

2<sup>nd</sup> International Conference on

# Rare Diseases: Greek Chapter

The Balancing Act between Equity and Sustainability

## PRESS RELEASE

**2nd International Conference on Rare Diseases: Greek Chapter**

**Balancing between Equality and Sustainability - Athens, 1,2 March 2022**

### **"RARE DISEASES: THE IMMEDIATE PRIORITY OF PUBLIC HEALTH"**

**57 Distinguished speakers and 450 delegates from 30 countries participated in the 2<sup>nd</sup> International Conference on Rare Diseases: Greek Chapter**

Athens, March 2, 2022

Following this year's World Rare Diseases Day, Association "95", Hellenic Alliance for Rare Patients and Health Daily, BOUSSIAS organized **online the 2nd International Conference on Rare Diseases: Greek Chapter** on March 1-2, 2022, with the participation of **57 distinguished Greek and foreign speakers and more than 450 participants from 30 countries** around the world. Following last year's success, the conference has attracted the attention of the international community this year focusing on: **"Balance between Equality and Sustainability"**, taking into account the recent United Nations Resolution. The conference was held under the auspices of the European Parliament and EURORDIS - Rare Diseases Europe.

The conference highlighted the need to promote and protect the human rights of all people, but especially those living with a rare disease estimated at 300 million worldwide, many of whom are children. The need to analyze the deeper causes of inequality and discrimination faced by people living with a rare disease was emphasized, as well as the need for policies and programs aimed at eliminating "stigma" in healthcare settings, to ensure holistic treatment approach to the patient with universal and equal access to quality health services, without financial difficulties. The conclusions stressed that tackling problems ranging from diagnosis and care, research and innovation, to access to innovative medicines for patients with Rare Diseases, requires coordination and participation. Greece and the Health System must face the challenges, but also take advantage of the opportunities and possibilities that are presented.

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The government was represented by the Minister of Health **Mr. Thanos Plevris**, the Minister of Labor **Mr. Kostis Hatzidakis**, **Ms. Mina Gaga**, Deputy Minister of Health and **Ms. Pavlina Karasiotou**, Secretary General of Social Security, the Ministry of Health and the Ministry of Social Affairs **Mr. Dimitris Papadimoulis**, Vice President of the European Parliament, **Mr. Stelios Kypouropoulos** MEP-psychiatrist, **Norbert Couspel**, Chair, Future-Proofing Health Systems Committee, European Health Parliament, 7th edition, **Tomislav Sokol**, MEP, EPP Group in the European Parliament and **Maria da Graça Carvalho**, Member of the European Parliament.

The opening of the Conference was announced by the coordinators **Dimitris Athanassiou**, EMA Pediatric Committee, Member of the Board. EPF, WDO, EAE and **Vassilis Karatzias**, President, Hellenic Association Ataxia Friedreich, Director of the Office of Legal Advisers, NATO Rapid Deployable Corps, Greece who welcomed the Minister of Health, **Thanos Plevris**. In his inaugural address, the Minister stressed that the Ministry of Health is going to keep the promises it has made to the representatives of people with rare diseases, which were delayed by the need to deal with the pandemic. He reiterated that Greece has set as its priority to develop the country's health system, to provide equal access to health and universal coverage to all citizens and of course total coverage and support to people with rare diseases and their families.

"The participation of people with rare diseases in society, with equal rights and without discrimination, is the cornerstone of our society, the very soul of our culture," stated in her speech **Mary Adamopoulou**, president of "95" Rare Alliance Greece.

"The treatment of rare diseases is a big bet that must be won", said Deputy Health Minister **Mina Gaga**, adding that the above must be achieved by all parties involved: State, patients, payers, insurance companies, medical community, academia, in a health system that is sustainable. She stressed that the beginning will be made with the digital file and patient records as a first step to know where we are in relation to rare diseases. She added that the ministry is interested in the European Reference Networks and seeks to participate, while setting as a priority the training of doctors on rare diseases, so that they can recognize and categorize them. Asked about the creation of a National Action Plan for Rare Diseases, she answered that this is a project that requires time, education, specific centers with a defined budget, a plan of overall support for patients and their families, as well as many more partnerships. However, she added, the first steps can be taken within the year.

The role of IFET in meeting the needs of people with rare diseases in Greece was developed by the CEO of the Organization, **Giannis Sotiriou**, who added that in 2021 triple quantities of drugs were distributed compared to 2020 to patients with rare diseases such as cystic fibrosis and Wilson disease. He underlined the difficulties of diagnosis, clinical trials and supply chain challenges presented by rare diseases, a fact that requires innovative solutions, which in 2021 IFET carried out regarding cystic fibrosis, with price negotiations, increase of credit limits, the

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need for electronic connection with the other institutions but also the activation of IFET in the field of research in order to be able to provide the necessary treatments.

