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- Research and Innovation Action -

D9.3
Research key target groups required for clinical trial development

WP 9 – PATIENT ORGANISATION INVOLVEMENT

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Lead beneficiary for this deliverable: BDFA

Last editor: Heather Band

Contributors: Heather Band, Laura Codd, Andrea West

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
01	26/10/2016	Heather Band	
02	03/11/2016	Sara Mole	

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Definitions and acronyms

BDFA	Batten Disease Family Association
NCL	Neuronal Ceroid Lipofuscinoses
EMA	European Medicines Authority
FDA	US Food and Drug Administration

1. Introduction

Deliverable 9.3, entitled “Research key target groups required for clinical trial development”, falls within WP9 “Patient Organisation involvement”, for which the Batten Disease Family Association is the lead beneficiary.

The BATCure consortium are working on three forms of a complex and challenging disease, NCL. Whilst one of the key aims of the project is to provide an integrated approach, the potential treatments may be diverse in nature and require different development paths to clinical application. For this reason, it is of utmost importance to set up an efficient and workable system for patient participation prior to any clinical work.

As established in Task 9.3, we have identified key target groups for clinical trial development, which will be disseminated within the consortium and to the stakeholders. For clinical trials in rare diseases where the number of affected and eligible patients can be expected to be small, it is particularly important for their success that all those who could potentially benefit are identified early in the process.

1.1 General Context

For any potential treatment or therapy to be translated effectively and as quickly as possible, the ability of the consortium to respond rapidly is essential. Prior mapping and research of key stakeholders, early interaction will be essential for success.

1.2 Deliverable objectives

Following Task 9.3 of the BATCure Description of the Action, part A, this deliverable aims:

- To research and map key stakeholders, set useful parameters for data collection and accurately record data.
- To provide comprehensive information on key stakeholders that can be easily provided by the BDFA to the consortium and any other future interested parties.
- To ensure that the “Patient Voice’ is central to any process ensuring that those who may benefit from such research are identified early in the process.

2. Methodological approach

A central aim of the method is that this is not a static activity. The research must be ongoing for the duration of the project, and the system for retrieving relevant information responsive and adaptable.

The BDFA already utilises a database[1], with informed consent, and details of relevant organisations, from the categories identified in Section 3, have been added. New fields have been designed to reflect the potential involvement of key stakeholders and for searches to be performed by the BDFA and/or at the request of consortium members.

The BDFA has 18 years experience in this field. The BDFA has an active role, including mapping key stakeholders, for the BMN190 trial and is currently working with the Pharmaceutical company 'BioMarin' on all aspects needed to progress the treatment to a licensed therapy for CLN2 disease in the UK and Europe.

Many beneficiaries of the BATCure consortium have previously successfully translated research to clinical application and we will make use of this expertise to inform and achieve our end goals of bringing a new therapy to clinic (e.g. applications for Orphan drug status).

The BDFA keeps all patient and family contacts confidential and will maintain this throughout the project as specified by the BDFA data protection policy. The BDFA is able to make patients and families aware of the latest progress of the research.

3. Summary of activities and research findings

Key groups have been identified as:

- Patients and families, Europe and worldwide
- NCL Patient Organisations, Europe and worldwide
- Clinicians, Europe and worldwide
- Regulatory Authorities (e.g. EMA, FDA)
- BATCure Consortium
- Researchers
- Pharmaceutical industry
- Research foundations and other charities, Europe and worldwide
- Healthcare, social care and education providers

Using this experience, relevant information has been collated and is in the process of being checked and added to the BDFA database.

Further web-based research has been undertaken and will be added in the next two months.

4. Conclusions and future steps

The research and use of the BDFA database provides a suitable working platform. Three monthly reviews will be undertaken by the BDFA with input from all consortium members.

A six monthly report will be produced for the consortium and feedback invited allowing the long-term effectiveness of the database will be assessed and fields updated if required.

A more defined mapping process will be undertaken as the specific need arises, depending on the direction of research results (e.g. for a Gene Therapy treatment, for drug development).

5. Publications resulting from the work described

N/A

6. Bibliographical references

[1] Donor Strategy, Web integrated Customer Management (CRM) operated by Advanced NFP, Registered in England and Wales: 04023140