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RARE DISEASES EUROPE

Juggling Care and Daily life:

The Balancing Act of the Rare Disease Community

Report on the launch of the Parliamentary Advocates for Rare Diseases

17th October 2017,

European Parliament

#ParliamentAdvocate4Rare

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On 17 October 2017, the new **network of Parliamentary Advocates for Rare Diseases**, an initiative piloted by EURORDIS-Rare Diseases Europe aiming to bring together members of the European Parliament and national parliaments, launched at the European Parliament in Brussels.

The creation of this network is the fruit of long-standing efforts to ensure that European stakeholders continue to unite and call for strong international and local action on rare diseases, and that they shape political input for current and future legislation, integrating rare diseases in all relevant policies at all levels.

The launch event ***'Juggling Care and Daily Life: The Balancing Act of the Rare Diseases Community'*** was organised under the auspices of MEP Francoise Grossetête and aimed to bring together MEPs and other key stakeholders to discuss ways to tackle the deep-seated challenges -and often also inequalities- which rare diseases create and which patients have to deal with on a day-to-day basis.

The event encouraged indeed a genuine discussion and exchange of ideas on the possibilities for policy making at EU level that can have a real impact on the daily lives of people living with a rare disease and their carers.

The meeting was well-attended with, in addition to Ms. Grossetête, MEPs Frédérique Ries, Kateřina Konečná, Marek Plura, Christel Schaldemose, and Bart Staes being present and introducing themselves. In addition, assistants to MEPs Elena Gentile, Patrizia Toia, Elisabetta Gardini and Olga Sehnalová also attended the event.

Programme

Event moderated by Vivienne Parry OBE, Science Writer and Broadcaster (The Guardian, The Times, BBC), Head of Engagement at Genomics England (100,000 Genomes Project).
15.45 - Introduction to Rare Diseases - Making the Invisible Visible (Video)
15.50 - Why should we act collectively on rare diseases? Françoise Grossetête MEP
16.00 - Juggling Care and Daily Life: The Balancing Act of the Rare Diseases Community – Avril Daly, EURORDIS Vice-President, CEO Retina International
16.10 - Patient Testimony. Lise Murphy, Swedish Marfan Association, ERN mentor, former Co-Chair of the Patient and Consumer Working Party (PCWP) at the EMA
16.20 - EU policy for rare diseases: what still needs to be done to improve patients' daily life – Martin Seychell, Deputy Director General DG SANTE, European Commission
16.30 - The network of Parliamentary Advocates for Rare Diseases: what it is and what it can do – Yann Le Cam, EURORDIS Chief Executive Officer
16.40 - Exchange of views: the EU added value for people living with rare diseases in research, access to health and social provisions Moderated debate with intervention from Marek Plura, MEP and Kateřina Konečná
17.20 – 17.30 - Concluding remarks – Terkel Andersen, EURORDIS President

Discussions

Françoise Grossetête MEP opened the event with a keynote recounting her long-term commitment to rare diseases and delineating the need to act collectively at EU level. As she pointed out, rare diseases represent much more than the commonly discussed topic of orphan medicines and their impact on health budgets; in fact, they pose challenges to *all* areas of life, for the *entire* lifespan of a person, and they need to be looked in a more holistic manner in order to break the isolation experienced by patients themselves and their families. For this, the **right level to tackle the challenges of rare diseases is the European one**, and the newly launched European Reference Network (ERNs) should be fully exploited towards this goal. In addition, social policy proposals like the new EU Directive on work-life balance, with its provision for five days a year of paid carers' leave, could also be a first step in addressing some of these issues, provided that it is not watered down.

EURORDIS Vice-President Avril Daly provided an introductory snapshot of the everyday life of European citizens living with a rare disease. She guided the audience through the main results of **the**

recent RareBarometerVoices survey, the first Europe-wide one on the social impact of rare diseases, carried out among 3,000 patients and carers in the context of the EU funded project InnovCare (EaSI).

Key results from this survey show that rare diseases have a serious impact on everyday life and imply a significant time and care burden for patients and carers. Challenges relate to self-care, domestic tasks, leisure, transport and mobility, as well as financial stability, with rare diseases having a particularly severe impact on work-life balance causing absence from work, reduction in professional activity and increasing the risk of loss of income.

Key figures

- **More than 70%** of patients have **difficulties with daily activities and tasks** such as preparing meals and handling household chores**, with motor and sensorial functioning** such as visual, hearing and body positioning issues, **and with social life** as maintaining relations with others.

- **30%** of carers spend **over 6 hours a day on disease-related tasks** with over **60% of these carers being women.**

- Having to **reduce or stop professional activity** due to illness occurs to **70%** of rare disease patients and carers.

Lise Murphy, EURORDIS volunteer and ERN mentor, shared her own testimony of the challenges she and her family faced due to the rarity of their condition, mirroring the key survey results. In particular, she highlighted how these difficulties are linked to **a health care and social services system that is not adapted to rare diseases**, often leaving patients wandering in search of a specialist, a diagnosis, and someone in the social services who can understand their condition and look at it in a holistic manner.

