FONDAZIONE
PER LA RICERCA FARMACOLOGICA
GIANNI BENZI
ONLUS

ASSOCIAZIONE CULTURALE Giuseppe DOSSETTI I Valori TUTELA E SVILUPPO DEI DIRITTI

RICERCA, INNOVAZIONE E COMPETITIVITÀ:

QUALI VANTAGGI PER IL PAESE E PER I PAZIENTI?

IL FARMACO: UN SETTORE TRAINANTE PER LA RICERCA E LO SVILUPPO?

DA INDUSTRIA 2015 A HORIZON 2020

Lunedì 3 dicembre 2012 ore 8.30 – 14.00

I network di ricerca in pediatria TEDDY e GRIP

Paola Baiardi

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TEDDY e GRIP oggi

TEDDY

Task-force in Europe for Drug Development for the Young

European multispecialty Network aiming at promoting paediatric drug development through good clinical research

GRiP

Global Research in Paediatrics

E' un progetto mondiale finanziato nell'ambito del 7° Programma Quadro (2011- 2015)

AIM: to implement an infrastructure matrix to stimulate and facilitate the development and safe use of medicine in children. This implementation entails the development of a comprehensive training programme and integrated use of existing research capacity, whilst reducing the fragmentation and duplication of activities.

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Teddy Network of Excellence

Funded under the Community's Sixth Framework Programme

TEDDY was established as a Network of Excellence funded under the 6 Framework Program (2005-2010), encompassing 19 Research Centers and 10 European Countries and Israel. The project involved with more than 200 researchers and experts

Aimed at increasing availability of paediatric safe and efficacious drugs

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...how TEDDY was born...

2003 FP6 LSH-2003-1.2.1.1

Medicines for children

aimed at structuring efforts on:

- Clinical and biological research
  - Hoping and reality
  - Ethics and methodology
- Drug use in children
- Dissemination of knowledge

...how TEDDY has grown...

TEDDY has grown in the context of the Paediatric Regulation entered into force since 2007

to share and support EU Authorities for:

- Therapeutic Needs and Research Priorities
- Methodology for Paediatric Trials
- Ethics
- Paediatric drug authorisation
- Database of paediatric drugs, prescriptions and AEs

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TEDDY is not a Scientific Society

TEDDY is not a Clinical Trials Center

TEDDY is not a Consultative Body

TEDDY IS A NEW RESEARCH INSTRUMENT

to straightening paediatric research and supporting both TEDDY Research groups and the existing external Networks, Societies and Experts to perform common and innovative initiatives

including those that would be more difficult to execute in lack **of a supportive action**.

Networking is the core of the mission of TEDDY

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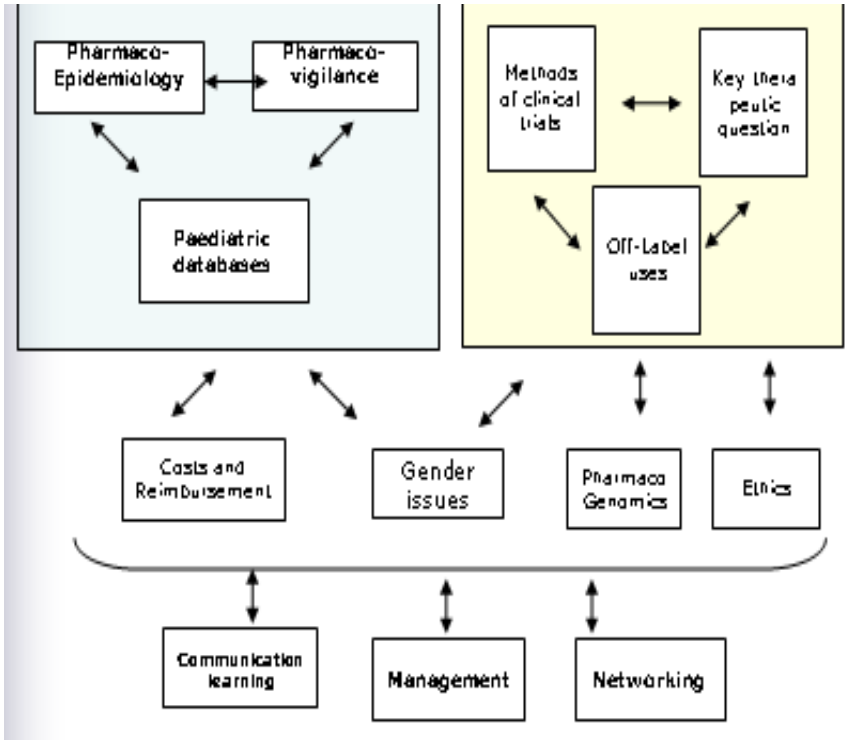
TEDDY NoE STRUCTURE
7 objectives/12 WPs



