The session consisted of three lectures. First, current medical therapies were reviewed. Secondly, new treatment approaches were discussed. Lastly, non-medical treatment options were considered.

In pediatric PAH, the main treatment goals are reducing symptoms, improving WHO-functional class and exercise capacity, and improving quality of life and survival. Patients should be able to live their lives as normal as possible. Next to PAH-targeted therapies, supportive care is important. Pediatric PAH-patients should be treated and followed in specialized centers to optimize treatment. Before starting treatment, an acute vasoreactivity response testing should be performed to determine whether a patient will or will not respond to calcium channel blocker (CCB) therapy. The testing should be repeated every 1-2 years, as responsive patients can become non-responsive, which would necessitate switching from CCB therapy to PAH-targeted therapy. Regarding, PAH-targeted therapy, treatment options have improved tremendously in the last twenty years with the introduction of prostacyclins in 1995, endothelin receptor antagonists in 2001 and 5-phosphodiesterase inhibitors in 2005. Unfortunately, these drugs do have side-effects and are not curative. Ideally, treatment of PAH would result in turning the disease from irreversible back to reversible and eventually back to a normal pulmonary vasculature.

Based on randomized controlled trials (RCTs), treatment algorithms for adult PAH patients have been established. In pediatric PAH, there are no RCTs and patients are treated according to the adult guidelines. Although these algorithms give directions on how to start treatment, it is not well known how to optimize treatment during follow-up. Would a wait-and-see or a goal-oriented (treat to target) treatment strategy be optimal? Goal-oriented treatment has been suggested to improve outcome in adult PAH patients. Such a strategy could be beneficial in children also. However, treatment targets (or surrogate endpoints) to guide therapy are not well defined nor validated in pediatric PAH. Although subjective, WHO-functional class may be a suitable surrogate endpoint, as well as the plasma level of NT-proBNP. The introduction of PAH-targeted therapies improved survival in (pediatric) PAH patients, however prognosis remains poor. Escalation to PAH-targeted combination therapy might improve survival in pediatric PAH patients. Given the fact that a large proportion of patients is currently treated with PAH-targeted mono therapy, there may be room for improvement in this respect. We should ‘treat early’ and ‘treat to target’.

If medical treatment fails, non-medical treatment should be considered to preserve cardiac output and to reduce the right ventricle workload. Non-medical treatment options are atrial septostomy, Potts shunt and lung transplantation. Balloon atrial septostomy is most frequently used in pediatric PAH patients and leads to reduced heart rate, oxygen saturation and right atrial pressure and improved or maintained cardiac output and exercise capacity. It is mainly used to abolish syncope spells in pediatric PAH patients. Creation of a Potts shunt has also been shown to have positive effects. Furthermore, aortic banding might be an option in humans with PAH in the future.
**Suggested literature**


