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3rd International conference on
Rare Diseases: Greek chapter

Leveraging the momentum for a comprehensive rare disease strategy

RARE DISEASE DAY®

PRESS RELEASE

3rd INTERNATIONAL CONFERENCE ON RARE DISEASES: GREEK CHAPTER

RARE DISEASES: THE IMMEDIATE PUBLIC HEALTH PRIORITY

Athens March 6, 2023

As a follow-up to this year's World Rare Disease Day, organized by Rare Diseases Greece (RDG), "95" Rare Alliance Greece and Boussias organized the 3rd International Conference on Rare Diseases: Greek Chapter at Technopolis City of Athens Gasholder 1 – Auditorium "Miltiadis Evert", on **February 28 (Hybrid) & March 1 (Digital)**, 2023 with the physical presence **of 28** and the online presence **of 40 distinguished Greek and foreign speakers,** and more than **350** participants from **20 countries around the world** . After last year's success, the Conference, which was held under the auspices of EURORDIS - Rare Diseases Europe, gathered the interest of the international community this year by focusing on the: **"Need for an integrated strategy for Rare Diseases".**

The opening of the Conference was announced by the coordinators **Dimitrios Athanasiou,** President RDG, EMA Pediatric Committee, EPF, WDO, EAE Board Member, and **Vassilis Karatzias**, RDG Vice President, President of the Hellenic Association Ataxia Friedreich, Director of the Office of Legal Advisers, NATO Rapid Deployable Corps, Greece. **Dimitrios Athanasiou** stressed the **need for the State and the involved bodies to make Rare Diseases a National Priority** and to support the efforts being made in Europe to create the European Action Plan. With Rare Diseases affecting up to **5% - 7% of the population, i.e. over 500,000 families, the immediate signing and activation of the National Registry of Rare Diseases** is important, as well as the **renewal and finally the implementation of the corresponding national action plan within 2023.**

The creation of a Patient Registry for Rare Diseases is in progress

The Ministry's actions to support people with Rare Diseases were mentioned by the Deputy Minister of Health, **Mina Gaga**, in her opening speech. As she mentioned, work has already begun on the creation of the Rare Disease Registry with the collaboration of IDIKA, in which pediatric cancers and Cystic Fibrosis have been included, and an effort is being made for the statistical classification of diseases (ICD 10). Referring to the issue of genetic molecular tests to which future parents should undergo, Ms. Gaga said that it is the Ministry's goal to be compensated in the future and to have Special Centers for Molecular Tests, while there is already collaboration with EKPA for genetic testing in neuromuscular and metabolic diseases, while genetic tests are already being made at the "Agia Sophia" Children's Hospital. The Deputy Minister of Health also referred to the need for Primary Health Care to collaborate with Secondary and Centers of Expertise, so that the needs of patients are effectively covered. From his part, the Mayor of Athens, **Kostas Bakoyannis**, in his greeting, stated that: "Unfortunately, we have not devoted the required study, energy and resources to rare diseases, and this must change", while patients with Rare Diseases in our country reaches 600,000 people, 3.5%-6% of the population, and if we also count families, the numbers multiply.

