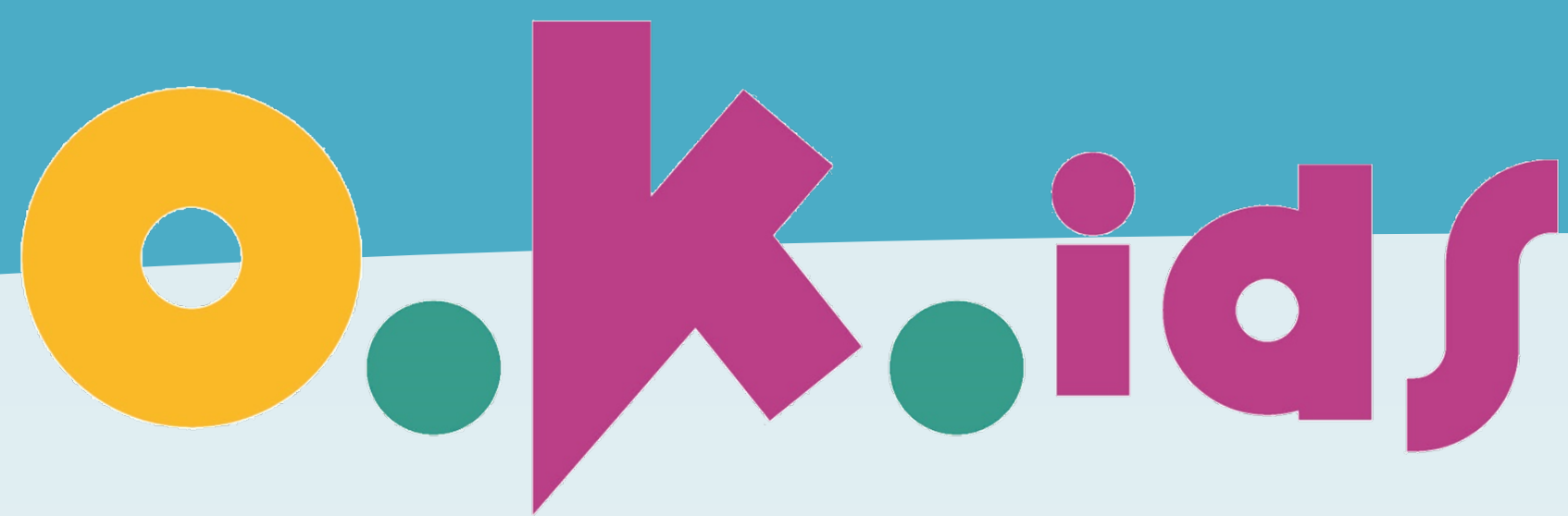


OKIDS Activities

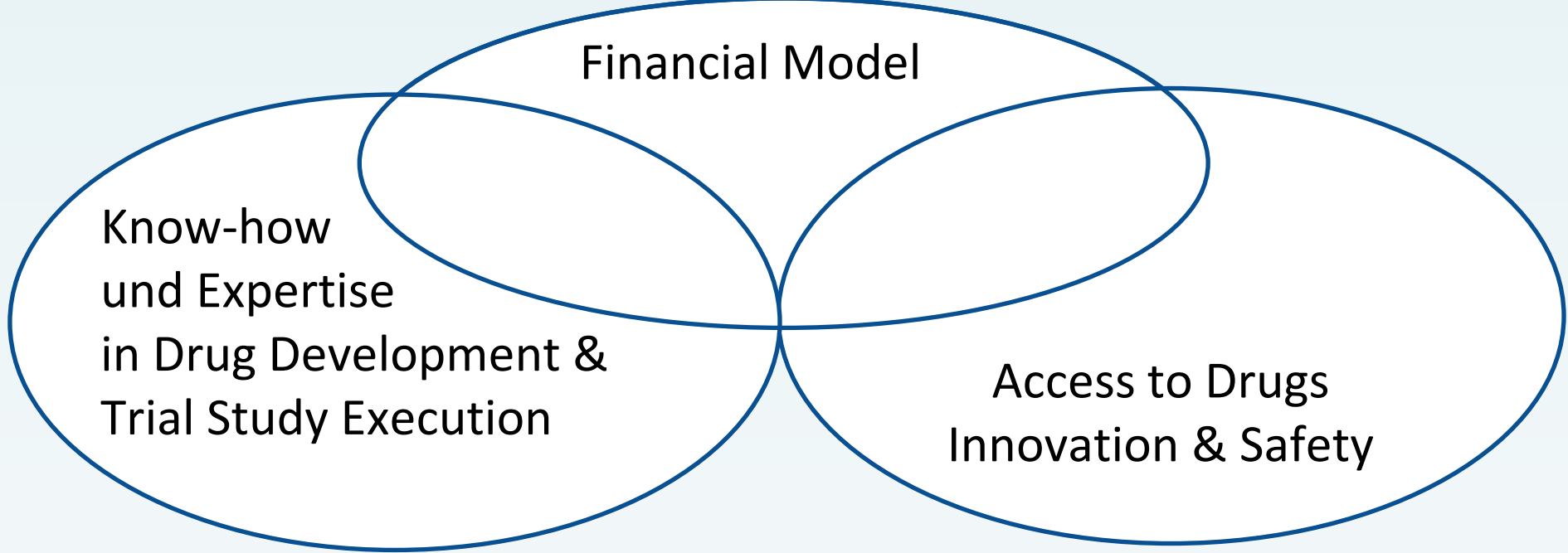
Austrian Organisation for Medicines in Children

