TYPE-APPROVAL

1st International Conference on Rare Diseases
"Building the path from Diagnosis to Access"

60 Distinguished speakers and 650 participants from 37 countries took part in the first International Conference on rare diseases held live from Greece

Athens, 9 March 2021 - The **1st International Conference on Rare Diseases (Greek Chapter)**, held online March 1 and 2 2021 focusing on: "Building the path from Diagnosis to Access" was completed with great success. The event coincided with awareness-raising activities on the occasion of World rare diseases Day which takes place on 28 February annually.

The Conference was organized by the "95" Rare Alliance Greece and Health Daily of BOUSSIAS, and hosted for the first time more than 60 distinguished speakers, who elaborated on the latest developments, strategy and important new data on research, new innovative therapies and policies promoted at a European level for the treatment of rare diseases, giving rise to further dialogue and analysis of the needs that exist in our country.

The online conference was attended by more than 650 participants from 37 countries around the world (Europe, USA and Asia), including health professionals, researchers, academics, policy makers, industry representatives and patients. For the first time, the event attempted to capture the entire journey that rare patients experience from diagnosis to access to the treatments and care they need.

The aim of the Conference was, through the exchange of knowledge, ideas, and proposals, to discuss appropriate practices and policies that will help health systems better respond to the needs of the **30 million people living with a rare disease in Europe**, while **in Greece it is estimated that rare diseases affect 350.000 to 600.000 citizens,** although accurate data is not available.

95% of the 6.000 – 8.000 known rare diseases have no treatments, **50%** of the patients are children with **30**% of them not reaching their **5th birthday,** while rare diseases are responsible for **35%** of infant deaths within the **1st year of life.**

The conference proceedings were inaugurated by **Dimitris Papadimoulis**, **Vice-President of the European Parliament**, who stressed the fact that: "*Although important steps have been taken in the EU, we are still a long way from a common European health policy and there is a lack of specific health policies for rare diseases. It is necessary at European level to strengthen cooperation between European programs and initiatives on health, research and technological development. The EU also needs to encourage and support Member States in developing national health strategies to ensure equal access and availability in the areas of prevention, diagnosis, treatment and rehabilitation of people with rare diseases*", adding that the 1st International Conference on Rare Diseases can become a starting point for a more permanent cooperation between the European Parliament and patients with rare diseases to help advance needed actions.

The **European Commission**, **Vice-President Margaritis Schinas**, during his opening speech referred to the initiatives taken by the European Commission to enhance the level of care for the citizens of Europe in general and for patients with rare diseases in particular. As he noted, in the last 12 years Europe has given over 1.4 billion euros for the development of 200 innovative medicines, and the European Rare Diseases Program is a remarkable example of the optimal finding and use of national and European resources.

The President of the "95" Rare Alliance Greece, Mary Adamopoulou, called on all interested parties involved in the field of rare diseases for cooperation, aimed at better prevention, diagnosis and treatment of patients affected by this disease group. "We call on the government to work with us and implement a national strategy on rare diseases before the end of 2021. We call on the government to

include trained patient representatives as equal partners in the decision-making of rare diseases policy," she said.

From the part of the Hellenic Government participated the Secretary General for Coordination of the **Presidency of the Government, Thanasis Kontogeorgis**, who stressed the government's and the competent Ministry's intention to create as soon as possible a national plan in Greece for the treatment of rare diseases. The **Deputy Minister of Development and Investments, Christos Dimas**, assured that Ministry's aim is to promote essential tools and policies that strengthen the innovation ecosystem, and to invest and support research on rare diseases.

Speaking about funding opportunities, the **coordinator of the European Joint Programme on rare diseases (EJP RD), Daria Julkowska**, stressed that: "*It is extremely important to have this contact both at a national, European and international level, so that together they can support and promote research*." She also called on the Hellenic Ministry to make use of European financial tools towards this goal.

Especially supportive towards the rare diseases' community was **MEP-Psychiatrist Stelios Kympouropoulos** during his opening remarks at the second day of the conference declaring that: "*Rare patients are also human, and medical needs are only a part of our lives, we want to ensure a decent living. The right to life and participation of patients with disabilities must be protected, and I hope that the quality-of-life factor will be a priority in policy-making in the future*." He also stressed the need for a minimum list of rare diseases for neonatal screening tests in each member state.

MEP Tomislav Sokol added his perspective on what the European Union needs to do in order to solve some of the problems faced in this area, adding that: "*I strongly believe that cross-border healthcare & centres of excellence can solve some of existing problems with whom rare disease patient are facing across whole EU*". In addition, he spoke about the need of Health Technology Assessment Organisations, of all member states, to be governed by common rules and the need for an EU legislation to help simplify procedures for cross-border healthcare.

Yann Le Cam, CEO of EURORDIS-Rare Diseases Europe, concurred, saying: "*There is a pressing need to reshape European strategy for rare diseases, as emerged from the multi-stakeholder Rare 2030 foresight study. We need to ensure that the future of 30 million people living with a rare disease is not left to luck or chance, by harmonising national policies for rare diseases with the same measurable objectives, providing better access to treatment, and developing sustainable healthcare pathways, from local to the European level. We, therefore, encourage continued investment in the field of rare diseases at both the European and national levels to ensure we do not lose momentum*". Moreover, he stressed how important is to have a new platform in Greece, "95" Rare Alliance Greece and welcomed the support and attention of the Hellenic government and the public administration towards the rare patient community.