WP1
Pharmacoepidemiology

WP2
Genomics and pharmacogenomics

WP3
Clinical Trial Methodology

WP4
Key Therapeutic questions

WP5
Rare Diseases

WP6
Post-marketing studies

WP7
Ethics

WP8
Paediatric Drug Database

WP9
Communication, Information

WP10
Project Management

WP11
Joint Initiatives

WP12
Gender Issues

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Networking Methodology

- Contact and interconnect ‘noncompeting’ people having similar interest or concerns

  → Search for sector of interest and knowledge

- Create consensus on content and instruments to be used for common research

  → Surveys, focus groups, questionnaires

- To circulate knowledge and skills along the ‘points’ and the ‘nodes’ of the Network

  → Personnel exchange, e-learning courses

- Favour relationship among interested parties

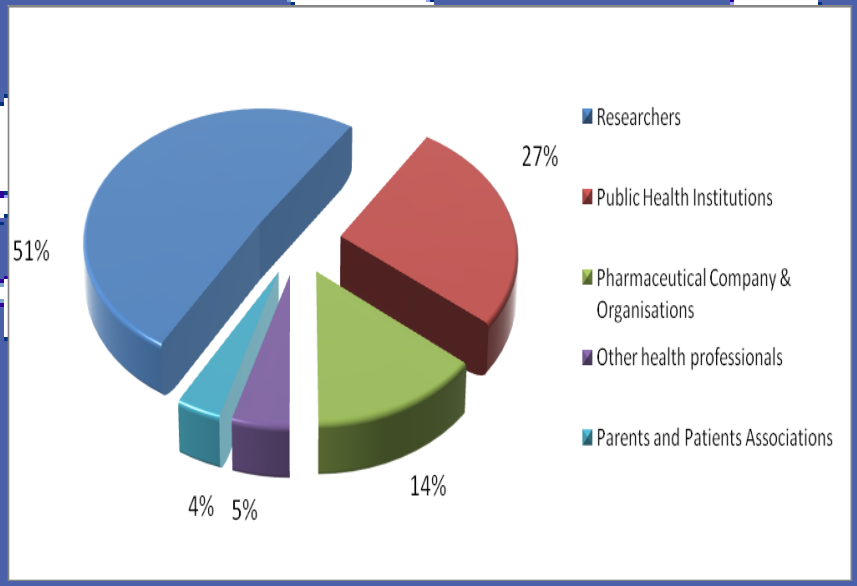
  → Open conferences, working groups

TEDDY networking

Internal (145 TEDDY Experts) and external (970 global Contacts) expertise

TEDDY collaboration with Paediatric Scientific Groups

SIOP Europe, EU Paediatric Cardiology Association (**AEPC**), EU Society for Paediatric Endocrinology (**ESPE**), EU Society for Paediatric Infectious Diseases (**ESPID**), EU Respiratory Society (**ERS**), Socirtà Italiana per le Malattie Respiratorie Infantili (**SIMRI**), EU Society for Paediatric Gastroenterology, Hepatology and Nutrition (**ESPGHAN**), Società Italiana di Pediatria (**SIP**) ,Societé Francaise de Pediatrie, Paediatric Spanish Society, **EUCROF**-Paediatric Initiative



European Countries: 25
Non EU Countries: 8

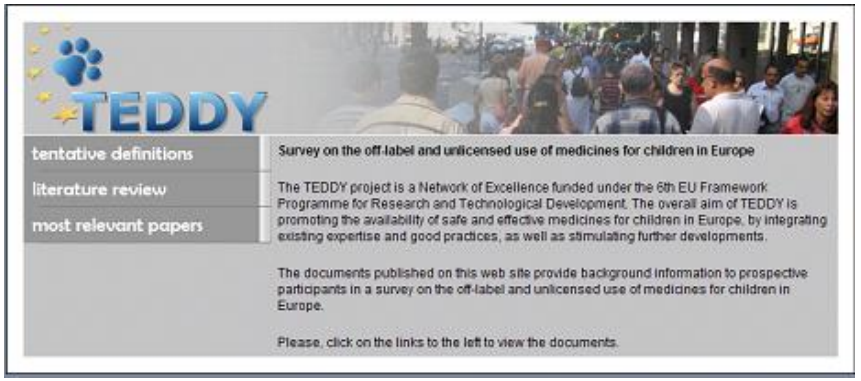
Defining off-label and unlicensed use of medicines for children: results of a Delphi survey