Our country lacks national planning for Rare Diseases

Both the Vice-President of the European Parliament **Dimitris Papadimoulis**, and **Yann Le Cam**, Chief Executive Officer, EURORDIS-Rare Diseases Europe, the President of "95" Rare Alliance Greece, **Mary Adamopoulou,** and the President of the Greek Patients' Association, **Nikos Dedes** emphasized the imperative need for a national Strategy for RDs, with the creation of a national action plan within 2023, which will go hand in hand with the formulation of the European policy for RDs, as well as the creation of Registries and reimbursement of Prenatal Screening. In fact, according to the Professor of Health Economics at the University of Piraeus and Director of the "Health Economics and Management" Laboratory of the Univ. Piraeus, **Athanasios Voziki** , a National Plan for Rare Diseases must primarily ensure social justice, i.e. the removal of all obstacles, so that all patients have the access they need to the appropriate care. As he mentioned, the ever-increasing research activity regarding Rare Diseases in recent years is impressive, but the process from the beginning of a research until an innovative drug reaches patients takes about 12 years. From 2015 to 2021, 20% of medicines approved by the E.U. concerned medicines for Rare Diseases, while in 2022 this percentage reached 25%. In Greece, only 11% of innovative medicines come without conditions, while 40% are imported with conditions. And much of the Innovation does not reach the patients. The time it takes for an innovative medicine to be available to the patient from the moment it is approved by EMA is 450 days. Referring to the pillars on which an integrated National Plan for Rare Diseases should be based, he said that they are five: a) early and valid diagnosis, so that the patient has access to appropriate treatments, specialized doctors, family planning and better management of the disease, b) Equal access and care within the NHS, given that many patients currently have to settle for the best doctor available, due to training gaps in Rare Diseases and face increased costs of tests, treatments and rehabilitation that are not covered, c) Equal access to better care and the importance of ERNs. Today, almost 1500 ERN units operate in 27 Member States and Norway. From the beginning of 2022, Greece joined with 18 Health units in the ERN, 9 in Laiko, 4 in Aeginetio, 1 in Attiko and 4 in the "Agia Sophia" Children's Hospital, d) Cross-border care, i.e. the right of European citizens to care in any EU country with reimbursement of expenses from the country of origin and e) Promotion of Research and Clinical Studies. As Mr. Vozikis noted, at the moment in our country the bureaucracy in clinical research acts as a deterrent to their implementation both in the academic circle and in the NHS hospitals. From the side of the pharmaceutical industry, the President of SFEE, **Olympios Papadimitriou,** proposed that the financing of Rare Diseases should not be included in the total pharmaceutical expenditure, but should be included in a separate expenditure budget, so as to avoid "irrational routes" of entry of these medicines in the country, such as the introduction through the IFET.

The President of Greek National Organization for Medicines (EOF), **Dimitrios Filippou**, referred to the weaknesses of the National Health System to manage clinical studies. As he mentioned, the creation of a platform in which the EOF and the patients will participate, which will record all information related to the studies currently being conducted in the country as well as the next ones, in order to provide the necessary information for the interested parties, is being considered. He emphasized, however, that this is a rather difficult undertaking requiring also the help of the pharmaceutical industry. In addition, the training of Health Professionals in conducting clinical studies is necessary in Greece, which is why we need to

create this culture among Health Professionals over a period of 5 years, so that we may have ambassadors of this culture in the future as well.

The value of prenatal screening and early and valid diagnosis of children - Significant savings from screening programs in RDs

Maria Kalogeropoulou, Head Value Access, Health Policy & RWE, IQVIA Hellas, spoke about prenatal and neonatal screening programs in Rare Diseases, as a tool for a sustainable Health System, as well as for the production of data for decision-making regarding Rare Diseases in Greece. In her speech she analyzed four steps of a strategy development plan for Rare Diseases in Greece, which are: 1. The collection of data on the prevalence of Rare Diseases and the creation of a list of diseases in Greece. 2. The collection of data on the financial burden of Rare Diseases, and the carrying out of a cost analysis of RDs in Greece. 3. The gathering of data on the humanitarian burden caused by the RDs in Greece, by measuring the quality of life and the burden of the patients. 4. The development of RDs awareness actions (eg media releases, white paper, etc.). Based on the above, there will be policy recommendations for RDs and the development of a roadmap for the National Action Plan for RDs in Greece.

