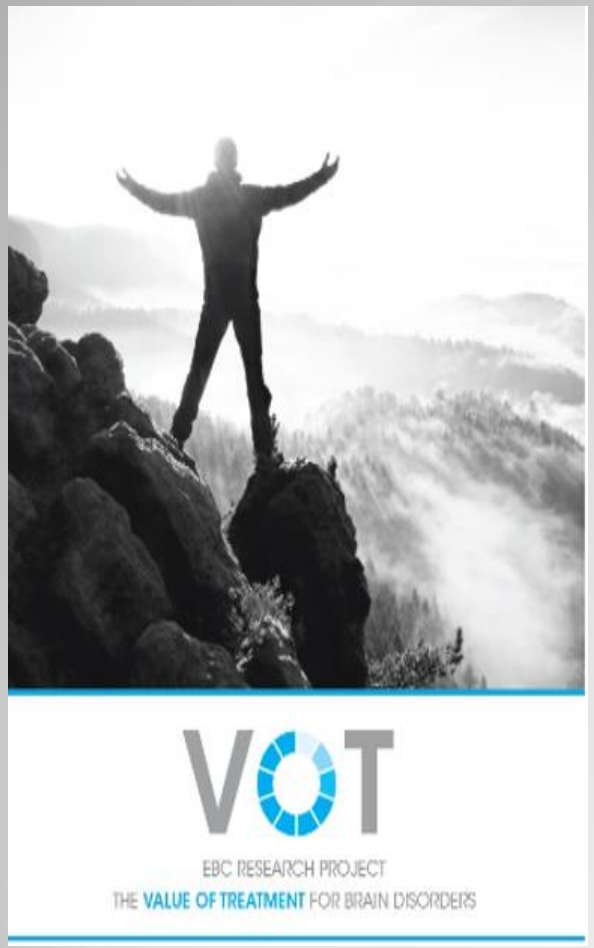
# European Brain Council Value of Treatment project – Ataxia Case study

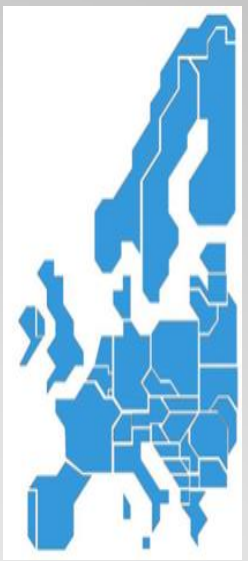
Dr Julie Greenfield
Ataxia UK

Euroataxia meeting 9th November 2018, Frankfurt

# The Value of Treatment for Brain Disorders




2015-2017 Pan-European Study



Value of Treatment for Brain Disorders

| | |
|---|---|
| **CASE STUDIES (9)** | **Mental Health:** Schizophrenia |
| | **Neurology (*)** Alzheimer's disease, Epilepsy, Headaches, Parkinson's disease, Multiple Sclerosis, Restless Legs Syndrome, Stroke |
| | **Neurosurgery/Neurology (*):** Normal Pressure Hydrocephalus |

EBC White Policy Paper released on 22 June 2017

# Rationale: VoT Research Methodology

## Case studies analysis aims to:

Patient care pathway analysis

What are the gaps/ unmet needs?

