Poster Number 30

Patient preferences regarding the use of combination ERA+PDE5i for the treatment of pulmonary arterial hypertension: Results from a discrete choice experiment

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Key takeaway

Patients prefer a single-tablet combination therapy (STCT) over traditional multi-pill regimens, mostly due to its ability to streamline their prescriptions and reduce pill burden. Costs remain a significant factor in determining whether patients can afford the convenience an STCT offers.

Background

- Upfront combination therapy with endothelin receptor antagonist plus phosphodiesterase 5 inhibitor (ERA+PDE5i) is the standard of care for patients with pulmonary arterial hypertension (PAH) due to improved patient outcomes.[1,2]
- Despite guidelines, many continue to be treated with monotherapy at 6 months post-initiation.[2,3]
- Reasons for this disconnect in real-world versus recommended prescribing practices are unclear.
- In this study, we examined treatment-level attributes affecting patients' perceptions and willingness to adopt dual combination of ERA+PDE5i.
- We also explored if a single-tablet combination therapy (STCT) might provide additional benefits and enhance patient participation in treatment choices.

Methods

- This was an online survey of 201 self-reported patients with PAH in the US from PHAR (Pulmonary Hypertension Association Registry), the largest active longitudinal registry tracking patients with PAH across the country.
  - Patients must have used an oral PAH medication for 3+ months in the past year.
  - Patients with a self-reported diagnosis of chronic thromboembolic pulmonary hypertension, interstitial lung disease, or diastolic heart failure were excluded.
- Seven attributes associated with ERA+PDE5i therapy were explored as part of a discrete choice experiment (DCE) to assess patients' willingness to adopt ERA+PDE5i (**Table 1**).
  - Attributes and levels were obtained via literature review and input from clinical advisors.
- Additional exploration of STCT on ERA+PDE5i acceptance was assessed via a supplemental questionnaire.

TABLE 1: Treatment Attributes and Levels in the DCE

Attribute	Level 1	Level 2	Level 3	Level 4	Level 5
Out-of-Pocket Costs	<$20	$20–$50	$50–$100	$100–$200	$200+
Dosing Frequency	One pill, once daily	Three pills, multiple times a day	Four pills, multiple times a day	Ten to thirteen pills, multiple times a day	NA
Discontinuation Due to Side Effects	7% of patients	8% of patients	9% of patients	12% of patients	NA
Prior Authorization	Requires 1 prior authorization	Requires 2 prior authorizations	NA	NA	NA
Pharmacies	Requires 1 pharmacy	Requires 2 pharmacies	NA	NA	NA
Dose Increase (Titration)	2 steps to reach goal dose	3 steps to reach goal dose	4–5 steps to reach goal dose	NA	NA
Patient Support Program	Available	Not available	NA	NA	NA

NA, not applicable.

- We utilized adaptive choice-based conjoint analysis methodology to ask patients to select the treatment profile they found most attractive (see **Table 2** for an example choice trial).
- These iterative choice trials calculated preference weights for each individual level for each attribute measured. These preference weights were used to calculate the relative importance of each attribute.
  - Larger differences across preference weights *within* a single attribute indicated greater influence in shaping patients' willingness to adopt ERA+PDE5i.
- Relative importance scores summarized the most/least influential attributes driving patients' willingness to adopt ERA+PDE5i.
- Patient characteristics and attitudinal beliefs were analyzed descriptively and in aggregate across the total sample.

TABLE 2: Example of a Choice Task From the DCE

"Please indicate whether each PAH treatment below is a possibility or not for you. Please assume that all treatments are similarly effective at treating PAH."