In the course of her speech Mrs. Kalogeropoulou presented part of the results of the "Rare Diseases Project " study and mentioned two characteristic examples of RDs: 1. Spinal Muscular Atrophy, from which it is estimated that 190-348 patients (0.003% of the total population) suffer, however the total cost (direct & indirect) per patient over a period of two years amounts to 514,654 euros (versus 7,111 euros/patient per year for diabetes – 10% of the population). This total cost jumps to 98-179 million euros over a period of two years. If a newborn screening program is implemented, the cost savings will be in the order of 106 thousand euros per patient. 2. Duchenne muscular dystrophy: It is estimated that 240-522 patients (0.005% of the total population) are affected, with a total cost (direct & indirect) of 254,053 euros per DMD patient over a two-year period. The total cost over a period of two years jumps to 61-133 million euros.

From the EODY's side, the President **Theoklis Zaoutis** mentioned that the EODY is participating in a pilot program for NGS screening to check infants for Rare Diseases. In the current phase, 1,000 infants are participating and will be screened for over 200 Rare Diseases. The forecasts for the price of screening in 3-5 years from today amount of 100 euros per sample, emphasized the president of EODY, adding that the Organization will be able to contribute to informing the public about the great importance of screening for Rare Diseases.

The Assistant Professor of Pediatric Endocrinology-Juvenile Diabetes at the First Pediatric Clinic of the School of Medicine of the University of Athens at the "Aghia Sophia" Children's Hospital, **Christina Kanaka-Gantenbein**, mentioned, among other things, the value of prenatal screening, and early and valid diagnosis of the children, in order to be able to ensure them a safe and as high-quality future as possible. Accordingly, Professor of Genetics at EKPA **Jan Traeger-Synodinou** emphasized that in the new national Action Plan for Rare Diseases, the necessary Genetic Tests should be prescribed and covered, so that couples and families have the possibility, regardless of their financial situation, to proceed with necessary preventive checks.

Formulation of policies for faster diagnosis, optimal treatment, and more effective follow-up of patients with RDs

Panagiota Mitrou, Head of the Independent Department of Therapeutic Protocols and of Patient Registries at the Ministry of Health, emphasized that the National Registry of Patients with rare diseases is expected to be a valuable tool for the formulation of Health policies. As she mentioned, there is a need for National Registries, as the available information systems for patient records are fragmented, contain duplicate records, and could have security and data protection issues, do not have the possibility of interoperability, nor a wide possibility of processing and analysis, and finally are tailored to the therapist /researcher and not the needs of the patient. The goals of the National Patients' Registry for Rare Diseases are to record all patients with rare diseases who are diagnosed and treated in the Greek territory, to collect accurate, necessary and useful data, in real conditions (real world data evidence). When asked about the progress of the RD Patient Registry, the Director of the Office of the Minister of Digital Governance, **Konstantinos Chambidis**, stated that the RD registry is in a very good place. In fact, in March there will be a temporary contractor, and in the coming months the registry will start working. A second big project according to Mr. Chambidis is the upgrade and interconnection of hospitals' digital systems which will equally help. 70 public hospitals are already interconnected in the data exchange. The rest of the total of 128 hospitals must also connect. In closing, he emphasized that there is a question of changing the mindset regarding the use of data. **Eleftherios Thiraios**, General Practitioner, Director of ESY - Vari Health Center, Head of the Health Services Quality Improvement Directorate - O.DI.P.Y. SA, General Secretary of the Medical Society of Athens, emphasized the necessity of data quality control. Safety and patient needs such as early diagnosis combined with the multi-morbidity accompanying the initial disease, development of digital tools for home care, effective training of primary care physicians, etc., must be taken into account, so that the right 'care pathway' is created. The patient registry is horizontal in nature, but in rare patients there must also be a vertical nature for the interconnection of data at European level. On this finding, **Andri Papadopoulou**, Scientific Officer at the European Commission, Joint Research Centre, Directorate F – Health and Food, JRC.F1 – Disease Prevention, said that the European data collection platform with tools such as cryptographic techniques, search tools for facilitation of clinical and pharmaceutical studies, etc. ensures access to safe and effective treatments by urging the Greek community to participate in the European platform with the appropriate training offered.

