

Advancement of Treatments  
FOR **RARE DISEASES**

# THE CYPRUS INSTITUTE OF NEUROLOGY & GENETICS

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Organisers:



THE CYPRUS INSTITUTE OF  
NEUROLOGY & GENETICS



DEPUTY MINISTRY OF  
RESEARCH, INNOVATION  
AND DIGITAL POLICY  
REPUBLIC OF CYPRUS



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# 1990 ESTABLISHMENT

SUPPORTED & FUNDED BY THE CYPRUS GOVERNMENT

NON-PROFIT

PRIVATE

SERVES THE PUBLIC

**1<sup>st</sup> CENTER IN  
CYPRUS TO  
INTRODUCE**

- SPECIALIZED NEUROLOGY
- MEDICAL GENETICS
- MOLECULAR DIAGNOSIS
- BIOMEDICAL SCIENCES

# WHAT

# WE DO

**3 PILLAR  
TRANSLATIONAL  
MEDICINE CENTER**

**MEDICAL**

**RESEARCH**

**ACADEMIC**

Advancement of Treatments  
FOR **RARE DISEASES**

# RARE DISEASES



# Advancement of Treatments FOR RARE DISEASES

## RARE DISEASES

<b>Muscular Dystrophies</b>	Rare forms of Puberty	<b>Bone marrow failure syndromes</b>
<b>Hereditary spastic paraparesis</b>	Inherited thrombophilias and bleeding disorders	<b>Congenital Adrenal Hyperplasia</b>
<b>β-thalassemia</b>	<b>Familial Mediterranean Fever</b>	<b>Inherited Deafness</b>
<b>Cystic Fibrosis</b>	<b>Congenital neutropenia and other primary immunodeficiencies</b>	<b>Rare forms of Obesity</b>
<b>Rare neurodegenerative diseases</b>	<b>Maturity Onset Diabetes of the Young</b>	<b>CADASIL</b>
<b>Multiple Endocrine Neoplasia type 2</b>	<b>Rare Epilepsies</b>	<b>Rare cancers</b>
	<b>Hereditary ataxias</b>	<b>TTR amyloid polyneuropathy</b>

## PATIENT CARE

### SPECIALIZED NEUROLOGICAL CENTERS

Neuromuscular Disorders

Multiple Sclerosis & Related Disorders

Movement Disorders

Dementia

Epilepsy

- INPATIENT WARD ■ OUTPATIENT CLINICS ■ PHARMACY ■ SOCIAL SERVICES ■ PHYSIOTHERAPY
- MULTI-PROFESSIONAL CARE TEAM
- CLINICAL LABORATORIES (*Electromyography, Neurophysiology, Neuropathology, Neurovascular*)

CHKS  
ACCREDITATION

### SPECIALIZED CLINICAL GENETICS CENTRE

TERTIARY CENTER

UNIQUE FOR CYPRUS

## PARTICIPATION IN INTERVENTIONAL CLINICAL STUDIES

1. Hereditary Transthyretin Amyloidosis with Polyneuropathy  
*TRITON-PN (Amylam) – Active, recruiting*
2. Acetylcholine Receptor Binding Antibody seronegative generalized Myasthenia Gravis  
*ADAPT SERON (Argenx) – Active, not recruiting*
3. Myositis or Idiopathic Inflammatory Myopathy  
*ALKIVIA and ALKIVIA+ (Argenx) - Completed*
4. Ocular Myasthenia Gravis  
*ADAPT OCULUS (Argenx) - Completed*
5. Hereditary Transthyretin Amyloidosis with Polyneuropathy  
*APOLLO, HELIOS-A (Amylam Pharmaceuticals) - Completed*
6. Hereditary Transthyretin Amyloidosis with Polyneuropathy  
*ION-682884-CS13 (Ionis Pharmaceuticals, Inc.) - Completed*

## NON-INTERVENTIONAL STUDIES

- ***Natural History of Charcot-Marie-Tooth Disease (CMT)***  
*Full member of the Inherited Neuropathy Consortium – Active site*
- ***Hereditary Transthyretin Amyloidosis with Polyneuropathy***  
*TEG4001 and TEG4002 studies (Akcea Therapeutics)*
- ***Registry and biomaterial project for inflammatory neuropathy (CIDP)***  
*INCbase, (based in Amsterdam)*

