A RETROSPECTIVE STUDY INVESTIGATING THE REAL-WORLD USE OF MONOTHERAPY IN PULMONARY ARTERIAL HYPERTENSION (PAH) IN THE US

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Background

- Observational studies can provide descriptive data regarding real-world management of people living with PAH
- The current standard of care for PAH is considered upfront combination therapy for most patients[1]
- This analysis characterized real-world treatment patterns in the US and investigated factors associated with, and reasons for, use of monotherapy based on a retrospective medical chart review

Methods

- An online questionnaire was fielded to PAH-treating physicians in the US between December 2023 and February 2024
- Respondents were required to:
  - Have 5–40 years of experience in their specialty
  - Be personally involved in management and treatment of ≥10 PAH patients in the previous month, with ≥5 patients treated with PAH-specific therapy
- Physicians provided deidentified medical record data for up to 7 of their most recent adult patients with PAH meeting the following criteria:
  - Diagnosed ≥1 year ago
  - World Health Organization (WHO) Functional Class (FC) II-IV (FC IV limited to ≤1 chart)
  - Currently receiving PAH-specific therapy
  - Primarily managed for their PAH by the respondent, and
  - Not currently participating in a clinical trial
- Questions about treatment regimens were focused on PAH-specific monotherapy, dual combination therapy, or triple combination therapy

Results

PHYSICIANS

- Medical chart data representing 768 patients was provided by 72 pulmonologists and 40 cardiologists from >80 institutions
- Physicians were associated with
  - Pulmonary Hypertension Association (PHA)-certified Centers of Comprehensive Care, CCCs (45%)
  - PHA-certified Regional Clinical Programs, RCPs (17%)
  - PAH centers without PHA certification, non-PHA (19%)
  - A non-PAH-focused institution, non-PAH center (20%)

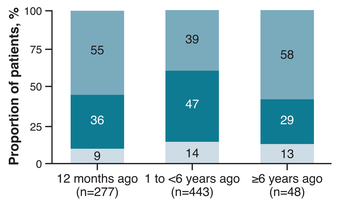
PATIENTS

- 53% of patients were female, with a mean age of 55 years, and were diagnosed with PAH 1–3 years ago
- PAH-specific monotherapy was used in 46% of patients at the time of the survey and consisted of
  - endothelin receptor antagonist (53%); phosphodiesterase 5 inhibitor (32%); prostacyclin, PC, oral/inhaled (8%); PC intravenous/ subcutaneous (5%); soluble guanylate cyclase stimulator (2%)
- Cardiopulmonary comorbidities were present in 68% of patients

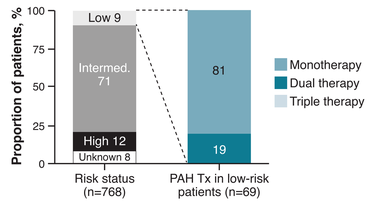
Results (continued)

- Patients diagnosed with PAH 1 or ≥6 years ago, or those with low-risk status (as recorded in the patient charts), were less likely to be prescribed a PAH-specific combination regimen

PAH Regimen by Time Since Diagnosis



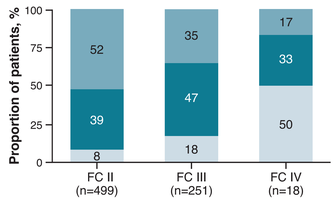
PAH Regimen for Low-Risk Patients



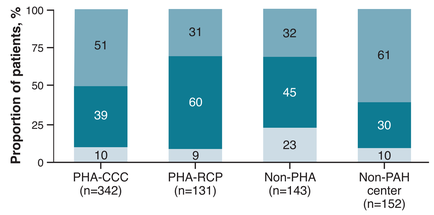
Intermed, intermediate; PAH, pulmonary arterial hypertension; Tx, therapy.