Need to create Centers of Expertise for Rare Diseases

The Centers of Expertise for rare diseases are all located in public hospitals and belong to the body of the National Health System regardless of the legal form they may have, said the Secretary General of Public Health, **Marios Themistokleous**. Regarding doctors' training on Rare Diseases, and especially those in Primary Health Care, so that they can easily proceed with a diagnosis and then guide the patient to the appropriate doctor or Center, Mr. Themistokleous stated that 14 million euros from the Recovery Fund will be allocated to the training of doctors with a special emphasis on general practitioners of Primary Health Care. In order to inform patients, he said that a website could be created where all the Centers of Expertise would be listed, an action in which the personal doctor could also contribute. From her part, **Kate Theochari,** President of the Panhellenic Association of Patient & Friends suffering from Lysosomal Diseases "The Solidarity", spoke about the need to create Expertise Centers for Rare Diseases and the participation of Patients in Research, and specifically in Clinical Trials.

The revision of the European pharmaceutical legislation is of key importance
Need to strengthen Innovation and ensure the competitiveness of the EU.

In the last decade innovation in the field of Rare Diseases has made leaps and bounds, transforming the lives of patients and their families, said **Nathalie Moll**, President of the European Federation of Pharmaceutical Industries & Associations (EFPIA) in her opening speech on the second day of the 3rd International Conference on Rare Diseases. Today, 200 innovative treatments are available, and another 1800 medicines are being developed. However, it is not certain that Europe will make progress in this area, as competition from other parts of the world is stiff. Recent research has shown that the number of treatments and clinical trials underway in America is 2 times greater than in Europe, and those in China 3 times. A fact that, as Nathalie Moll pointed out, is worrying for European patients, especially now that the European pharmaceutical legislation is being revised, after 20 years. The new legislation including orphan drugs presents recommendations to limit the definition of unmet medical needs, which should really be defined by patients, and is important both for them and for research. These recommendations carry the risk of interrupting or halting research into various diseases, including rare ones. A correct framework of the new European pharmaceutical strategy could make Europe a world leader in the field of Health, the president of EFPIA stressed. At today's rates, it will take 100 years to find cures for all rare diseases, Nathalie Moll said, which is why the innovative pharmaceutical industry supports «the rare disease moonshot» initiative which, together with other organizations, is trying to bring together private and public bodies, so that more patients have faster access to Innovation. The President of the Committee for Orphan Medicines of the European Medicines Agency (EMA), **Violeta Stoyanova-Beninska**, noted that, although for 95% of Rare Diseases there are still no treatments, she believes that this percentage has decreased, even if only slightly in recent years, and in this direction, we should continue, in order to respond to the needs of Rare Patients. The necessity of a broad consensus to promote a European Action Plan for Rare Diseases was pointed out by **Alexander Natz**, Secretary General, European Confederation of Pharmaceutical Entrepreneurs (EUCOPE), representing a broad set of European small and medium-sized pharmaceutical companies, many of which are active in the area of Rare Diseases. Referring to the upcoming revision of the EU pharmaceutical policy, which will shape the overall landscape for the next two decades at least, he stressed that it is important to emphasize the prevention and early diagnosis of patients with Rare Diseases, to ensure access for all of patients in treatments, and at the same time to strengthen Innovation and ensure the competitiveness of the EU relative to other international markets. **Thomas Bols**, Head of Government Affairs and Public Policy for EMEA & Asia Pacific Region, PTC Therapeutics, also focused his speech on the pivotal importance of the review of European pharmaceutical legislation. As he said, the existing European pharmaceutical legislation can no longer cover the issues that have arisen due to the different conditions, the rapid development of technology, the complex legal and regulatory issues, and clearly much more needs to be done, for a real review. To this end, all stakeholders need to focus on how to make this review better for patients. The encouragement of Innovation and the common effort to make Europe competitive in the international environment regarding medicine must be promoted. He also mentioned that there are points in this revision that need further clarification, such as the 10-year patent protection, which will increase or decrease depending on whether certain criteria are met by the pharmaceutical companies, such as e.g. to have access to the medicine in all EU countries etc. He emphasized that it is not always easy to have a medicine available in every country, for example some genetic therapies have to be channeled through special

