

EnprEMA 02-06-2016

OKIDS Activity Update, Learnings and Improvements

“OKIDS in the European Context”

Univ.-Prof.Dr. Ruth Ladenstein, MBA, cPM

OKIDS Director

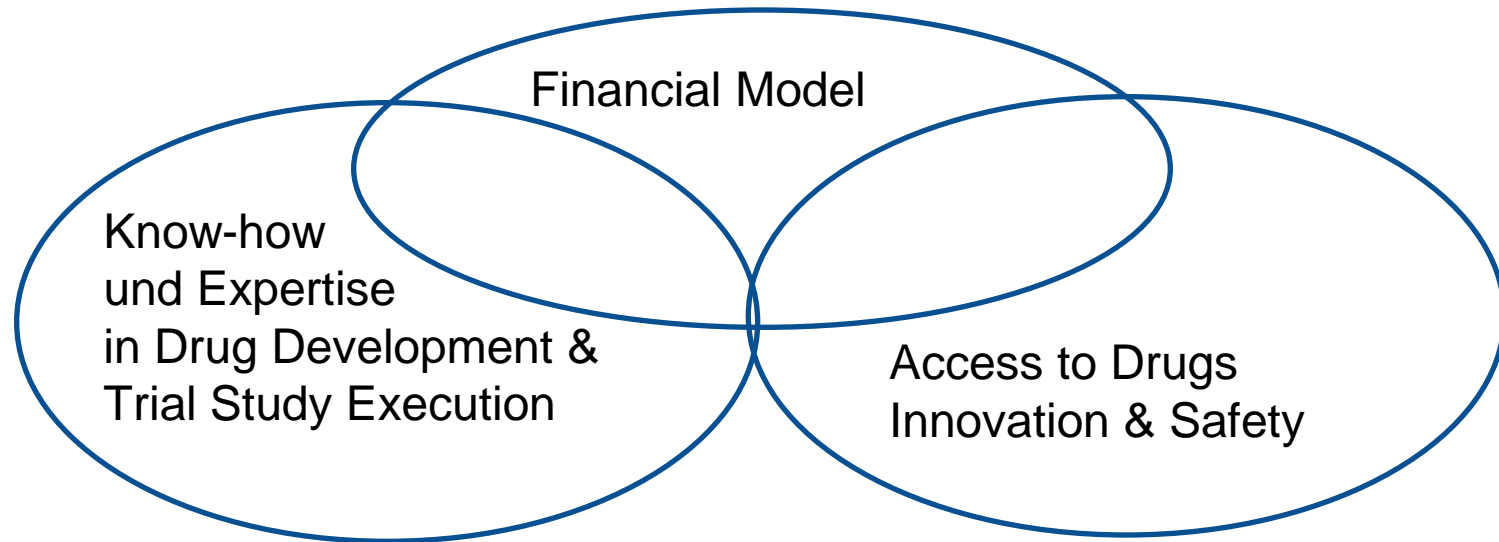
For the OKIDS Projekt Management Team

OKIDS USP

- OKIDS is the only **Organisation for Medicines for Children in Austria** coordinating the numerous tasks through its networking at home and abroad.
- **OKIDS is a subsidiary company of the ÖGKJ (Austrian Society of Paediatrics) founded in 05-2013** and acts in close co-operations with all stakeholders to facilitate information, training and top-quality clinical trials to foster drug development in Austria



Background



Private – Public Partnership

Academia
ÖGKJ
Experts
Med.Universities - KKS

Industry
Pharmig

Politics
Ministry of Health
Competitive Grant :
Public Insurers - Pharmig

Parents & Patients



OKIDS Sites

- 5 OKIDS sites provide a **single point of contact (SPC) at site for industry** to run feasibility requests, clinical trial inquiries and trials set up phase.
- OKIDS sites provide also support to academic researchers to build IDCT and provision of associated tasks like provision of insurance, monitoring, data management and pharmacovigilance.



Who is Who in OKIDS ?

