



To:
Ms. Ursula von der Leyen
President of the European Commission
Rue de la Loi 200
1049 Brussels

Copy to :
Ms Stella Kyriakides
Commissioner for Health and Food Safety

Mr. Nicolas Schmit
Commissioner for Jobs and Social Rights

Brussels, 28th February 2023

Rare Disease Day call for a European strategy on rare diseases

Dear Madam President,

28 February 2023 marks the 16th Rare Disease Day, a global awareness day celebrated in over 100 countries around the world. This year, Rare Disease Day puts the focus on equity.

As Members of the European Parliament and on behalf of the Network of Parliamentary Advocates for Rare Diseases, we have the honour to once again call on you to introduce a comprehensive European strategy on rare diseases to better meet the needs of the 30 million European citizens living with a rare disease.

Hearing the first-hand experiences of our constituents living with a rare disease, **we know that there is still a long way to go to reach equity in the European Union for this vulnerable population.** While an individual's condition may be rare, people living with a rare disease face common challenges:

- The average time for accurate diagnosis of a rare disease is about 5 years¹;
- Only 5% of people living with a rare disease have received a transformative treatment approved for the entire EU²;

¹ EURORDIS, 2022. *Rare Barometer survey on the Journey to Diagnosis for People Living with Rare Diseases, with over 10,000 respondents in Europe.* Pending publication.

² Results from EURORDIS Rare Barometer Survey, 2018. Available upon request.

- 22% of people with rare diseases could not get the treatments they needed because they were not available where they live, reflecting the fragmentation of the market across the Member States³;
- 52% of people living with a rare disease or their carers say that their condition has a **severe** impact on everyday life⁴.

The European added value to addressing the needs of people living with a rare disease is exceptionally high, due to the rarity of patients, experts, data and resources. It is true that the European Union has fostered tremendous progress in this area. The upcoming revisions of the general pharmaceutical legislation and Regulation on Orphan Medicinal Products will also offer the opportunity to tackle some of the identified shortcomings and needs. What the European Union is lacking today, however, is an updated cohesive strategy to improve the lives of people living with rare diseases.

An overarching strategy on rare diseases was proposed in the recommendations of a two-year Foresight Study, Rare 2030, initiated by the European Parliament and funded by the European Commission⁵. This is needed to bridge national and European legislation, policies and programmes across the Pharmaceutical Package, the European Health Data Space, European Reference Networks, Europe’s Beating Cancer Plan, EU4Health, Horizon Europe, the Long Term Care Strategy, the European Disability Strategy, and beyond.

The introduction of such a strategy, penned a **European Action Plan for Rare Diseases**, has recently gathered the support of 21 Member States who endorsed a Call to Action under the Czech Presidency of the Council⁶, hereby joining the Parliament⁷, the European Economic and Social Committee⁸, the European Court of Auditors⁹ and the rare disease community at large¹⁰. In December 2021, the General Assembly at the United Nations adopted a Resolution on Addressing the Challenges of Persons Living with a Rare Disease¹¹ recognising the global impact of rare diseases.

There is no option but to work collaboratively in the area of rare diseases. The Conference on the Future of Europe singled out rare diseases as an area to “improve the effectiveness of European governance towards the development of the European Health Union”¹². In this vein, the ENVI Committee has recently put forward rare diseases to be included as a European competency in the proposals of the Parliament for the amendment of the Treaties¹³.

³ Ibid

⁴ EURORDIS, 2017. [Juggling care and daily life: The balancing act of the rare disease community](#)

⁵ Rare 2030 Foresight Study, 2021. [Recommendations: The future of rare diseases starts today](#)

⁶ Czech Presidency of the EU Council, December 2022. Call to Action from the Expert Conference on Rare Diseases: Towards a new European framework on rare diseases. Attached to email.

⁷ European Parliament Debate, 2021. [A European Action Plan Against Rare Diseases.](#)

⁸ European Economic and Social Committee, 2022. [Ensuring strong European solidarity for rare disease patients](#)

⁹ European Court of Auditors, 2019. [Special Report of the European Court of Auditors on Implementation of Directive 2011/24/EU.](#)

¹⁰ [#30millionreasons for European Action on Rare Diseases](#), over 2000 testimonies of why action is needed.

¹¹ United Nations, 2021. [Addressing the challenges of persons living with a rare disease and their families : resolution / adopted by the General Assembly.](#)

¹² Conference on the Future of Europe, 2022. Report on the Final Outcome, p.52.

¹³ Proposals of the European Parliament for the amendment of the Treaties (2022/2051(INL))

Fourteen years after the last comprehensive EU strategy on rare diseases, it is time to act and propel rare diseases as an engine for a European Health Union.

This Rare Disease Day, we stand with the rare disease community and respectfully urge you to take forward the recommendation for a European strategy on rare diseases.

Yours sincerely,

Frédérique Ries, RE, Belgium	Ewa Kopacz, EPP, Poland
Alex Agius Saliba, S&D, Malta	Stelios Kypouropoulos, EPP, Greece
João Albuquerque, S&D, Portugal	Katrin Langensiepen, Greens/EFA, Germany
Barry Andrews, RE, Ireland	Peter Liese, EPP, Germany
Marc Angel, S&D, Luxembourg	Colm Markey, EPP, Ireland
Pascal Arimont, EPP, Belgium	Radka Maxová, S&D, Czechia
Brando Benifei, S&D, Italy	Tilly Metz, Greens/EFA, Luxembourg
Izaskun Bilbao Barandica, RE, Spain	Alin Cristian Mituța, RE, Romania
Jordi Cañas, RE, Spain	Dolors Montserrat, EPP, Spain
Sara Cerdas, S&D Portugal	Juozas Olekas, S&D, Lithuania
Rosanna Conte, ID, Italy	Piernicola Pedicini, Greens/EFA, Italy
Deirdre Clune, EPP, Ireland	María Soraya Rodríguez Ramos, RE, Spain
Josiane Cutajar, S&D, Malta	Monica Semedo, RE, Luxembourg
Clare Daly, GUE/NGL, Ireland	Tomislav Sokol, EPP, Croatia
Frances Fitzgerald, EPP, Ireland	Susana Solís Perez, RE, Spain
Claudia Gamon, RE, Austria	Nicolae Ștefănuță, RE, Romania
Maria da Graça Carvalho, EPP, Portugal	Véronique Trillet-Lenoir, RE, France
Christophe Hansen, EPP, Luxembourg	István Ujhelyi, S&D, Hungary
Adam Jarubas, EPP, Poland	Petar Vitanov, S&D, Bulgaria
Radan Kanev, EPP, Bulgaria	Hilde Vautmans, RE, Belgium
Karin Karlsbro, RE, Sweden	Mick Wallace, GUE/NGL, Ireland
Billy Kelleher, RE, Ireland	Maria Walsh, EPP, Ireland
Ondřej Knotek, RE, Czechia	Isabel Wiseler-Lima, EPP, Luxembourg
Kateřina Konečná, GUE/NGL, Czechia	
Athanasios Konstantinou, NA, Greece	