

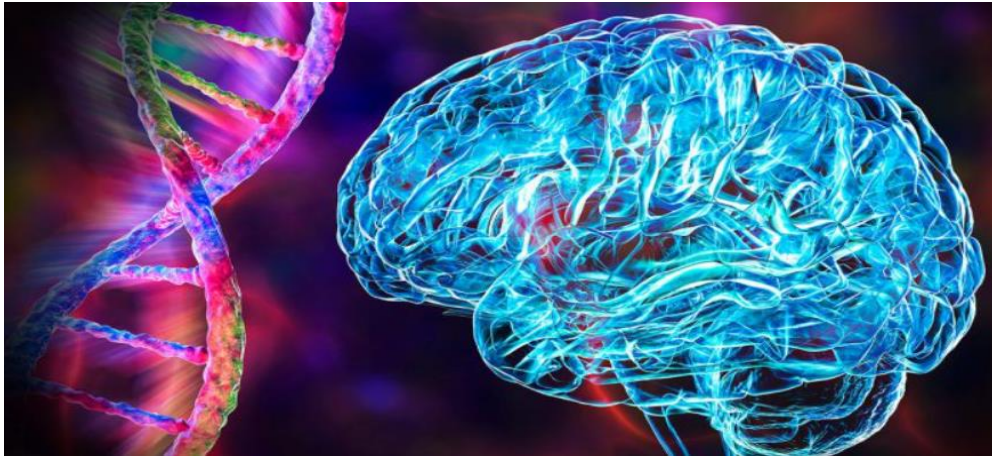
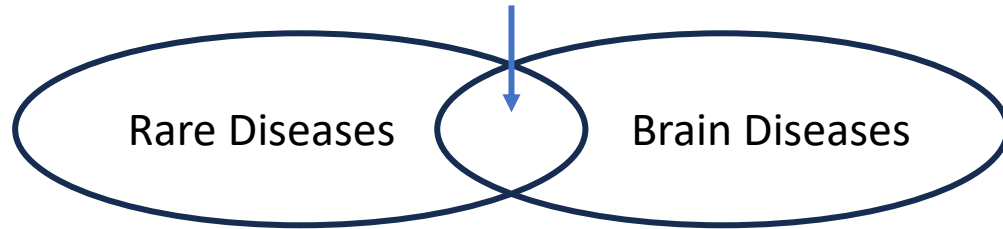
ALS Side Event – Session 3:

Action at European level

Vinciane Quoidbach, Research Project Manager, European Brain Council



Why are rare brain diseases important?



- **75% of rare diseases have neurological symptoms**
- **Rare brain diseases** involve complex genetic, molecular, or cellular abnormalities.
- **Amyotrophic Lateral Sclerosis (ALS), Huntington's Disease, Ataxia, Dystonia, Phenylketonuria, ...** are just a few examples, and there are many more RBDs that impact individuals and their families.
- **Multidisciplinary care and health digitization** are the cornerstone of RBDs management

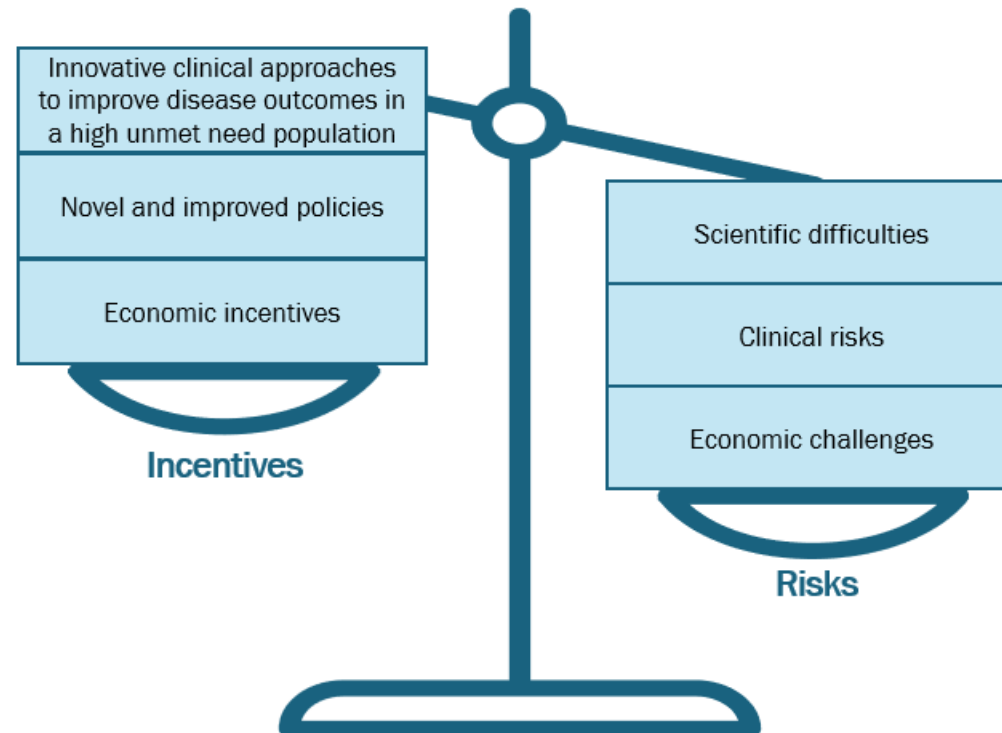
Challenges linked to ALS

- Societal

In Europe ALS about 50.000 individuals of middle age are affected by ALS
=> Adapted care along the patient pathway as the disease evolves