Innsbruck

Salzburg

Wien

Wien

Graz

Management



Mag. (FH) Sabine
Embacher-Aichhorn



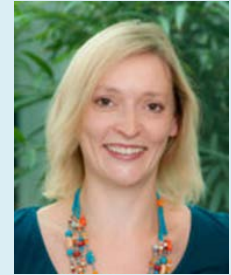
PD Dr. med.
Florian B. Lagler



OKIDS Director:
Univ.-Prof. Dr. Ruth
Ladenstein, MBA, cPM



A.o. Univ.-Prof. Dr.
Christoph Male



Mag. Gabriele
Pfaffenthaler

OKIDS Personell at Sites



Mag. Dr. Barbara
Lanthaler



Andrea Hörwertner



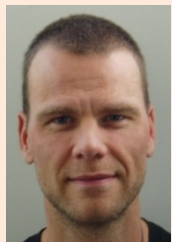
Andrea Mikolasek



Mag. Dr. Alexandra
Kreißl



Ulrike Trummer



Christian Reif



Brigitta Fragner



Christiana Echtner Heather Ablaza



- **OKIDS CLINICAL TRIAL
ACTIVITY UPDATE 2016**

Number of Enquiries

60 feasibilities / enquiries for trials

- 26 industrial partners
- 24 indications

	2013	2014	2015 /2016	Total
PHF	8	9	2+4	23
CRO/PHF	1	6	7	14
CRO	4	7	9+3	23
Total	13	22	25	60

OKIDS Trial Site Support Services

OKIDS staff supported at 5 sites local teams with:

- Advise
- Initial approaches to launch a trial – contract support –initiation /
- Study Nurse activities: Trial administrative and communication tasks , CRF completion, Sampling
- Monitoring

Site	CRCS	MUG	MUI	MUW	SAK	Total
Industry driven	7	12	11	10 (+22)*	19	59
Investigator driven	9	5	6	9 (+9)*	7**	36
Total	16	17	17	19 (+31)*	26 (+44) ***	95

* (ongoing without OKIDs support)

** SAK- S²IRP CCRI: ITCC trials (early investigator driven trials with industry support)





*** SAK – S²IRP CCRI in addition :

25 IDCT and 18 Registries in Paediatric Hemato-Oncology ,
1 Medical Product Study

Broad range of requested indications...

- **Neonatology**
- **Metabolic Diseases**
 - M. Fabry
 - M. Gaucher
 - Phenylketonurie
- **Tuberose Sclerosis**
- **Haemophilia**
- **Anticoagulation**
- **Epilepsy**
- **Pulmonale art. Hypertension**
- **Cardiology**
- **Hypercholesterinaemia**
- **Acute Gastroenteritis**
- **Palliative Care**
- **Chronic Pain**
- **Haemtology- Oncology**
 - Acute Leukaemai & Solide Tumors
 - ALL Relapse / Refractory ALL
 - Consolidation ALL Relapse
 - Newly diagnosed MDS & MML
 - Ph pos CML / ITCC
 - Refract. & Relapse Neuroblastoma
 - Rhabdomyosarcoma/ITCC
 - Conditioning allogeneic HSCT in malignant diseases
 - Condit. allogeneic HSCT in non malignant diseases
 - M. Hodgkin
 - Malignant brain tumors
 - Neuroectodermal tumors