Director: Univ.-Prof. Dr. Ruth Ladenstein
Project Management: Andrea Mikolasek



Background

OKIDS is a subsidiary company of the ÖGKJ (Austrian Society of Paediatrics) founded in 05-2013 based on a private-public partnership initiative to foster drug development for children and young people in Austria and to interact closely with medical experts and renown sites in Austria together with concerned patient groups.



Private – Public Partnership

Academia ÖGKJ Experts Med. Universities - KKS	Industry Pharmig	Politics Ministry of Health Competitive Grant : Public Insurers - Pharmig	Parents & Patients
--	----------------------------	--	-----------------------------------

Advantages for industry with OKIDS

„RIGHT“ PATH	„RIGHT“ SITES	„RIGHT“ PATIENTS	„RIGHT“ START
Realistic feasibilities	Choice of right centres & interested PI / local team willing to engage	Identification of patient accrual potential in rare sub-entities	Cooperation Common aims & shared experience
<input type="checkbox"/> Indication <input type="checkbox"/> Therapeutic proposal / Standard of care <input type="checkbox"/> Time of PI	<input type="checkbox"/> Hospitals • Severe diseases • Hospitalisation / day care contacts • Speciality consultations • Trained personal / study teams • Infrastructure <input type="checkbox"/> Private practice • Less severe diseases • Any indication • Practice assistant / patient administration • Basic infrastructure	<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 medial colleagues	<input type="checkbox"/> Cooperation between Sponsor (Industry) and OKIDS <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

OKIDS achievements after 4 years

Feasibilities and Clinical Trials

- **80 feasibilities for 35 industry partners** (CROs and Pharma)
- Total of **149 trials supported**, 92 industry, 57 academia
- **45 trials “OKIDS” initiated**
- **Activity in 35 paediatric diseases**
(23 non-malignant and 12 malignant indications)

Continued education support

Annual GCP courses at the Congress of the Austrian Society for Paediatrics (ÖGKJ) and at respective Working Group Meetings reflecting the scope of paediatric specialities.

Public appearance & Media

- **> 25 topic specific meetings**
- **7 press conferences, 124 total hits of press media**
- **OKIDS Web Page German-English**
- **4 movies** (<https://www.okids-net.at>)
- **2 publications**
- **15 new guidelines** (ÖGKJ Web Page)

1st OKIDS-EUPATI congress / workshop / press conference 15.11.2017
96 participants, 14 pharmaceutical companies

1st OKIDS-EUPATI Congress
Opening speech of SC Dr. Arrouas (BM ASKG) and Prof. Dr. Sperl (president of the ÖGKJ)

Round Table
Dr. Hemma Bauer (BM BfW), Dipl.-Ing. Dr. Michaela Fritz (University of Vienna), DDR, Karl-Heinz Huemer (AGES-MEA), Univ.-Prof. Dr. Ruth Ladenstein (OKIDS GmbH), Mag. Martin Munte (Pharmig and Amgen GmbH), PD Dr. Johannes Pleiner-Duxneuner (Roche Austria GmbH) and Mag. Dominique Sturz (patient representative)

