



BATTEN DISEASE FAMILY ASSOCIATION

*Together we WILL make a difference*

*Registered Charity No. 1084908*



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Lab Open day  
MMU  
30<sup>th</sup> March 2017



*This project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement No 666918*



BATTEN DISEASE FAMILY ASSOCIATION

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To ensure that everyone living with a diagnosis of Batten disease has access to the best quality services and support to enable them to live life to the full and to change lives by funding research into potential therapies and ultimately a cure.

- ▶ To support families and the professionals who work with them
- ▶ To raise awareness and advocate for better services and treatments
- ▶ To directly fund research into potential therapies and ultimately a cure





Development of screen using Patient derived CLN5 cells

Gene therapy to treat visual failure in CLN6/3 & CLN2 Include other NCLs

Support and Advocacy for families on BMN190 clinical trial in the UK and worldwide

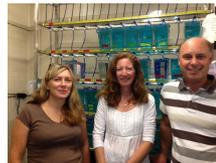
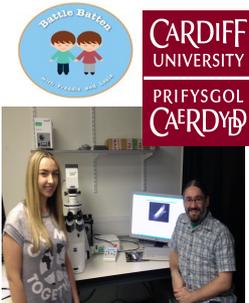


Uncovering fundamental differences in cell biology in CLN5

DanioVision Locomotion detection system Zebrafish studies

Assessing the efficacy of gene therapy upon neuropathology in CLN5 sheep

NCL International Registry



**BATCure** What is it? → A **3** YEAR RESEARCH PROJECT FUNDED BY THE **E.U.**s "New Therapies for Rare Diseases"

THE THERAPEUTIC OPTIONS FOR PATIENTS & FAMILIES LIVING WITH **CLN 3, 6** OR **7** BATTEN DISEASE



WE ENSURE THE VOICES OF PATIENTS AND AFFECTED FAMILIES ARE **HEARD!**



**BDFA** PROJECT CO-ORDINATOR **LAURA CODD**

[lauracodd@bdfa-uk.org.uk](mailto:lauracodd@bdfa-uk.org.uk)  
[www.bdfa-uk.org.uk](http://www.bdfa-uk.org.uk)



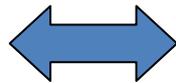
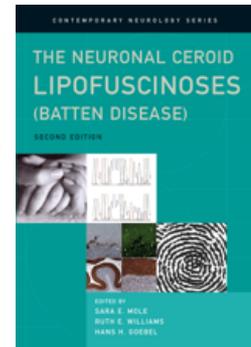
**BDFA** PROJECT CO-ORDINATOR **PROF. SARA E. MOLE**

WHO IS INVOLVED?  
A CONSORTIUM FROM **7** E.U. COUNTRIES, **10** SCIENTIFIC RESEARCH GROUPS, **3** COMPANIES AND THE **BDFA**



The BATCure project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement No. 444018

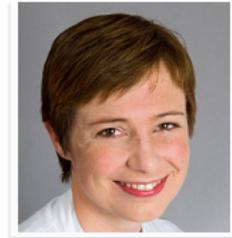
- Interactive Website and Social Media, lab open days
- Lay information and translation of resources



Summer 2017  
Feedback to consortium members

- Develop a methodology to enable all patients and patient groups to engage and participate in study

# NCL International Disease Registry



## B:OMARIN



**Norway**  
Ingrid Helland, MD  
Oslo University Hospital



**USA**  
Ron Crystal, MD PhD  
Weill Cornell Medical College



**Germany**  
Angela Schulz, MD , Co-ordinator  
University of Hamburg



**Denmark**  
Jon R. Ostergaard, MD  
Aarhus University Hospital



**Argentina**  
Ines Noher de Halac, MD  
Universidad Nacional de Cordoba



**Italy**  
Alessandro Simonati MD  
University of Verona



**France**  
Catherine Caillaud MD PhD  
INSERM, Paris



**Brazil**  
Charles Lourenco, MD PhD  
University of São Paulo



**UK**  
Ruth Williams, MD  
GSST, London



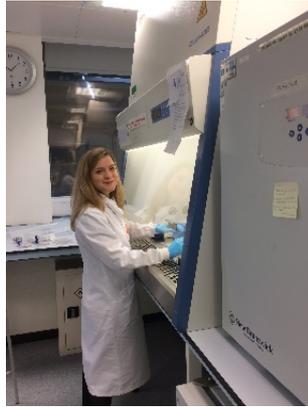
**India**  
Pratibha Singhi, MD  
PGIMER, Chandigarh



**Turkey**  
Meral Topcu, MD PhD  
University Children's Hospital, Ankara



**Finland**  
Laura Aberg  
Folkhälsan, Helsinki



Manchester  
Metropolitan  
University



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*BATCure research consortium by Tristan McKay  
McKay group Batten Disease research by Lorna FitzPatrick  
Question & Answer session  
Lunch  
Tour of the labs with some observation of their work  
Tea, summary and farewell*



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