Advantages for industry to cooperate with OKIDS

<p>"RIGHT" PATH</p> 	<p>"RIGHT" SITES</p> 	<p>"RIGHT" PATIENTS</p> 	<p>"RIGHT" START</p> 
<p>Realistic Feasibilities</p>	<p>Choice of right centres & interested PI /local team willing to engage</p>	<p>Identification of patient accrual potential in rare sub- entities</p>	<p>Cooperation Common aims & Shared experience</p>
<ul style="list-style-type: none"> <input type="checkbox"/> Indication <input type="checkbox"/> Therapeutic proposal / Standard of Care <input type="checkbox"/> Time of PI 	<ul style="list-style-type: none"> <input type="checkbox"/> Hospitals <ul style="list-style-type: none"> ▪ Severe Diseases ▪ Hospitalisation / day care contacts ▪ Speciality consultations ▪ Trained Personal / Study teams ▪ Internal knowledge about Infrastruktur <input type="checkbox"/> Private Practice <ul style="list-style-type: none"> ▪ Less severe diseases ▪ Any Indication ▪ Prcatice Assisitant / patient administration ▪ Basic Infrastruktur 	<ul style="list-style-type: none"> <input type="checkbox"/> Prevalence / Incidence of diseases <input type="checkbox"/> Age groups of patients <input type="checkbox"/> Accrual area of hospitals / private practices <input type="checkbox"/> Transferral of possible patients by informed medical colleagues 	<ul style="list-style-type: none"> <input type="checkbox"/> Cooperation between Sponsor (Industry) and O.K.ids <input type="checkbox"/> Know – How about local trial portfolio <input type="checkbox"/> Contacts to possible trial sites <input type="checkbox"/> Experience with indications (experts at sites) <input type="checkbox"/> Support in decision making

Florian Lagler and Christoph Male
For the OKIDS PMT Team

- **INTERACTION WITH EXPERTS
AND FAMILIES**

Expert panel – revisited

- Paediatric experts
- Patients/parents with study experience
(experts by own experience)
- Broadened focus
- Pro-active acquisition of studies

The EUPATI Toolbox www.eupati.eu



European Patients' Academy

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[Home](#)

[What is EUPATI?](#)

[EUPATI Network](#)

[Blog](#)

[Glossary](#)

The A to Z of how medicines are developed

Patient education!

Search our library by keyword

For example: drug discovery process

Search

 Search our library by category

Basics of Medicine

Pharmaceutical Development

Regulatory Affairs

Types of Medicine

Clinical Development and Trials

Health Technology Assessment

Drug Discovery

Personalised Medicine

Non-Clinical Studies

Safety of Medicines

Benefit and Risk Assessment

Pharmacoepidemiology

OKIDS Initiatives – Partner with EUPATI

- Objectives 2016

- Information folder for patients- young people
- Workshops for patients- young people / parents
- Integration of young people into the OKIDS expert panel –
including training to the topic

OKIDS Brochures for Teams and Parents



o.k.ids
Organisation Kinderarzneiforschung
Medicines for Children Research Organisation · AUSTRIA

German 2015
English 2016

o.k.ids
Organisation Kinderarzneiforschung
Medicines for Children Research Organisation · AUSTRIA

KLINISCHE STUDIEN BEI
KINDERN UND
JUGENDLICHEN

INFORMATION FÜR ELTERN

CLINICAL STUDIES WITH
CHILDREN AND
ADOLESCENTS

INFORMATION FOR PARENTS



**FU Action after
1st OKIDS Parent Meeting Salzburg March 2015**

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Organisation Kinderarzneiforschung
Medicines for Children Research Organisation · AUSTRIA

Sabine Embacher-Aichhorn

für das OKIDS Projekt Management Team

TRAININGS FOR INVESTIGATORS AND STUDY PERSONNEL



Organisation Kinderarzneiforschung
Medicines for Children Research Organisation · AUSTRIA

Basic offer

➤ **OKIDS benefits from existing training programme**

(KKS Wien, KKS Graz, KKS Innsbruck, CRCS)

- **GCP Basics**
- **GCP Refresher**
- **Principal investigator training**
- **Basic course for Study Nurses & administrative study persone**
- **Specific trainings on clinical trials (AMG & MPG)**
- **Basic course „Statistics“**



- **INFORMATION & MEDIA ACTIVITY
IN AUSTRIA AND BEYOND**

German 2013
English 2015

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Organisation Kinderarzneiforschung
Medicines for Children Research Organisation · AUSTRIA

