

# Hall Skåra

## PHA Europe

EMA  
London  
March 20th, 2018

# Initiatives

Ongoing initiative following the EMA, FDA and health Canada workshop June 2017

- Endpoints:
  - Non-invasive endpoints are being developed (e.g. 6MWT, actigraphy, NT-pro-BNP, MRI and echocardiography)
- Patient Reported Outcomes: New pediatric PROs should be implemented
- Clinical aspects: Patients and carers should be involved early in pediatric study designs

# Initiatives

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- Registries:
  - Challenges identified
  - Limitation of existing registries and difficulties in existing data
  - Redesign important in order to use registries in research
- Operational aspects of conducting pediatric PAH studies:
  - Lack of existing pediatric cardiology network - being addressed by developing a global multi-stakeholders pediatric PAH network

# Criteria & Methodology

- Criteria:
  - disease seriousness (progression, morbidity, QOL)
  - adult medication availability
- Methodology
  - Best results are achieved if all key stakeholders work together to help identify diseases & subgroups
    - Key stakeholders: Regulators, pharma, clinicians, researchers, patients and carers
  - Improved registries could be very useful
  - Pediatric networks could help develop criteria & methodology

