Challenges and Opportunities in the Patient's Pathway to Access

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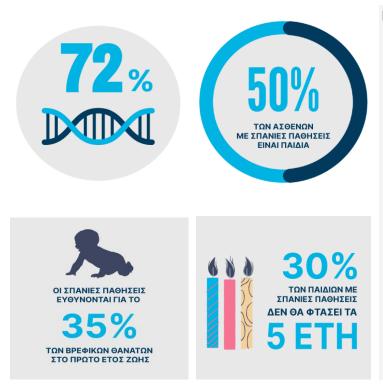
Disclosures

I have no real or apparent relevant financial relationships to disclose



Epidemiological data

237k to 630k patients in Greece



Rare Diseases in a Nutshell # of Rare Diseases # of Patients Affected 237K 468 Top 10 Rare Diseases (based on # of patients) Brugada syndrome Protoporphyria, erythropoietic Guillain-Barre syndrome Melanoma, familial Autism, genetic types Tetralogy of Fallot Scleroderma Familial long QT syndrome Polycystic kidney disease, Autosomal ... 4.2K Romano-Ward syndrome 4.2K





- Consists of 30 regular members, including Associations, Societies, and NGOs representing patients with Rare Diseases across the country.
- Is the largest representative body for Rare Disease patients in Greece and an institutional partner of all public and non-public entities.
- Participates in Committees and Working Groups, such as the Rare Diseases Committee, the Registry Working Group, the Pharmaceutical Expenditure Committee of the Ministry of Health, and others.
- Is the official representative body of Greece abroad, a member of the European Organization for Rare Diseases (EURORDIS).





EUROPEAN AND NATIONAL PROJECTS



UNIVERSIDAD POLITÉCNICA DE MADRID

IMI PROJECT: ENKORE (GPA / RARE DISEASES GREECE)







An Roinn Sláinte Department of Health

EU4 HEALTH - DEPARTMENT OF HEALTH IRELAND Project: Myhealth_at_myhands (rare diseases greece & ellok)



GRECRIN

GRECRIN aims to create a reliable network to expedite clinical trial research in Greece.

Created in 2023, GRECRIN aims to support and facilitate academically initiated multinational clinical trials and to provide affiliated countries with all the necessary services for clinical research. By fostering communication among life scientists, stakeholders, authorities, and patients, GRECRIN aspires to overcome obstacles in conducting clinical trials in Greece. GRECRIN will



ecrin

INTERNATIONAL PROJECTS









eptri

EUROPEAN PAEDIATRIC TRANSLATIONAL RESEARCH INFRASTRUCTURE





European Alliance for Transformative Therapies



Project Portfolio Μνημόνια Συνεργασίας













12™ EUROPEAN CONFERENCE ON RARE DISEASES & ORPHAN PRODUCTS

EARN MORE AT <u>Rare-Diseases.eu</u>





Project Portfolio Ερευνητικά Πρόγραμμα



ΕΘΝΙΚΟ ΚΑΙ ΚΑΠΟΔΙΣΤΡΙΑΚΟ ΠΑΝΕΠΙΣΤΗΜΙΟ ΑΘΗΝΩΝ ΙΑΤΡΙΚΗ ΣΧΟΛΗ Β' ΝΕΥΡΟΛΟΓΙΚΗ ΚΑΙΝΙΚΗ, ΠΑΝΕΠΙΣΤΗΜΙΑΚΟ ΓΕΝΙΚΟ ΝΟΣΟΚΟΜΕΙΟ «ΑΤΤΙΚΟΝ» ΑΙΕΥΘΥΝΤΗΣ: ΓΕΩΡΓΙΟΣ Κ. ΤΣΙΒΓΟΥΑΗΣ ΚΑΘΗΓΗΤΗΣ ΝΕΥΡΟΛΟΓΙΑΣ ΕΚΠΑ

PKU RESEARCH AND SUPPORT : Project Code 16843

Rare Diseases Greece is willing to support with a Grant of 13.000 a dedicated program to support the under-served PKU community, to design well targeted activities and build a pathway that helps PKU patients to reach a better treatment outcome.

PKU Support Program Purpose and Detailed Budget:

The Association will support an one-year program (April 2023 - April 2024) with the aim to enable the participating HCPs and patient advocates to develop a consensus on training guidelines and discussion guidelines for the clinic, a registry of the adult PKU population in Greece, tools to monitor patient compliance and to operate a PKU hot-line within the Clinical Center with the support of the Association.





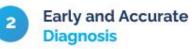
Access from Prevention to Treatment





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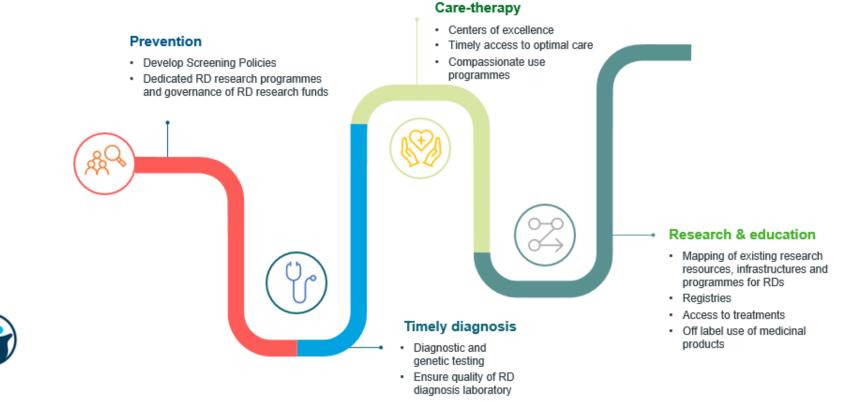
Access to Optimal Care & Innovative Treatments