Therapy Characteristic	Therapy A	Therapy B	Therapy C
Out-of-Pocket Costs	<$25	$50–100	$50–100
Dosing	One pill, once daily	Three pills, multiple times a day	Ten to thirteen pills, multiple times a day
Prior Authorizations	Requires 1 prior authorization	Requires 2 prior authorizations	Requires 1 prior authorization
Pharmacies	Requires 1 pharmacy	Requires 1 pharmacy	Requires 2 pharmacies
Discontinuation Due to Side Effects	8%	7%	9%
Dose Increase	2 steps to reach goal dose	3 steps to reach goal dose	4–5 steps to reach goal dose
Patient Support Program	Available	Available	Not available

Results

- The most common regimens at the time of the survey were triple therapy PDE5i+ERA+prostacyclin (36.8%), double therapy PDE5i+ERA (17.9%), and monotherapy PDE5i (15.4%) (**Table 3a**).
- Respondents were primarily White (86.1%) and female (88.6%). A majority were not employed (70.2%), with over one-third reporting a disability (38.3%) (**Table 3b**).

Factors Influencing ERA+PDE5i Adoption

- The 2 most important factors influencing ERA+PDE5i adoption were the out-of-pocket costs (33.7) and dosing frequency (31.5) (**Figure 1**).
- Individual preference weights confirmed that patients were most accepting of ERA+PDE5i therapies when available at the lowest out-of-pocket cost and the least frequent dosing regimen (i.e., one pill, once daily) (**Figure 2**).
- Availability of patient support programs, discontinuation due to side effects, number of pharmacies, number of prior authorizations, and dose increase (titration) were less important.

TABLE 3a: Patient Characteristics

Characteristic	N=201
Current Treatment*, n (%)	
PDE5i+ERA+prostacyclin	74 (36.8)
PDE5i+ERA	36 (17.9)
PDE5i monotherapy	31 (15.4)
ERA monotherapy	22 (11.0)
Other	16 (8.0)
sGC+ERA+prostacyclin	15 (7.5)
sGC+ERA	7 (3.5)
Time Since Diagnosis, years	
Mean (SD)	10.6 (8.0)
Median (Range)	9 (1–38)

*PDE5i (sildenafil or tadalafil); ERA (bosentan, ambrisentan, or macitentan); prostacyclin (selexipag, treprostinil, epoprostenol, or iloprost); sGC (riociguat).
SD, standard deviation; **sGC**, soluble guanylate cyclase.

TABLE 3b: Patient Characteristics

Characteristic	N=201
Sex, n (%)	
Female	178 (88.6)
Race, n (%)	
White	173 (86.1)
Black or African American	10 (5.0)
Asian	
Native Hawaiian or Other Pacific Islander	1 (0.5)
Two or more races	5 (2.5)
Prefer not to answer	6 (3.0)
Employment, n (%)	
Employed full time	35 (17.4)
Self-employed	11 (5.5)
Employed part time	14 (7.0)
Manage family/household	21 (10.5)
Not employed and looking for work	3 (1.5)
Not employed and not looking for work (e.g., student)	33 (16.4)
Not employed and unable to work (e.g., disability)	77 (38.3)
Prefer not to answer	7 (3.5)

FIGURE 1: Relative Importance Scores of Each Attribute

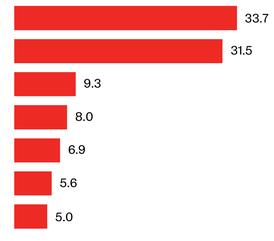
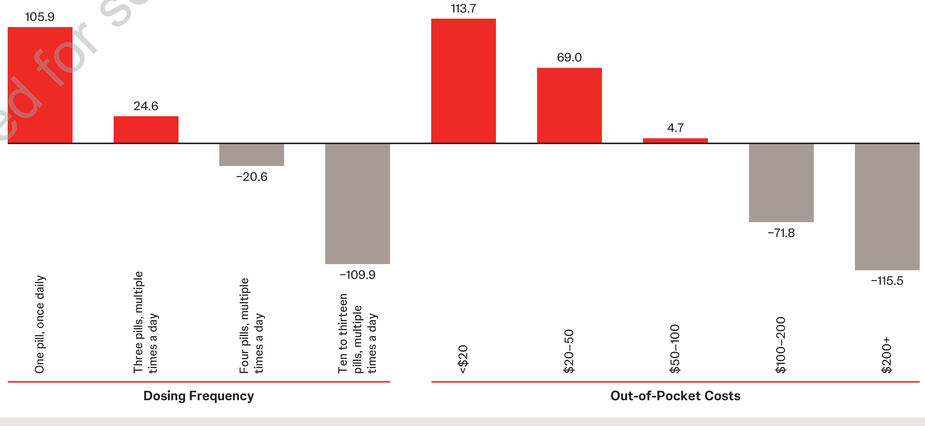


