

## RATIONALE FOR THE WORKSHOP

The new Paediatric Regulation requires the submission of a Paediatric Investigation Plan (PIP) to the Paediatric Committee of the EMEA "upon completion of human pharmacokinetic studies in adults as specified in Section 5.2.3 of Part 1 of Annex I to Directive 2001/83/EC" and to update this PIP whenever new information allows the refinement. The intention of the PIP is to stimulate identification of new (and marketed) compounds' suitability for meeting treatment needs for children early in the clinical development.

At the end of Phase I, no proof of efficacy and only marginal safety information in adults have been accomplished. For new compounds, most sponsors consider the preparation of a first PIP at that stage a challenge as the low level of information, stemming from literature and potential experience with similar compounds, makes planning at least highly speculative.

In addition, the resources for preparation of a PIP run the risk of being wasted when considering the high attrition rates after Phase I: with only 10-15% success to launch, there is a high chance that a submitted PIP would have to be withdrawn if the compound fails.

Therefore, sponsors are looking for efficient ways to generate information suitable for a PIP without requiring disproportionate resources. At this stage of development, human pharmacology may play a significant role.

### **This workshop is intended**

- **to establish a common understanding of available experience and tools in human pharmacology for drug development in paediatrics with new and marketed products and**
- **to identify the areas in which better knowledge should be developed in order to enable human pharmacology supporting a more substantiated PIP.**

The results of this workshop and further work from expert groups may lead to the development of guidance for human pharmacological contributions to a PIP.

## PROGRAMME COMMITTEE

### **Ingrid Klingmann**

Pharmaplex,  
Brussels, Belgium

### **Wolfgang Seifert**

Seifert Research,  
Berlin, Germany

### **Thomas Sudhop**

Bundesinstitut für Arzneimittel und Medizinprodukte, Bonn,  
Germany

## REGISTRATION

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Workshop fees:

EUR 300 Members of AGAH, Club Phase I, BAPU, AHPP1  
EUR 500 Non-Members

Fee includes admission to all sessions, lunches and coffee breaks.

Accommodation:

A number of rooms at special discounted rates are blocked and available via INTERCOM Dresden GmbH

Registration form available at:

Web: [www.agah-web.de](http://www.agah-web.de)



## WORKSHOP

# PAEDIATRIC INVESTIGATION PLAN -

## How to Adapt Clinical Development to the Particularities of Paediatrics?

### **An International Workshop on Human Pharmacology Strategies and Tools**

**13.-14. 01. 2009**

Gustav-Stresemann-Institut e.V.  
Langer Grabenweg 68  
D-53175 Bonn - Bad Godesberg  
Germany

## PROGRAMME

### Day 1

10:00 Registration

10:45 Welcome and Introduction  
*Ingrid Klingmann, Pharmaplex, Belgium*  
*Wolfgang Seifert, Berlin, Germany*

#### Session 1:

##### Requirements for PIPs and Current Practice

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*Chair: Chair invited*

11:00 EMEA's expectations and experiences with submitted PIPs, regulatory requirements for PIPs with new drugs, marketed and out-of-patent drugs as well as new paediatric formulations  
*Birka Lehmann, BfArM, Germany*

11:30 The European PIP as a step in industry's paediatric drug development process  
*Thomas Severin, Novartis, Switzerland*

12:00 Maturing physiological systems over life time – relevance of the ripening process for paediatric treatments  
*Speaker invited*

12:30 Lunch break

#### Session 2:

##### First in Child Studies With New Drugs: How to Find the Appropriate Age-Related Dose for Minors?

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*Chair: Thomas Sudhop, BfArM, Germany*

13:30 What works in current paediatric practice of off-label dose adjustment of adult doses?  
*Lothar Bernd Zimmerhackl, University of Innsbruck, Austria, Member of the Expert Panel for Paediatric Medicinal Products, BfArM, Germany*

14:00 Consideration on first paediatric dose, safety and the therapeutic range for different age groups  
*John Van den Anker, Children's National Medical Center, USA & Medicines for Children Research Network (MCRN), The Netherlands*

14:30 Intelligent early paediatric study designs for new drugs with small patient groups  
*Speaker invited*

15:00 Biometrical considerations on covariates: weight, age, gender, metabolic conditions...  
*Willi Weber, Sanofi-Aventis, Germany*

15:15 Progressive methods and tools to approach PK in minors like low dose administration for the assessment of clearance and for modeling  
*Joachim Grevel, Merck-Serono, Germany*

15:45 Coffee break

#### Session 3:

##### Human Pharmacology's Role for PIPs on Authorized Products

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*Chair: Monika Seibert-Grafe, KKS Mainz, Germany*

16:15 Consideration on the dose selection in different age groups and on safety  
*Wolfgang Rascher, University of Erlangen, Chair of the Expert Panel for Paediatric Medicinal Products, BfArM, Germany*

16:45 New paediatric indication for a marketed drug: Preclinical extrapolation vs bridging?  
*Klaus Olejniczak, BfArM, Germany*

17:15 New paediatric formulations: How and when should they be developed?  
*Jörg Breitzkreutz, University of Düsseldorf, Germany*

17:45 End of Day 1

### Day 2

#### Break-out Groups

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*After a scene-setting presentation by the Chair, the Break-out Groups will exchange information, brainstorm and discuss possibilities for human pharmacology to generate relevant information for PIPs on the respective category of drugs:*

8:00 **Group 1:**  
Human Pharmacology's contribution for PIPs with new drugs  
*Chair: Hannsjörg Seyberth, University of Marburg, Germany*  
*Rapporteur: invited*

#### Group 2:

Human Pharmacology's contribution to PIPs for paediatric indications for authorized products  
*Chair: Gerd Bode, University Marburg, Germany*  
*Rapporteur: invited*

#### Group 3:

Human Pharmacology's contribution to PIPs for new paediatric formulations  
*Chair: Martina Uttenreuther-Fischer, Boehringer-Ingelheim, Germany*  
*Rapporteur: Birka Lehmann, BfArM, Germany*

#### Group 4:

Human pharmacology's contribution to PIPs for rare indications  
*Chair: Ralf Herold, EMEA*  
*Rapporteur: Joachim Boos, University of Münster, Germany*

10:30 Coffee break

#### Session 4:

##### Reports from the Break-out Groups, Open Forum Discussion of Suggestions

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*Chair: Ralf Herold, EMEA*

11:00 Reports from the Break-out Groups  
*Rapporteurs*

12:00 Open Forum Discussion  
*Break-out Group Chairs and Rapporteurs*

13:15 Conclusions and next steps

**13:30 Farewell Lunch**