The Need for a National Action Plan





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Social/ economic burden and healthrelated quality of life in patients with Spinal Muscular Atrophy (SMA) in Greece

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Keywords:

Spinal muscular atrophy, cost of illness, quality of life, disease costs, disease burden

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The Health Budget Implications

DMD patients 240-522 patients (0.005% of pop) Total cost of DMD over a two-year timeframe is 0.8% of the total health expenditure in Greece

SMA patients 190-348 patients (0.003% pop) Total cost of DMD over a two-year timeframe is 1.2% of the total health expenditure in Greece

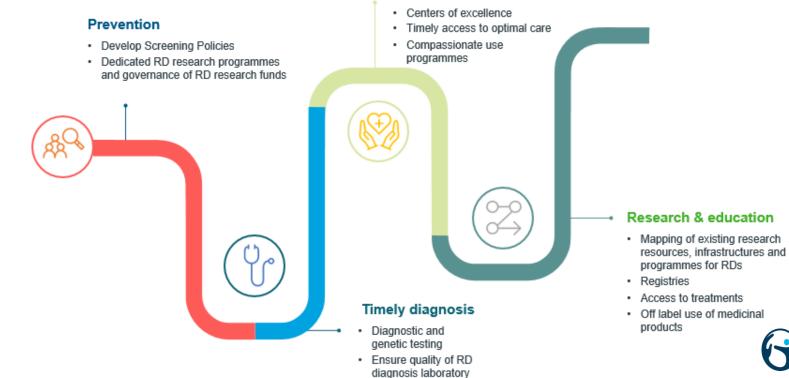




€7,111/patient per year for diabetes - 10% of population

RARE DISEASE ODYSSEY

Care-therapy





Barriers to Accessibility

- Lack of information
- Lack of communication
- ➤ Language
- Time Consuming
- Support

Patient's Needs

- Access to innovative treatment
- Better communication and collaboration
- ➤ Facilitation
- Guidelines for the procedure
- Follow Up Care

PROTON THERAPY

18 FRANCE
48 GERMANY
177 SWITZERLAND
8 GREAT BRITAIN
1 USA
29 ITALY
2 CZECH REPUBLIC

<u>PHOTON CARBON ION</u> <u>THERAPY</u>

1 GERMANY

<u>CARBON ION</u> <u>THERAPY</u> 9 GERMANY 3 ITALY

HADRON ION THERAPY 1 ITALY HEAVY ION THERAPY 1 GERMANY

How can we change it?

- Train Health care Professionals & Providers
- Train Patients Experts & advocates
- Guidelines for Access
- Eliminate Paperwork
- Facilitate Cross Border Health
- Guidelines for Follow Up
- Establish National Expert Centers

Patients' Organizations Role

- Representation and advocacy: Patient organizations play a central role, as they represent the rights and needs of patients. They actively participate in shaping policies and promoting the improvement of healthcare services.
- Awareness and education: Organizations inform patients about the opportunities provided and encourage them to seek specialized care.
- Participation in decision-making: They actively participate in working groups and health committees, contributing to the formation of strategies for better integration of networks into national healthcare systems.

Patients' Organizations Role

- Promotion of Participation in Research: Patient organizations encourage participation in clinical trials by informing patients about their options and the benefits of their participation.
- Liaison with the Pharmaceutical Industry and Regulatory Authorities: Patient organizations maintain contact with pharmaceutical companies and regulatory authorities to express patients' views and concerns, and to pursue improvements in research and drug development.
- Site Support and Data Collection: Patient organizations maintain connections with clinical sites conducting trials, patients, and often ensure the collection and access to high-value data such as PROMS, RWE, Patient Preferences Data, Patient Experience Data, and many others.

A Case to Follow & Share



A HOMEPAGE → ADMINISTRATION + → SERVICES + 1 PATIENTS + ● INFORMATION + ? QUESTIONS CONTACT

Transnational - Clinical access to doctors and patients

In order to schedule a patient's visit to one of the androtherapy centres, the following must first be completed electronically using the form available at https://www.litriplus.ou/transnational-access-ca/

Through the electronic form, the applicant will be able to the doctor (medical oncologist or radiotherapist) submit the patient application for access to the TNA clinic available from HITRIplus partners.

Please note that in order for your application to be assessed, you must meet the following requirements:

· Have the patient consent to provide his or her details for the eligibility assessment committee.

· Send the necessary documentation in English, original or appropriately translated by clinical experts.

Know and inform the patient that his/her data will be used for the sole purpose of assessing his/her case and your
request for its treatment, and if the case is not positively assessed, it will be deleted within 3 months of your request

The programme grants the amount of 3.000,00 €, which covers the expenses (travel, accommotation, are 1.of the doetne whe wishes to accompany the patient to the treatment centre.

Persons insured under the Greek social security system have the right to travel abroad to

planned care, especially in cases where the care is not provided in Greece or cannot be provided within the medically acceptable time/rame for their health condition.

For its insured persons, EOPVY authorises cross-border healthcare (healthcare provided outside the patie



Home Organizers Objectives and Scientific Programme Despite their physical and biological limitations, most cancer radiation treatments worldwide are delivered with high-energy X-rays. However, particle therapy using protons and heavy ions has many advantages over conventional X-Ray radiotherapy. Heavy ion beams radiate turnors by focusing on cancerous tissue whilst spring healthy tissue around it, making the treatment more effective than any other irradiation treatment.







Thank you!