Identify treatment gaps *and causing factors*

Propose solutions *« best practice healthcare interventions »*

Measure their socio-economic impact *versus standard of care or non treatment*

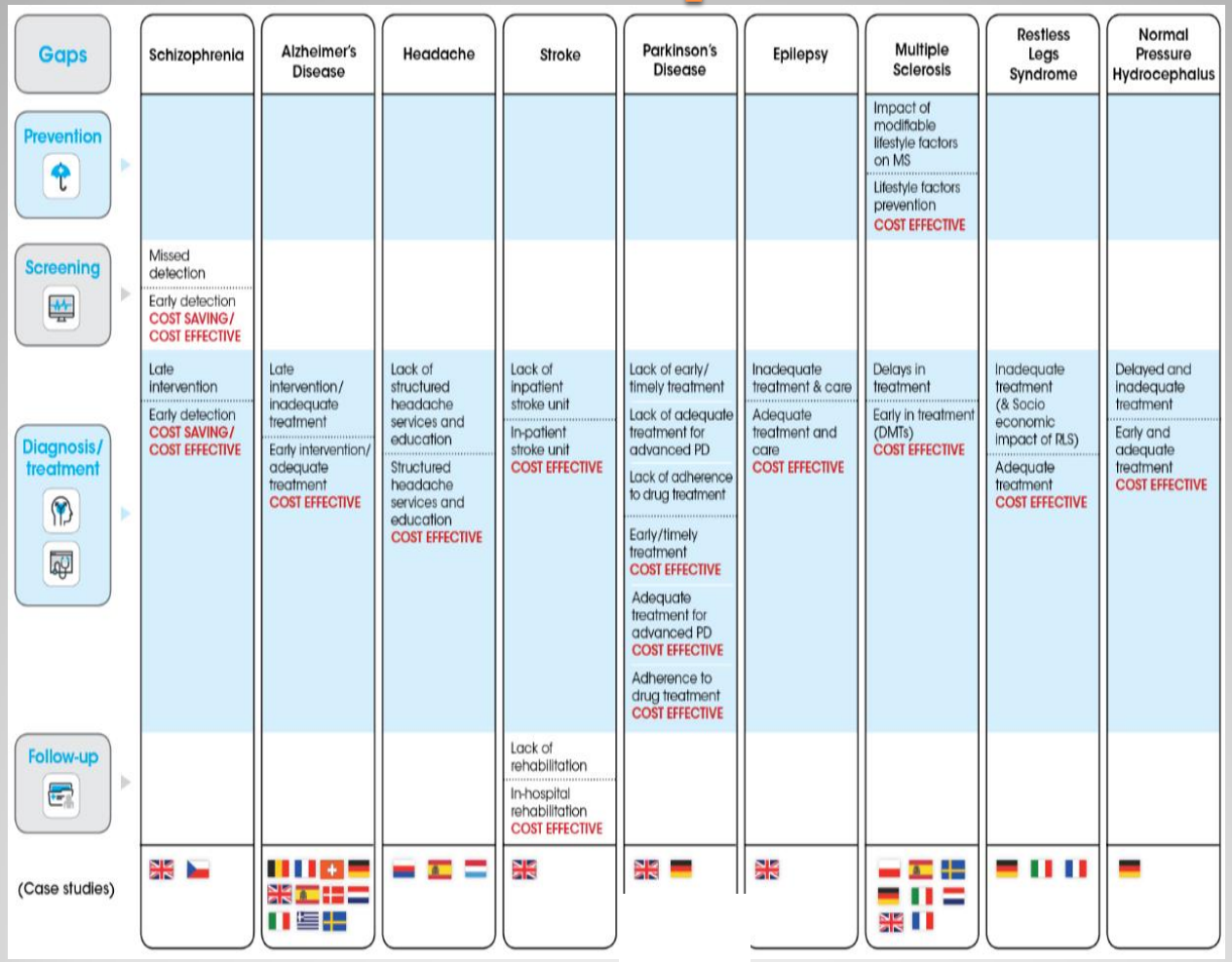
Cost effectiveness analysis

What are the benefits of targeting these gaps?

# Rationale: VoT Research methodology

**Major goal of the Value of Treatment (VoT) project** is to *demonstrate the benefits* of well targeted and patient centred healthcare interventions to close the treatment gap in Europe, and *converge evidence to policy*

