EBC Research Project

The Value Of Treatment for brain disorders in Europe

Value of Treatment research (VoT2)

Case studies on Rare Neurological Disorders (RNDs): Ataxia, Dystonia, Phenylketonuria & Mental Disorders (MDs): Autism Spectrum Disorder, Eating Disorder, Major Depressive Disorder

PROJECT OVERVIEW

The Value of Treatment for brain disorders (VOT) is a health economics and outcomes research project addressing all brain disorders and coordinated by the European Brain Council (EBC). Brain disorders - both neurological and mental alike - constitute a major factor, alongside cancers and cardiovascular diseases, driving the overall burden of diseases in Europe. Neurological and psychiatric disorders are the leading cause of poor health and disability in Europe. But this is not considered. The conclusions of the VOT research project first round released in 2017 (which included case studies on disorders ranging from schizophrenia to Alzheimer's disease, epilepsy, headache, normal pressure hydrocephalus, Parkinson's disease, multiple sclerosis, restless legs syndrome and stroke) highlighted the need for more research, early, if possible prodromal, diagnosis and intervention, integrated seamless care underpinning timely care pathways as a solution to address value-based health care in Europe and access to the best treatments available. In the continuity of these conclusions and in the framework of the VOT research, new case studies (VOT2) have been developed on new therapeutic areas focusing on rare neurological diseases [Ataxia, Dystonia and Phenylketonuria] and mental disorders [Anorexia Nervosa, Autism Spectrum Disorder, Major Depressive Disorder] launched respectively in 2018 and in 2019.

OBJECTIVE

The Value of Treatment (VoT) project aims to to examine health gains and socio-economic impacts resulting from optimized healthcare interventions in comparison with current care or no treatment, and converge data evidence to policy recommendations on how to improve the care pathway(s).