VÖGKJ
ÖSTERREICHISCHE GESELLSCHAFT
FÜR KINDER- UND JUGENDGERIATRIE

BUNDESMINISTERIUM
FÜR GESUNDHEIT

SEKUNDRÄRE
BEWEISSTELLE
FÜR KLINISCHE PRÜFUNGEN

PHARMIG
ÖSTERREICHISCHE
PHARMA-INDUSTRIE

Contact Us | Intranet

OKIDS > default

Aktuelles

Netzwerk

Industrie

Kinder & Familie

Wissenswertes

17.05.2013
OKIDS Kick-off Veranstaltung

Kontakt

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OKIDS - Netzwerk

Im Rahmen der Kindergesundheitsstrategie des Bundesministeriums für Gesundheit (BMG) wurde die Verbesserung von kindgerechten Arzneimitteln als Ziel formuliert und in der Arbeitsgruppe 6 des Kindergesundheitsdialogs umgesetzt. Das österreichische Studiennetzwerk für Arzneimittel und Therapien "OKIDS" wird als kompetenter Partner und Serviceleister für Universitäten/spezialisierte Zentren und die pharmazeutische Industrie agieren und durch die Bündelung der Kompetenzen und Ressourcen vermehrt akademische und Industriestudien mit pädiatrischen Patienten rasch und effizient durchführen, um damit die notwendige klinische Forschung für Arzneimittelsicherheit zu stärken.

Kinder & Familie

Medikamente müssen an Kindern speziell geprüft werden, um ihre besonderen Auswirkungen auf den kindlichen Organismus festzustellen. Über Studien haben Kinder Zugang zu neuen Behandlungsmöglichkeiten und da sie in Studien besonders ausführlich beobachtet werden, werden Kinder in Studien meist sicherer behandelt als außerhalb. mehr...

Wissenschaft

Klinische Forschung für Kinder ist wichtig um Medikamente und andere Innovationen speziell für Kinder bereit zu stellen. Dies wurde auch von der Europäischen Behörde (EMA) mit einer hohen Priorität eingestuft und ein entsprechendes gesetzliches Umfeld für Forschung mit Kindern geschaffen. mehr...

Industrie

Vor der Initiierung einer klinischen Studie am Prüfzentrum sind zahlreiche Schritte notwendig, die vom Abschluss von Verträgen bis zur Organisation des Zahlungsverkehrs reichen. Die Netzwerkzentrale von OKIDS bietet den Service eines One Stop Shops an, der die notwendigen Schritte an den OKIDS Standorten zentral koordiniert. mehr...

Netzwerk

Ein Aspekt, der für die erfolgreiche Durchführung von klinischen Prüfungen eine wesentliche Rolle spielt, ist geschultes und erfahrenes Personal. An den OKIDS-Standorten in Wien, Graz, Innsbruck und Salzburg werden regelmäßig Schulungen rund um das Thema klinische Prüfungen angeboten. mehr...

14:34
16.05.2013

OKIDS Organisation

Brochures Deutsch & English 2014

OKIDS GmbH Head Office

OKIDS Management

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Who we are?

Spectrum of Services

Children & Family – Science – Industry

Service for Industrial Studies in accordance with Catalog of Benefits

Feasibility Check – fast and valid

Utilize the knowledge of the network of OKIDS operational sites to evaluate the feasibility of studies in Austria in cooperation with pediatric centers to make optimal use of the existing resources.

Study Contract Management – custom-made and comprehensive

OKIDS operational sites ensure rapid review of contracts by taking center-specific features into account.

Site Management –



Organization for Research in Children's Medicines
 Medicines for Children Research Organization – Austria



Your competent partner
 for research in children's medicines

OKIDS Network Austria

Operational Site: Vienna



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Operational Site: Salzburg



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Operational Site: Innsbruck



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Operational Site: Graz



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 gabriele.pfaffenthaler@okids-net.at
 www.medunigraz.at/kks

Supported by the following companies pursuant to an agreement with Pharmig (Association of the Austrian Pharmaceutical Industry).



History

OKIDS is the result of Task Group No. 6 of the Pediatric Health Dialog and is part of the general National Health Targets of the Federal Ministry of Health. OKIDS was developed in cooperation with the Federal Ministry of Health and Pharmacy.



