



Cardiology Network

AEPC – Task Force for Drugs and Therapeutics in Paediatric Cardiology

EnPR EMA Workshop

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AEPC

www.aepc.org

- **Association for European Paediatric Cardiology**

- Need for organization and coordination of Research and Trials in Paediatric Cardiology
- Most of drugs are off-label used
- Very few PIPs

- **Objectives**

- *Promote Research on therapeutics (medicines, devices) to increase the availability of authorised products.*
- *To create a European network of research centres and networks of excellence*
- *Define therapeutic needs and priorities*
- *Provide a platform for initiation of trials*
- *Focus on quality, safety and efficacy for their use in children*
- *To facilitate multicentre trials and European centres collaboration*
- *To improve the development and utilization of therapeutics in children with cardiac disease.*

Task force for drug studies

- ✓ Get approval from AEPC council
- ✓ Nucleus: Chair : J. Stein
 - Secretary: S. Di Filippo
 - Treasurer: J. Stein (current AEPC Council treasurer)
 - Councillors: C. Male, G. Oskarsson, M. Burch
- ✓ Call for interest
 - Contact national representatives
 - Identify centres from former industry trials

Task Force for Drugs and Therapeutics in Paediatric Cardiology



- Nucleus
- Network of members : expert centres of excellence in Europe
- Collaboration with Enpr EMA
- First meeting in London April 9th, 2013
- First business meeting in London, AEPC annual meeting May 25th, 2013
- Second business meeting in Helsinki, AEPC annual meeting May 24th, 2014



Cardiology Network

- **54 centers from 17 countries applied**
 - 13 UK
 - 8 France
 - 4 Germany
 - 3 Austria
 - 2 Italy, 2 Spain, 2 Portugal
 - 2 Netherlands, 2 Finland, 1 Sweden, 1 Iceland, 1 Ireland
 - 4 Romania, 5 Russia, 3 Czech Republik and 1 Poland.

Issues

- Define standards (treatment, diagnostics, biomarkers, etc)
- Research portfolio
 - Drug priority list – review paed needs CV drugs
 - Formulations
 - Dosing schemes
 - Pharmacokinetics in children
 - Guidelines and recommendations
- Methodological standards
- Inventory of centres
 - Centres with experience, infrastructure
 - Centres with patients
- Linking up with other working groups within AEPC
- Linking with EnprEMA

Initiation of TF organization

- **Network:**
 - 54 European centers / 17 countries
- **Cooperation with AEPC working groups:**
 - 11 WGs
 - Any field of Pediatric and Congenital Cardiology
 - Trials and registries ongoing
- **Links with Enpr EMA**
 - Lists of drugs
 - Interaction with industry

Initiated studies

- **Scimitar Registry**
 - Initiated in Italy (Padova)
 - Spreading through AEPC TF network
 - Include patients with any forms of Scimitar anomaly to improve knowledge, management, therapie, and guidelines
- **Cooperation with AEPC Arrhythmias group**
 - Ablative therapy in children < 15kg
 - First step= make a registry
 - Further steps: identify target issues and provide protocols and recommendations for ablation in infants

Second Task Force business meeting

Helsinki, 24th may 2014

- **Propranolol availability** -
 - Paediatric formula provided : short notice and no way of alternative-
 - Availability in Europe up, see the attachment.
- **Major issue** :
 - use of drugs off label,
 - no pediatric trials,
 - very few data on pharmacokinetics,
 - new formulas with no alternative
- **Paediatric drugs**
 - EMA
[listhttp://www.ema.europa.eu/docs/en_GB/document_library/Other/2013/04/WC500142474.pdf](http://www.ema.europa.eu/docs/en_GB/document_library/Other/2013/04/WC500142474.pdf)
- **AEPC Taskforce to approach industry**
 - not just taking paediatric formulas from the market without notice and given alternatives
 - but be in contact and interact before that step.

AEPC TF next steps

- List of drugs to work on
- Contacts with industry
- Identify and support drug trials of interest (EMA)
 - Beta blockers
 - Anti arrhythmic drugs
 - Anti hypertensive drugs
 - Anti pulmonary hypertensive drugs
 - Inotropes (levosimendan)
 - Anti thrombotic drugs
- Include devices