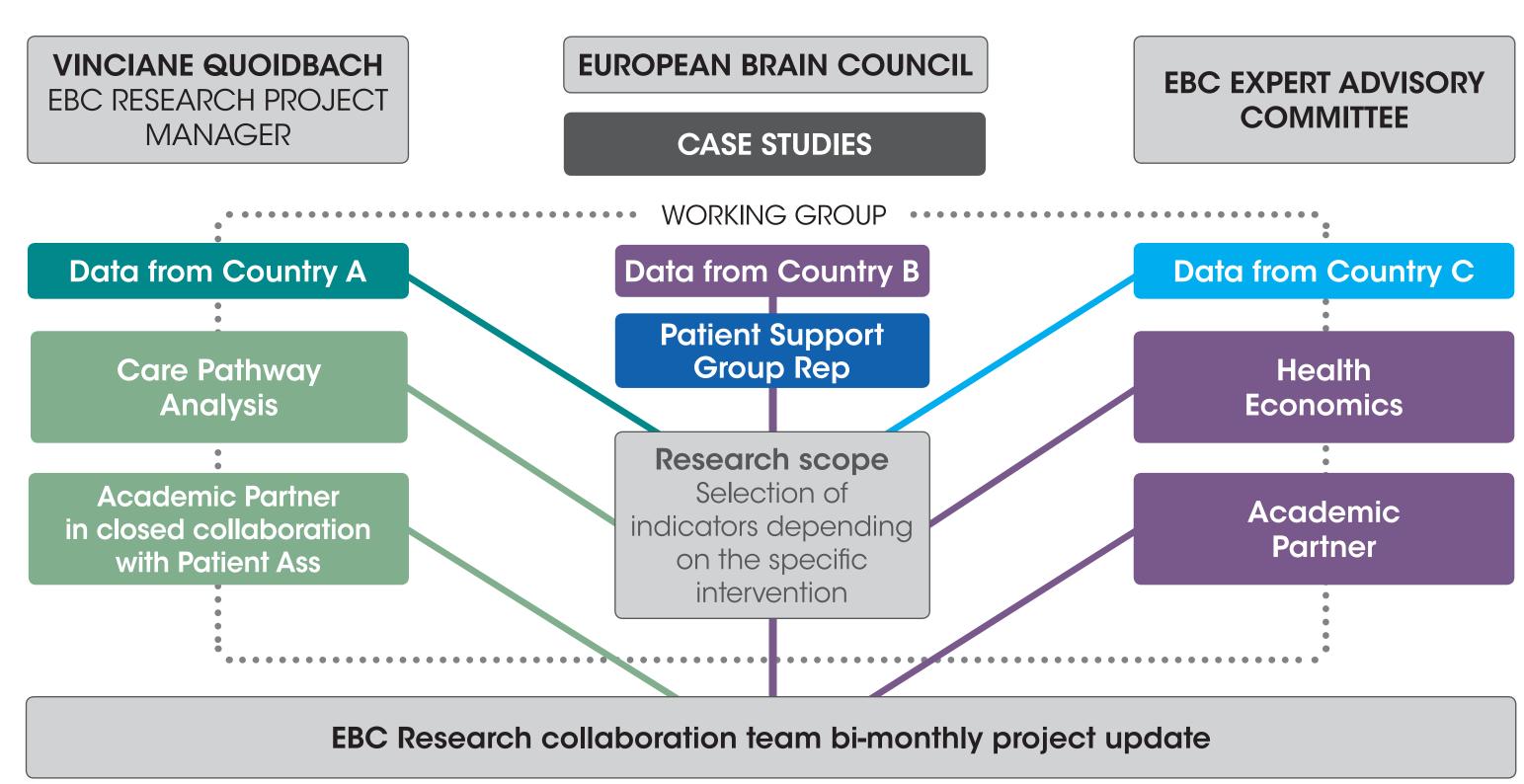
OUTCOMES

The study on Ataxia together with Dystonia and Phenylketonuria will provide insight on the value of specialist centres in terms of diagnosis, management of patients with rare conditions and its cost implications in several European countries. For mental disorders, lack of seamless care between multiple services and between patients and services is commonly seen as a major problem for people affected by mental disorders. The study's research framework will include the testing of a comprehensive care model and the development of a series of qualitative and quantitative benchmarks to identify treatment gaps and causal factors along the continuum of care in a patient care pathway analysis. The study also will estimate the socioeconomic impact and health gains from optimized health-care interventions with an economic evaluation. Case studies will be analysed in collaboration with experts from the European Brain Council's scientific societies in line with the research framework, applying empirical evidence from different European countries.

Results of the research are to be released by end 2021. Findings will be disseminated via newsletters, scientific publications and an overarching policy paper.

Accessible online: VoT2 - rare neurological disorders article (CMJ 2019) on "Toward earlier diagnosis and treatment of rare neurological disorders: the value of coordinated care and specialist centers". Link to PubMed: https://www.ncbi.nlm.nih.gov/m/pubmed/31044588/?i=6&from=croat%20med%20j

PROPOSED STRUCTURE



What is crucial is to harmonize datasets for the 3 case studies on RNDs based on a standardized approach, same for MDs.

² Bulletin of the World Health Organization 2018;96:298-298A. doi: http://dx.doi.org/10.2471/BLT.17.206599