Sponsored by the Federal Ministry of Health. Supported by Pharmig – the Association of the Austrian Pharmaceutical Industry. Sponsored by a grant of the "Joint Health Objectives of the Pharma Master Agreement, a cooperative project of the Austrian Pharmaceutical Industry and social insurance".



OKIDS

A network for clinical studies on children's medicines.

Child-appropriate medicines

The greatest need for child-appropriate medicines is in the fields of neonatology and oncology (80 - 90% of current use are „off-label“). However, new medicines and child-appropriate formulations are needed for the entire spectrum of pediatric conditions.

- OKIDS serves as a competent partner and service provider for universities/pediatric departments and the pharmaceutical industry.
- By combining competences and resources, OKIDS allows to perform industry sponsored as well as academic studies in children and adolescents.
- OKIDS improves clinical research to increase drug safety in children.

OKIDS Expert Forum

Particularly in very young and sick children, there is a significant lack of tested and approved medicines. Consequently there is an urgent need to investigate medicines for children and it is essential to set clear priorities.

OKIDS has assembled a Forum of Austrian pediatric Experts representing all subspecialties which ensures prioritization of pediatric therapeutic needs and of the most promising new developments.

OKIDS Movies in 2015 - Example MPS

„Research in Medicinal Products as Chance “



WEB-Page
German 2015
English Subtitles 2015

- Importance of trials as access to drugs
- Importance of trial sites in Austria
- The view of parents & patient in affected families

OKIDS Press Conference 01.12.2015

11 Clippings:

- Der Standard und Der Standard online
- Kleine Zeitung
- Kronen Zeitung
- Kurier
- Medianet
- Medical Tribune
- Pharmaceutical Tribune
- Pharma-Time
- Pharmig Info
- Tiroler Tageszeitung



15 Journalists



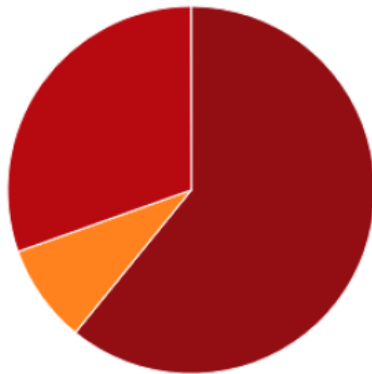
- Radiobericht in Ö1



OKIDS Presence in Media 2015

23 Clippings im Jahr 2015, davon 14 in Printmedien, 7 auf News Sites und 2 Agenturmeldungen

Aufteilung der Meldungen auf Quellenbündel



Radio & TV (0)
Agenturen (2)
Printmedien (14)
News Sites (7)

Quellen

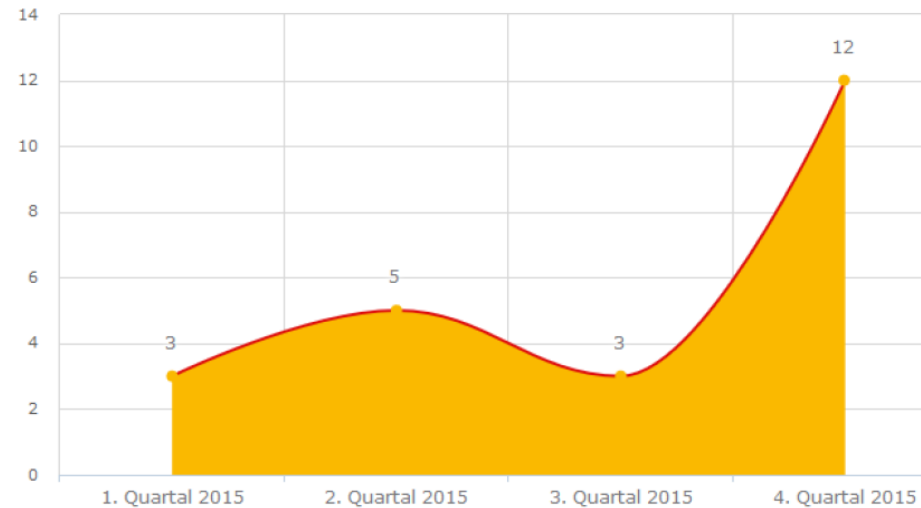
PHARMA-TIME (2)
DER STANDARD (2)
APA - AUSTRIA PRESSE AGENTUR (1)
KLEINE ZEITUNG (1)
MEDIANET (1)
PHARMACEUTICAL TRIBUNE (1)
CLINICUM (1)
APA - OTS (1)
WECARELIFE.AT (1)
PRESSEMELDUNGEN.COM (1)
DERSTANDARD.AT (1)
SPEZIALQUELLE (1)
MEDIANET.AT (1)
LIFESCIENCEAUSTRIA.AT (1)
KURIER (1)
PHARMAUSTRIA (1)
ÄRZTE KRONE (1)
PROFIL.AT (1)
MEDICAL TRIBUNE (1)
PROFIL (1)
OE-JOURNAL.AT (1)