Greece has a satisfactory pace regarding the entry of new drugs for rare diseases, having from 2016-2019 brought 30 of the 50 drugs circulating in the EU, stressed the General Director of SFEE, **Michalis Himonas**. However, he added, only 19% of these drugs are available and most of them are imported by IFET, while in other countries these drugs are imported only through risk-sharing agreements. He said the country needs digital tools to measure spending, innovative ways of evaluating and financing products and a review of pharmaceutical spending by the State, especially in hospitals.

In Greece, it is estimated that there are 350,000 to 590,000 patients with rare diseases. Taking into account the family members and their caregivers, there are more than 1,500,000 people in our country who are directly or indirectly affected by rare diseases. The above was mentioned by **Nikos Kostaras**, General Manager of IQVIA Greece. As he mentioned, rare diseases result in high mortality, and disability. This leads to low quality of life for the patient, increased psychological, financial, and social burden for his family, increased public spending and use of resources for the Health and Social System Insurance. Patients with rare diseases do not have accurate and timely diagnosis, up-to-date and reliable information and support, optimal disease management by interdisciplinary teams in specialized centers of excellence and access to innovative, effective and affordable treatments. The development of long-term and effective National Action Plans, from prevention to care, is essential for building sustainable and resilient national health systems. Early access to appropriate services results in reduced mortality, disability and resource utilization that ensure sustainability for health systems and improves the quality of life of patients and their families. Nikos Kostaras stated that IQVIA is launching a major project at the request of the "95" organization, with the aim of a clearer picture of the situation in Greece regarding rare diseases. The project concerns the production of data for decision making for rare diseases in Greece. The main objectives are the creation of data and the development of a strategy for rare diseases in Greece, which will ensure access to optimal care and treatment for all patients living with Rare Disease in a sustainable and resilient Health System. It includes, collection and analysis of Real Data for Rare Diseases in Greece based on Quantitative and Qualitative Methodology, Strategy Development, Policy Recommendations, Roadmap of National Action Plan for rare diseases, from prevention, diagnosis, care, research and access to new treatments.

It is important for KESY to examine all medical specialties to make sure that there will be adequate training, because rare diseases usually require many specialties, stressed the president of KESY **Dimitris Boubas**. He also stated that there is a need to disconnect the expert groups operating in hospitals-centers for rare diseases from the hospital budget, and to be financed from the budget of the Ministry of Health.

The legislative, regulatory and political environment does not give the academic community separate tools and procedures to help shape a new framework, so the issue of rare diseases and their treatment remains only a political decision, taken either through a ministry or

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through IFET, pointed out **Athanassios Vozikis**, Associate Professor of Economics and Health Management at the University of Piraeus. He added that other countries have separate procedures for the evaluation of orphan drugs, either by raising the threshold (eg .00 100.00 - NICE) or by evaluating quality-adjusted life years or by using multi-criteria analysis and not just economic criteria.

"To talk about equality in social benefits, we must develop a system for assessing disability", noted **Pavlina Karasiotou**, Secretary General of Social Security. She added that the development of support policies for disability and illness requires that there be no fragmentation of responsibilities, an inherent pathogen of the public system. She stressed that the entire regulation of the operation of KEPA has progressed, something that has not existed since their establishment. Rare diseases have also been included in the regulation. Not all rare diseases can be included in the disability assessment table, due to the large number of cases. Also imminent is the signing of the government ministerial decision that abolishes the "threshold" of 67% to be considered a rare condition irreversible from the second crisis onwards.

During his speech, **Dimitris Papadimoulis**, Vice President of the European Parliament, noted: "Immediate strengthening of cooperation in the EU, through European programs and initiatives for research and development. As a European family, we cannot be indifferent to the 6,000 rare diseases with 300 million patients worldwide." Mr. Papadimoulis also spoke about common policy guidelines regarding the Centers for Disease Control, disease research and detection, and the improvement of patient identification.

"This is an important time to talk about rare diseases viability," said **Nathalie Moll**, Director General of the European Federation of Pharmaceutical Industries and Associations (EFPIA). As she mentioned, Europe was slower compared to the USA, Japan, etc. to recognize the issue of rare diseases, perhaps due to the complex environment that is taking shape in Europe, with many countries, different research centers and the small number of people with rare diseases being spread across different countries. Ms. Moll underlined that after the European Parliament, the pharmaceutical industry, as well as all other stakeholders in health, must create a clear and sustainable framework for rare diseases. The EU must provide a stable legal framework to facilitate investment in drug research and development. "We also need to work more closely with patient groups, hospitals and national authorities", Nathalie Moll concluded.