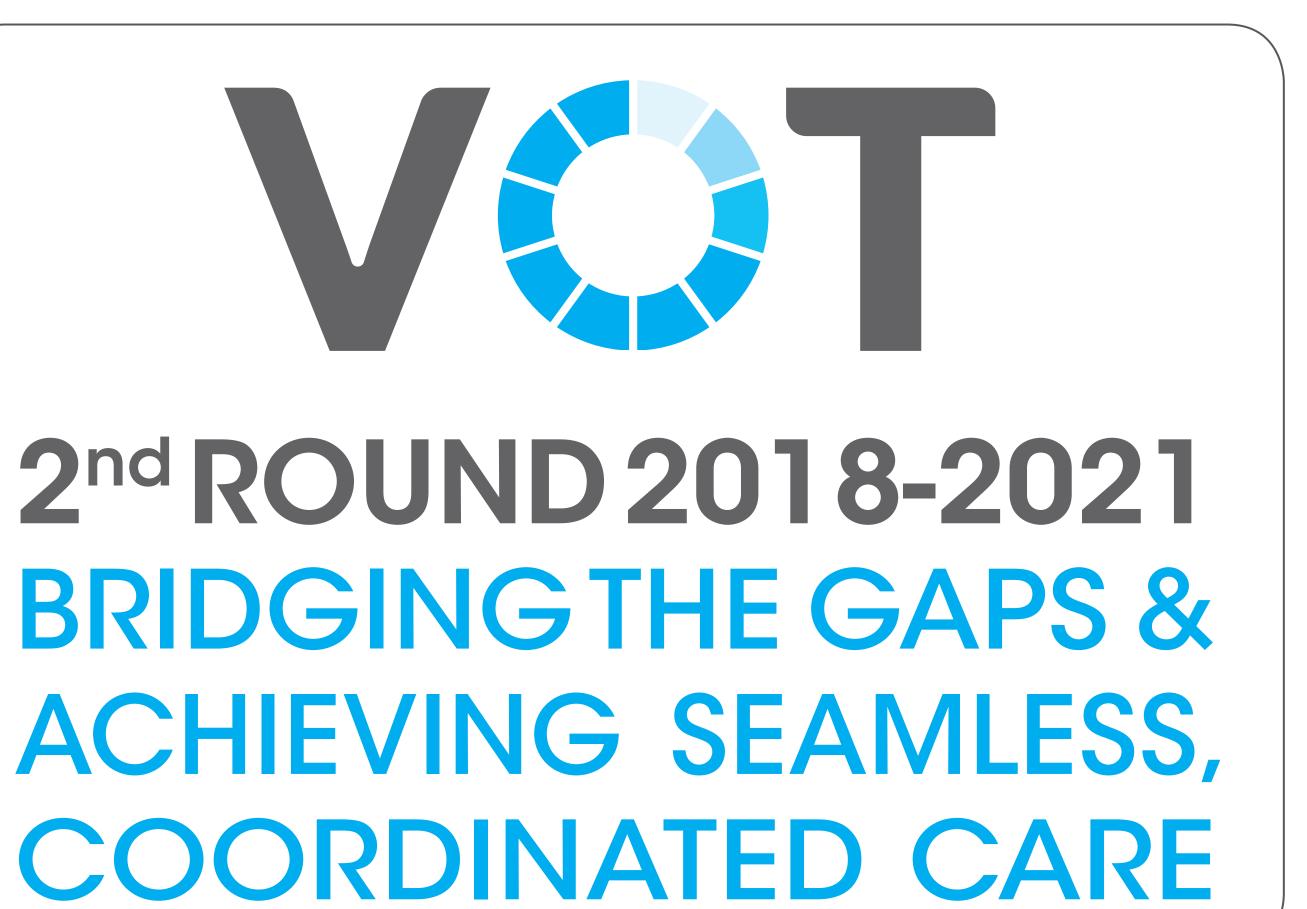












VOT2 - PROJECT RESEARCH COLLABORATION

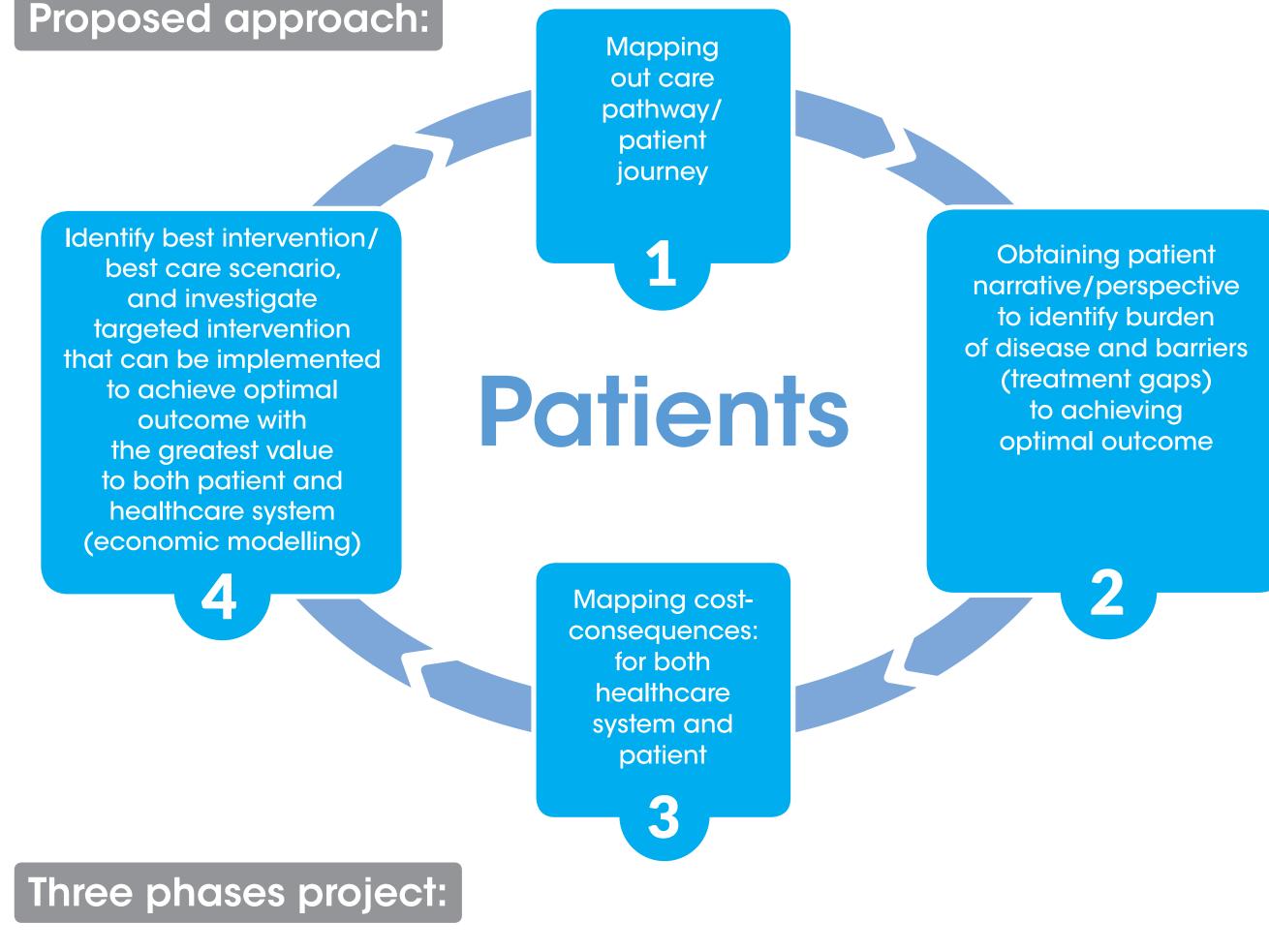
Study includes: Academic partners, clinicians, health economists, epidemiologists with the participation of patient associations (*).

