

Improving access to care and treatment for Huntington's Disease patients and families

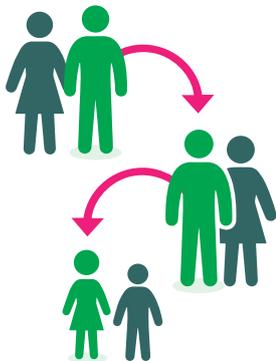
Following a virtual policy roundtable held on 3 December 2020, organised by the European Huntington Association (EHA) and the European Federation of Neurological Associations (EFNA) with financial support from Roche, the following organisations and stakeholders call for further EU and national action to improve the quality of care and treatment for people living with Huntington's Disease.

Support from policymakers at national and international levels is crucial to enable the necessary steps to be taken to improve the quality of life of those affected by Huntington's Disease.

This Consensus Statement sets out a number of recommendations where multi-stakeholder action could help overcome some of the key challenges faced by those affected by Huntington's Disease.



About Huntington's Disease



Huntington's Disease is a rare, genetic, neuro-degenerative and ultimately fatal disease that has a devastating impact on families across generations. Living with Huntington's Disease comes with a number of health and societal challenges, including serious multifaceted symptoms, long-term disability, battling stigma, social isolation, loss of income and social support and increased care burdens for families, to name a few.

There is a lack of understanding of Huntington's Disease among healthcare professionals, with many patients struggling to receive the care they need and at times receiving incorrect information from healthcare professionals. In addition, opinions vary on what best practice in Huntington's Disease looks like, given the many differing approaches to care and treatment across countries, both in Europe and globally.

It is clear that change is required within healthcare systems to support better access to care and treatment for Huntington's Disease patients. Increasing knowledge amongst healthcare professionals and social services, as well as a multidisciplinary and holistic approach to care and treatment, are critical to close the gaps which currently exist. To address these gaps, we need well informed and activated patients and caregivers; an understanding of where to access support for patients and families; opportunities for user involvement; joined up support for physical health, mental wellbeing and social care; and, importantly, we need to fight the stigma that surrounds this patient community.

Speeding up research into treatments for Huntington's Disease and other neurodegenerative diseases is paramount. In addition, methods to measure cognitive functions and non-motoric symptoms, such as emotional control, need to be improved. Another factor which has a significant impact on the quality of life of Huntington's Disease patients and their families is mental health, an area in which research is currently very minimal. **EU research and innovation programmes, such as Horizon Europe, can be leveraged to support this type of research by bringing together expertise from all EU Member States to improve how care is delivered and how research is conducted for rare diseases.**





In addition, challenges continue to exist around patient access to medicines for rare diseases, with major inequalities in access between Eastern/Western and Northern/Southern Europe.

The European market is fragmented with multiple HTA, pricing and reimbursement processes. In general, no individual country has enough knowledge of rare diseases to assess them on its own. We need to coordinate efforts to ensure a stronger focus on research, better clinical trials, early dialogue on the value of therapies, and a joint HTA discussion at European level.

Renewed EU momentum on health policy as a result of COVID-19, and recent proposals to build a European Health Union, are encouraging signals for the rare disease community. We must leverage this momentum as an opportunity to ensure that we improve healthcare service provision to Huntington's Disease patients, and rare disease patients more broadly, across the EU. Strong collaboration between policymakers and other stakeholders will be essential to make this happen.

The Pharmaceutical Strategy for Europe, published in November 2020, touches upon many areas where more cooperation is needed, including data, transparency of costs and pricing, and joint procurement of high-cost therapies. The Strategy has a lot of potential, but it will be important that the initiatives it includes really link together to achieve better, more affordable treatment while also ensuring Europe remains at the forefront of innovation in healthcare.

In the post COVID-19 recovery phase, we need to give a voice to those diseases which have not received the attention they deserve over the past years and ensure that they are not overshadowed by the pandemic, but rather that rare disease patients are a central focus in the recovery phase and beyond.

Some of the lessons learned from the COVID-19 pandemic are applicable to rare disease management, including tackling the health inequalities which persist across the EU. This health crisis has shone a light on how essential healthcare systems are, and has opened the field of possibilities with telemedicine, increased investment in healthcare and accelerated clinical trials, amongst others. This should now become a reality in other areas of healthcare, including rare diseases.



