1010

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

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 \geq one PAH medication
- diagnosed with PAH for \geq six months, and never participated in a clinical trial
- The survey protocol met regulatory guidance for patient-focused drug development and was approved by a central institutional review board (Advarra, Inc.). All respondents provided informed consent prior to survey initiation.

RESULTS

102 patients completed the survey

| Table 1. Demographics and baseline characteristics (N=102) | | |
|--|--------------|--|
| Age, mean | 51.4 years | |
| Female, % | 88 | |
| College or graduate degree, % | 51 | |
| Time since PAH diagnosis, mean | 8.3 years | |
| 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, (1/2/3), % | 25 / 38 / 37 | |

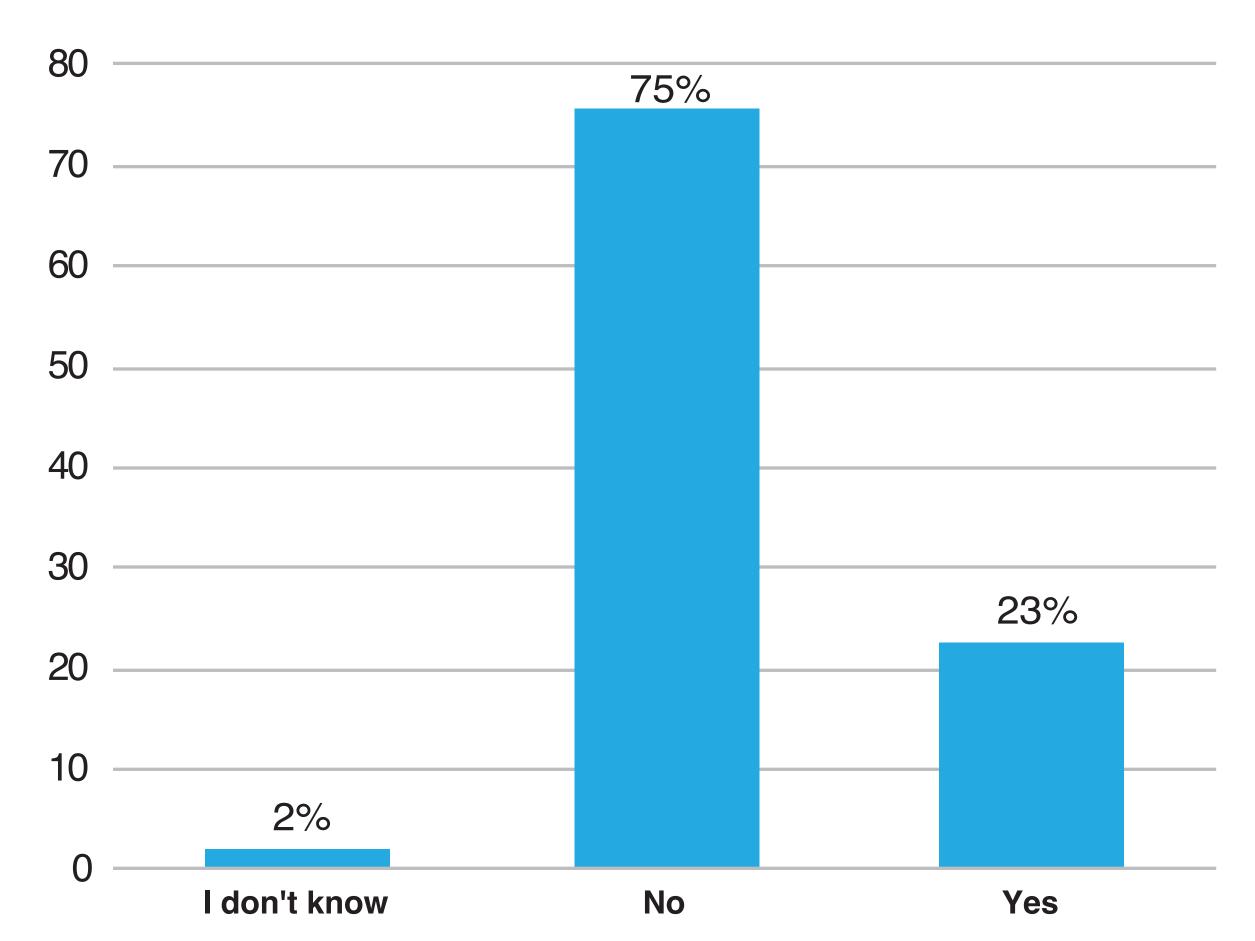


| RESU |
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| Figure 1. Patier | nt |
|--------------------|----|
| (B) clinical trial | Ķ |

| Α | Q: H |
|---------|------|
| Percent | 60 — |
| | 50 — |
| | 40 — |
| | 30 — |
| | 20 — |
| | 10 — |
| | 0 — |





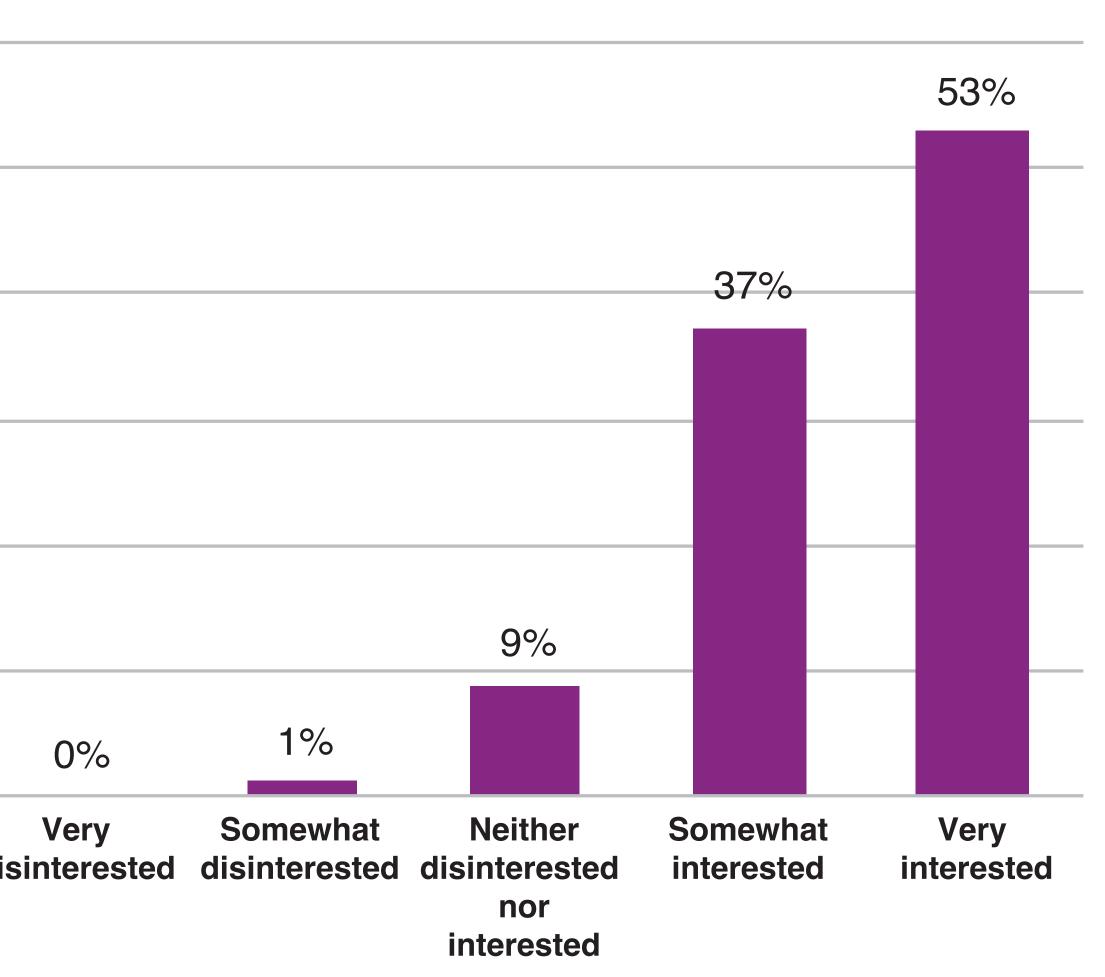
- (Figure 1A)