centers that are not always available in every country. Another point, finally, is to clarify the term unmet medical needs, which plays an important role especially in Rare Diseases. Europe's intention with regard to Rare Diseases is to create an integrated plan to deal with them and to recognize their existence and make patients more "visible", said psychiatrist, MEP **Stelios Kympouropoulos**. According to Mr. Kympouropoulos, the goals of the new European pharmaceutical legislation should be, among other things, the simplification of the structure of the European Medicines Agency, so that there are more flexible procedures, the clarification of the term uncovered medical needs, which plays an important role especially in Rare Diseases, access to data with respect for personal data protection as well as the development of new medicines so that patients have more treatment options. The creation of National Action Plans to deal with Rare Diseases is essential, the MEP stressed, adding that these should, among other things, include prenatal screenings and ways to increase awareness for Rare Diseases.

Nicolas Garnier, Senior Director, Patient Advocacy Lead, Global Product Development, Pfizer Rare Disease, spoke about the European plans for Rare Diseases and the national importance of policies to cover the distance with the progress of science that is constantly accelerating, citing that today there are over 500 ongoing gene therapy clinical trials. In fact, according to the FDA, 10 to 20 gene and cell therapy approvals are expected each year from 2025 onwards. Therefore, he stressed, it is urgent to prepare Health Systems and ensure we have the right policies in place to offer these innovative treatments to RD patients. Mr Garnier highlighted three points: a) data collection, RWE b) evaluation of the value of treatments and c) cross-border healthcare and cross-border participation in clinical trials, in which the European Reference Networks play a crucial role. For RWE, EMA launched DARWIN project which can facilitate regulatory decisions throughout Europe. Post-approval safety studies are also required for gene therapies in Europe. However, due to data protection rules in Europe and GDPR, the process of implementing and using RWD and registries to demonstrate long-term safety and effectiveness, but also to measure effectiveness, is complex and time-consuming. When it comes to assessing the value of highly innovative treatments for RDs, we face a fragmented market access landscape, which makes launching in the 27 EU countries very complicated. We only need to consider that pricing and reimbursement remain national competences to understand how much progress needs to be made in these policies. Finally, he highlighted the need to adopt value-based agreements and leverage RWEs and registries to facilitate access and assessment of the value provided by these treatments to patients on an individual basis. Regarding cross-border healthcare in the EU, he noted that there are too many practical obstacles, as the EU legislation does not really effectively facilitate the transfer of patients from center to center and from country to country.

Hana Horka, Policy Officer, European Commission Directorate-General for Health and Food Safety Unit B3, European Reference Networks (ERNs), spoke about the European Reference Networks (ERNs). The network was created 5 years ago, in 2017. Dealing with Rare Diseases requires a lot of expertise, and the 24 national networks symbolize solidarity at the European level. After 5 years, these Networks are at a pivotal point, as a new financial framework is being prepared to support them for the next 4 years, amounting to 77,000,000 euros. They also focus on improving the system they work with, so that it becomes clear what is effective and where improvements can be made. At this point the evaluation of the Reference Networks is already in place, and in the autumn they will be able to know where they should focus in the coming years for the best result. The next goal is to integrate the European

Reference Networks (ERN) into the national Health Systems. Concluding her presentation, Mrs Horka referred to DG SANTE's support for the revision of the registration of orphan and pediatric medicines for Rare Diseases, in the Orphanet program which provides information through official networks on a specific rare disease or group of Rare Diseases, and close cooperation with Patient Associations that rightfully have an important say in the decision-making centers of Health policies.