# VoT Scope


Gaps
Prevention
Screening
Diagnosis/ treatment
Follow-up
(Case studies)
Schizophrenia
Missed detection
Early detection COST SAVING/ COST EFFECTIVE
Late intervention
Early detection COST SAVING/ COST EFFECTIVE
Alzheimer's Disease
Late intervention/ inadequate treatment
Early intervention/ adequate treatment COST EFFECTIVE
Headache
Lack of structured headache services and education
Structured headache services and education COST EFFECTIVE
Stroke
Lack of inpatient stroke unit
In-patient stroke unit COST EFFECTIVE
Lack of rehabilitation
In-hospital rehabilitation COST EFFECTIVE
Parkinson's Disease
Lack of early/ timely treatment
Lack of adequate treatment for advanced PD
Lack of adherence to drug treatment
Early/timely treatment COST EFFECTIVE
Adequate treatment for advanced PD COST EFFECTIVE
Adherence to drug treatment COST EFFECTIVE
Epilepsy
Inadequate treatment & care
Adequate treatment and care COST EFFECTIVE
Multiple Sclerosis
Impact of modifiable lifestyle factors on MS
Lifestyle factors prevention COST EFFECTIVE
Delays in treatment
Early in treatment (DMTs) COST EFFECTIVE
Restless Legs Syndrome
Inadequate treatment (& Socio economic impact of RLS)
Adequate treatment COST EFFECTIVE
Normal Pressure Hydrocephalus
Delayed and inadequate treatment
Early and adequate treatment COST EFFECTIVE

# VoT2 continuity: sustaining into next phase

## Rare Neurological Diseases

- **Ataxia** – *Started (1st scoping meeting on 26/3)*
- **Dystonia** – *Started (VoT2 strategic meeting on 24/9*
- **Phenylketonuria (PKU)** – *Started*


2018-2020 VoT Research Study 2nd round

# Value of treatment project 2 - Ataxia

- European Parliament
  Prof Paola Giunti invited to speak at the launch of White Paper on VoT on 'Ataxia Specialist Centre'.

- Submission to the EBC of the Ataxia case study (Nov 2017)
- Established Working Group
- Funding secured from Takeda and now Reata. Seeking further sponsors
- Meetings with EBC and Sponsor Takeda (March and Sep 2018 in Brussels)

***Aim:*** **To understand differences in care between Specialist Ataxia centres compared with non specialist care for inherited ataxias**

***Research Plan:***

1. Literature review of treatment care pathways in Europe
2. Ataxia care pathways survey of patients in UK and other EU countries
3. Economic evaluation

2-year project

EBC European Brain Council

ATAXIA UK

UCL

# Ataxia Working Group

| Name | Affiliation | Expertise |
|---|---|---|
| **Paola Giunti** | UCL/UCLH | Clinician |
| **Steve Morris** | UCL | Health economist |
| **Julie Greenfield** | Ataxia UK and Euroataxia | Patient group representative |
| **Barry Hunt** | Ataxia UK | Patient group representative |
| **Vinciane Quoidbach** | EBC | Policy |
| **Deborah Hoffman** | Takeda | Industry representative |
| **Cathalijne Van Doorne** | EFNA | Policy, patient group representative |
| **Holm Gressner** | ERN-RD coordinator | Policy |

# Patient care pathway analysis

- Literature review complete
  - Diagnosis
  - Treatment guidelines
  - Treatment Patterns in Europe
    - Time to diagnosis
    - Specialist treatment
    - Management of emergencies
    - Use of specialist settings
- Conclusions: Unmet Needs and Evidence Gaps

# Treatment Guidelines (1)

- Only **4** evidence-based consensus clinical management guidelines were identified[1-4]
- **No approved therapy** to treat inherited ataxias and most treatment is purely symptomatic[1-4]
  - No high-quality evidence to support efficacy of any disease-modifying treatment

## Treatment of Cerebellar Motor Dysfunction and Ataxia[1,5]

- Systematic review of evidence for ataxia treatment (not diagnosis or other areas of management)
- 17 global experts: US, Japan, and Germany
- Covers all cerebellar ataxias, not just inherited
- **No approved therapy** to treat cerebellar motor dysfunction and **no pharmacologic or surgical treatment routinely used**

## Consensus on the Diagnosis and Management of Chronic Ataxias in Adulthood[2]

- European guideline developed by 10 experts aimed at clinical neurologists and specialists
- Focuses on heredodegenerative ataxias and covers diagnosis and treatment
- Most treatment is purely symptomatic with no strong evidence to support effectiveness of any pharmacological therapy
- Class II evidence to recommend physiotherapy and Class III data to support occupational therapy

AAN = American Association of Neurology; EFNS/ENS = European Federation of Neurological Societies/European Neurological Society; FARA = Freidreich's Ataxia Research Alliance.