Calls for action

1

ADDRESSING CARE AND TREATMENT GAPS FOR PATIENTS LIVING WITH HUNTINGTON'S DISEASE



National level action: **Integrating the European Reference Networks in national healthcare systems**

To implement the changes necessary to support people living with Huntington's Disease, we need EU Member States to integrate the European Reference Networks (ERNs) within their rare disease strategies. This will ensure ERNs become **a centralised go-to point for patients** and ensure **harmonisation of information and standards of care across Member States**.

Sharing disease specific expertise through the European Reference Networks

EU Member States must be more proactive, increase resources for their ERN members, as well as organise national networks to allow the expertise to flow from the ERN to the patient and vice versa. Member States should share best practices to ensure that solutions are implemented effectively across Europe.

Adopting international guidelines on Huntington's Disease

The European Reference Network for Rare Neurological Diseases (ERN-RND) has published **International Guidelines for the Treatment of Huntington's Disease**. These international guidelines should be endorsed and adopted by all national healthcare authorities and should form the direction for the development of services. This would help ensure consistency and access to high quality, multidisciplinary care and support for everyone affected by Huntington's Disease.

2



Ensuring a multidisciplinary approach to care

Because no disease modifying or curative treatment is available yet for Huntington's Disease, **it is crucial to focus on symptom management and improving quality of life.** This includes treatment of psychiatric symptoms, physiotherapy, nutritional management, psychological support, occupational therapy, among others. Access to these options is highly variable in different countries and many patients have limited or no access. The support of multidisciplinary teams is also essential and the ERN-RND international guidelines on Huntington's Disease reflecting this should be adopted in all European countries to ensure patients have access to the high quality care they need.

EU level action: **Optimising healthcare professional training programmes and fellowships/exchange programmes**

ERNs go beyond clinical cooperation; they can also offer **professional training activities** to share experts' knowledge and provide young professionals with in-depth training. Steps should be taken to ensure these training opportunities reach as many healthcare professionals as possible. ERNs should also be focal points for research and for exchanges with patients and patient representatives.

Implementing legal frameworks for the services provided by European Reference Networks

COVID-19 has highlighted **the importance of legal frameworks** for serious cross-border threats to health, and the importance of strengthening the position of the ECDC and the EMA. For Huntington's Disease patients, we also need legal frameworks for the services provided, for example, **by developing an education programme to attract a larger number of experts on rare diseases.**



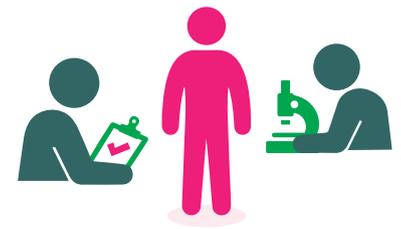
2 ENSURING RESEARCH PRIORITIES HAVE PATIENTS IN MIND

EU level action: **Improving the involvement of patients in R&D**

Huntington's Disease patients and their caregivers should be actively involved in research to ensure their voice is included. Usually research is executed in easy-to-reach populations before looking at how real-life populations react to treatments. By improving the involvement of a variety of Huntington's Disease patients in research, the best possible research outcomes can be ensured. European research and innovation programmes, including Horizon Europe and the Innovative Health Initiative, should ensure that meaningful patient involvement is embedded.

Leveraging European research and innovation programmes

We need to **seize opportunities for Huntington's Disease research in calls under European research and innovation programmes, such as Horizon Europe.** These programmes should include research calls which provide opportunities for Huntington's Disease research, including calls around rare diseases and neurology, as well as around patients' unmet needs.



3 IMPROVING RARE DISEASE PATIENTS' ACCESS TO TREATMENT BY ENCOURAGING EU-WIDE COOPERATION ON HTA AND PRICING AND REIMBURSEMENT

EU and national level action:

Improving HTA, pricing and reimbursement processes across the EU for better and more equal access to rare disease treatments

To tackle inequalities in access to rare disease treatments across the EU, we need better cooperation. EU Member States should work to reach an agreement on **joint European HTA assessment.** The EU should provide a platform for early dialogue on the value of therapies involving all Member States.