The aim of this Delphi survey is to develop common definitions for unlicensed and off-label drug use in children to be used for research and regulatory purposes. After a literature review on the current status of unlicensed/off-label definitions, a two-stage, web-based Delphi survey was conducted among experts in Europe. Results were then consulted with the European Medicines Agency (EMA)

The lowest level of consensus reached was for questions related to a different formulation or if a drug was given although contraindicated. At the final step, **85% of the responding experts agreed on the proposed definition for off-label** (use of a drug already covered by a Marketing Authorisation, in an unapproved way) **and 80% on the definition for unlicensed.**

Table 1: Composition of the Expert Panel

	Invited % (n)	participants % (n)
Health Professionals	14.29 (12)	20.59 (7)
Industry	8.33 (7)	8.82 (3)
Regulatory	20.24 (17)	14.71 (5)
Scientists	52.38 (44)	55.88 (19)
Other	4	0
Total	84	34



Pharmacol Res. 2008 Nov-Dec;58(5-6):316-22. Epub 2008 Sep 18

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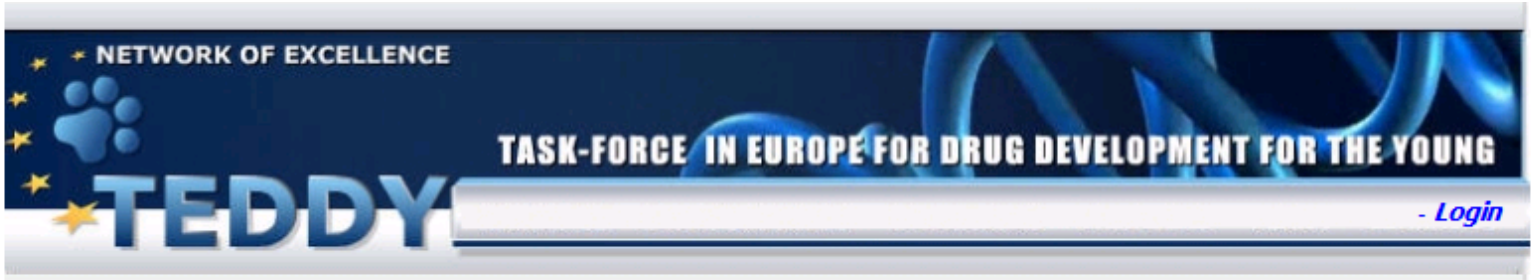
E-learning courses

www.teddylearning.com



- » Home
- »
- » Courses
- » Courses Directors
- » Teachers
- » Scientific Coordinators
- » Courses Presenter
- » Course structure
- » Registration Form
- » IT Tools Management
- »
- » TEDDYOUNG
- » Home Page
- »
- »

TEDDY

The Courses in the **TEDDY e-learning Platform** summarize the recent advancement in the sector of paediatric medicines and are based on the results of the Network as well as the contribution of well recognise high level experts collaborating with TEDDY.

The following Courses are available or are on-going:

- » CHILDREN ARE NOT LITTLE ADULTS
- » THE EUROPEAN PAEDIATRIC REGULATION
- » PAEDIATRIC STATUS AND OFF-LABEL USE OF DRUGS IN CHILDREN
- » CLINICAL TRIALS INVOLVING CHILDREN - Methodological aspects
- » CLINICAL TRIALS INVOLVING CHILDREN - Ethical aspects
- » PHARMACOEPIDEMIOLOGY AND DRUG UTILISATION IN CHILDHOOD
- » PHARMACOVIGILANCE AND DRUG ADVERSE REACTIONS IN PAEDIATRICS
- » INNOVATIVE MEDICINES AND ORPHAN DRUGS FOR CHILDREN
- » THERAPEUTIC NEEDS IN THE PAEDIATRIC POPULATION
- » TEDDY RESULTS

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Multidisciplinarità

Pubblicazioni

Recommendation for drug development for children

M. Catapano[a], C. Manfredi[a], P. Paolucci[b], H. Cross[c], K. Verhamme[d], M.J. Mellado Peña[e], I. Grosch-Wörner[f], C. Knibbe[g] and A. Ceci[a,*]

