

BRAINS FOR BRAIN FOUNDATION MANIFESTO

Hunger for Knowledge: Investing in Brains to deliver research value in the EU

Addressing the gaps in knowledge to unleash the potential of Research and Innovation in Europe

On 16 October 2013, MEP Sartori and MEP Berlinguer presented a manifesto¹ “European Research Area: a Maastricht for Research”, which aims to rally the European scientific community and institutions. A key objective is to seek consensus on a series of actions and initiatives that will enable the completion of the European Research Area. Building on the political momentum gained by this initiative, **the Brains for Brain Foundation aims to propose a model of cross-sector cooperation to overcome the gaps in research in the area of rare neurometabolic paediatric diseases.** The intention is to create a model that could facilitate the set-up of a European PhD Programme in the area of rare neurometabolic paediatric diseases, which could be replicated in other disease areas and sectors.

Given the challenges posed by an ageing health workforce on the healthcare sector, it is now fundamental to consider how Europe will ensure continuation of the essential public health activities of biomedical laboratories specialised in neurodegenerative disorders affecting children. These are currently at risk of closure. This is even more problematic in rare paediatric diseases which are characterised by a limited number of patients and scarcity of relevant knowledge, which requires close cooperation across the EU as a whole.

The challenge lies in addressing the gaps in research in the field of rare paediatric diseases and limiting the brain drain created through the lack of career opportunities in this field in the EU.

To ensure continuity within the research community, the Brains for Brain Foundation aims to create a network of experts committed to launch a scientific doctorate and post-doctorate to train the future generation of physicians and scientists. To ensure a multidisciplinary approach, the scientific programme will be supported by leading scientists, biotech companies, policy-makers, patient and healthcare professional groups.

The implementation of this model of cross-sector cooperation aims to achieve the following:

- create synergies in order to coordinate research endeavours,
- incentivise the application of research into clinical practice,

¹ The “European Research Area: a Maastricht for Research” Manifesto paves the way for an ERA Framework Directive. It also identifies a number of proposals to overcome the following problems: 1) Low level of investment and human resources, 2) Fragmentation and low coordination, 3) Knowledge application, 4) Research infrastructures, and 5) Research Careers and Mobility.

- improve the knowledge of the health workforce,
- invest in the most talented biomedical researchers,
- ensure mobility of careers.

This will contribute to EU leadership in research and limit the brain drain in the field of rare neurometabolic paediatric diseases in the European Union.

The need for a model of cross-sector cooperation in the area of rare neurometabolic paediatric diseases

The WHO has estimated that 1 in 10 people will be diagnosed with a severe and sometimes rare brain disorder in their lifetimeⁱ.

There are 5000 to 8000 rare diseases affecting 5 to 8% of the population in the EUⁱⁱ, many of which have neurological manifestations. Rare neurometabolic diseases in children, such as lysosomal storage diseases, and inherited metabolic disorders affecting the brain, such as phenylketonuria, have a low prevalence. However, when combined the numbers represent a large burden for European society.

Lysosomal storage disorders (LSDs) are the cause of significant morbidity and mortality rates for those affected. Most LSDs patients begin showing signs of the disease early in childhood, often in the first year or two of life. To date, 300 inherited disorders are currently known to result in brain damage in children.

These diseases are characterised by a limited number of patients and scarcity of relevant knowledge that require a close cooperation across the EU as a whole. Research results must be transferred in a more active way to the communities and professionals who need this information in their daily lives. Because LSDs are so rare it remains difficult to find health care professionals knowledgeable about the diagnosis, management and treatment of these conditions.

The objective of investing in high quality healthcare via a more efficient use of public and private resources can only be pursued if an integrated and coherent policy framework is developed that mainstreams excellence across sectors. Sustainable investments in health should therefore address the mismatch between the educational needs of healthcare professionals and bio-medical scientists with the existing societal needs of children.

