

PHenomenal Hope 2024

Knowledge, Research & Advocacy in PH

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Pulmonary arterial hypertension clinical trial endpoints and outcomes survey: patient perspectives and preferences

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Introduction

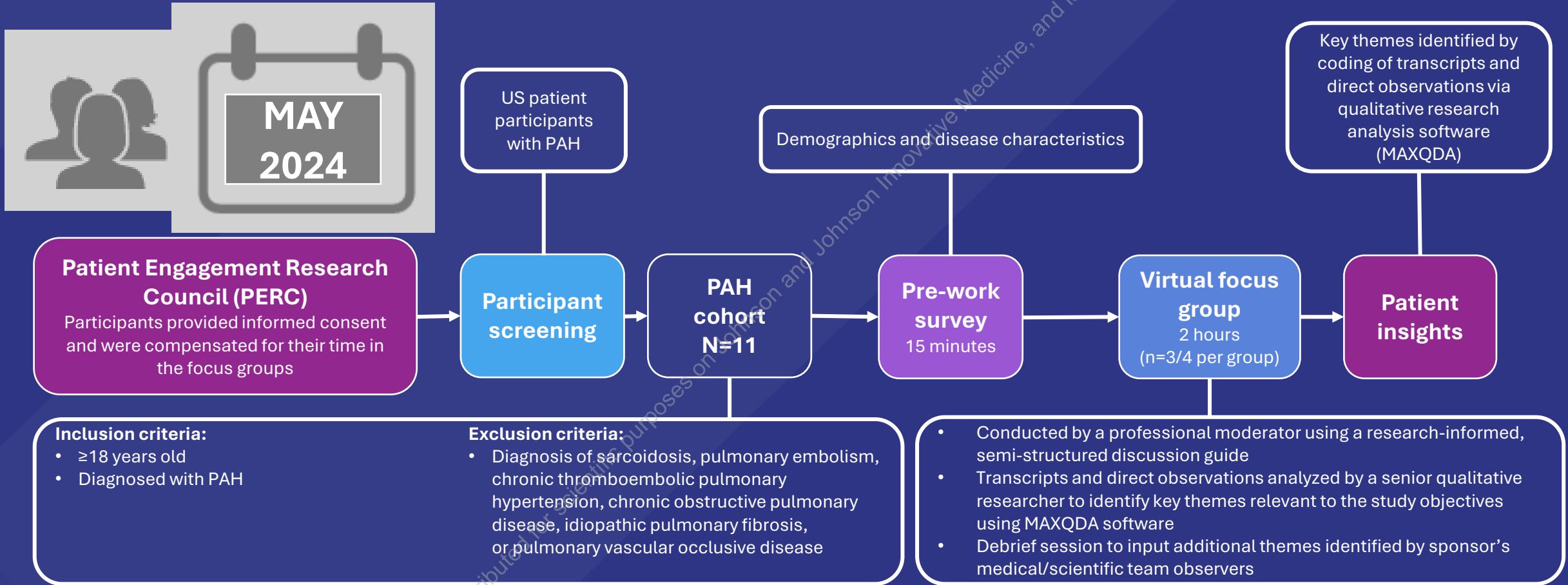
- Pulmonary arterial hypertension (PAH) is a complex, progressive, and fatal disease with a profound impact on the quality of life of affected individuals¹
- Recently, greater focus has been placed on the patient perspective in PAH, specifically as it relates to disease management
- Insights from the patient perspective are critical to understand what is important to patients with PAH and may help to inform the development of new medications
- These insights can be leveraged for clinical trial design and may enable shared decision-making to improve patient outcomes in PAH