Challenges linked to ALS

- Scientific
- Clinical



Consequences: a higher risk of clinical trial failure, discouraging R&D investment in ALS

Addressing treatment gaps set the case for renewed European action on rare diseases

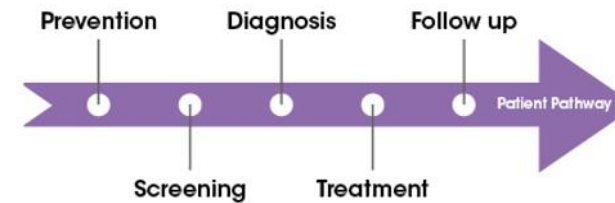
EBC RESEARCH PROJECT - THE **VALUE OF TREATMENT** FOR BRAIN DISORDERS IN EUROPE

BRAIN COMMUNITY JOINS CALLS FOR A RENEWED **EU** **ACTION PLAN ON RARE DISEASES**



[Value of Treatment: 2nd Round – European Brain Council \(EBC\)](#)

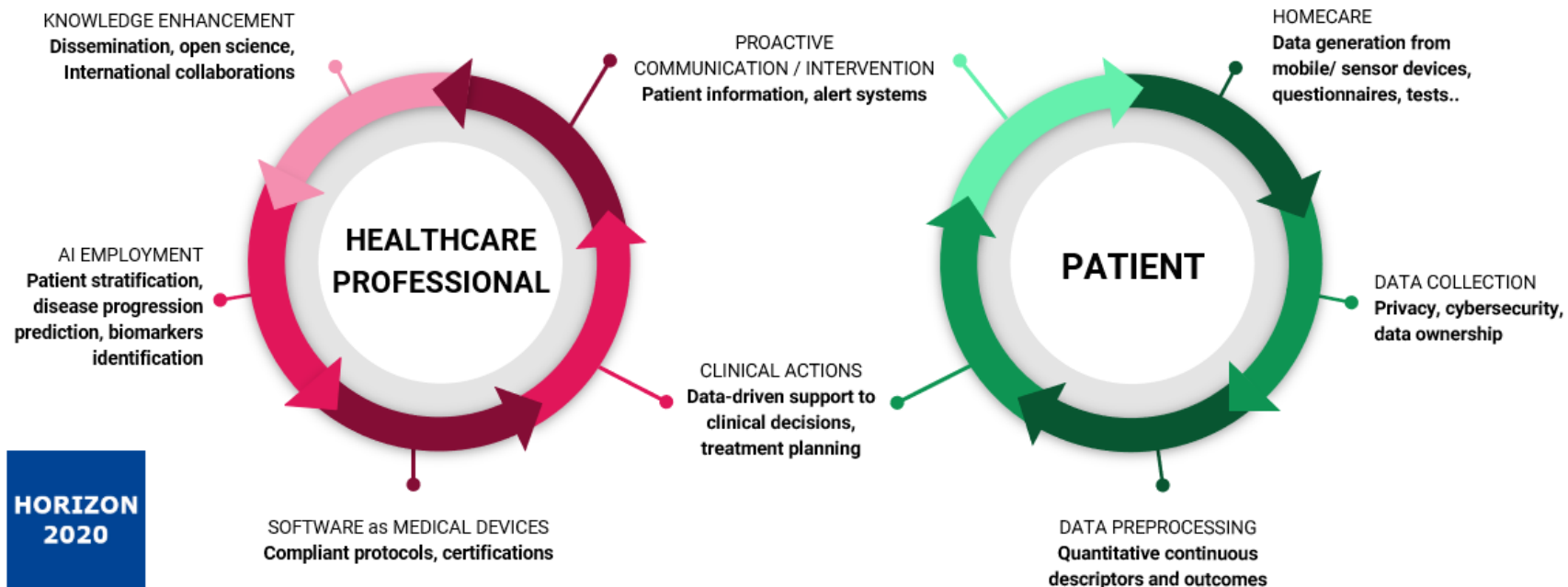
Treatment gaps along the care pathway



- ▶ Low insight into pathological mechanisms, delayed detection and diagnosis
- ▶ Barriers to access specialized care and treatment options
- ▶ Uncoordinated care, discontinuity, drop-outs
- ▶ High prices and lack of reimbursement
- ▶ Lack of knowledge raises concern to health professionals
- ▶ Limited sharing of best practice and expertise

Bringing AI at home for Better care of Amyotrophic Lateral Sclerosis (ALS) patients

- Developing predictive/ preventive **AI models** for **patient stratification and disease progression**
- Using these models in real life to **inform patients, help clinical decision** making, **simulate in silico trials**, and provide **personalized medicine** suggestions



Necessary collaboration amongst all stakeholders



The EU ALS Coalition published its Policy Paper with policy recommendations, for improving the diagnosis, care and prognosis of PLWALS

Brain Innovation Days

26-27 October 2023, Brussels



**Brain
Innovation
Days** 26-27 October 2023
Brussels, Belgium

Thank you

