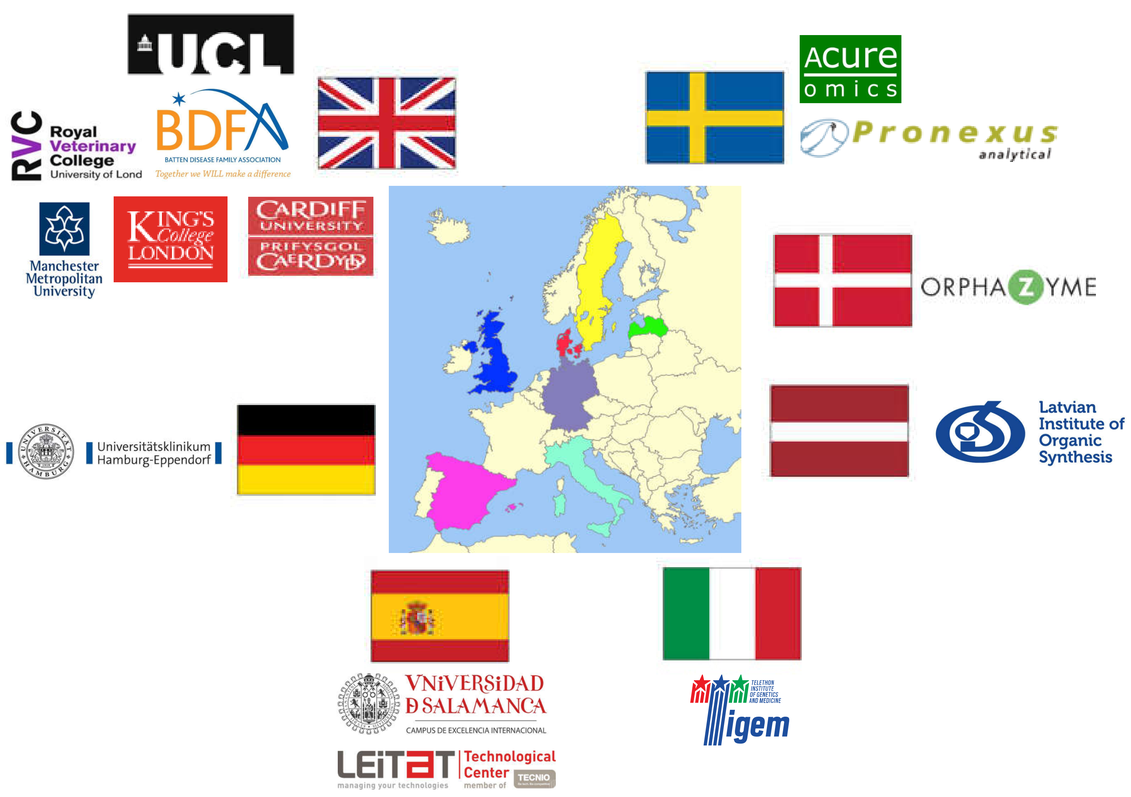
Developing new therapies for neuronal ceroid lipofuscinoses or Batten disease

Maija Dambrova[1,2*] and Sara E Mole[3] on behalf of the BATCure Consortium
[1]Latvian Institute of Organic Synthesis, Latvia; [2]Riga Stradins University, Latvia; [3]University College London, UK

