

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 briefing October 2019

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PROJECT OVERVIEW

The European Brain Council (EBC), an organization promoting research on brain health and disorders in Europe, initiated a European study called the Value of Treatment (VoT) for Brain Disorders. The project (2015-2017) 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. The value of treatment for brain disorders – policy white paper published in June 2017¹ provided important new insights into recent progress in the areas of pharmacology and the biopsychosocial approach, and into health-care service delivery. Conclusions of this first phase of the VoT project highlighted the need for a more seamless management of brain diseases, and led to a second phase of the project "VoT2" (2018-2020) focusing on Rare Neurological Diseases [Ataxia, Dystonia, Phenylketonuria] and on Mental Disorders [Autism Spectrum Disorder, Eating disorder, Major Depressive Disorder]. This new phase initiated by EBC mid-2018 for the case studies on Rare Neurological Disorders is looking at the value of early diagnosis and intervention, with an aim to assess the benefits of coordinated care and multidisciplinary care patterns on patient outcomes. New projects on mental disorders were launched in January (Eating disorder and Major Depressive Disorder) and in May (Autism Spectrum Disorder) this year in the context of this second phase of the project. Following initial discussions to identify treatment needs for people affected by mental disorders, it was decided that the overarching theme of the VoT 2 case studies would be the role of transition, continuity of care, and its impact on health service use and patient outcomes. A VoT2 joint meeting took place on 15 May 2019. Next VoT2 joint meeting (only with Working Group leaders and researchers) will take place on 27 November 2019.

OBJECTIVE

Objective of the Value of Treatment (VoT) project is to examine health gains and socio-economic impacts resulting from best practice healthcare interventions in comparison with current care or no treatment, and to converge evidence to policy.

OUTCOMES

We hope to analyse patient views and assess the cost-effectiveness of specialist centers for managing care of people with ataxia or dystonia in several European countries; and the cost-effectiveness of care coordinators for people with phenylketonuria to reduce drop-outs, encourage the compliance with the Phe-free diet, and improve overall health outcomes (previous scope). The aim of the economic analysis is now to report on the costs and consequences of standard treatment for sufferers of PKU who are unable to comply with a low Phe diet. The objective of the work is to determine the quality of life of patients and costs to families, the health care system and society of standard care (no treatment). Outcome measures to be determined. Our aim is also to examine the role of national policy and programs, including National Rare Diseases Plans on the effective implementation of coordinated comprehensive services directed to ataxia, dystonia, and phenylketonuria. 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 issues associated with continuity are non-disease specific; they apply to all mental health disorders. There are clear benefits from effective maintenance, continuity of care for patients with complex needs, but there is strong evidence that optimal continuity care does not occur in most cases. We hope to assess the patients' needs and to evaluate the costs and consequences of transition, continuity of care for mental disorders in several European countries.

Ultimately the EBC Value of Treatment project aims to influence the policy towards better treatment and care for people with rare neurological diseases and mental disorders across Europe. Results of the research are to be released by end 2020. During 2021, findings will then be disseminated via newsletters, scientific publications and an overarching policy paper.

