

# Challenges and Opportunities in the Patient's Pathway to Access

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**ΕΣΑΕ**  
ΕΝΩΣΗ ΣΠΑΝΙΩΝ  
ΑΣΘΕΝΩΝ ΕΛΛΑΔΟΣ

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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

468

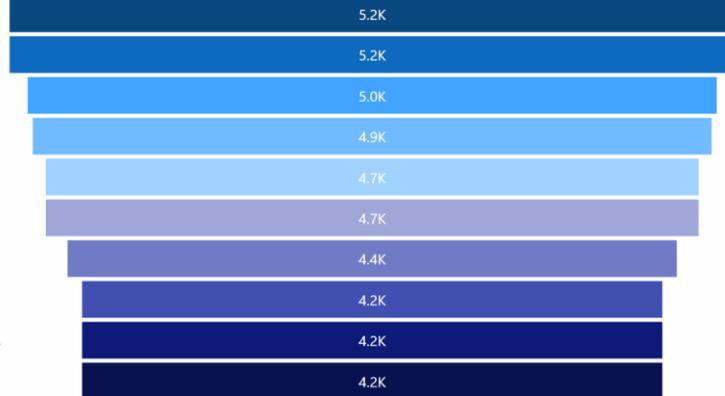
# of Patients Affected

237K

IQVIA

### Top 10 Rare Diseases (based on # of patients)

Brugada syndrome  
Protoporphyrin, erythropoietic  
Guillain-Barre syndrome  
Melanoma, familial  
Autism, genetic types  
Tetralogy of Fallot  
Scleroderma  
Familial long QT syndrome  
Polycystic kidney disease, Autosomal ...  
Romano-Ward syndrome





- 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)**



# ERDERA

EUROPEAN RARE DISEASES RESEARCH ALLIANCE

2024 – 2034



An Roinn Sláinte  
Department of Health

**EU4 HEALTH - DEPARTMENT OF HEALTH IRELAND  
PROJECT: MYHEALTH\_AT\_MYHANDS  
(RARE DISEASES GREECE & ELLOK)**

GET TO KNOW OUR  
NATIONAL PARTNERS  
  
**GREECE**

**GRECRIN**  
Greek Clinical Research Infrastructure



**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



## INTERNATIONAL PROJECTS



# Project Portfolio

## Μνημόνια Συνεργασίας



15 MAY ► 16 MAY 2024

12<sup>TH</sup> EUROPEAN CONFERENCE ON  
RARE DISEASES & ORPHAN PRODUCTS

LEARN MORE AT [RARE-DISEASES.EU](http://RARE-DISEASES.EU)



# Project Portfolio

## Ερευνητικά Πρόγραμμα



ΠΑΝΕΠΙΣΤΗΜΙΑΚΟ ΓΕΝΙΚΟ ΝΟΣΟΚΟΜΕΙΟ  
**ΑΤΤΙΚΟΝ**



ΕΘΝΙΚΟ ΚΑΙ ΚΑΘΟΔΙΣΤΡΙΑΚΟ ΠΑΝΕΠΙΣΤΗΜΙΟ ΑΘΗΝΩΝ  
ΙΑΤΡΙΚΗ ΣΧΟΛΗ  
Β' ΝΕΥΡΟΛΟΓΙΚΗ ΚΛΙΝΙΚΗ, ΠΑΝΕΠΙΣΤΗΜΙΑΚΟ ΓΕΝΙΚΟ  
ΝΟΣΟΚΟΜΕΙΟ «ΑΤΤΙΚΟΝ»  
ΑΙΕΥΘΥΝΤΗΣ: ΓΕΩΡΓΙΟΣ Κ. ΤΣΙΒΓΟΥΛΗΣ  
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.



