The ASCENT Study: An Open-Label Prospective Multicenter Study to Evaluate Safety and Tolerability of DPI LIQ861 (YUTREPIA™) in Pulmonary Hypertension

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Background

Pulmonary hypertension (PH) is present in an important proportion of patients with ILD encompassing a large, heterogeneous group of diffuse parenchymal lung diseases. Idiopathic pulmonary fibrosis (IPF), chronic hypersensitivity pneumonitis, connective tissue disease related ILD, and combined pulmonary fibrosis and emphysema (CPFE) are the ILDs most commonly associated with PH [1]. Notably, up to 86% of patients with ILD develop PH[2]. Development of PH-ILD is linked to diminished exercise capacity, increased O_2 requirements, impaired quality of life, and higher mortality rates [3]. Available data also suggest a 3x increase in mortality in PH-ILD compared to ILD alone[4]. Dry powder inhaled (DPI) treprostinil has the potential to deliver treatment directly to well-ventilated lung tissue near the pulmonary arteries, and is approved for management of both pulmonary arterial hypertension (PAH, WHO Group 1) and PH-ILD. YUTREPIA™ is a novel DPI formulation of treprostinil, engineered with proprietary PRINT® technology to create precise and uniform drug particles, and enable more robust pulmonary drug delivery[5]. The open-label ASCENT study is the first prospective study to evaluate the safety and tolerability of DPI treprostinil in PH-ILD, addressing a critically unmet need in PH research.

Methods

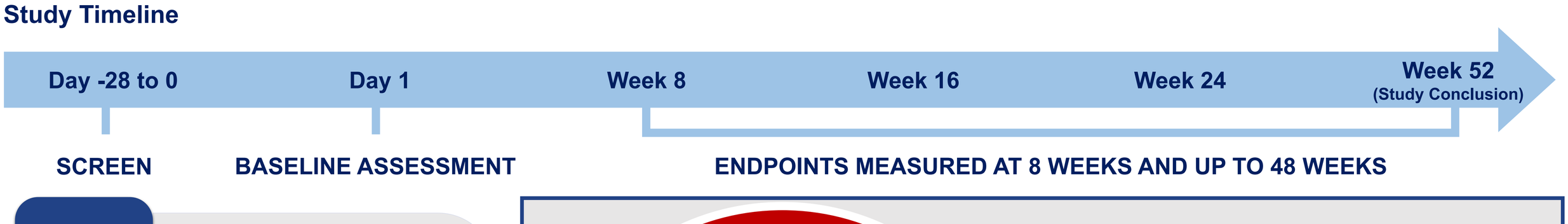
ASCENT is an open-label, prospective multicenter study that aims to evaluate the safety and tolerability of DPI treprostinil, YUTREPIA™ in subjects who have WHO Group 1 & 3 PH [6].

Study Design

COHORT A
- Target enrollment ~60 patients
- WHO Group 3 PH associated with interstitial lung disease (PH-ILD)
  - Up to 20% of the cohort may include combined pulmonary fibrosis and emphysema-associated ILD
- Additional limited exploratory subset of patients with mPAP ≥ 21 mmHg and PVR ≥ 3 WU*