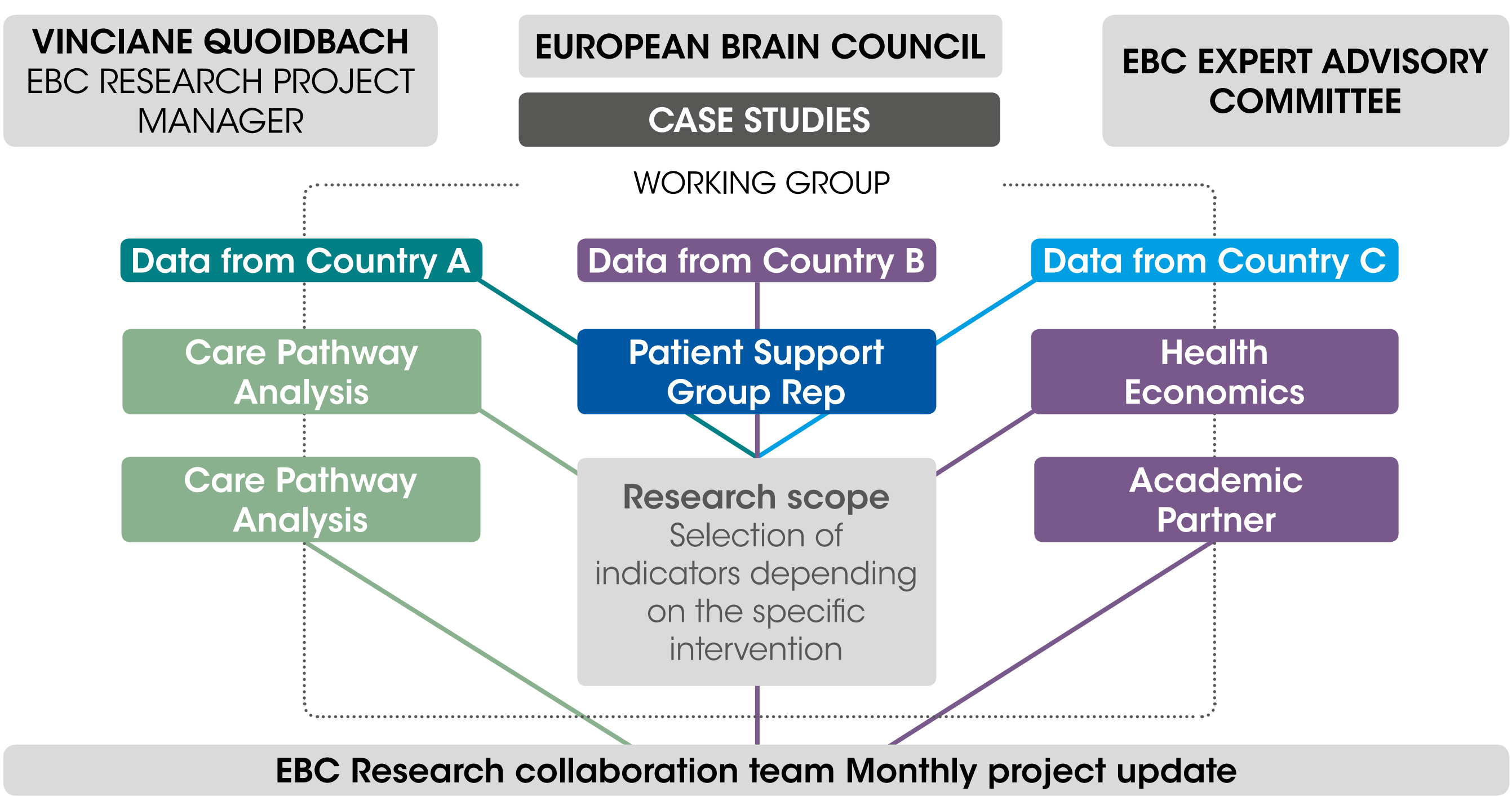
Accessible online: VoT2 – rare neurological disorders article on "Toward earlier diagnosis and treatment of rare neurological disorders: the value of coordinated care and specialist centers and link to Pubmed: www.ncbi.nlm.nih.gov/m/pubmed/31044588/?i=6&from=croat%20med%20j

¹ The value of treatment policy white paper: towards optimizing research and care for brain disorders. Brussels: European Brain Council; 2017. Available at: www.braincouncil.eu/wp-content/uploads/2017/06/EBC_white_policy_paper_DEF26072017_Low.pdf

2nd ROUND 2018-2021

BRIDGING THE GAPS & ACHIEVING SEAMLESS, COORDINATED CARE

PROPOSED STRUCTURE



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

VOT2-PROJECT RESEARCH COLLABORATION

Researchers collaboration for RNDs case studies:

- UCLH: Paola Giunti and Julie Vallortigara – Ataxia care pathways analysis
- University of Cambridge [since June 2019]/UCL [since June 2019]: Steve Morris and Emma Hudson– Ataxia AND PKU health economic studies
- University of Zagreb Medical School: Maja Relja, prof.dr.sc. Vladimir Trkulja Pharmacologist and Health economist prof.dr.sc. Stipe Oreskovic, health economist– Dystonia care pathways analysis AND economic evaluation
- UCD: Gregory Pastores and Thilo Kroll – PKU care pathways analysis