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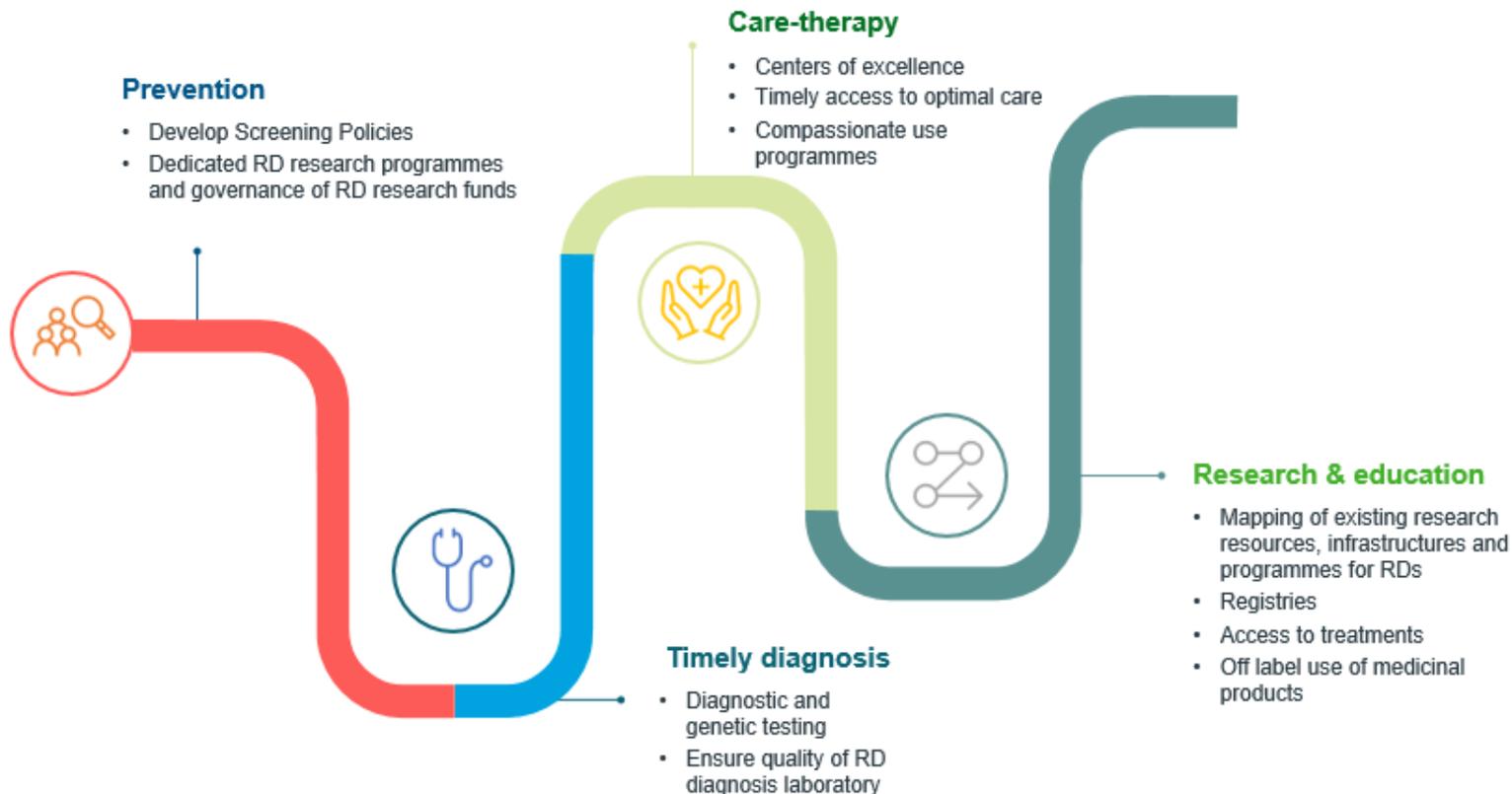


# Access from Prevention to Treatment

- 1 **Prevention & National Plans**
- 2 **Early and Accurate Diagnosis**
- 3 **Access to Optimal Care & Innovative Treatments**
- 4 **Access to Social Care & Research**