THE CHALLENGES OF THE NEW EU PHARMACEUTICAL LEGISLATION

Health-care Development

Challenges in prescribing drugs for children with cancer

THE LANCET Oncology

EJCP European Journal of Clinical Pharmacology

BMJ

RESEARCH

Drug use in children: cohort study in three European countries

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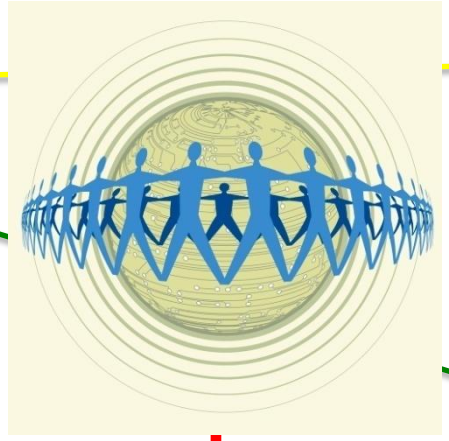
124 Deliverables

NETWORK



Scientific Pubblication certifying the multidisciplinary /multinational efforts in the paediatric research domain

FP6-EU - LSHB-CT-2005-005216

NEW PROJECTS generated in the European Paediatric Research context

E-learning Platform

Website

Database

Products demonstrating the practical interest around the paediatric issue

Based on the multidisciplinarity of the Network
Dermatology, Gastroenterology, Haematology, Infectious disease, Intensive care, Neonatology, Nephrology, Neurology, Oncology, Pain, Rare diseases, Respiratory

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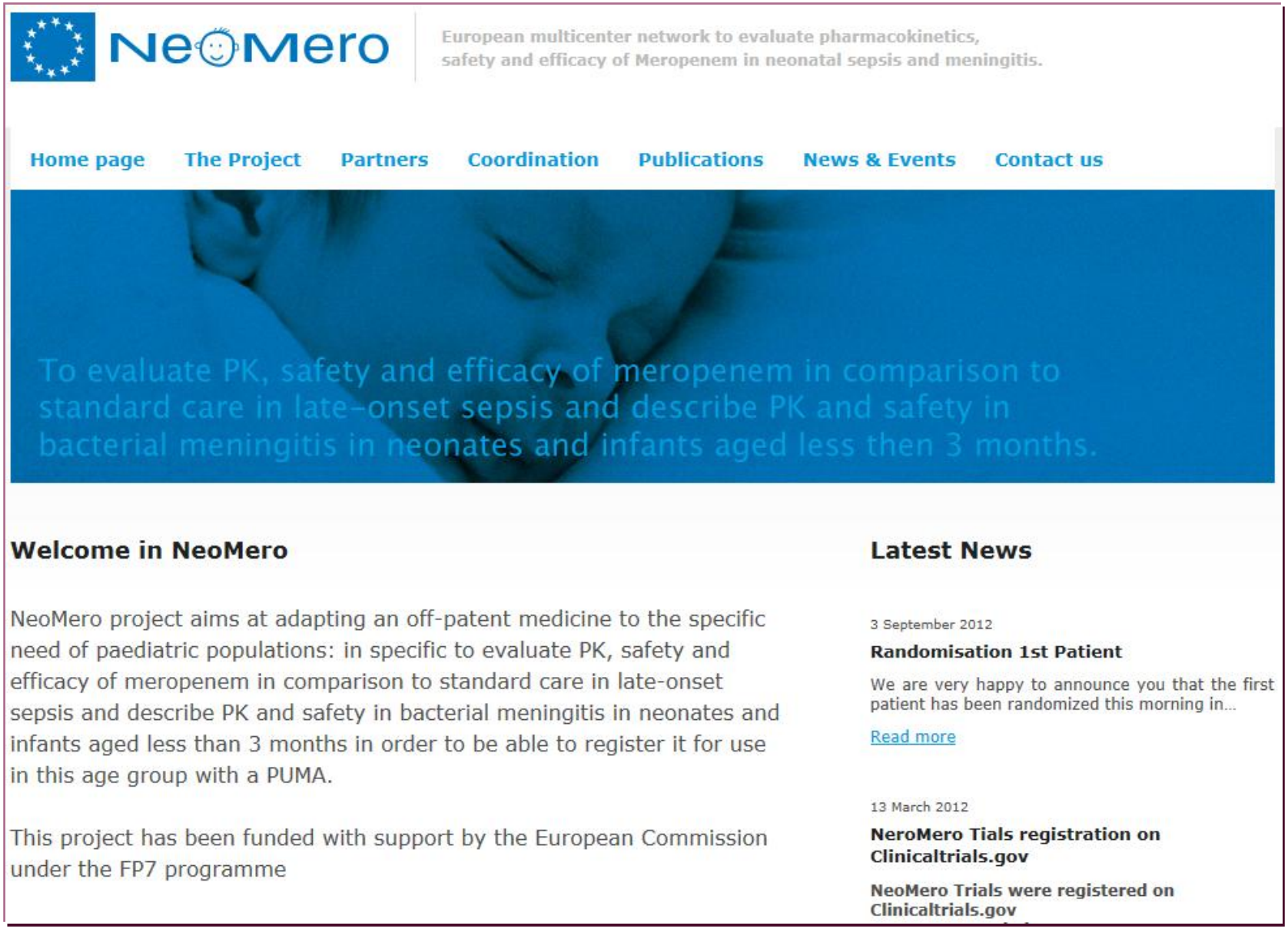
Progetto in Neonatologia