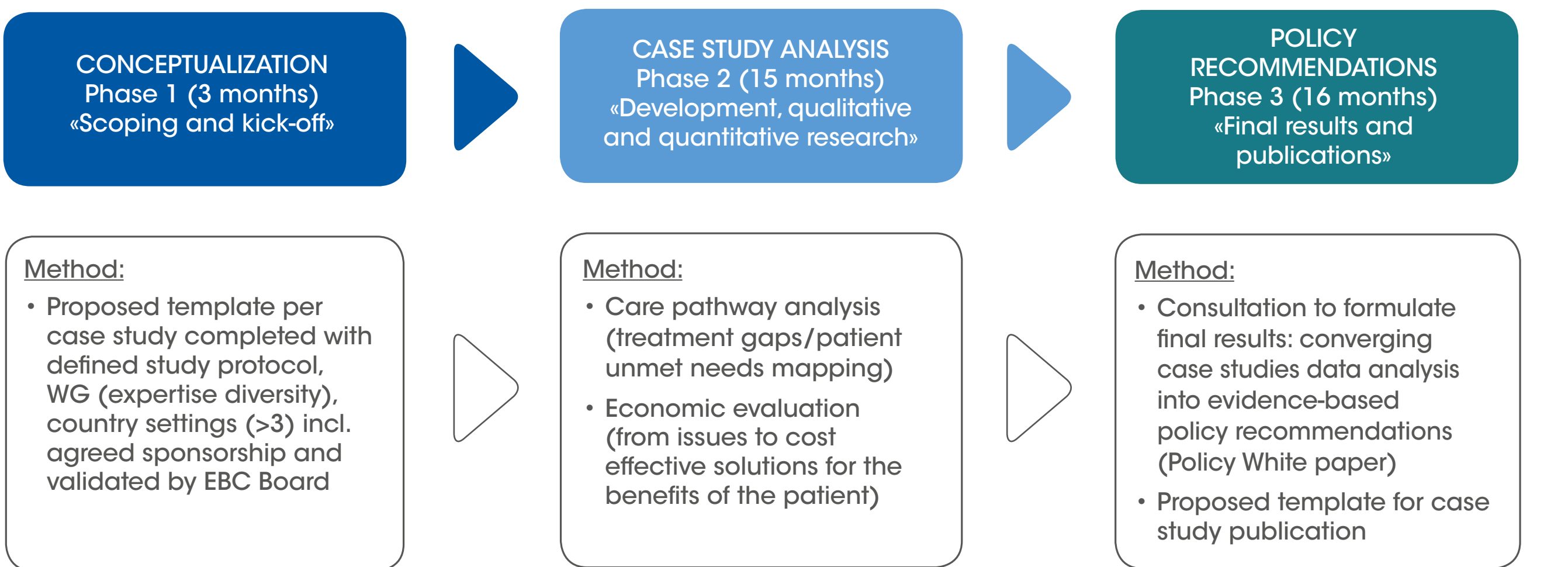
Researchers collaboration for MDs case studies:

- Anorexia Nervosa (ED): LSE (collaboration is being formalized)
- Major Depressive Disorder (MDD): King's College London (collaboration is being formalized)
- Autism Spectrum Disorder (ASD): LSE (collaboration is being formalized)

Proposed approach:

1. Mapping out care pathway/patient journey
2. Obtaining patient narrative/perspective to identify burden of disease and barriers (treatment gaps) to achieving optimal outcome
3. Mapping cost-consequences: for both healthcare system and patient
4. Identify best practice, and investigate targeted intervention that can be implemented to achieve optimal outcome with the greatest value to both patient and healthcare system (economic modelling)

Three phases project:



Milestones for 2019 and 2020:

- 15 May 2019: VoT2 Joint Meeting
- 27 November 2019 (afternoon meeting): VoT2 Joint Researchers Meeting (with researchers and WG leaders only)
- May 2020 (date tbc): VoT2 Joint Meeting
- November 2020 (date tbc): last VoT Joint Researchers Meeting
- December 2020: all analyses completed and written up - reporting and recommendations: Submission to EBC

Note: scientific publications, reporting and policy recommendations will be released during the first semester of 2021.