OKIDS Press Conference Q4/2015

Aufteilung der Clippings nach Quartal 2015

Etwas mehr als die Hälfte aller Clippings im 4. Quartal 2015 (OKIDS Pressegespräch, Pharmig Pressemitteilung)

Meldungen im Zeitverlauf



+ 1 Radiobericht in Ö1



Organisation Kinderarzneiforschung
Medicines for Children Research Organisation · AUSTRIA

OKIDS & Pharmig:

Informative Meetings and Discussion Rounds

- 13-07-2012:** Information for funding industry
- 20-11-2013 :** 6 Months OKIDS – First Experiences of Operations
- 18-11-2014:** Role as Facilitator for pharmaceutical companies and CROs in paediatric trials:
Feedback, Co-operations with CROs
- 01-12-2015 :** How to position paediatric trials in Austria
- 25-04-2016 :** OKIDS in the European Context



OKIDS in the European Context

April 25th 2016, PHARMIG (Association of Industries Austria)

EnprEMAs Mission and Insights in European Paediatric Networks

Recent EnprEMA activities to foster Paediatric Network funding: Current and Upcoming opportunities

Mark Turner BSc, MBChB (Hons), PhD, MRCP(UK), MRCPCH, DRCOG

- Interaction with Industry
- Expected synergies to foster drug development for children in Europe

Industry expectations

Dr. Sylvia Nanz (Pfizer)

PD Dr. Johannes Pleiner-Duxneuner (Roche)

- Current view on the European competition for paediatric trials – Preferred countries, visibility and more
- How we foster paediatric research-our devoted programmes

The parent perspective (MPS Association)

Michaela Weigl

News from OKIDS :

- **Activity update, learnings and improvements (Prof.Dr.Ruth Ladenstein)**
- **Interaction with Families and Experts Revisited (PD Dr. Florian Lagler , Prof.Dr. Christoph Male)**
- **Training of investigator teams (Mag. Sabine Embacher-Aichhorn)**



OKIDS & ÖGKJ Activities (Austrian Society of Paediatrics)