FIGURE 2: Individual Preference Weights – Top 2 Attributes



Blinded Choice of PAH Therapies

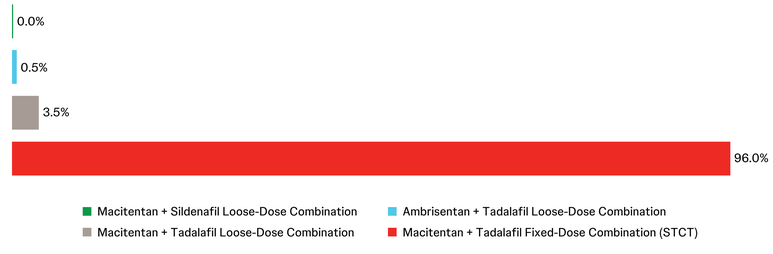
- After each DCE trial, patients were asked to select their preferred therapy from an array of four blinded (unlabeled) treatment profiles (**Table 4**).
- Patients displayed an overwhelming preference for an STCT of macitentan-tadalafil (96.0%) over alternative traditional "loose-dose" profiles (<5%) (**Figure 3**).

TABLE 4: Blinded Choice Exercise – STCT Preference

Treatment Profiles Shown	Treatment A	Treatment B	Treatment C	Treatment D
Dosing Frequency	1 tablet once daily	3 tablets per day	Up to 10–13 tablets per day**	Up to 3 tablets per day
Dose Increase (Titration)	Available	Available	Available	Available
Discontinuation Due to Side Effects*[4–6]	8%	7%	9%	12%
Pharmacies Required	Requires 1 pharmacy	Requires 2 pharmacies	Requires 2 pharmacies	Requires 2 pharmacies
Prior Authorizations	Requires 1 prior authorization	Requires 2 prior authorizations	Requires 2 prior authorizations	Requires 2 prior authorizations

Treatment profiles were based on existing PAH dual oral combination therapies; safety is reported as the unadjusted proportion of participants who experienced edema in the available clinical trials. All therapies were presented in blinded (unbranded) fashion (e.g., "Treatment A").
*Presented to respondents in the DCE as "the percentage of patients that stopped medication due to side effects."
**Assumes maximum dose of 60–80 mg sildenafil.

FIGURE 3: Blinded Choice Exercise – STCT Preference



Perceptions of an STCT for ERA+PDE5i Adoption

- Results from a supplemental questionnaire confirmed that most patients believed that an STCT would reduce pill consumption (83.1%) and time spent managing prescriptions (68.7%) (**Figure 4a**).
- Over one-third of patients identified benefits to compliance (42.3%), cost (39.8%), and dose burden (37.3%).
- Approximately one-third of patients reported benefits to treatment initiation (34.8%), followed by adherence (39.3%) (**Figure 4b**).

FIGURE 4a: Perceptions of STCT – Pros

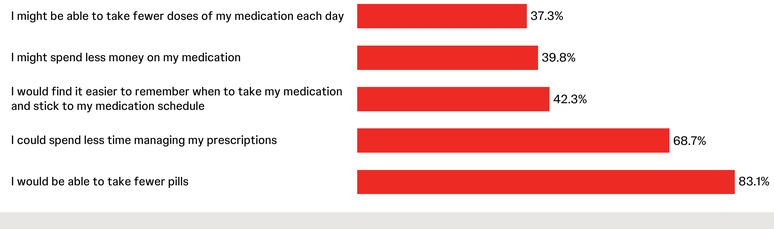
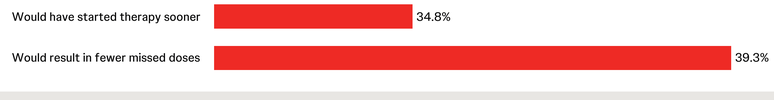


FIGURE 4b: Influence of STCT on Treatment Behavior



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