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LTS

t interest in (A) and HCP communication about participation

low interested are you in participating in a clinical trial for PAH at some time in the future?



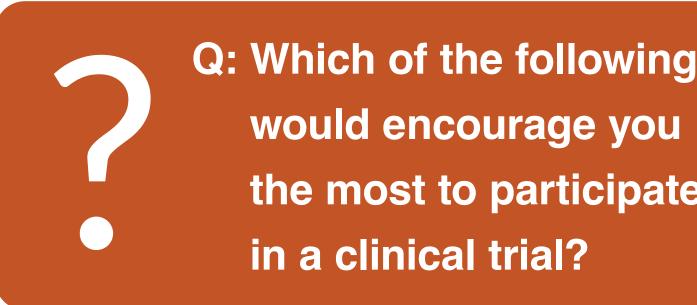


• Fifty-three and 37% of patients responded to be "very interested" or "somewhat interested", respectively, in participating in a clinical trial

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 suggested clinical trial enrollment, participants provided the following top emotive responses: 'interested' (80%), 'excited' (41%), 'good' (30%), 'grateful' (28%), and 'thankful' (27%)

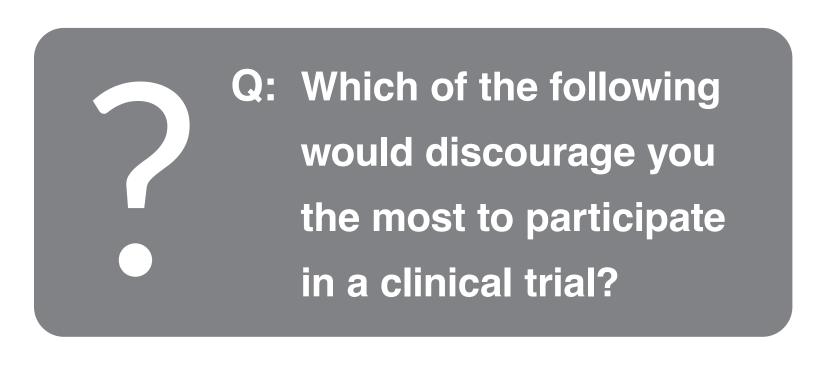
Figure 2. Top patient-reported factors encouraging clinical trial participation



My doctor recommends I participate in the study The study does not require me to come off one or more of my current PAH therapies The investigational drug targets a new potential treatment pathway My doctor is participating in the study, so I would not have to go to someone new After the trial, there is an option to receive the investigational therapy (not placebo) After the study, I would receive the results of the study back There is the option for some site visits to be done via tele visit and/or home heath care I know someone else who is participating in the trial 1%

Q: Which of the following the most to participate My doctor provides me with easy to understand materials explaining the study / requirements for participation Company sponsoring study is a known supporter of the patient organizations supporting my disease community (e.g. phaware)

Figure 3. Top patient-reported factors discouraging clinical trial participation



LIMITATIONS

CONCLUSIONS

- and requirement for invasive procedures

Study would require me to stop one or more of my current PAH meds My doctor is not participating in the study, so I would have to see a new PAH specialist A chance for getting placebo Study would require one or more invasive procedures, eg. right heart cath After the trial, I would not be allowed to stay on the investigational therapy Multiple days off work required for study visits 4% Concerns about COVID-19 3%

• 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

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 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,

• Further education is needed for both patients and clinicians to encourage participation in clinical trials

