Initial Clinical Experience With Oral Treprostinil (UT-15C) In Pediatric Pulmonary Hypertension at a Single Center

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**Purpose:** The specific aims of this retrospective study were to evaluate: 1) dosing and tolerance of oral treprostinil in patients 10-20 years old with weight >25 kg; and 2) clinical stability of pulmonary arterial hypertension with transition from parenteral, inhaled treprostinil, or add-on initiation of oral treprostinil.

**Background:** Pulmonary arterial hypertension (PAH) is an important cause of morbidity and mortality in pediatric patients. Advances in understanding the pathobiology of PAH have led to novel therapies. Administration of drug therapies targeting the prostacyclin pathway have historically been complex requiring an indwelling central venous line with risk of line infection and dislodgement, or placement of a subcutaneous catheter complicated by site pain and skin infections. Inhaled therapies require frequent dosing with compliance issues. These factors have increased the risk for delivery of prostacyclin therapy as well as impacted the patient’s quality of life. In December 2013 oral treprostinil (UT-15C) received FDA approval as the first orally administered prostacyclin for any disease.

**Methods:** A retrospective chart review of 8 patient initiations (5 IPAH, 3 CHD). All patients were WHO Class I. Patient selection was based on clinician assessment of clinically stability, including WHO FC II-III, 6MWD >425 meters, without RV failure, IV/SQ dose <125 ng/kg/min. All patients were on background ERA, PDE5i, and/or CCB therapy.

**Results:** Seven patients transitioned from prostacyclin (5 inhaled treprostinil, 1 inhaled iloprost, 1 SQ treprostinil). In 1 patient UT-15C was added to background therapy. Seven transitions were successful and did not require change in therapy. In 1 patient admitted for SQ to oral transition, clinical deterioration occurred during down-titration of SQ therapy and up-titration of UT-15C, resulting in immediate re-initiation of SQ therapy. One patient discontinued UT-15C after 4 months due to recurrent migraines. Six minute walk distance improved in 4/5 patients with repeat measurement. Most common adverse events (AE’s) included: headaches (88%), flushing (88%), nausea and vomiting (75%), migraines (25%), heart burn (25%), anorexia (25%), diarrhea (25%). Most AE’s were moderate, transient, and improved with slowing up-titration.

**Conclusion:** Oral treprostinil was well-tolerated in some pediatric PAH patients with clinical stability or improvement in 75%. Twenty-five percent of patients discontinued UT-15C because of worsening PAH and migraines.