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PHC-14-2015 'New therapies for rare diseases'

- Research and Innovation Action -

D9.12
Host NCL- 2018

WP 9 - Patient Organisation involvement

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Dissemination Level		
PU	Public	
PP	Restricted to other programme participants (including the Commission Services)	
RE	Restricted to a group specified by the consortium (including the Commission Services)	
CO	Confidential, only for members of the consortium (including the Commission Services)	

History table

Version	Date	Released by	Comments
V1	12/06/2019	Sara Mole	First draft

Table of contents

Key word list

NCL

Definitions and acronyms

Acronyms	Definitions
NCL2018	16th International Conference on Neuronal Ceroid Lipofuscinoses (Batten disease).

1. Introduction

D9.12 'Host NCL2018' is part of the communication strategy of WP09, including public and professional engagement as well as the sharing of scientific data and discussion as appropriate to a scientific meeting.

1.1 General context

NCL2018 is the 16th International Conference on Neuronal Ceroid Lipofuscinoses (Batten disease), that took place on 12 - 16 September 2018. It is a scientific meeting that takes place every 2 years, hosted by leading scientists in the field, and is attended by Patient Organizations as well as individual family members interested in progress. As such, there is always an important element of public engagement. The Coordinator of BATCure, Prof Sara Mole, offered to be Chair of the next international NCL meeting to be held in Europe, NCL2018, at a previous meeting, with BATCure participants playing a central role in its organization as well as its contents.

1.2 Deliverable objectives

D9.12 was to ensure appropriate organization in order to deliver an excellent scientific meeting, NCL2018.

2. Methodological approach

Delivering D9.12 required the early setting up of a local logistics planning who then took responsibility for all aspects before, during and after the event itself.

3. Summary of activities and research findings

NCL2018 took place 12-16 September 2018, Royal Holloway University of London, Egham, London UK

Scientific Organising Committee (SOC):

Sara Mole	University College London, London, UK, Chair (BATCure)
Susan Cotman	Harvard Medical School, Boston, USA
Jonathan Cooper	Harbor UCLA Medical Center, Los Angeles, USA (BATCure)
Angela Schultz	University Medical Center Hamburg-Eppendorf, Hamburg, Germany (BATCure)
Alexander Smith	University College London, London, UK (BATCure)
Heather Band	Batten Disease Family Association, Farnborough, UK (BATCure)
Ruth Williams	Guy's and St Thomas' Hospital, London, UK
Emyr Lloyd-Evans	Cardiff University, Cardiff, UK (BATCure)
Jill Weimer	Sanford Research, Sioux Falls, USA
Juan Bolanos	University of Salamanca, Salamanca, Spain (BATCure)
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 (BATCure)
Dr Claire Russell	Royal Veterinary College, London, UK (BATCure)
Heather Band	Batten Disease Family Association, Farnborough, UK (BATCure)
Dr Ruth Williams	Guy's and St Thomas' Hospital, London, UK
Dr Alexander Smith	University College London, London, UK (BATCure)
Dr Ahad Rahim	University College London, London, UK (BATCure)
Dr Brenda Williams	King's College London, London, UK

Administrative support:

Bioscientifica	Bristol, UK

Programme

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 *sli.do* for interactive questions and polls to widen engagement, as well as allowing anyone to submit a question for the speaker during a talk.

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 10 minutes. 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 (BATCure), Christoph Schwering, Miriam Nickel

2 UCL Batten disease Centre: Wendy Heywood, Elisa Tinelli (BATCure)

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 (BATCure), Claire Russell (BATCure), Tom Wishart

8 Cellular models: Gemma Gomez-Giro, Favio Pesaola, Stephane Lefrancois

9 UCL Gene Therapy Group: Sophia kleine Holthaus (BATCure), Ahad Rahim (BATCure), Sander Smith (BATCure), Mikel Aristorena

10 University North Carolina Gene Therapy Group: Steven Grey, Alejandra Rozenberg, Elena Batracova

11 More than the brain: Jon Cooper (BATCure), Hemanth Nelvagal

12 Drug Therapies: Rafael Badell Grau, Katie Shipley (BATCure), Paul Trippier, Ritva Tikkanen, Helen Waller-Evans (BATCure)

