



Welcome to the second BATCure Newsletter. Our goal is to keep you informed about progress on the BATCure project and to provide an insight into the BATCure-funded research taking place at institutions across the European Union (EU). BATCure is a 3-year research project funded by the European Union's Horizon 2020 Research and Innovation Programme that began in January 2016. The BATCure project consortium is comprised of fourteen institutions across the EU. These consist of ten leading scientific research groups, three companies and the Batten Disease Family Association (BDFA) who is leading part of the project and ensuring that the voice of patients and affected families is heard. Whilst BATCure focuses on the development of new therapies for CLN3, CLN6 and CLN7 diseases, it is hoped that the research results will ultimately be of benefit to patients with all forms of Batten disease.

BATCure so far!

In August, the BATCure 18-month report was successfully submitted to the EU, and we are pleased to report that the project is in good shape as we reach the mid-way point. Good progress has been made generating new models, tools and technologies in preparation for developing and testing emerging therapies.

The patient voice has also been represented with the BATCure Family Survey available online in 10 European languages from July to September 2017. The survey explored the perspective of EU families affected by all forms of Batten disease, gathering their thoughts about research and clinical trials to help develop and plan for future treatments. A survey update can be found on the back of this newsletter.

We asked Sara Mole, Professor in Molecular Cell Biology at University College London and overall Coordinator for BATCure, to share her thoughts on project progress so far...

"The first 18 months of BATCure has been a great success. We have created new disease models for CLN3, CLN6 & CLN7 diseases and these are being used to identify new compound leads. We have learnt a great deal more about disease at a cellular level as well as in patients. In particular, using a gene therapy approach we have successfully prevented the loss of photoreceptor cells in the CLN6 disease mouse model, a necessary step to developing treatment to prevent loss of sight in Batten disease. With the BDFA and many other Patient Groups across Europe, we have enabled patients, families and the wider public to engage with BATCure. The consortium members work very hard to support each other – they are a talented and dedicated group of researchers."

Professor Sara Mole, BATCure Coordinator



Batten Disease Awareness Day – Friday 9th June 2017

The BATCure consortium pulled together on Batten Disease Awareness Day to help raise awareness of the disease across Europe. They took an active role in our social media campaign, generating a buzz by 'Passing the baton' for Batten disease.



Dr Kate Bennett and the AcureOmics team passing the baton in Umeå, Sweden.



Universitätsklinikum
Hamburg-Eppendorf



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Spotlights on...

In each edition of the newsletter, we shine a spotlight on some of the institutions and the individuals involved in BATCure.

Evghenia Scripnic, University College London

Evghenia is the European Project Manager at the UCL European Research and Innovation office where she is responsible for monitoring and supporting the work of institutions who are part of BATCure. She joined the BATCure project in April 2017 and in collaboration with the Project Coordinator, she monitors project progress against the plan and liaises with the dedicated EU Project Officer in Brussels.

"I truly enjoy working with the top researchers from all around Europe who are devoted to developing treatments for the Batten disease. My role within the project is to provide financial, contractual and administrative support and to ensure the timely delivery of all the results. So far, significant progress has been achieved in various Work Packages and I look forward to continue accompanying the consortium in their research work."



Evghenia Scripnic

Dr Ahad A. Rahim, University College London

Dr Rahim is a Lecturer in Translational Neuroscience at School of Pharmacy. His group at University College London (UCL) focus on conducting pre-clinical gene therapy studies in mouse models of paediatric neurological conditions. This includes CLN3, CLN6 and CLN7 Batten disease as part of the BATCure project. Dr Rahim is the BATCure Work Package 1 co-ordinator and works on gene therapy for the brain in CLN3, CLN6 and CLN7 disease as part of Work Package 7.

"Gene therapy is a very multidisciplinary field of research and testing it for these three forms of Batten disease is challenging. They involve different defective genes, have their own distinct mouse model and a slightly different type of gene therapy is required for each one. However, UCL has a great track record in gene therapy and together with Dr Saul Herranz Martin in my lab and our collaborators on BATCure we are making progress. Being part of the BATCure project provides a great research opportunity."