Nathalie Moll, Director General of EFPIA spoke of a different type of conversation between patients, industry, health systems and governments to collectively discuss how to ensure access to new treatments and technologies today, medical innovation for tomorrow and sustainable healthcare systems in a globally competitive Europe. " *We share two aspirations with the rare disease community: to ensure faster, more equitable access to treatment for people living with rare disease in Europe and to meet the needs of people living with rare conditions where treatment options are suboptimal*".

Olympios Papadimitriou, President of the Hellenic Association of Pharmaceutical Companies (SFEE), stressed that "*governments and the pharmaceutical industry must find the appropriate balance of funding of treatments and sustainability of the systems though collaborative approaches and the participation of patients, so that existing treatments are available to patients and so that pharmaceutical research can continue to develop treatments for the hundreds of rare diseases that currently do not have one"*.

Finally, the important role of the **Institute of Pharmaceutical Research and Technology (IFET)**, the body responsible for the introduction of orphan medicines in Greece, was highlighted by **CEO Giannis Sotiriou**,

who stressed that: "The Agency is trying to find ways to tell patients with rare diseases that the fact that their disease is rare does not mean that they will not have the best possible care, and to offer them answers and hope".

Among final conclusions, a number of issues were summarised including the problems from diagnosis and care, research and access to innovative medicines for patients with rare diseases, which require greater coordination and stakeholder participation. The Greek Health System must immediately cover the distance that separates it from the rest of Europe, to face the challenges, but also to take advantage of the opportunities and possibilities that are presented.

The Patient Community and the "95" Rare Alliance Greece called for:

- **The creation of a high-level patient-centered Think Tank with active participation and representation of all stakeholders, with the aim of developing the National Strategy for rare diseases.**
- **The review and implementation of the National Action Plan for Rare Diseases by the end of 2021, with specific qualitative and quantitative objectives for the development and improvement of the institutional framework in Greece, in order to ensure prevention, accurate and timely diagnosis, immediate and unhindered access to optimal care and treatment for all Greek citizens with rare diseases.**
- **Design and implementation of a long-term sustainable political, economic and technical investments development model for rare diseases by the end of 2022, in line with European Policies, with the aim of promoting research, attracting clinical trials and research funding, yielding significant benefits for patients, the National Health System and the Greek economy as a whole.**

Before the end of proceedings, the organizers announced the organization of the **2nd International Conference on Rare Diseases - Greek Chapter to be held on 28 February and 1 March 2022.**

The 1st International Conference on Rare Diseases was held with the invaluable support of major international organisations, such as EURORDIS, EFPIA, EUCOPE, EuropaBIO, while in Greece the Hellenic Patients Association of Patients, the Institute of Child Health, the Laboratory of Genetics of the National Kapodistrian University of Athens, the Hellenic Association of Pharmaceutical Companies (SFEE), the Institute of Pharmaceutical Research and Technology (IFET) provided their support and from Cyprus the Cyprus Alliance of rare diseases also supported the event.

Gold Sponsor of the Conference was Takeda. Grand Sponsors were Alexion, Biogen, Chiesi, Novartis, Pfizer, PTC Therapeutics and Roche. Sponsors were Genesis Pharma and Healthink. Supporters were Brain Therapeutics, CSL Behring, Innovis, Sanofi and Specialty Therapeutics.

ADDITIONAL STATEMENTS

Alexander Natz, Secretary-General of EUCOPE

"*Cooperative and multi-stakeholder initiatives are key to develop effective and evidence-based policies. Hence, they are an integral part of EUCOPE's work. Our organisation is co-leading and taking part in several platforms gathering partners around the entire lifecycle of rare disease therapies. The core objective and mission of these initiatives is improving the life of people living with rare diseases and developing solutions that are centred around their needs*".

Bernard J Grimm, Healthcare Biotechnology Director, EuropaBIO

"*Working together to foster the rare diseases ecosystems in Europe is the only way forward. It means strengthening the communities of care from research to patient care and powering the innovation engine with strong biotechnology SMEs*".

Albert Bourla, Chairman and Chief Executive Officer, Pfizer

"The Covid-19 pandemic was and is a unique threat to global health in our century, a threat not only to those who are sick or dying from the virus, but also an indirect threat to people with serious illnesses such as patients with rare diseases. The Pfizer-BioNTech vaccine took just 248 days for regulatory authorities to approve. This is clearly a top success, but at the same time, it raises an essential question: If we can achieve so much for Covid-19 infection, why not for other diseases? Only 5% of the 7,000 rare diseases have an approved treatment option. This gap in care leads to new approaches, which can change the lives of thousands of people. Gene therapies can transform the global healthcare landscape worldwide in the coming years, removing significant weight from health systems. National health policy frameworks will need to change to adapt to developments so that vulnerable populations, such as those with rare diseases, have access to innovation".

For more information about the Conference:
Natalia Toubanaki, Tel: **+306947936708, e-mail: ntoubanaki@boussias.com**
For more information on rare diseases "95" Rare Alliance Greece:
Elina Miaouli, Tel: **+306977263301, e-mail: rarealliance.gr@gmail.com**