1. Zesiewicz et al., 2018; 2. van de Warrenburg et al., 2014; 3. Ataxia UK, 2016; 4. Corben et al., 2014; 5. AAN, 2018.

# Treatment Guidelines (2)

- One **country-specific guideline** identified for the UK (Ataxia UK, 2016)[1]
- One **global FRDA guideline** identified: addresses the nonneurological problems associated with FRDA, including the high incidence of cardiovascular problems and diabetes[2,3]

**Management of the Ataxias: Towards Best Clinical Practice[1]**

- UK-specific guideline developed by 32 UK experts aimed at primary, secondary, and tertiary care
- Covers a broad range of hereditary and idiopathic ataxias, including episodic ataxias (but not AT)
- Comprehensive guideline covering diagnosis, specialist referral, symptomatic treatment, speech/physio/ occupational therapy, palliative care, and research
- Specific recommendations for management of cardiac problems in FRDA
- There are **no approved treatments** for the majority of inherited ataxias in the UK

**Consensus Clinical Management Guidelines for Friedreich's Ataxia[2]**

- 39 experts from Europe, US, Canada and Australia
- All areas of clinical management covered (but not diagnosis), including neurological problems, QOL, mental health, rehabilitation and palliative care
- Nine potential treatments identified but none proven to slow disease progression: **not recommended to routinely prescribe any medication for FRDA**
- Multiple gaps in the evidence for service delivery were identified as well as the need for further research

1. Ataxia UK, 2016; 2. Corben et al., 2014; 3. Zeitlberger et al., 2018

# Patient survey in the UK

- Survey outline is complete
- Being piloted in a few people with ataxia
- To be submitted for Ethical Review
- Plan to distribute survey to ataxia patients in the UK via Ataxia UK in January
- Plan to extend to two other countries

# Patient Survey: Outline

- Introduction and Background
  - Demographics such as ataxia type and Specialist Ataxia Centre usage will be collected
- Time to Diagnosis
  - Questions to determine if Specialist Ataxia Centres usage is related to earlier diagnosis
- Access to Coordinated Care
  - Questions to determine whether Specialist Ataxia Centres may improve access to care
- Quality of Care
  - Questions to determine factors that patients feel affect their quality of care
- Resourse use (Costs)
    - Numbers of misdiagnoses and contacts with doctors received before and after ataxia diagnosis
    - Details of treatments received, referrals made, including the level of information that Specialist Ataxia Centres provide about treatment
    - Info on Hospital admissions

# Economic evaluation

- Work to be undertaken by Steve Morris, health economist at UCL
- Feasibility phase (until Jan 2019)
- Report and meeting
- Main phase Jan – Dec
- Data: Survey + pre-existing data + published and grey literature
- UK and 2-3 other European countries

# Ataxia: evaluating the costs and benefits of specialist centres - diagnosis

- Measuring costs:
  - Contacts with local and specialist services from initial symptoms to diagnosis
  - Include time and travel cost for family members/carers
  - Include treatment for symptom alleviation and incorrect diagnoses?
  - Compute on an annualised basis then multiply by time to diagnosis?
- Measuring consequences & effects:
  - Time to diagnosis
  - Number of incorrect diagnoses before obtaining a correct diagnosis

# Ataxia: evaluating the costs and benefits of specialist centres - treatment

- Measuring costs:
  - All contacts with local and specialist services
  - Include treatments as well as contact (e.g. drug therapy)
  - Include time and travel cost for family members/carers
  - Compute on an annualised basis, then multiply by pre-agreed time period
- Measuring consequences & effects:
  - Health-related quality of life
  - Patient and family satisfaction
  - Disease-specific indicators
  - Process measures reflecting quality of care

# Future plans

- Complete and circulate UK survey
- Analyse data
- Identify 2 more countries/ teams to work with and do survey (Centres within ERN)
- Economic evaluation study in UK and 2 other countries
- Write results and policy papers
- Dissemination