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Current Management and Treatment Result of PAH

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[II-S13-03]Pediatric Pulmonary Arterial Hypertension:A Worldwide View

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Despite enormous advances in the understanding and treatment of pulmonary arterial hypertension (PAH), the prognosis of children with this disorder remains poor. The approval of several PAH-targeted drugs and the introduction of evidence-based treatment algorithms, leading to improved outcomes in adult patients, seem to have benefited children with PAH substantially less.
Reasons for this " pediatric PAH-lag" include the heterogeneity of the disease in infancy and childhood, the lack of robust data on disease characteristics and the lack of controlled efficacy trials, where unique features of pediatric PAH preclude simple extrapolation from adult studies.
Recently, important observational data have become available from relatively large pediatric registries (TOPP, Reveal) and national or single-center pediatric cohorts. In the absence of controlled trials, disease registries are powerful tools and an invaluable source of information on epidemiology, diagnostics, outcome and current treatment strategies in pediatric PAH.
All etiologies of PAH described in adults, do occur in the pediatric patient population, however, in children with PAH, types and distribution of associated conditions and comorbidities differ importantly. Pediatric PAH most commonly presents either as idiopathic/heritable PAH (IPAH/HPAH) or as PAH associated with congenital heart disease (PAH-CHD). Further, PAH in infancy and childhood presents with specific features, such as underdevelopment or maladaptation of the pulmonary vasculature, lung hypoplasia, complex cardiovascular anomalies, associated congenital malformations, or genetic disorders, which might substantially affect pathophysiology, prognosis, and treatment options in pediatric pulmonary vascular disease.
Further, analyses of registry data indicated that although survival in pediatric PAH improved in the current era of PAH-targeted therapies it remains very unsatisfactory. Using the observational data from pediatric cohorts with standardized follow up, prognostic factors, treatment targets and clinical end points in children with PAH could be defined.
These insights have led the pediatric taskforce of the World Symposium on Pulmonary Hypertension in Nice 2014, to make some first steps in customizing the WSPH-Clinical Classification of Pulmonary Hypertension for pediatric PH and to propose diagnostic and treatment algorithms, specifically for children with PAH.
This presentation will summarize current insights in epidemiology and survival of pediatric PAH, address

differences and discrepancies in available contemporary data, and will propose goal-oriented treatment strategies for children with PAH.