Strategic partnerships in Austria - Prof. Dr. Rumler (Pfizer Austria GmbH), Ing. Claas Röhl (EUPATI Austria), Prof. Dr. Ruth Ladenstein (OKIDS GmbH) und Prof. Dr. Susanne Greber-Platzer (Medical University of Vienna)

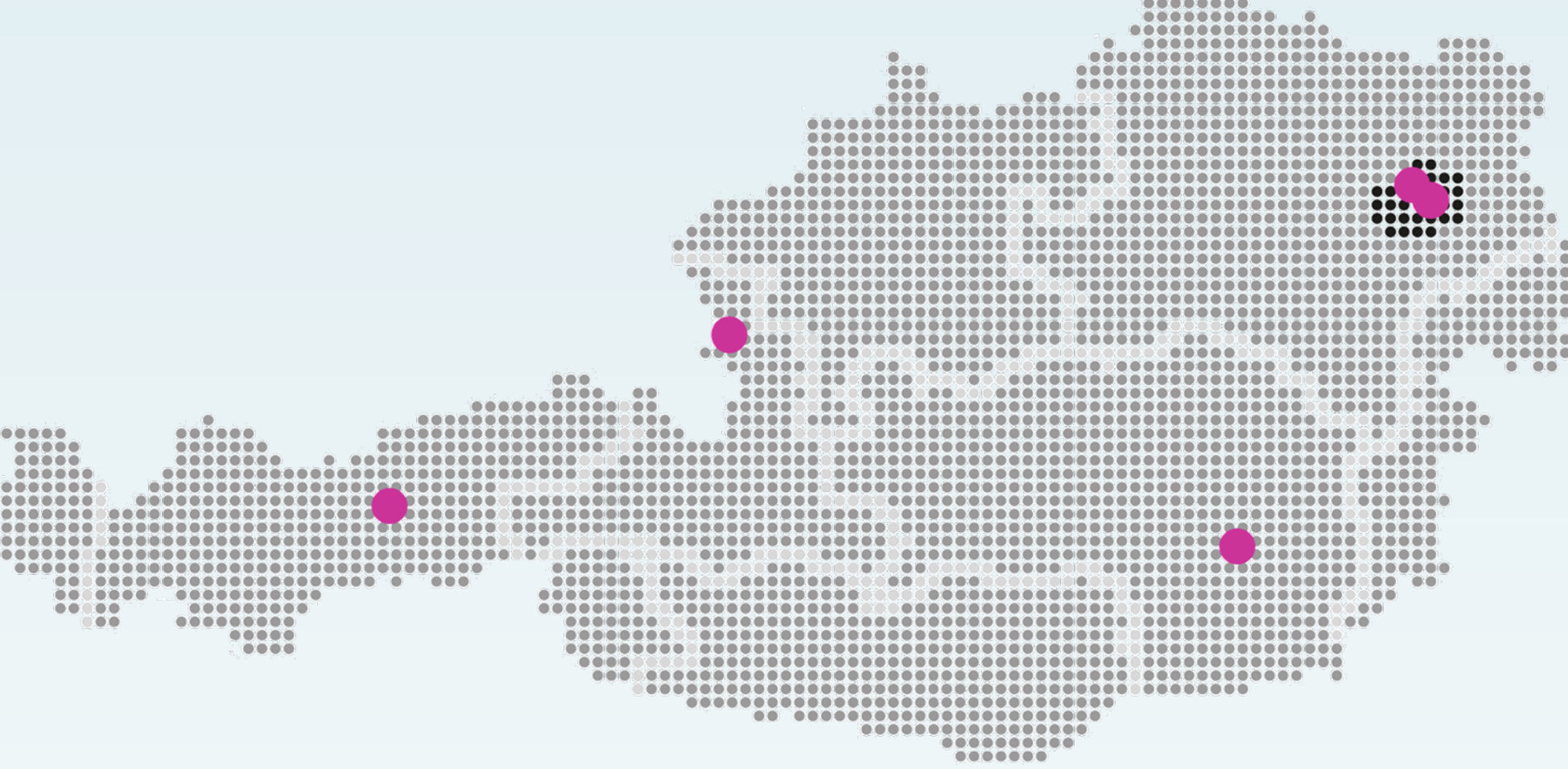
Communications on major achievement in drug development in children and their ongoing needs in the fields of: Neurology (Prof. Dr. Bernert), Neonatology (Prof. Dr. Berger), Metabolic diseases (Prof. Dr. Greber-Platzer, PD Dr. Lagler), Oncology / Car- T Cells (Prof. Dr. Peters), Rheumatology (Prof. Dr. Brunner), Drug Development (Prof. Dr. Male)