Martin Seychell, Deputy Director General of DG Sante (European Commission (EC)), shared his perspectives in a talk entitled 'EU policy for rare diseases: what still needs to be done to improve patients' daily life'. Rare diseases have been and will continue to be a strong priority for the EC which supported the setting up of Orphanet 20 years ago, encouraged the development of National Plans for rare diseases in Member States and the establishment of 24 ERNs. The next big step for the EC is to disseminate and scale up best practices on cooperation. There is strong momentum for closer collaboration in research with the European Joint Programme (EJP) being launched in 2018, and in clinical care with the ERNs harnessing the potential of knowledge and data. In addition, the added-value of cooperation is not just demonstrable in clinical aspects, but also in social services and support to patients and carers. In fact, his key message was that **"there is a need to move from best practices to standard practices"** and this is one of the activities that the new Steering Group on Prevention and Promotion is undertaking.

On the topic of ERNs, **Yann Le Cam, CEO of EURORDIS,** mentioned the need to make them a 'one-stop-shop' for patients, following an integrated approach of 'case

management' that encompasses the clinical, medical and social aspects. In addition, he stressed the need to engage in systematic registration of patients, and ERNs being an opportunity to do this.

For the rest of his presentation as panellist, Yann Le Cam focused on the rationale behind the choice of the topic for the launch of the Parliamentary Advocates for Rare Diseases. As the RareBarometer survey reveals, rare disease patients are affected by **accumulative vulnerabilities that go beyond the field of health and enter the realm of social protection and social justice**. Considering the current international framework, with the adoption of the United Nations 2030 agenda, its Sustainable Development Goals (SDGs) and its bearing principle to 'leave no one behind', Yann Le Cam emphasised the need for the rare disease community, an often invisible population, to mobilise more than ever and demonstrate its relevance in this global ecosystem, and to engage committed policy makers in all fields that can advocate for this holistic approach.

During the exchange of views, **Dorica Dan, Member of EURORDIS Board of Directors,** stressed that we are in a good moment when all elements are coming together i.e. evidence on the situation of rare disease patients through these survey initiatives, the EC Expert Group recommendations on Centres of Expertise and the recommendations on the incorporation of rare diseases into social services and policies, and the ERNs; but that there is a need to better assess the situation on the ground. In the same line, **Victoria Hedley, RD-ACTION,** highlighted that achieving such recommendations was the fruit of painstaking discussions until agreement by all Member States and it is time to think how the newly created network can support the implementation of these. This comment emerged particularly in the context of a general worry in the audience by the lack of a forum for all stakeholders to discuss with the Member States since the dismantlement of the EC Commission Expert Group on rare diseases. Martin Seychell indicated that the governance of the ERNs in time may be able to offer such platform for discussion.

MEP Marek Plura commended the launch of the Parliamentary Advocates for Rare Diseases as the fact that both Members of the European Parliament and of the different national Parliaments can integrate this network and discuss is key. In his view, national parliaments have a crucial role to play when it comes to translating EU recommendations into national practices.

MEP Kateřina Konečná, who has been very engaged with patients groups in the Czech Republic, attested of the difficulties faced not only by patients but also families and society as a whole and hoped for the achievement of concrete steps politically through the cooperation of the ENVI Committee at the European Parliament and the European Commission.

Terkel Andersen, President of EURORDIS, gave the closing remarks to the event reminding the participants of the particularity and unique aspects of rare diseases and how the event's mission had been to highlight the daily challenges faced by this group of European citizens and how therefore, solutions required action at the European level. In particular, he alluded to the Council Conclusions of June 2016 which called upon the EC to assess how far we have come in implementing the 2009 Council Recommendation on an action in the field of rare diseases and to ensure that Member States adapt their national plans and strategies to new initiatives like the recently launched ERNs; an assessment that the rare disease community deems absolutely necessary.

Contact the EURORDIS team to find out more about becoming a member

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Parliamentary Advocates for Rare Diseases

List of Members of the European Parliament who expressed their interest to engage in the Network of Parliamentary Advocates for Rare Diseases

Françoise Grossetête

European People's Party (EPP)
France

Brando Benifei

Group of the Progressive Alliance of Socialists and Democrats (S&D)
Italy

Frédérique Ries

Group of the Alliance of Liberals and Democrats for Europe (ALDE)
Belgium

Seb Dance

Group of the Progressive Alliance of Socialists and Democrats (S&D)
UK

Elena Gentile

Group of the Progressive Alliance of Socialists and Democrats (S&D)
Italy

Christel Schaldemose

Group of the Progressive Alliance of Socialists and Democrats (S&D)
Denmark

Cristian Bușoi

European People's Party (EPP)
Romania

Marek Plura

European People's Party (EPP)
Poland

Kateřina Konečná

Group of the European United Left - Nordic Green Left (GUE – NGL)
Czech Republic

Elisabetta Gardini

European People's Party (EPP)
Italy

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