PARTNERS



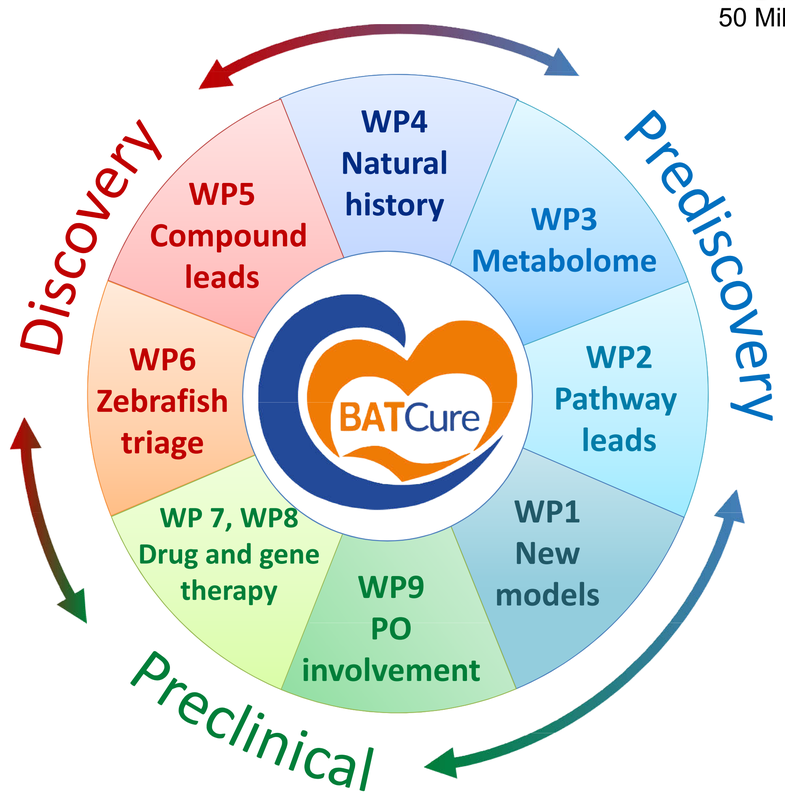
PROJECT DESIGN

8 experimental WPs:

NEW MODELS	Development, validation and optimisation of essential new models & tools
PATHWAY LEADS	Identifying therapeutic target pathways using genetics & biology
METABOLOME	Identifying therapeutic target pathways and developing new diagnostic & monitoring techniques using metabonomics
NATURAL HISTORY	Natural history of the brain and beyond
COMPOUND LEADS	Identifying new therapeutic compound leads
ZEBRAFISH TRIAGE	Determination of therapeutic potential using zebrafish
GENE THERAPY	Gene therapy for the brain, eye and other organs
DRUG THERAPY	Delivering new small molecule therapy to the mouse

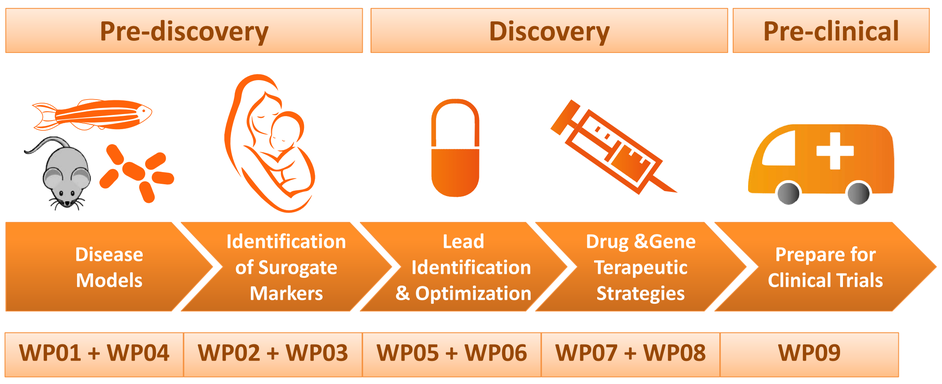
3 facilitating or supporting WPs:
Patients' Organisation involvement
Management
Ethics

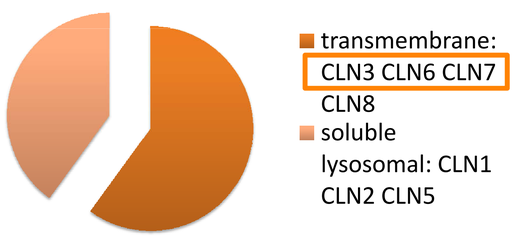
4-5 Tasks/WP
90 Deliverables
50 Milestones