Cecile de Coster, Executive Director, Global Regulatory Affairs - Development Strategy, Alexion International, spoke about how the medicines' regulatory environment has evolved and how it has helped usher in new treatments for Rare Diseases. As she mentioned, January 2023 marked the 40th anniversary of the Orphan Drug Act in the US, which brought tremendous progress in the development of treatments for RDs. We currently have over 200 medicinal products approved in Europe for Rare Diseases. 85% of them have been approved in the last 10 years. There is a success story behind this evolving regulatory environment, but this does not mean that patient needs are being met, there is still much work to be done. Working together, we must continue to build on the remarkable progress we have made over the past 40 years. It is important that we maintain the spirit of the legislator's original intent for orphan drugs and continue to improve patient access to treatment, ensuring that it remains at the top of the agenda. From her part , **Mencía de Lemus**, Alternate Member of the Committee for Advanced Therapies, EMA, Trustee at FundAME, Spanish Delegate at SMA Europe, spoke about the important role that patients can play in planning and conducting Clinical Studies, incorporating their voice from the beginning to the end of the process, which is why training and empowering them is absolutely necessary, something that the European Medicines Agency also supports.

Data quality issues at both European and global level

The session under the theme " The Value Patient Data and RWE", which was coordinated by **Anja Schiel**, Special Adviser, Lead Methodologist in Regulatory and Pharmacoeconomic Statistics, NoMA, Teamleader international HTA (iHTA, NoMA), Vice-Chair CSCQ JSC (EUnetHTA21), Member Scientific Advice Working Party (SAWP), EMA, Member Methodology Working Party (MWP) with the participation of **Denise Umuhire**, Pharmacoepidemiologist and RWE specialist, he Data Analytics and Methods Taskforce, European Medicines Agency (EMA), **Ana Rath**, Coordinator of the Orphanet Work Direct Grant and of the RD-CODE Project, **Luca Pani**, Professor of Psychiatry at University of Miami and Professor of Pharmacology at Università di Modena e Reggio Emilia, Chief Innovation and Regulatory Officer, Nurosene, Chief Clinical Operations and Strategic Development, Relmada Therapeutics and **Simona Martin** Scientific Officer, Project Leader at the European Commission Directorate General Joint Research Centre, Directorate F – Health, Consumers and Reference Materials, Unit F1 – Disease Prevention was of great significance. All speakers referred to the problems of data quality both at European and global level. Patient data is the voice of patients, and they are the ones who know their disease best by providing real testimonials and evidence. However, the wealth of this data does not ensure correct information, and the information does not provide knowledge, valuable for the effective treatment of Rare Diseases.
In this direction, Ana Rath referred to the Orphanet program, which is implemented in at least 40 countries. Orphanet provides information on formal networks of research projects, multinational clinical trials, patient registries and biobanks for a specific rare disease or group of Rare Diseases. Depending on the type of network, Orphanet includes networks officially

defined by the country's health authorities, funded at European or national level, or open for collaboration. With 30.000,000 rare disease patients across Europe, the European Commission had no choice but to take action, continued Simona Martin, by creating the EU Rare Disease Platform. The EU platform for Rare Diseases aims to provide researchers, healthcare providers, patients and policy makers with a means to improve knowledge, diagnosis and treatment of Rare Diseases. The European Rare Disease Registry Infrastructure (ERDRI) makes Rare Disease registry data searchable. With tools to securely search and cross-reference information, it supports and facilitates diagnoses and clinical research and trials. It also provides statistical information on Rare Diseases. ERDRI supports existing registries through their interoperability and the creation of new registries. Concluding the very interesting discussion, all participants agreed that the safe and effective collection of quality data is a multi-dimensional problem, but also a great challenge. There is a pressing need for well-structured and coded data and everyone with new tools and proper methodology should aim in this direction.

