FONDAZIONE
PER LA RICERCA FARMACOLOGICA
GIANNI BENZI
onlus

EJP on Rare Diseases Info Day and Brokerage Event

Fondazione per la Ricerca Farmacologica Gianni Benzi Onlus

Annalisa Landi

Sabancı Universitesi
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Business Support on Your Doorstep
European Commission
TÜBİTAK
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TURKEY in HORIZON 2020
COOPERATION. INNOVATION. COMPETITIVENESS
This project is co-financed by the European Union and the Republic of Turkey
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REPUBLIC OF TURKEY
MINISTRY OF INDUSTRY
AND TECHNOLOGY
SUNUM

Fondazione per la Ricerca Farmacologica Gianni Benzi Onlus

Fondazione per la Ricerca Farmacologica Gianni Benzi Onlus *is a registered not-for-profit Scientific Research Organization, established in 2007, inspired to the work carried on by professor Gianni Benzi, expert in bioscience and regulatory issues, with the aim* ***to promote research in biological, medical and pharmaceutical areas*** *with strict and continuous attention to the innovations and opportunities resulting from the scientific progress.*

Wellbeing of patients affected by rare diseases and other special populations, like children, are our dimension and final horizon!

DEferiprone Evaluation in Paediatrics

ePTRI
European Paediatric Translational Research Infrastructure

conect 4children
COLLABORATIVE NETWORK FOR EUROPEAN CLINICAL TRIALS 4 CHILDREN

PedCRIN

GRiP

EPICAL

InNerMeD
Inherited NeuroMetabolic Diseases Information NETWORK

GAPP GAbapentin in Paediatric Pain
Toward new therapies to challenge chronic pain in children