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Progetto in Ematologia

DEFERIPRONE EVALUATION IN PAEDIATRICS

SEVENTH FRAMEWORK PROGRAMME

Home | Background | DEEP Project | Project partners | Project Structure | News and Events | Reserved Area

• • • NEWS • • •

place at the Grand Nile Tower Hotel in Cairo, Egypt.

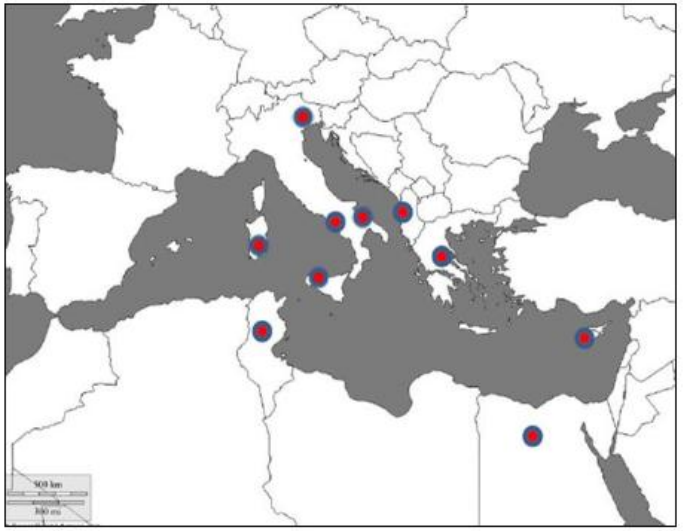
DEEP Partners will discuss the state-of-the-art of the project after the submission to the European Commission of the first official report and the relevant issues linked to the start of the clinical trials.

Nicosia (Cyprus), May 16th - 18th, 2012

Cleopatra Hotel - Workshop on GCP & Investigator Meeting DEEP1/DEEP3

DEEP Project: what it is?

- DEEP is a 4 years multinational Project
- The DEEP Consortium is composed of 12 recruiting European Centres (from Italy, Greece, Cyprus) and 3 recruiting non-European Centres (from Egypt, Albania, China) with scientific partners from EU
- A pharmaceutical group (ApoPharma and APOTEX) based in Canada and in Europe, is part of the DEEP Consortium



