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A forward-looking agenda for rare diseases: bringing Europe closer to its people

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Event report

On Tuesday, 20 November, the European Idiopathic Pulmonary Fibrosis & Related Disorders Federation (EU-IPFF) and MEP Elena Gentile (S&D, IT) hosted an event at the European Parliament on November 20, 2018. The event titled, "*A forward-looking agenda for rare diseases: bringing Europe closer to its people*", reviewed what has been achieved on the topic of rare diseases in the past four years and what remains to be done. Ultimately, the event aimed to discuss solutions to improve the quality of life of patients with idiopathic pulmonary fibrosis (IPF).

The meeting brought together thought leaders in the IPF/rare diseases field, including EU policy-makers, healthcare professionals, patients' organizations and international advocacy groups. Speakers included MEP Elena Gentile, EU-IPFF President Carlos Lines Millán, EU-IPFF Chair of the Scientific Advisory Board Francesco Bonella, Member of the Cabinet of Health Commissioner Andriukaitis Annika Nowak, Policy and Programme Officer at DG Research and Innovation Iiro Eerola, Chair of the ERNs Coordinators Group Franz Schaefer, and EU-IPFF Board Member and Chair of Action for Pulmonary Fibrosis Steve Jones.

MEP Elena Gentile (S&D, IT) opened the meeting by advocating for the EU to play a more important role in supporting research and innovation and eliminating inequalities in access to medicines, health and social care between and within Member States. She acknowledged the support of her colleagues during the last legislative cycle, especially the 388 MEPs who signed the Written Declaration on Idiopathic Pulmonary Fibrosis (2016), thus raising awareness of a disease unknown to most people. MEP Gentile also encouraged the future legislature to build upon the progress achieved so far, and continue to invest in research and cross-border cooperation to improve the lives of patients with rare diseases.

On the patient-side, ***Carlos Lines Millán, President of EU-IPFF***, acknowledged the important role that the EU Institutions play in addressing the challenges and needs of IPF patients. He also underlined the importance of implementing concrete actions to achieve the objectives expressed in the Written Declaration on IPF. Mr Lines Millán finished his presentation with a short introduction of two projects that were presented during the event- *the EU-IPFF Position Paper on the Patient's Journey* and the EU-IPFF Benchmarking Report - and called on future European leaders to continue working on improving the quality and life expectancy of patients with IPF.

Annika Nowak, Member of the Cabinet of Health Commissioner Andriukaitis, provided up-to-date insights into the 24 European Reference Networks (ERNs), which connect physicians specialized in rare and complex diseases. Thanks to these networks healthcare professionals can now collaborate with their cross-border peers to improve diagnosis and care for rare disease patients. Ms Nowak presented the key achievements of the ERNs, but also the challenges they are facing in the current deployment phase, including the incomplete integration of the networks into the national healthcare systems, inter-operability issues and

limited human resources available for ERN-related initiatives. Ms Nowak further outlined other Commission initiatives that aim to support the diffusion of high-quality knowledge and the exchange of good practices, such as the Steering Group on Promotion and Prevention, the new legislative proposal on Health Technology Assessment (HTA) and the Communication on the Digital Transformation of Health and Care. All these actions put forward by the European Commission aim in particular to support the Member States' strategies on reforming their national health systems.

Mr Iiro Eerola, Policy and Programme Officer at DG Research and Innovation, delivered a presentation about EU-funded research contributing to the goals of the International Rare Diseases Research Consortium (IRDiRC). Mr Eerola presented the achievements of IRDiRC in bringing together cross-border expertise to identify and study rare diseases. He also explained that IRDiRC has set itself three main goals for 2021-2027, namely: diagnosis of patients with a suspected rare disease within one year, approval of 1000 new therapies for rare diseases, new methodologies to measure impact of diagnosis and therapies. Mr Eerola further introduced other EU-funded research projects through the 7th Framework Programme for Research and Technological Development or Horizon 2020. At the end of his presentation, Mr Erola highlighted that under the new Horizon Europe programme for 2021-2027, a specific cluster is dedicated to healthcare and one of the six areas of intervention will be non-communicable and rare diseases.