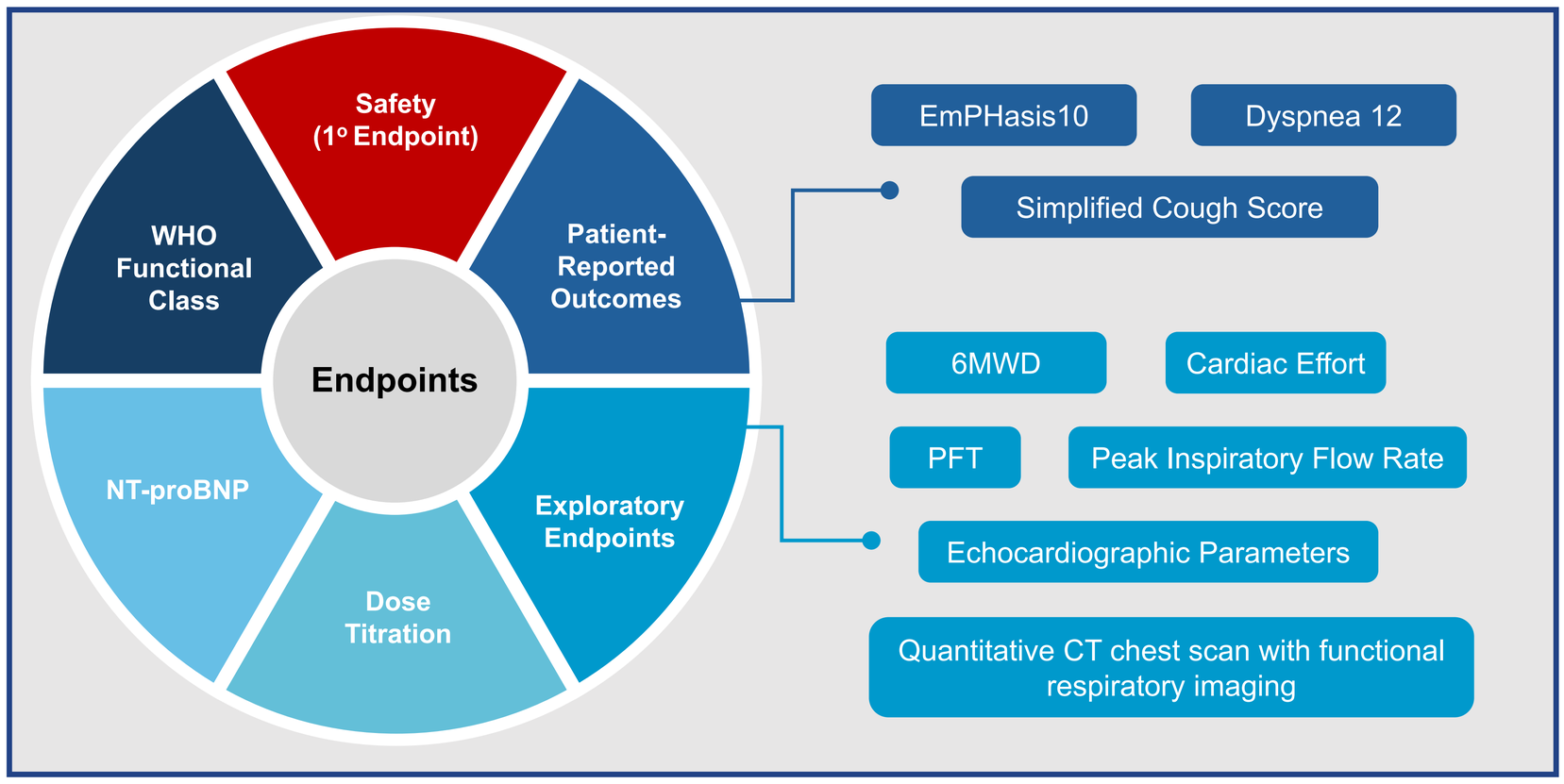
Additional Cohorts
- Additional cohorts with Group 1 or Group 3 PH may be defined in a future protocol amendment

Study Timeline

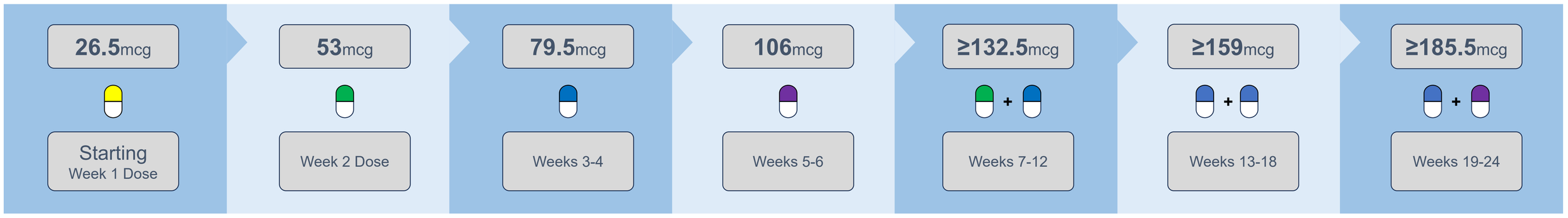


Key Inclusion Criteria

- mPA ≥30 mmHg and PVR ≥3 WU
- mPA >21 mmHG and PVR ≥3 WU*
- Baseline 6MWD ≥125m
- CT chest imaging consistent with ILD or CPFE
- FEV1/FVC ≥70%



ASCENT Dosing Guidance



Conclusions

ASCENT is the first open-label, prospective, multicenter investigation designed to evaluate the safety and tolerability YUTREPIA™ in prostacyclin naïve patients with PH-ILD, including CPFE patients.

References and Disclosures

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Conflict of Interest disclosures: **S. Patel**, **A. Galloway**, **G. Gwyn**, and **R. Saggar** are salaried employees and shareholders of Liquidia Corporation.