# The Need for a National Action Plan





# Social/ economic burden and health-related quality of life in patients with Spinal Muscular Atrophy (SMA) in Greece

DOI:10.7365/JHPOR.2023.2.6

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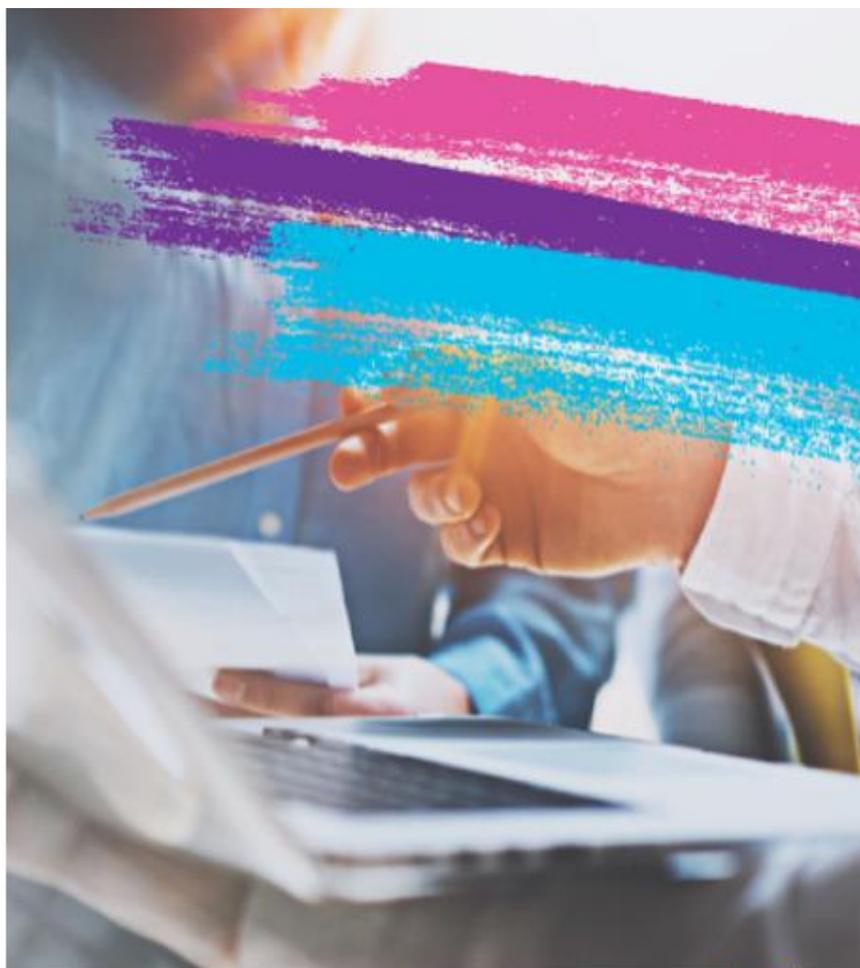
## Keywords:

