Evaluating markers of positive response on therapy and survival of children with pulmonary arterial hypertension in Russia

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Objective: to evaluate an association between clinical, functional and haemodynamic features, current treatment options and outcomes in children with pulmonary arterial hypertension (PAH).

Methods: 50 consecutive patients with PAH were included and followed up through Jan 2011 - Dec 2014; mean age at 9.4±5.4 and 51% were males. Tests included routine cardiological examination; echocardiography, CT angiography and scintigraphy; six-minute walk test (6MWT) in children aged 7+; right right heart catheterization (RHC) and acute pulmonary vasodilator testing in 63% of pts.

Kaplan Meier Method and Cox proportional hazard model were employed for testing differences in survival.

Results: PAH was associated with congenital heart defects in 42 pts (84%), with portal hypertension in 2 pts (4%). In 21 pts (42%) more than one associated conditions were found. 34% of children were with Down's syndrome. Associated conditions were not identified in 12% patients. They were classified as having idiopathic PAH. Late diagnostics of PAH in children was observed in 71% of pts. At first examination 29% of pts had functional class (FC) II, 65% of pts had FC III; and 6% – FC IV.

Mean pulmonary artery pressure varied from 30 to 105 mmHg, and pulmonary vascular resistance (PVR) varied from 8.5 to 29.3 UW. Median cardiac index was 1.9±0.5 L/min/m² and median arterial oxygen saturation was 90±2.7%. Patients were treated with sildenafil (n=4), bosentan (n=25), combination therapy by bosentan and sildenafil (n=16), bosentan, sildenafil and Inhaled iloprost (n=5). The atrioseptostomy was performed in 4 pts. Median duration of follow-up was 42 months. Reduction of dyspnea and improvement of RV function were observed in 22% of pts. 6MWT distance increased by 116.2±12.7 m, PVR decreased by 8.9±3.4 UW. Five IV class pts have died. Reduction of dyspnea, improvement of RV function and increased cardiac index are significantly associated with positive response to therapy.

Conclusions: Pediatric PAH frequently presents with associated conditions and syndromal abnormalities. FC and hemodynamic parameters are the strongest predictors of survival in children with PAH. There are no statistically significant sex differences in incidence, age at onset, disease severity.