Research collaboration with academic institutions for RNDs case studies:

- University College London Hospitals: Ataxia care pathway analysis
- University of Cambridge: Ataxia AND Phenylketonuria health economic studies
- University of Zagreb Medical School: **Dystonia care pathway analysis AND** economic evaluation
- University College Dublin: Phenylketonuria care pathway analysis

Research collaboration with academic institutions for MDs case studies:

- King's College London and London School of Economics: Anorexia Nervosa care pathway analysis AND health economic studies
- King's College London and Greenwich College London: Major Depressive Disorder care pathway analysis AND health economic studies
- London School of Economics: Autism Spectrum Disorder care pathway analysis AND health economic studies



CASE STUDY ANALYSIS CONCEPTUALIZATION Phase 2 (15 months) Phase 1 (3 months) «Scoping and kick-off»

«Development, qualitative and quantitative research» Method:

Care pathway analysis

POLICY RECOMMENDATIONS Phase 3 (6 months) «Final results and **publications**» Method: Consultation to formulate final results: converging case studies data analysis

case study completed with (treatment gaps/patient defined study protocol, unmet needs mapping) WG (expertise diversity), Economic evaluation country settings (>3) incl. (from issues to cost agreed sponsorship and effective solutions for the validated by EBC Board benefits of the patient)

study publication **<u>Timeline:</u>** all case studies and relevant reports to be **completed by end 2021** (rare neurological disorders) and by June 2021 (mental disorders). Publications

into evidence-based

(Policy White paper)

policy recommendations

Proposed template for case

Milestones for 2020 and 2021:

Method:

Proposed template per

will be released in 2021.

VoT2 - MDs: Final case study deliverables	Phase 3 «Final results and publications» June 2021 - Dec 2021 (project last 7 months)						
MONTH	Jun	Jul	Aug	Sep	Oct	Noc	Dec
A1. Results data collection, analysis and interpretations via literature review, landscape assessment and survey + Economic evaluation based on care pathways analysis: CASE STUDY POSTER FOR EBC FINAL PAPER	End						
A2. EBC FINAL PAPER with Part1 and Part 2 (posters)							End
A3. Case study full article: JOINT SCIENTIFIC PUBLICATION							End

- VOT2 Joint Meeting (18 May 2020)
- Last VOT2 Joint Meeting (June 2021) date tbc
- A1: final by WGs (June 2021)
- A2 Part 1: draft for consultation by EBC & Health economist experts (June 2021)
- A2 Part 1, Part 2 by WGs and A3 by WGs: final (December 2021)

(*) The European Brain Council (EBC) is a network of key players in the "Brain Area", with a membership encompassing scientific societies, patient organisations, professional societies and industry partners.

¹ The value of treatment policy white paper: towards optimizing research and care for brain disorders.

Brussels: European Brain Council; 2017. Available at: https://www.braincouncil.eu/wp-content/uploads/2017/06/EBC_white_policy_paper_DEF26072017_Low.pdf