Dr Francesco Bonella, Chairman of the EU-IPFF Scientific Advisory Board, talked about the *EU-IPFF Position Paper on the Journey of Patients living with Idiopathic Pulmonary Fibrosis (IPF) and other forms of Pulmonary Fibrosis (PF)* which is one of the main projects the EU-IPFF worked on in 2018. The key objectives of this project are to increase and share knowledge on IPF / PF care pathway, and to shed light on the unmet needs of patients with these rare conditions. Dr Bonella also explained that the Position Paper was developed on the basis of a literature review and two surveys shared amongst healthcare professionals (HCPs), and patients and carers. He then presented the main findings for the five areas that have been assessed in the Position Paper: the overall interaction between HCPs and patients, diagnosis and referral, access to pharmacological treatment (specific to IPF patients), access to non-pharmacological treatment, and research and clinical trials.

Mr Franz Schaefer, Chairman of the ERNs Coordinators Group, delivered a presentation on how research and development initiatives can be scaled-up within the ERNs. Mr Schaefer provided an update into the wide range of activities of the ERNs, including developing new disease guidelines, monitoring performance and patient outcomes, as well as setting up a virtual consultation system that would allow healthcare professionals to provide via an online platform expert advice on challenging cases. Mr Schaefer also talked about the emerging European Rare Disease Registry Framework, which is a core registry of five ERNs that will provide information about disease demographics, and will help to identify patients who might be eligible for clinical trials. Mr Schaefer highlighted that despite the many challenges that lie ahead, the ERNs represent a success story of what the EU Institutions are doing for the health and welfare of the European citizens.

The final speaker, ***Mr Steve Jones, EU-IPFF Board Member and Chair of Action for Pulmonary Fibrosis,*** introduced the *EU-IPFF benchmarking report*, which compares how European countries are performing on different areas of IPF care and management, identifying

best practices and gaps where urgent policy action is needed. For the report, 16 patient organisations from 13 EU Member States were interviewed in order to collect data on diagnosis and access to specialised care, access to pharmacological and non-pharmacological treatments, and access to palliative care and end-of-life care. Mr Jones presented the main results of the interviews, which were fed into a European scorecard illustrating country performances according to specific indicators. Mr Jones also called upon current and future European and policy-makers to ensure that funding is earmarked for rare diseases in the future European research programmes, as well as for patient groups to collaborate and capacity-build, along with greater support for the integration and sustainability of the ERNs.

During the subsequent discussions, MEPs ***Tilly Metz (Greens, LUX)*** and ***John Procter (ECR, UK)*** took the floor to reiterate their support for IPF patients. They also acknowledged the central role played by the European institutions in bringing all the relevant stakeholders together.

In addition, patients also had the opportunity to provide their perspective on the most important challenges and needs that they face in their daily lives. They highlighted the importance of setting up support groups and the need to close the gap between prevalence and incidence of rare diseases.

During the Questions & Answers session***, Simone Boselli, Public Affairs Director at EURORDIS, Rare Diseases Europe*** called on the European Commission to provide the adequate financial resources for the ERNs, and inquired on the position of the EU Institutions to support holistic approaches to diseases management. Furthermore, ***Dr Francesco Bonella*** highlighted that the compliance rules that are currently in place prevent patients from getting direct exposure to treatment information and clinical trials as they cannot take part in medical congresses where such information is presented. ***Bernard Grimm, Director at EuropaBio*** inquired on the progress made in terms of having quality data on rare diseases.

In her reply about the resources allocated to the ERNs, ***Ms Annika Nowak*** confirmed that there will be funding available from the ESF+, Horizon Europe and Digital Europe programmes, but acknowledged that increased funds would be necessary to implement all the envisaged activities. With regards to the compliance rules, ***Mr Franz Schaefer*** informed the audience that discussions have been re-started on this topic and a proposal was put forward to create a blind fund where industry could contribute to support the ERNs, and an independent committee would review how these funds are distributed. On the same topic, ***Mr Iiro Eerola*** outlined that as part of EU-funded clinical trials projects, dissemination activities for patient organisations are usually foreseen. On quality of data, ***Mr Franz Schaefer*** acknowledged that systematic data collection in rare diseases is still in a very early phase, and more funding was requested for the creation of patient registries. In addition, the ERNs are currently working with EURORDIS on finding ways to identify what is relevant for patients and what their real needs are.

The meeting was closed by ***Carlos Lines Millán, President of EU-IPFF,*** who encouraged the European institutions and the Member States to continue working together to improve the quality and life expectancy of patients with IPF. Although progress on rare diseases has been made during the current legislature, many challenges still lie ahead, so it is important that future EU leaders continue promoting initiatives that lead to tangible outcomes in the field of rare diseases.