The great importance of this year's international celebration of the World Rare Diseases Day, with the lighting of over 100 emblematic buildings around the world, was pointed out by **Yann Le Cam**, Chief Executive Officer, EURORDIS-Rare Diseases Europe. The reason is that for the first time the issue of rare diseases is becoming "visible" in so many countries, with information and awareness actions. He then hailed as extremely important the fact that 27 associations of patients with rare diseases in Greece were officially united in the national Rare Disease Greece delegation. He described as particularly important the adoption of the Resolution on Rare Diseases by the UN General Assembly. In closing, he spoke about the

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important steps that the European Parliament has recently made towards a common European strategy on rare diseases.

The need for holistic and personalized care for every patient with a rare condition in all European countries, stressed the MEP-psychiatrist **Stelios Kypouropoulos**. By the end of 2022, there will be a sustainable framework for tackling rare diseases in Europe, the MEP said, stressing once again the importance of patient involvement in decision-making and calling on all stakeholders to support equal diagnosis and treatment for patients with rare diseases.

"Cooperation" between all stakeholders as well as Member States is the key word in Europe for finding solutions to rare diseases, said **Andrzej Jan Rys**, Director for Health systems, medical products and innovation responsible for Directorate B in DG SANTE, to the European Commission. The European Commissioner also stressed the important role of data both in developing new therapies and in evaluating existing ones. Regarding Orphanet, Mr. Rys said that the high-quality information on rare diseases that he has, ensures equal access to knowledge for all stakeholders and makes patients visible in the Health Systems of each country and therefore will be supported in Greece.

The unmet medical needs of patients with thalassemia in 48 countries around the world, brought to light by a recent report, were mentioned by the Executive Director of the International Thalassaemia Federation (TIF), Dr. **Androulla Eleftheriou**.

The significance of the recent United Nations (UN) Resolution on Rare Diseases was mentioned by **Flaminia Macchia**, Executive Director, Rare Diseases International. According to her, the three bodies that played a key role in the resolution were Rare Diseases International, the MKO Committee for Rare Diseases and Eurordis. The countries at the beginning of discussions are Spain, Brazil and Qatar. It is the first UN text to call for action to address the challenges that Rare Patients and their families face on a daily basis and it includes human rights, quality and equal access to quality care while ensuring global legitimacy in all is done at national level. It is supported by the 193 member states of the Organization and has sponsors 54 countries including Greece. "Equality means doing something for those who have less," said Macchia, adding that Rare Diseases International was also aiming for a resolution from the World Health Organization that is likely to take place in May 2024.

**Alba Alconchea Diaz**, Member of the BoD, Eurordis, CEO of Feder, referred to the significant disparities in the treatment of patients with rare diseases both between EU Member States and between regions within the same country.

**Alexandra Pacurariu**, Scientific Administrator, Data Analytics and Methods Task Force, European Medicines Agency (EMA) presented the important role of the federal data network, expertise and services, DARWIN EU, in rare diseases.

A very optimistic note on the second day of the conference was given by the Minister of Labor **Kostis Hatzidakis**, pointing out that in Greece, after close communication of all parties, the ministry took measures to support people with rare diseases in their lives and work. The



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Minister also stated that in cooperation with the Ministry of Digital Policy, the entire architecture for disability certificates is being revised, with the possibility of having only one digital certificate for people with rare diseases that certifies their condition and is used everywhere, in order to reduce bureaucracy and make their lives easier.

The dynamic landscape, with the introduction of many new drugs and the entry for the first time of many foreign companies is evolving lately in Europe, was stressed by **Alexander Natz**, Secretary General, European Confederation of Pharmaceutical Entrepreneurs (EUCOPE). He added that there has been a lot of discussion about how pharmaceutical companies should turn in new directions and cover the diseases that have not been treated so far. He stressed that three important steps could help in this direction: the use of real-world evidence (RWE), the cooperation of all national health systems, with the aim of compensating medicines for rare diseases and finally the existence of a European HTA that support the entry of many more new drugs.

**Simone Boselli**, Public Affairs Director, EURORDIS – Rare Diseases Europe, presented the actions of the Rare Impact initiative which works to improve patients' access to advanced medicines and innovative treatments.

Finally, **Fernando Arnaiz**, Global Access & Healthcare Funding & Financing Solutions Lead, Roche, said that Roche's team is working on adequate funding to ensure that patients with rare diseases have access to innovation.

The 2nd International Conference on Rare Diseases: Greek Chapter, was held with the support of major international organizations, such as the European Federation of Pharmaceutical Industries and Associations (EFPIA), the European Gastrein Health Forum (EHFG), the European Health Organization (EF) Confederation of Pharmaceutical Entrepreneurs (EUCOPE), the Institute of Pharmaceutical Research and Technology (IFET), the Hellenic Patients Association, and the Association of Pharmaceutical Companies of Greece (SFEE).

Gold Sponsor of the Conference was Takeda. Major sponsors were Novartis Gene Therapies and Roche. Sponsors were Alexion, Chiesi, Genesis Pharma, IQVIA, Pfizer and PTC Therapeutics. Supporter was Ardius Pharma.