A model of cross-sector cooperation is needed to achieve an EU knowledge-economy

A model of cross-sector cooperation is needed to optimise the value chain from biomedical research to translational application and health outcomes. The challenge lies in matching

the educational needs of biomedical scientists with employment opportunities, in order to advance the available treatment and improve the care delivered to children with rare paediatric diseases and their families. Investments in child health should be combined with investments in human capital and a strengthened interaction between key sectors such as education, excellence in research, employment, and quality of healthcare.

This approach is fundamental for a disease area where available knowledge is limited. At times of economic austerity, **it is crucial that the EU continues implementing actions to limit the brain drain due to a lack of career opportunities for researchers in the EU.**

Despite a higher percentage of science graduates in EU Member States compared to the US, the EU lags behind in terms of number of researchers per working populationⁱⁱⁱ. Over the years the Commission and Member States have dedicated resources to curb this trend, notably by facilitating youth mobility within the Marie Curie Actions Programme. The European Commission has recognised the prominent role and impact of a knowledge-based economy in driving sustainable and smart growth in Europe. Europe 2020 flagship initiatives closely intertwine critical aspects such as investment in research to address societal challenges, encourage youth mobility and lifelong learning to attract younger generations towards science and technology careers.

Yet, more has to be done to foster cooperation and leadership in Europe. **The EU has to undertake action to ensure continued mobility of PHD and post-doc students in Europe and greater transparency in recruitment appointments in research universities. PHD and post-doc curricula should also include extensive interaction with biotech and pharmaceutical companies.**

Such activities can contribute to maintaining the EU at the cutting-edge of fundamental and translational research in rare diseases, as well as investing in forward-looking and innovative technologies to address our society's challenges.

European pilot PhD programme on rare paediatric neurometabolic diseases

Since 2007, the Brains for Brain Foundation has invested resources to bring to life projects in the field of rare neurometabolic paediatric disorders, such as the 2013 exchange programme on lysosomal storage disorders. This programme gave the opportunity for the most deserving PhD students from across Europe to come together to present their research and progress in the area, as well as receive guidance on conducting further research projects. Given the specificities of rare neurometabolic diseases, we believe that greater results could be achieved if we pool together the resources at European level and invest in the brains of our biomedical scientists, to advance the treatments of rare neurometabolic diseases affecting children.

Building on EU initiatives to achieve the Europe 2020 objectives through a strong knowledge-based economy, the signatories of this paper call upon the European institutions and national political and health authorities to:

- **Support the establishment of European pilot PhD and post-doc programmes on rare paediatric neurometabolic diseases**

In order to address the disconnect between societal needs in the area of rare neurometabolic diseases affecting children and the curricula of healthcare professionals and biomedical scientists, the PhD programme should be linked to the dedicated European Reference Networks (ERN) so as to achieve and maintain excellence in Europe.

Further we call on you to:

- Foster interaction between key sectors such as education, employment, research and healthcare in the field of rare neurometabolic diseases affecting children
- Retain and invest in the students who study the brain and contribute to the development of our knowledge economy
- Promote policy activities that address the disconnect between excellence and societal needs
- Promote and facilitate partnership and collaboration between physicians, researchers, patient advocates, carers, policymakers and industry
- Capitalise on knowledge gained through EU projects for the benefit of society
- Encourage and support research and the translation of scientific breakthroughs into clinical practice
- Contribute to the establishment of a standard of care for patients with rare neurological disorders which is agreed across Europe, and to ensure equity of access to diagnosis, treatment and care
- Increase exchanges and mobility of biomedical researchers and healthcare professionals

The signatories of this paper believe that the dissemination of new scientific knowledge developed via European collaborative research projects should be directed to our European biomedical scientists, in order to advance research and care for children with rare neurometabolic diseases.

We invite you to join us in supporting this new initiative.

Brains for Brain Foundation

ⁱ WHO, Neurological disorders: public health challenges,2006

ⁱⁱ European Medicines Agency

ⁱⁱⁱ Science and Technology Indicators 2003