Präsident:
Prim.Univ.-Prof.Dr.Wolfgang Sperl



<http://www.docs4you.at>



Vizepräsident:
Prim.Univ.-Prof.Dr.Reinhold Kerbl

OKIDS Presence in Board ÖGKJ Board Meetings combined with Experts Meeting

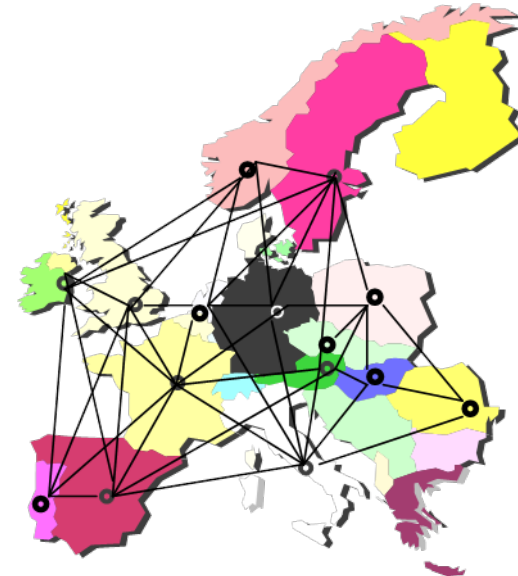
- Annually March 2013 /2014/2015/2016

OKIDS Involvement in Annual ÖGKJ Society Meetings

- 51. Annual Meeting ÖGKJ 26. – 28.09.2013 in Innsbruck
- 52. Annual Meeting ÖGKJ am 18.09.2014 in Wien
- 53. Annual Meeting ÖGKJ: 24. - 26.9.2015 in Eisenstadt
- 54. Annual Meeting ÖGKJ: 24. - 26.9.2015 in Feldkirch



Organisation Kinderarzneiforschung
Medicines for Children Research Organisation · AUSTRIA



■ POSITIONING IN EUROPE



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

European Network of Paediatric Research at the European Medicines Agency

**OKIDS is an Enpr-EMA Category 1 Member
since Mai 2015**



- 1. European Visibility and raised awareness for an Austrian Pediatric Medicines for Children platform for future cooperation with pharmaceutical industry (benchmarking effect)**
- 2. Expert – Know-How exchange and transfer between paediatric networks**
- 3. Lobbying for and common applications for relevant EU support programmes to increase funding**

PCT Congresses – Barcelona 2014 / Hamburg 2015

Round table:

“The role of the academic research sites and clinical research units in clinical trials”



Total of contact talks with 12 pharmaceutical companies and 20 CROS (1-2x)

2014: OKIDS first participation

2015: OKIDS appears in program 1st time

2016: OKIDS will be present in program 2 sessions

Lessons learned

- **Most pharmaceutical companies Clin Ops moved medical operations away from Austria;** most headquarters abroad: little direct contacts in Austria possible
- **Changing industry landscape with growing importance of CRO's as first contact** (feasibilities for free or moderately priced) - Feasibilities don't work as business model.
- **Multiple CRO contacts (efforts) prior trial arrives in Austria – if at all;** OKIDS role vis a vis CRO needed new definition ; CROs often prefer to run early feasibilities quickly via direct contacts based on address data bases (quality of responses?)
- **Shared study nurse capacity is a big asset at sites to encourage PIs to take new/additional responsibilities and to run trials (fair cost sharing model at sites 60:40 to nourish shared study nurse pools)**
- **Long term sustainability: a big challenge !**
 - Patients accrual low – effort compensation comes late with PPF
 - PPFs hardly compensate for efforts around study initiation phase – upfront fees modest
 - New /additional base funding streams needed

Actions taken: OKIDS & ÖGKI engage both

- to improve further **public awareness and acceptance of clinical trials for children in Austria** (congresses, media, webpages, movies, brochures) to increase annual initiation of important clinical trials investigating new medications for children to improve save paediatric drug supply.
- to act as **facilitator of interactions of parents/patients , medical experts and pharmaceutical industry** to serve the patients interest and needs in the best possible manner.
- to position **OKIDS as central point of access for all interest groups involved** in improved supply of medicine for children
- to **provide and integrate experts knowledge** to achieve high quality and feasible trials
- to **improve training of medical teams**
- to **provide treatment guidelines**
- to **maintain and upscale the Public Private Partnership-Modell** integrating the relevant the stakeholders in Health and Research in Austria and EU

Funding Support- Thank You!

- In order to establish a network for the research of medicinal products suitable for children and adolescents in Austria a public-private partnership was developed including
 - Federal Ministry of Health,
 - the Austrian Association of Pharmaceutical Manufacturers (Pharmig)
 - the industry (see next slide)
 - the mutual health objectives of the Pharma framework agreement
 - the Austrian Society for Paediatric and Adolescent Medicine (ÖGKJ)
 - the respective universities and Co-ordination Centres for Clinical Studies



Funding Support - Thank You!

Supported by the following companies as part of an agreement with Pharmig (Association of the Pharmaceutical Industry of Austria)



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