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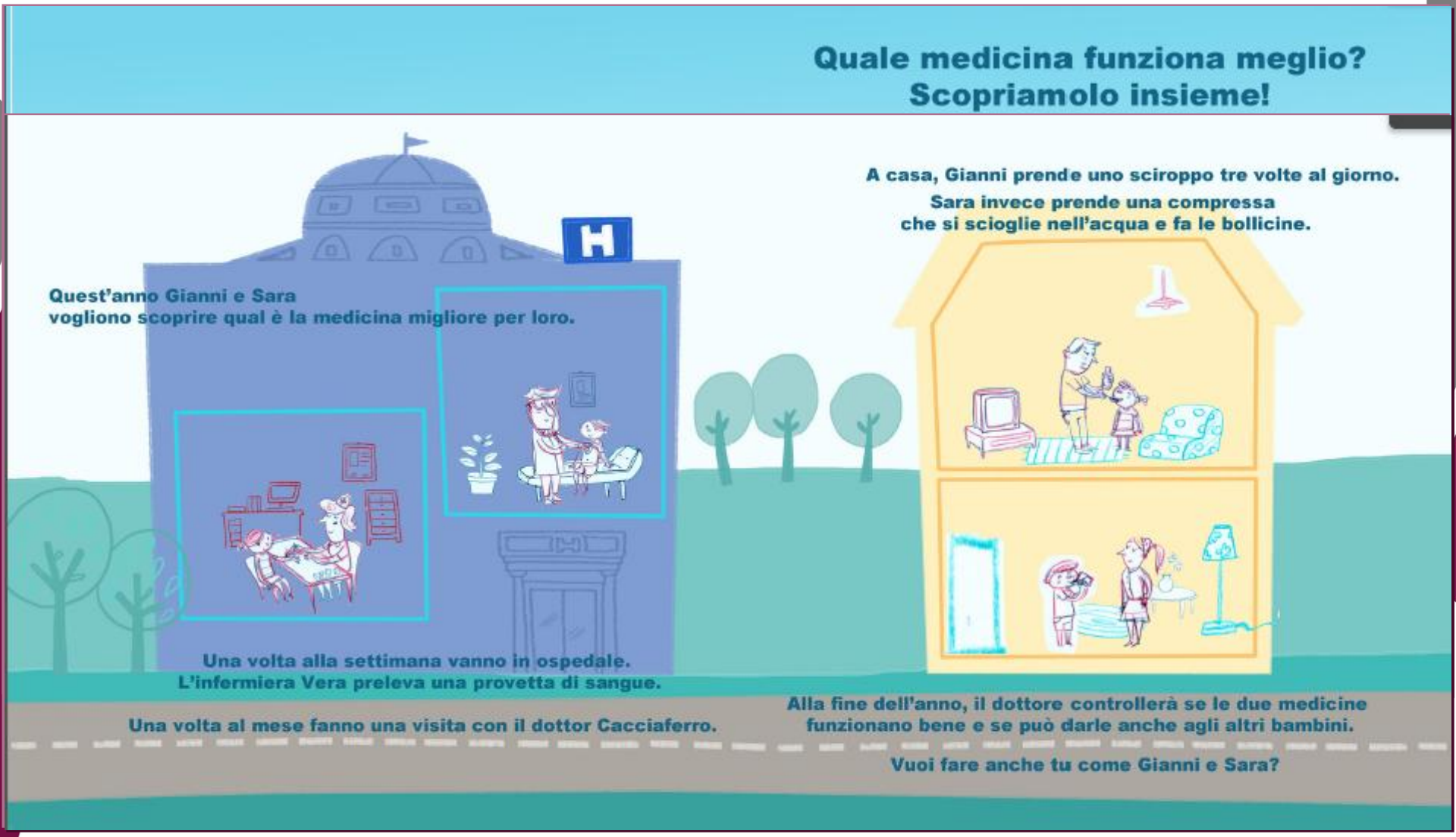
Informative Package for children

Age < 6 years



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Informative Package for children

Age from 6 to 12 years

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Progetto GRiP

Http://www.grip-network.org/

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GRiP Global Research in Paediatrics

GRiP Partnership

Id.	Participant organisation name	Acronym	Country	Lead Scientist
1	Azienda Ospedaliera di Padova	AOPD	Italy	Carlo Giaquinto
2	National Institutes of Health	NICHD-NIH	USA	Steven Hirschfeld
3	European Medicines Agency	EMA	UK	Agnes Saint-Raymond
4	Erasmus Universitair Medisch Centrum Rotterdam	EMC	The Netherlands	M Sturkenboom
5	University of Liverpool, MCRN	ULIV-MCRN	UK	Mark Turner
6	Ospedale Pediatrico Bambino Gesù	OPBG	Italy	Paolo Rossi
7	Institut national de la santé et de la recherche médicale	INSERM	France	Evelyne Jacqz Aigrain
8	National Center for Child Health and Development	NCCHD	Japan	Hidefumi Nakamura
9	St George's Hospital	SGUL	UK	Mike Sharland
10	Consorzio per le Valutazioni Biologiche e Farmacologiche, TEDDY	CVBF-TEDDY	Italy	Adriana Ceci
11	Rijksuniversiteit Leiden	UL	The Netherlands	Oscar Della Pasqua
12	Academic Medical Center	AMC	The Netherlands	Martin Offringa
13	Fundación Vasca de Innovación e Investigación Sanitarias	BIOEF	Spain	Adolfo Valls-i-Soler
14	Instytut Pomnik Centrum Zdrowia Dziecka	PCZD	Poland	Marek Migdal
16	World Health Organization	WHO	Switzerland	Sue Hill
17	School of Pharmacy	SoP	UK	Ian Wong
18	Helsingin Ja Uudenmaan Sairaanhoitopiirin Kuntayhtymä	HUS	Finland	Kalle Hoppu
19	Brighton Foundation	BF	Switzerland	Jan Bonhoeffer
20	PENTA Foundation	PENTA	Italy	Silvia Faggion
21	Vereniging Samenwerkende Ouder - En Patientenorganisaties (NL)-EGAN	VSOP-EGAN	The Netherlands	Cor Oosterwijk
22	The Hospital for Sick Children	SICKKIDS	Canada	Shinja Ito