4

LEVERAGING RENEWED EU MOMENTUM ON HEALTH POLICY

EU level action: Ensuring the Pharmaceutical Strategy for Europe concretely leads to better and more equal access to treatment for rare disease patients across the EU

The EU must ensure that the initiatives set out in the Pharmaceutical Strategy are executed effectively to improve EU cooperation, including, amongst others, a legislative proposal on a European Health Data Space and non-legislative measures, such as guidelines, to improve transparency of costs.

EU and national level action:

Advocating for Member State governments to support the development of a strong European Health Union

In order for proposals to build a **European Health Union** to be successful, all Member States need to be on board. All stakeholder associations with a vested interest in a strong European Health Union, in which all EU countries work together to better protect citizens' health and improve treatment, should ensure their voices are heard at national level and advocate for Member State governments to agree to be part of the European Health Union.



Active patient group participation and engagement with policymakers to ensure Huntington's Disease has a higher profile on the policy agenda at regional and national levels

Policymakers need input and feedback from patient representatives to ensure they fight for policies that address the needs of all people. Patient groups must ensure a strong presence among policymakers both at European and national level to raise awareness of Huntington's Disease and ensure that the **Huntington's Disease patient perspective** is integrated into relevant policies and initiatives.

5

PUTTING RARE DISEASE PATIENTS AT THE CENTRE OF POST COVID-19 RECOVERY



National level action: Implementing national rare disease strategies which include specific measures to improve treatment, care and support for everyone affected by Huntington's Disease, including patients, families and carers



Up to date national rare disease strategies are an important step to improve rare disease care in each country. All EU Member States should adopt and implement a national rare disease strategy including specific measures and targets on Huntington's Disease. **National strategies are needed in order to have a European strategy.**

Increasing investment in national healthcare systems to increase capacity and resilience and ensure all rare disease patients can access the care they need

EU Member States need to learn the lesson from the COVID-19 pandemic and continue to increase investment in their healthcare systems to ensure they are robust and with sufficient capacity to provide excellent care and support to everyone affected by a rare disease, including in the event of future pandemics.

EU level action: **Scaling up the European Reference Networks**

The new **EU4Health programme** should be used to scale up the European Reference Networks, including through more sustainable funding and integration into standard care pathways, and to support European level disease registries, or alliances of national registries with common core data sets. The **EU legal framework on cross-border healthcare** should be strengthened to support the further development of the ERNs.

Calls for action

1 ADDRESSING CARE AND TREATMENT GAPS FOR PATIENTS LIVING WITH HUNTINGTON'S DISEASE

- Integrating the European Reference Networks in national healthcare systems
- Sharing disease specific expertise through the European Reference Networks
- Adopting international guidelines on Huntington's Disease
- Ensuring a multidisciplinary approach to care for patients with Huntington's Disease
- Optimising healthcare professional training programmes and fellowships/exchange programmes
- Implementing legal frameworks for the services provided by European Reference Networks

2 ENSURING RESEARCH PRIORITIES HAVE PATIENTS IN MIND

- Improving the involvement of patients in R&D
- Leveraging European research and innovation programmes

3 IMPROVING RARE DISEASE PATIENTS' ACCESS TO TREATMENT BY ENCOURAGING EU-WIDE COOPERATION ON HTA AND PRICING AND REIMBURSEMENT

- Improving HTA, pricing and reimbursement processes across the EU for better and more equal access to rare disease treatments

4 LEVERAGING RENEWED EU MOMENTUM ON HEALTH POLICY

- Ensuring the Pharmaceutical Strategy for Europe concretely leads to better and more equal access to treatment for rare disease patients across the EU
- Advocating for Member State governments to support the development of a strong European Health Union
- Active patient group participation and engagement with policymakers to ensure Huntington's Disease has a higher profile on the policy agenda at regional and national levels

5 PUTTING RARE DISEASE PATIENTS AT THE CENTRE OF POST COVID-19 RECOVERY

- Implementing national rare disease strategies which include specific measures to improve treatment, care and support for everyone affected by Huntington's Disease, including patients, families and carers
- Increasing investment in national healthcare systems to increase capacity and resilience and ensure all rare disease patients can access the care they need
- Scaling up the European Reference Networks

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WITH THE SUPPORT OF EURORDIS



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Neurological Diseases
(ERN-RND)