Dialogue and cooperation of all stakeholders is needed for the R&D of new medicines for Rare Diseases

Diego Ardigó, Head of Research & Development, Global Rare Diseases, Chiesi, spoke about the collaboration as a key factor in facilitating the creation of new medicines. As he said, the lack of knowledge about RDs cannot be solved locally or nationally by a single drug manufacturer or a single researcher. What is needed is a dialogue table where everyone can have a place, drug manufacturers, funders (public and private), researchers and patients. He referred to the development of the Orphan Drug Development Guidebook, which is a list of all the incentives, designations, practices, tools, procedures that exist and support Rare Disease Research and Development and is intended for all drug researchers and manufacturers. Finally, he spoke about another important project, which is identifying the neglected diseases which are so rare and far from being understood, and far from the research target for academics and far from the pipeline for drug development. He said they have found that half of the identified Rare Diseases have never been studied in a clinical trial, and more than 90% of them have a prevalence of less than 1 million, and perhaps less than 100-200 patients in total. That's why we need collaboration between many agencies and stakeholders around the world, in order to identify patients, diseases that can be cured, to identify missing pieces of knowledge to facilitate the creation of new medicines.

Advanced therapy medicines (ATMPs) have potential benefits and should be viewed as investments in healthcare, not just costs

Umang Ondhia, Global Access Strategy Lead for Rare Diseases, Neuroscience & Gene Therapies Roche, spoke about the enormous value of the European Alliance for Transformative Therapies (TRANSFORM), focusing on 3 of the 7 recommendations in its charter where progress can be made, which will help unlock the potential of ATMPs in Europe. First of all, he stressed, RWD should be allowed to be used for ATMP matters. There is also a need for closer collaboration in the early stages of development to ensure outcomes that matter to patients, physicians, healthcare systems and payers are captured; We may have data for many Rare Diseases, but sometimes it is scattered across Europe. Therefore, it is very important to combine and harmonize data sets and ensure the right quality. Umang Ondhia also spoke about HTA and early access to new treatments. As he mentioned, in 2025 we

expect ATMPs to go through the first joint HTA process at EU level. He emphasized that sometimes the value is not sufficiently recognized, so it is critical to work with multi-stakeholder communities to avoid overlap in clinical assessment at a local level, build on the work that has been done and work with EMA to recognize the wider value of ATMPs. Finally, he spoke about promoting new access routes and supporting the sustainability of health care systems. We need to recognize, he stressed, that ATMPs are new and different from other treatments we have, and therefore we need to create and implement new models of access to ATMPs. To do this we must first ensure that we have adequate funding for Rare Diseases. ATMPs have potential benefits and should be treated as investments in health care, not just costs. Next, we need to make sure we have the right infrastructure. We need to ensure we have the right support for gene therapy centers, enabling the procurement, management and data collection associated with the successful introduction of innovative flexible payment solutions to support ATMPs for patients across Europe. Finally, we need to implement the right payment solution for the right setting. Gene therapies require new payment and accounting models that allow long-term assessment of laid costs and benefits for patients and the NHS. To address issues of political uncertainty and economic sustainability we need to adopt innovative flexible solutions, including risk sharing and outcome-based agreements, financial agreements with stagern payments. So, we need to engage in early discussions to adopt them, work with different parts of the healthcare system including payers, treatment centers, patients, and physicians.

The Conference was held **under the patronage of the European Organization for Rare Diseases (EURORDIS - Rare Diseases Europe)** and **Rare Disease Day** , while it was held **with the support of the Hellenic Ministry of Health, the European Federation of Pharmaceutical Industries and Associations (EFPIA), the European Joint Programme on Rare Diseases (EJP RD), the European Confederation of Pharmaceutical Entrepreneurs (EUCOPE), the Greek Patient Association (EAE), the Greek Branch of the European Patient Academy (EUPATI Greece)**, the **Institute of Pharmaceutical Research and Technology (ΙΦΕΤ)**, the **Hellenic Association of Pharmaceutical Companies (SFEE)** and the **PhRMA Innovation Forum (PIF)**.

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