Dr Ahad A. Rahim

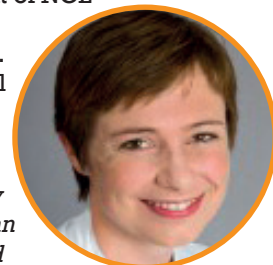
The Universitätsklinikum Hamburg-Eppendorf (UKE)

UKE is a clinical centre with long-standing expertise in the diagnosis and management of neuronal ceroid lipofuscinosis (NCL). The institution is an internationally acknowledged clinical reference and diagnostic centre for all NCL diseases and has a specialised NCL clinic.



Dr Angela Schulz is a paediatrician and has run the NCL clinic at the UKE Children's Hospital for more than 10 years. Dr Schulz coordinates the recruitment of NCL patients, collection of patient data for natural history studies for the project. She also coordinates the international DEM-CHILD NCL patient database.

"Collecting patient data is vital for clinical trials but is important for many other reasons – current patient data can identify early indicators of disease and help improve diagnosis."



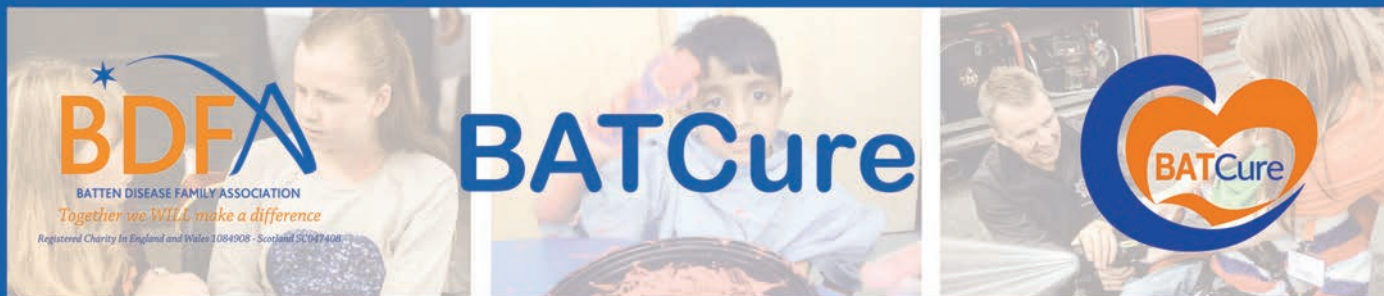
Dr Angela Schulz

Professor Thomas Braulke is Head of Lysosome/NCL Research Group at the Children's Hospital and has a long-standing expertise in the field of lysosomal storage disorders including the NCLs. In collaboration with **Dr Georgia Makrypidi**, they will guide the work on generating the required experimental tools to further study what is happening in the cell for BATCure.

Dr Stephan Storch coordinates the work on disease biology and CLN7 gene function using their CLN7 mouse model. Dr Storch and the team are also providing vital resources for other consortium members for the next phase of the project. The experiments are contributing to understanding the function of CLN7. Dr Storch works closely with Professor Lundstedt at AcureOmics in Sweden to see if where CLN7 acts in the cell can be exploited to find potential new therapies.



Dr Stephan Storch



BATCure Family Survey 2017

Thank you!

The survey is now completed.

The BATCure team would like to thank all the families who took the time to complete it and to have their voice heard in the BATCure research.

We would also like to thank the BDFA's partner Patient Organisations and contacts across the EU.

With their help, we achieved a total of 142 surveys from families in 15 different European countries.

What about the results?

The first survey results will be shared at the BDFA Family Conference in Stratford-Upon-Avon (24th - 26th November 2017). A written summary of survey results will be available in the next BATCure newsletter (early 2018).

Need further information?

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To find out more about BATCure

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