OKIDS-EUPATI Press Conference
a family affected with spinal muscular atrophy with Prof. Dr. Bernert and Prof. Dr. Ladenstein

OKIDS-EUPATI Press Conference 19.12.2016
EUPATI (Ing. Röhl, U. Holzer) and OKIDS Team (Prof. Dr. Ladenstein, PD Dr. Lagler) with patient representatives for neurofibromatosis and cystic fibrosis

OKIDS Sites

KKS Netzwerk



- **OKIDS has a single entry portal (network headquarter) in Vienna**

- **5 OKIDS AUSTRIAN sites** (4 universities and the St. Anna Children's Hospital) provide a **single point of contact (SPC) at site** for industry to run feasibilities and clinical trials: **Dedicated personnel (1 -2 study nurses / site) to support clinical trials.**

- OKIDS sites support also academic researchers & principle investigators to build **investigator driven clinical trials** (skill sets including i.e. data management and pharmacovigilance, insurance, monitoring, ...)

- **Site contract durations evaluation:**
Median 4 months (range 1-10 months), Mean 3.5 months

European Involvement



European Network of Paediatric Research at the European Medicines Agency

- **OKIDS benefits to be a recognised Enpr-EMA category 1 member**

The European Network of Paediatric Research at the European Medicines Agency (Enpr-EMA) strives to facilitate studies in order to increase availability of medicinal products authorised for use in the paediatric population. Enpr-EMA works by allowing networking and collaboration with members from within and outside the European Union (EU), including academia and the pharmaceutical industry. (<http://enprema.ema.europa.eu/enprema/showall.php> and <http://www.ema.europa.eu/ema>)

Links to the European Reference Networks
Part of Enpr-EMA working group on trial preparedness

- **European visibility and sustainability support through participation in European projects**

OKIDS is partner in PedCRIN: PedCRIN, funded by the Horizon 2020 INFRADEV-3 call, grant agreement nr 731046)

brings together ECRIN and the European Paediatric Clinical Trial Research Infrastructure (EPCTRI) to develop capacity for multinational paediatric clinical trials and develops tools specific for paediatric and neonatal trials

- **OKIDS is partner in the Collaborative Network for European Clinical Trials For Children**

c4c is a IMI2 call, fully granted as of April 2018. The principle objective is the development, implementation and evaluation of robust, sustainable and integrated arrangements for trial delivery to enhance and support paediatric drug development. c4c will be a collaborative effort between industry and non-industry partners addressing the needs of all participants and stakeholders. OKIDS will help to built a single point of contact structure in Europe for the collaborative networks.

USP



OKIDS is the only Organization for Medicines for Children in Austria coordinating the numerous tasks through its networking at home and abroad.

OKIDS is the Austrian partner for drug development and clinical research acting in close co-operations with all stakeholders to facilitate information, training and top-quality clinical trials.

Austrian Core Team 2018

	MANAGEMENT	OKIDS Personell at Sites			
WIEN	 Univ.-Prof. Dr. R Ladenstein	 A. Mikolasek	 Ch. Echtenr	 H. Ablaza	
WIEN	 Univ.-Prof. Dr. S. Greber-Platzer	 Univ.-Prof. Dr. C. Male	 Mag. DDr. A. Kreissl, MSc	 Ch. Wittmann, BSc	
GRAZ	 S. Sach-Friedl, BSc, MSc		 T. Esterl, BSc		
INNSBRUCK	 Mag. (FH) S. Embacher-Aichhorn		 M. Wenzel, MSc., BSc		
SALZBURG	 PD Dr. F.B. Lagler		 S. Geiersberger, BSc		



EUPATI–AUSTRIA focuses on patient education and involvement and interacts closely with patient organisations, academia, industry and government representatives as well as with health care provider teams to achieve a better involvement of patients in drug development. The EUPATI-Toolbox for patient representatives as well as an online library support education in the complex field.

OKIDS and EUPATI have signed a memorandum of understanding to act closely and in synergy in the field. (15.01.2017)

OKIDS and EUPATI organised a patient workshop at the 1st OKIDS Congress held in Vienna/Austria on 15.11.2017. OKIDS and EUPATI also convene press conferences together.

Acknowledgement