Small Medicines Advanced Research Training

ARISE
African Research And Innovative Initiative For Sickle Cell Education

CLOSED

EUROPEAN JOINT PROGRAMME RARE DISEASES

BiOmis

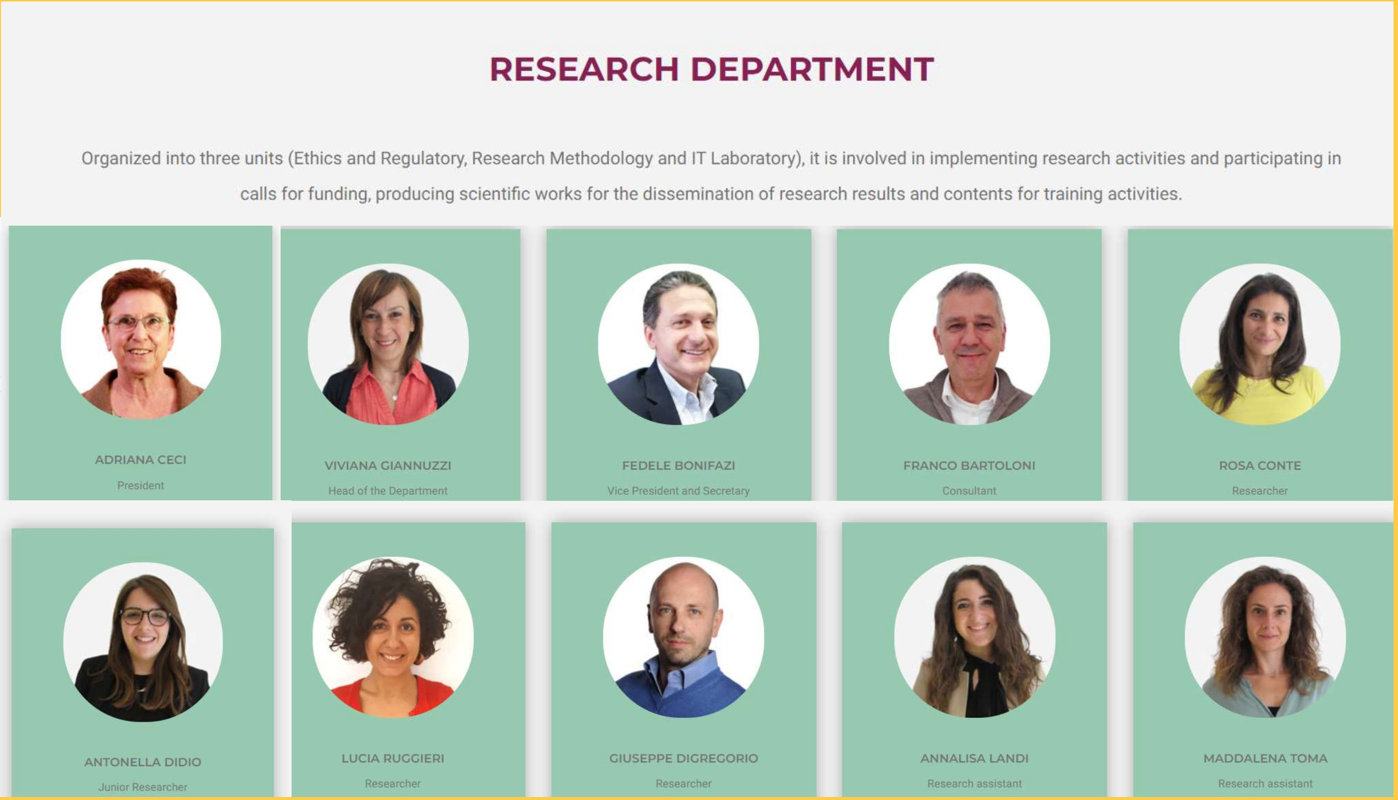
PEPPER

FGB RESEARCH INTEREST

ACTIVITIES

- ***Research methodology***
- ***Information technology and data science***
- ***Regulatory sciences***
- ***Orphan medicines***
- ***Knowledge Translation and Patients Empowerment***

RESEARCH TEAM



RESEARCH DEPARTMENT

Organized into three units (Ethics and Regulatory, Research Methodology and IT Laboratory), it is involved in implementing research activities and participating in calls for funding, producing scientific works for the dissemination of research results and contents for training activities.

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Project Title: FGB PROJECT IDEA

European portal for Paediatric Orphan Diseases

BACKGROUD

"The availability of drugs for rare diseases still represents a challenging objective, since research and development (R&D) in this field is characterised by many well-known difficulties. This is even more relevant if we consider that a large part of these patients are children, since paediatric trials are more challenging due to methodological, regulatory, ethical and economic reasons…." (Giannuzzi V. et al. 2017)

CHILDREN ARE "ORPHANS" TWO TIMES!

OBJECTIVE

To create **a publicly-accessible online portal dedicated to paediatric orphan diseases**:

- ✓ Information on the developmental status of orphan medicinal products both designated and approved for the treatment, diagnosis and prevention of rare diseases,on the availability of registries, biobanks, etc..
- ✓ Dedicated section for children and adolescents affected by rare diseases to foster the empowerment of paediatric patients in the rare disease field
- ✓ Collaboration with patients' associations, ERNs, existing paediatric initiatives (e.g. c4c, EPTRI)

INFORMATION on the PORTAL

❖ **ORPHAN DISEASES AFFECTING CHILDREN**
(information on the disease, on the availability of registries/ERNs, availability of biobanks, patients' associations)

❖ **ORPHAN MEDICINES**
(information on orphan medicinal products under R&D and on medicines approved for paediatric orphan diseases in Europe)

❖ **REGULATORY ASPECTS**
(information on clinical trials, PIPs)

❖ **SECTION DEDICATED TO CHILDREN/ADOLESCENTS**
(age-appropriate information for children/adolescents on the paediatric rare diseases, availability of existing initiatives for children/adolescents, dedicated space for collecting their suggestions/requests for collaboration)

AVAILABLE RESOURCES

- EuOrphan database

- European Paediatric Medicines Database (EPMD)

Proposed Partners

…and others!

No	Partner Name	Type	Country	Role in the Project
01	**INSERM**	Public research organisation	France	Collaboration with Orphanet
02	**TEDDY**	European Network for Paediatric Clinical Research	Italy	Hosting the European Paediatric Medicines Database –EPMD
03	**LUMC**	Leiden University Medical Centre	The Netherland	FAIRification procedures
04	**ERN EuroBloodNet**	European Reference Network	France	Collaboration
05	**ERN PaedCan**	European Reference Network	Austria	Collaboration
06	**EURORDIS**	Patient associations	France	Collaboration for improving the platform
07	**BBMRI-ERIC**	Research Infrastructure	Austria	Information on paediatric biobanks
08	**ELIXIR**	Research Infrastructure	Italian node	Data interoperability

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