Understanding Drivers and Barriers to Participation in Clinical Trials for Patients With Pulmonary Arterial Hypertension (PAH)

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BACKGROUND
• Clinical trials are key to providing access to novel therapies and improving outcomes, but recruitment is challenging
• To our knowledge, patient perspectives regarding trial enrollment are not well understood
• We conducted a patient survey to better understand the needs of patients when considering clinical trial involvement

OBJECTIVE
• Identify factors that motivate or discourage patients with PAH to participate in a clinical trial

METHODS
• A 33-question survey was administered through the Rare Patient Voice, an online platform comprising ~850 patients with PAH
• Patients were eligible to participate if
  – ≥ 21 years of age
  – currently treated with ≥ one PAH medication
  – ≥ 21 years of age
  – diagnosed with PAH for ≥ six months, and
  – never participated in a clinical trial
• The survey protocol met regulatory guidance for patient-focused trial enrollment and was approved by a central institutional review board (Acorda, Inc.). All respondents provided informed consent prior to survey initiation.

RESULTS
• 102 patients completed the survey
• Table 1. Demographics and baseline characteristics (N=102)

<table>
<thead>
<tr>
<th>Characteristic</th>
<th>%</th>
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<tbody>
<tr>
<td>Age, mean</td>
<td>51.4 years</td>
</tr>
<tr>
<td>Female, %</td>
<td>88</td>
</tr>
<tr>
<td>College or graduate degree, %</td>
<td>51</td>
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<tr>
<td>Time since PAH diagnosis, mean</td>
<td>8.3 years</td>
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Patient perception of disease status over past 60 days, %
• Getting a lot better: 3
• Getting a little better: 8
• Staying the same: 69
• Getting a little worse: 21
• Getting a lot worse: 0

Number of current PAH medications, (10/9/7), %
• 25 / 38 / 57

Fifty-three and 37% of patients responded to be “very interested” or “somewhat interested,” respectively, in participating in a clinical trial (Figure 1A). Seventy-five percent reported not having discussion about clinical trial participation with their clinicians (Figure 1B); however, 93% reported they would welcome information and discussion. When asked how they would feel if a health care provider recommended I participate in a clinical trial, 92% would be very interested or somewhat interested. Seventy-three percent of patients responded to be “very interested” or “somewhat interested” in receiving information about clinical trial participation. Seventy-two percent of patients reported to be “very interested” or “somewhat interested” in receiving information about clinical trial participation.

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CONCLUSIONS
• To our knowledge, this is the largest survey of PAH patients who have never participated in a clinical trial
• Although PAH patients are interested in clinical trials, in this survey, three quarters of participants were not approached about clinical trial enrollment
• Most encouraging factors provided by respondents included doctor recommendation, ability to continue current PAH therapies, study drug targets a new potential treatment pathway, and current health care provider is an investigator, allowing continuity of care
• Most discouraging factors included need to discontinue current PAH therapies, need to receive care from a new PAH specialist, possibility of placebo assignment, and requirement for invasive procedures
• Further education is needed for both patients and clinicians to encourage participation in clinical trials

LIMITATIONS
• Data regarding healthcare provider practice setting (academic vs community) was not available at the time of this survey
• Because only patient perspectives were captured, health care provider reasons for not offering study information is unknown

Presented at the PHA 2022 International PH Conference and Scientific Sessions, June 10-12, Atlanta, GA