# Advancement of Treatments FOR RARE DISEASES

## LABORATORY SERVICES FOR RARE DISEASES

Diagnostic  
Testing

National  
Screening  
Programmes

National  
Referrals

### ACCREDITATION

- ISO 17025
- ISO 15189
- College American Pathologists (CAP)

## RECOGNITION BY THE CYPRUS MINISTRY OF HEALTH

### NATIONAL CLINICAL CENTRES OF EXCELLENCE/ REFERENCE CENTRES

- Rare Neurological diseases
- Rare syndromes of congenital anomalies
- Rare Vascular Diseases, in collaboration with Makarios Hospital
- Rare and Complex Epilepsies, in collaboration with Makarios Hospital
- Rare Neuromuscular diseases, in collaboration with Makarios Hospital

## PARTICIPATION IN EUROPEAN REFERENCE NETWORKS

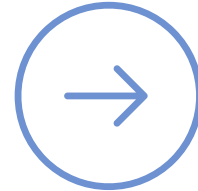
### FULL MEMBER:

1. ERN on rare neurological diseases (ERN-RND)
2. ERN on malformation syndromes and rare intellectual and other neurodevelopmental disorders (ERN-ITHACA)
3. ERN on rare respiratory diseases (ERN-LUNG)
4. ERN on rare hematological diseases (ERN-EurobloodNet)

### AFFILIATED MEMBER:

1. ERN on rare endocrine diseases (ENDO-ERN)
2. ERN on rare neuromuscular diseases (ERN-NMD)
3. ERN on rare multisystemic vascular diseases (ERN-VASCERN)
4. ERN on rare and complex epilepsies (EpiCARE ERN)
5. ERN on genetic tumour risk syndromes (ERN-GENTURIS)

# BENEFITS THROUGH RESEARCH



# Advancement of Treatments FOR RARE DISEASES

## BENEFITS FOR RARE DISEASES PATIENTS

Diagnosis

Treatment

Indicators for  
monitoring  
diseases

Disease  
mechanisms

Epidemiological  
studies

*Contribution towards the improvement of the quality of life of patients*

# STATE-OF- THE-ART RESEARCH FACILITIES

GENOMICS

PROTEOMICS

BIOBANK

MICROSCOPY  
(INCL. ELECTRON MICROSCOPY)

MOUSE FACILITY

# FUNDING

## LOCAL & INTERNATIONAL FUNDING EU & OTHER EUROPEAN COUNTRIES, USA, CYPRUS

- Horizon Europe
- Horizon Europe ERC
- EU4Health
- HORIZON-MSCA Staff Exchange
- EU - ERASMUS+
- Charcot-Marie-Tooth Association (USA)
- ELA Research Foundation (France)
- Association Francaise Centre Les Myopathies (AFM) (France)
- Ackea Therapeutics INC (USA)
- ARGENXbv (USA)
- Patient Family Association (Italy)
- Ionis Pharmaceuticals Inc (USA)
- Research and Innovation Foundation (Cyprus)
- Anastasios G. Leventis Foundation
- Cyprus Seeds

# EDUCATION

**7 MSc & PhD Programs**



Medical Genetics



Molecular Medicine



Neuroscience



Biotechnology

Accredited by

Cyprus Agency of  
Quality Assurance  
and Accreditation

**ERASMUS+**

Recognized by  
other countries

**COLLABORATION WITH MEDICAL  
SCHOOLS IN CYPRUS**

## OTHER ACADEMIC ACTIVITIES

- Medical Residency in Neurology
- Training of Medical Students
- Fellowships
- Laboratory Training and Internships

# EXPANSION OF CING

# NEW STATE-OF-THE-ART PREMISES

11,400m<sup>2</sup>

| SUPPORTED BY THE CYPRUS GOVERNMENT

- | **New research teams in new evolving areas**
- | **Further expansion in applied research and innovation**
- | **New technologically advanced infrastructure**
- | **More competitive projects**
- | **More opportunities for commercialization**
- | **New horizons for further expansion**
- | **More opportunities for researchers, students, scientists and doctors**
- | **More for the patients**



# Advancement of Treatments FOR RARE DISEASES



## **NEW PHYSIOTHERAPY CENTER | 400m<sup>2</sup>**

- | Aqua therapy services
- | Enhanced physiotherapy services
- | Better patient care

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