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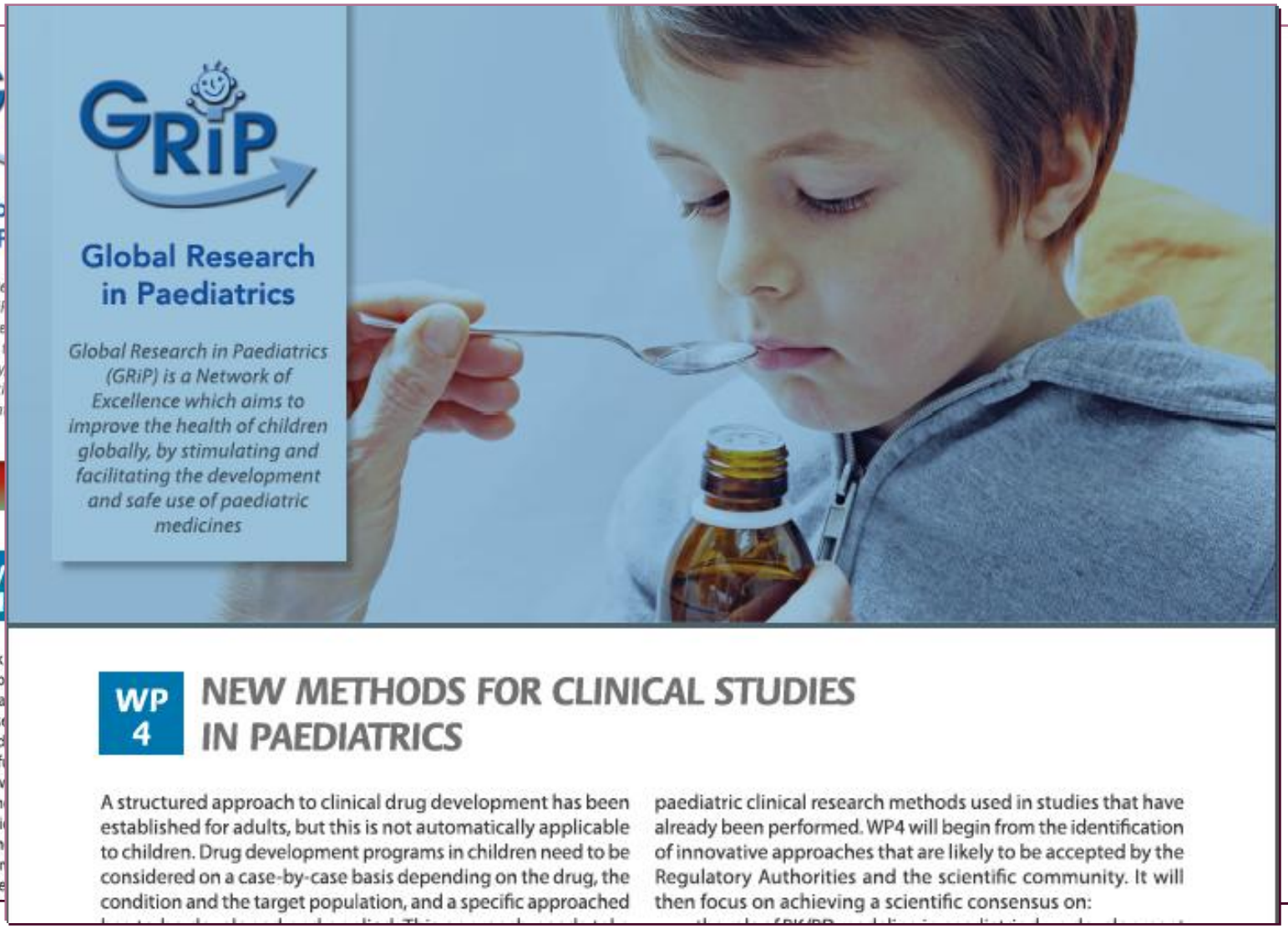
Struttura in WPs

Global Research in Paediatrics



WP 4

NEW METHODS FOR CLINICAL STUDIES IN PAEDIATRICS

A structured approach to clinical drug development has been established for adults, but this is not automatically applicable to children. Drug development programs in children need to be considered on a case-by-case basis depending on the drug, the condition and the target population, and a specific approached

paediatric clinical research methods used in studies that have already been performed. WP4 will begin from the identification of innovative approaches that are likely to be accepted by the Regulatory Authorities and the scientific community. It will then focus on achieving a scientific consensus on:

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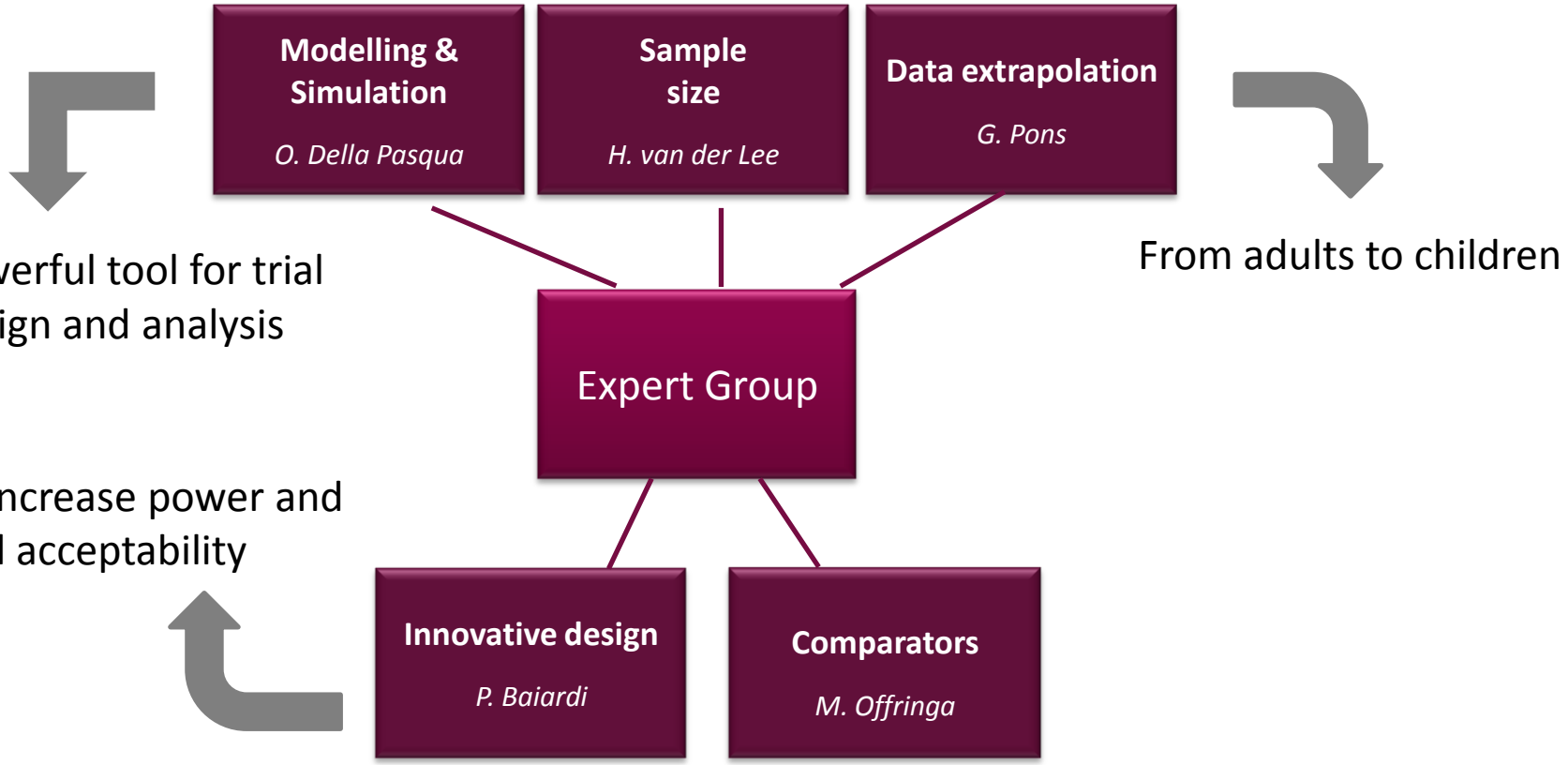
WP4 - New methods for clinical study in paediatrics



From "test of hypothesis" to "evidence synthesis"

To create «new» protocols

GRiP Global Research in Paediatrics

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Conclusioni

- Networking in the Paediatric Research sector is well accepted and represent an useful tool
- Collaboration with Existing Networks, Regulatory Bodies, Professional Associations, Pharmaceutical Industry, Patients/parents Associations is possible
- Multidisciplinarity and Multisectoriality of participants lead to building up critical mass of capacities and knowledge