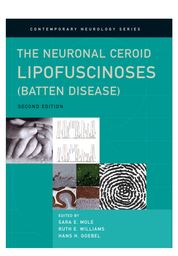


CONCEPT

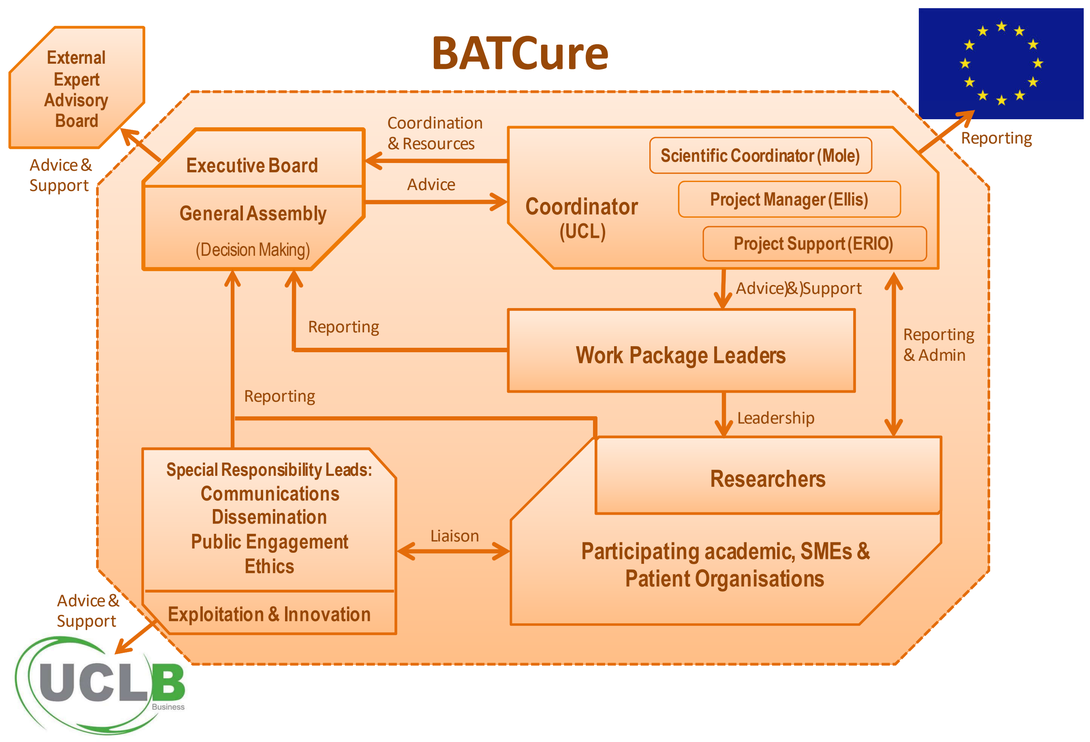
BATCure will investigate the natural history of Batten disease, elucidate the function of key proteins and determine disease mechanisms, as well as develop new therapies for three forms of the disease. Batten disease is one of approximately 50 lysosomal storage disorders, in which genetic mutations disrupt the cells ability to recycle waste. Children and young adults with Batten disease suffer progressive neurological impairment, which includes: seizures, visual impairment or blindness, personality and behaviour changes, dementia, loss of motor skills and loss of the ability to walk, talk and communicate. There is currently no treatment. This consortium brings together ten leading scientific research groups, three companies and one patient organization from across Europe, with half applying their expertise and skills to Batten disease for the first time.







ORGANISATIONAL STRUCTURE



H2020-PHC-14-2015: New therapies for rare diseases

Project: 666918
Duration: 36 months
Start date: 1/1/2016
Award: €5 995 769

Web site: www.batcure.eu
Twitter: @BAT_Cure
Facebook: BATCure

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