

**Current medical treatment in Pediatric Pulmonary Hypertension (PH) - Insights from the Global Registry Tracking Outcomes and Practice in Pediatric Pulmonary Hypertension (TOPP)**

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**Objectives:** The TOPP global registry provides demographic, clinical and outcome data in pediatric PH. One of the primary objectives was to describe current treatment. Treatment decisions were made by site clinicians without TOPP involvement.

**Methods:** 31 sites from 19 countries enrolled patients, diagnosis on/after January 2001, enrollment 2008-2010, age 3 months - 18 years at confirmatory right heart catheterization). PH was defined as: mPAP  $\geq$  25 mmHg, PCWP  $\leq$  12 mm Hg and PVRI  $>$  3 units  $\times$  m<sup>2</sup>.

PH targeted therapy (PHTT) included prostacyclin and its analogs (PGI<sub>2</sub>s), endothelin receptor antagonists (ERAs) and phosphodiesterase inhibitors type 5 (PDE5Is). Calcium channel blockers (CCBs) were considered as PHTT in responders to acute vasodilator testing. Supportive therapy included anticoagulation, oxygen, diuretics and/or digitalis.

**Results:** Of the 456 patients enrolled, 362 (79%) met all entrance criteria with 357 (99%) based on right heart catheterization and 5 (1%) on independently reviewed echocardiography. Of the PH confirmed patients, 102 (28%) were incident (defined as diagnostic right heart catheterization  $<$ 3 months prior to enrollment) and 260 (72%) were prevalent patients. For the present analysis only incident patients were included.

At diagnosis 10% (n=10) of incident patients were on PHTT, 76% (n=77) were treatment naïve and 15% (n=15) had incomplete data regarding treatment. PDE5Is were used in 52% (n=53), whereas 17% (n=17) of the patients received ERAs and 11% (n=11) PGI<sub>2</sub>s. CCBs for PHTT were used in 13% (n=13).

Supportive therapy in form of anticoagulation was provided in 17% (n=17) of the patients, oxygen in 27% (n=27) and diuretics in 30% (n=31). Digitalis was used in 16% (n=16) of the patients.

Of the incident patients, 66% (n=51) were started on PHTT monotherapy at diagnosis and 16% (n=12) started with dual therapy.

**Conclusion:** The largest proportion of pediatric PH patients enrolled in TOPP and recently commenced treatment start on PDE5Is (as monotherapy) at diagnosis. Long-term follow-up should provide invaluable data on how treatment patterns affect outcomes.