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

# 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**

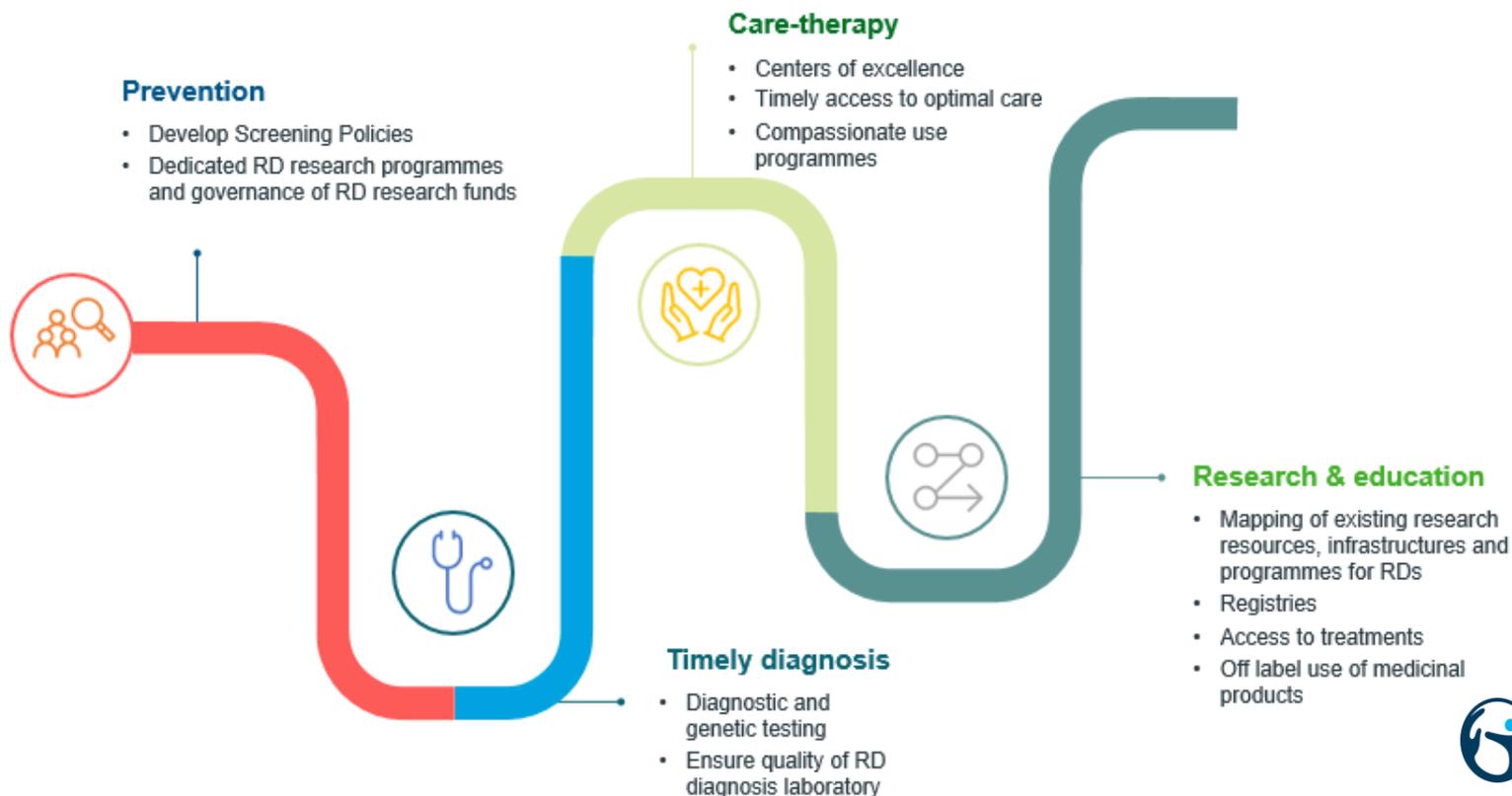
SMA patients 190-348 patients (**0.003% pop**)  
Total cost of DMD over a two-year timeframe is  
**1.2% of the total health expenditure in Greece**



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



# RARE DISEASE ODYSSEY



## Barriers to Accessibility

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- Lack of information
  - Lack of communication
  - Language
  - Time Consuming
  - Support
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## Patient's Needs

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- Access to innovative treatment
  - Better communication and collaboration
  - Facilitation
  - Guidelines for the procedure
  - Follow Up Care
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How many patients do have access? (Greek National Health Services Organization, 2013-2022)

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**PROTON THERAPY**

18 FRANCE

48 GERMANY

177 SWITZERLAND

8 GREAT BRITAIN

1 USA

29 ITALY

2 CZECH REPUBLIC

**PHOTON CARBON ION  
THERAPY**

1 GERMANY

**HADRON ION THERAPY**

1 ITALY

**CARBON ION  
THERAPY**

9 GERMANY

3 ITALY

**HEAVY ION THERAPY**

1 GERMANY

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## How can we change it?

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- 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
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# Patients' Organizations Role

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- **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.
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# Patients' Organizations Role

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- **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.
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Thank you!



ΕΣΑΕ

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ΑΣΘΕΝΩΝ ΕΛΛΑΔΟΣ