13 BATCure Consortium: Emyr Lloyd-Evans (BATCure), Juan Bolanos (BATCure), Diego Medina (BATCure)

14 BATCure Patient Organisation Involvement: Heather Band (BATCure), Evghenia Scripnic v

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

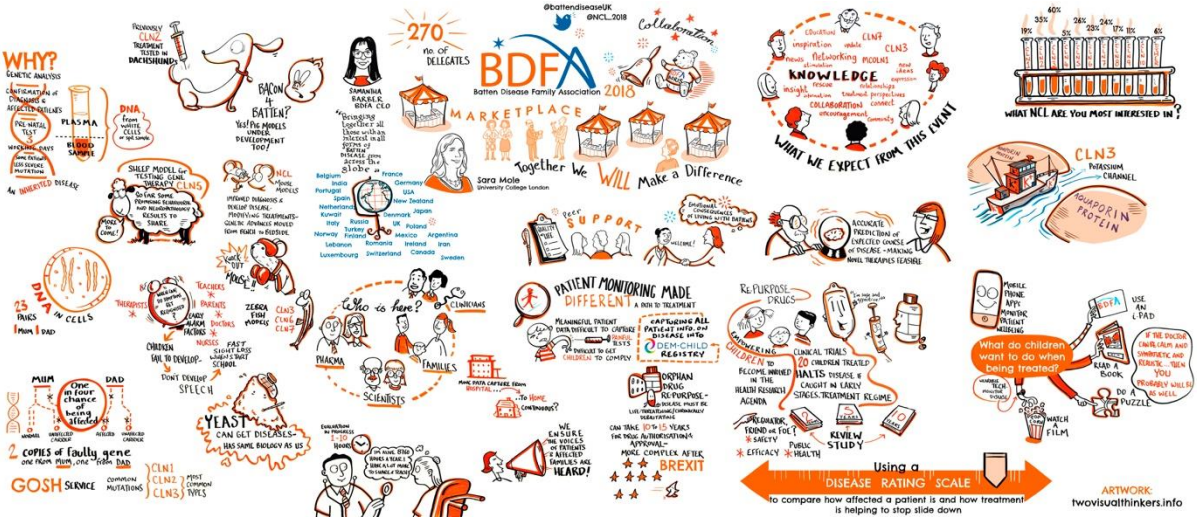
20 Laboratory diagnosis: Ines Hoher de Halac, Marina Trivisano

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.



Speakers

Forty-six oral presentations in 5 scientific sessions:

Genetics and Biology (Chairs: Marco Sardiello, Thomas Wishart)
Nicole Miller, Emyr Lloyd-Evans (BATCure), Alberto di Ronza

'Omics approaches (Chairs: Marco Sardiello, Thomas Wishart)
Stephano Doccini, Monther Abu-Remaileh, Wendy Heywood, Rachel Kline, Hannah Best, Stephan Storch (BATCure), Torbjörn Lundstedt (BATCure)

Disease Models and Mechanisms (Chairs: Jon Cooper (BATCure)*, Jill Weimer)*
Rebecca Ahrens-Nicklas, Meaghan McLaren, Jonathan Cooper (BATCure), Katie Shipley (BATCure), Rafaella Magnoni, Madeleine Klein, Emyr Lloyd-Evans (BATCure), Christopher Minnis (BATCure), Kevin Koster, Juan Bolanos (BATCure)

Translational Research – Preclinical (Chairs: Adriana Cismondi, Alexander Smith, Steve Gray, Stephanie Hughes)
Marco Sardiello, Elena Batrakova, Luis Tecedor, Nadia Mitchell, Alejandra Rozenberg, André Marques, Tyler Johnson, Shibi Likhite, Kathrin Meyer, Marco Peviani, Stephanie Hughes, Mikel Aristorena, Saul Herranz-Martin (BATCure), Sophia kleine Holthaus (BATCure)

Translational Research – Clinical (Chairs: Alessandro Simonati, Miriam Nickel, Jonathan Mink, Ruth Williams)
Heather Adams, Tufikameni Brima, Angela Schultz v, Charlotte Camp (BATCure), Christoph Schwering, Simon Dutz, Jonathan Mink, Eva Wibbeler, Emily de los Reyes, Kristin Page

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) (BATCure), *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.*

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.

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

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 sponsored attendees (covering registration costs).

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)

Travel awards

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

Sponsorship

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

Comments of at least 3 attendants about the Meeting

Many comments were overwhelmingly positive, offering the opinion that it was one of the best NCL meetings ever.

Prof Alfried Kohlschutter, keynote speaker, by email: *"'looking back' on earlier meetings of this kind, I have never experienced so much and so valuable exchange. Once again thanks for the invitation and congratulations to you and your team."*

Prof Alessandro Simonati, delegate, by email: *"I wish to thank you once more for the extraordinary organisation of NCL2018, which will remain in our memory as a pleasant mix of science and entertainment."*

One family attendee commented, *'Thank you all for putting it together- it was a brilliant event*'.

Sli.do recorded 78 responses to the question 'what is the main insight you are taking away from NCL2018?', presented as a word cloud.

4. Conclusions and future steps

NCL2018 was a resounding success, featuring scientific presentations from many BATCure partners, taking public engagement to a new level, and raising expectations for the standard and content of future international meetings.

Web site: http://www.ncllondon2018.com/