- Patients with WHO FC II, potentially considered to have less severe disease, were more likely to be treated with monotherapy
- PAH care at CCCs coincided with higher monotherapy use, as compared to in other treatment settings except for non-PAH-focused settings

PAH Regimen by Current Functional Class



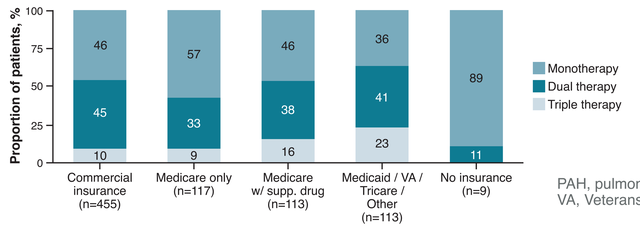
PAH Regimen by PHA Center Type



CCC, Centers of Comprehensive Care; FC, Functional Class; PAH, pulmonary arterial hypertension; PHA, Pulmonary Hypertension Association; RCP, Regional Clinical Programs.

- Combination therapy use was lower for patients with Medicare-only or no insurance compared to patients with other types of insurance coverage

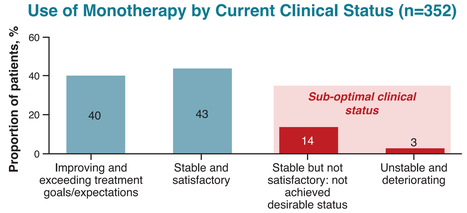
PAH Regimen by Insurance Type



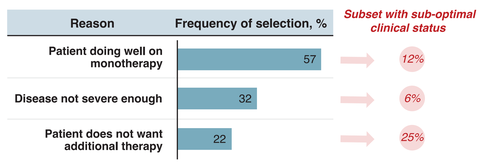
PAH, pulmonary arterial hypertension; supp, supplemental; VA, Veterans Affairs; w, with.

- The most frequently selected reasons for not prescribing combination therapy were focused on low disease severity and patient choice
- Of the 57% of patients considered to be doing well on monotherapy, 12% had suboptimal status (ie, "stable but not satisfactory" or "unstable and deteriorating")
- Of the 22% of patients on monotherapy who did not want additional therapy, 25% had suboptimal status

Use of Monotherapy by Current Clinical Status (n=352)



Reasons for Treatment With Monotherapy for PAH* (n=352)



*Other reasons for monotherapy treatment included: drug out-of-pocket cost (5%), not enough evidence that combination therapy is better than monotherapy in this patient subgroup (4%), not recommended in guidelines (4%). Concerns related to reimbursement, compliance, side effects, regimen complexity, and patient support were selected for ≤2% of patients on monotherapy.

What are the limitations of this observational study?

- This patient sample may not represent most patients with PAH, as 36% of patients were diagnosed 12 months ago
- Respondent practice settings were self-reported

KEY RESULTS

- Monotherapy use was higher in patients diagnosed with PAH 1 year or ≥6 years ago, WHO FC II, receiving care at CCCs or non-PAH centers, on Medicare-only or no insurance
- Top reasons for monotherapy: patients doing well on monotherapy, disease not severe enough, and patient choice of no additional therapy
- Sub-optimal clinical status was reported for 17% of patients in total, including 12% of the patients considered to be doing well on monotherapy, 6% of the patients with disease not considered severe enough for combination therapy, and 25% of patients who did not want additional therapy

WHAT DO THE RESULTS OF THIS STUDY MEAN?

- Heavy use of monotherapy in patients with PAH contrasts with recent guidelines[1] and suggests an unmet need for health care provider (HCP) education or alternative treatment options
- Monotherapy use in patients treated at a CCC or a non-PAH center may point to the complexity of PAH treatment in the real world, requiring individualized care in patient situations not addressed by guidelines
- In patients with Medicare-only or no insurance, cost of therapy may contribute to higher monotherapy use
- The main reasons for prescribing monotherapy focused on low disease severity and patient choice, even when the patient's PAH status was considered suboptimal. This highlights an important role of patient choice and disease education in PAH treatment

Conclusions

- This observational study demonstrates significant monotherapy use in an era with expanding evidence that combination therapy should be considered for the majority of patients with PAH
- Real-world treatment patterns suggest PAH treatment selection is complex and individualized, possibly following an escalate-as-needed approach based on physician perceptions of disease severity or patient stability or patient decisions
- The drivers and barriers of patient choice in PAH treatment, particularly in the setting of unsatisfactory or declining PAH status, warrant further exploration in future research

Reference: 1. Humbert M, et al. *Eur Heart J.* 2022;43(38):3618-3731.

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