Philadelphia	USA
Bettina	Thede	Hamburg	Germany
Carsten	Thiel	New York	USA
Niklas	Thießen	Kiel	Germany
Christopher	Thornton	Manchester	UK
Ritva	Tikkanen	Giessen	Germany
Elisa	Tinelli	London	UK
Charlotte Iversholt	Toft	Middelfart	Denmark
Anne-Grethe	Tøssebro	Trondheim	Norway
Paul	Trippier	Amarillo	USA
Marina	Trvisano	Rome	Italy
Herman	van der Putten	Hamburg	Germany
Linda	van Eck	Doorn	Netherlands
Marjolein	van Keulen	Zeist	Netherlands
Tracy	VanHoutan	Downers Grove	USA
Amy	Vierhile	Rochester	USA
Helen	Waller-Evans	Cardiff	UK
Jill	Weimer	Sioux Falls	USA
Katherine	White	Springfield	USA
Rebecca	Whiting	Columbia	USA
Eva	Wibbeler	Hamburg	Germany
Katarzyna	Więciorek	Göttingen	Germany
Ruth	Williams	London	UK
Brenda	Williams	London	UK
Thomas	Wishart	Roslin	UK
James	Yarrow	Royston	UK
Seda	Yasa	Laval	Canada

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n. Photos