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

Objectives

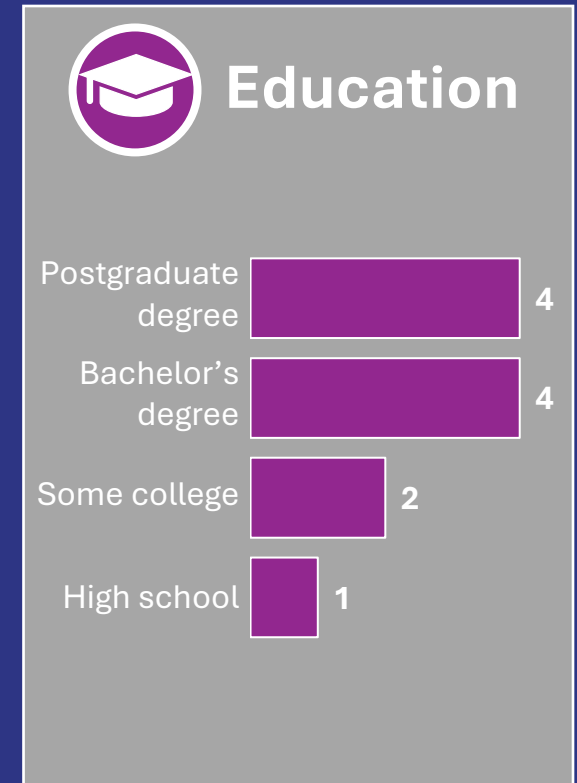
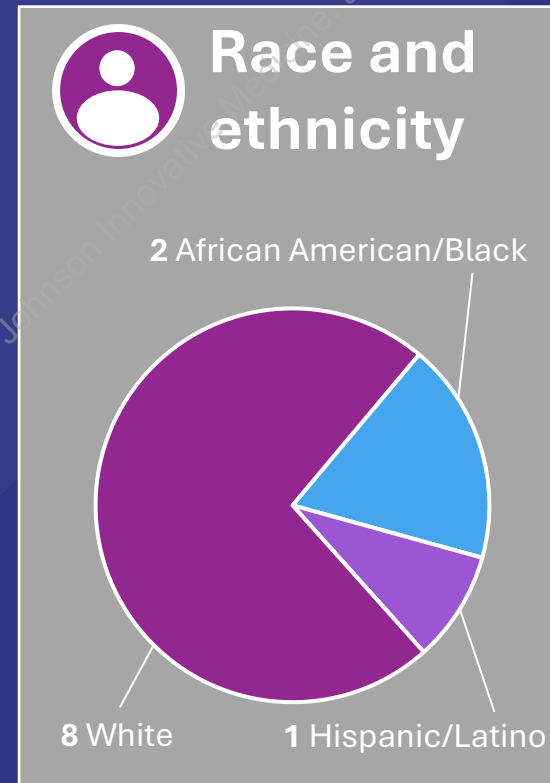
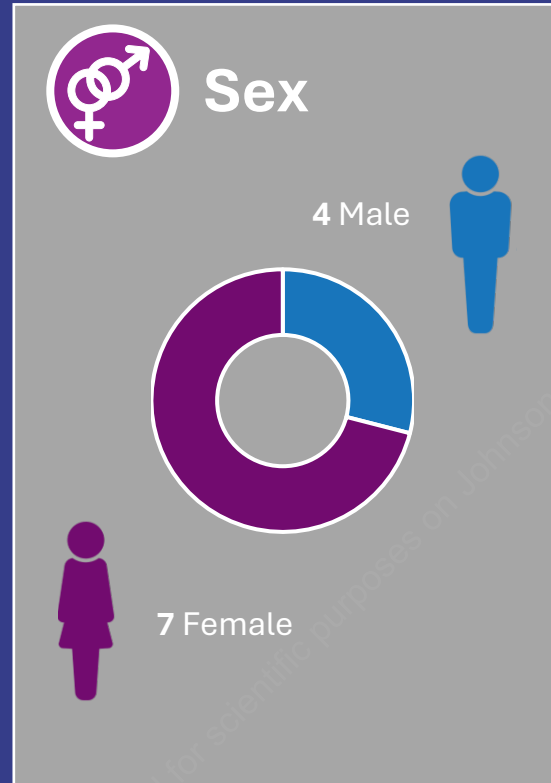
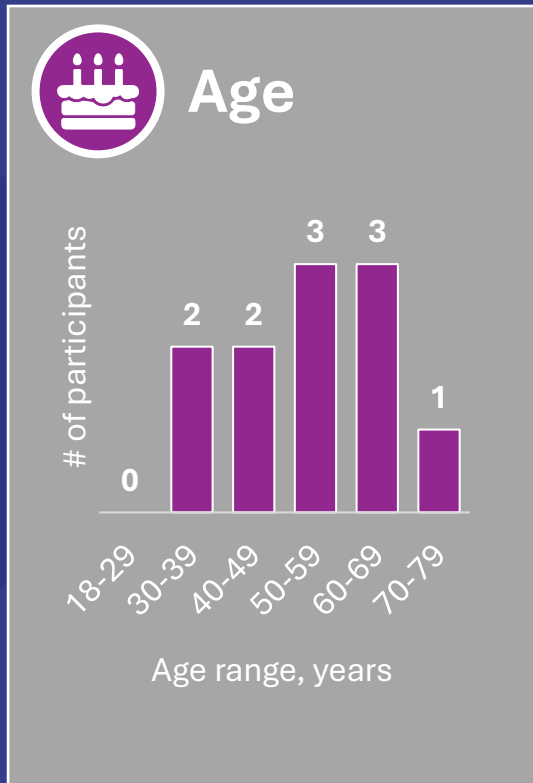
- To explore how participants in the PAH Patient Engagement Research Council (PERC) understand and assign importance to clinical trial endpoints and outcomes related to PAH
- To understand the extent to which these outcomes are discussed between patients with PAH and healthcare providers and whether these outcomes inform treatment decision-making

Methods



PAH, pulmonary arterial hypertension; US, United States.

Self-reported participant characteristics



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Learning about outcomes along the patient journey



- Participants reported **processing very little information at diagnosis** and said that their doctors spent little time discussing outcomes



- Participants recalled **learning about outcomes gradually during their disease**, including doing their own research



- **Input from family members and the impact on them influenced** how participants perceived outcome importance

Most important clinical outcomes to avoid, ranked by participants

#1: Mortality

#2: Hospitalization due to worsening PAH

#2: Lung transplant

#3: Worsening quality of life

#4: Decrease in WHO functional class

Less important outcomes to avoid:

- Increase in prostacyclin dosage
- Decrease in 6MWD
- Newly diagnosed ascites/peripheral edema
- Addition of another oral medication
- Addition of long-term oxygen at home

Mortality

(#1 most important outcome to avoid)

“I think [death] is a very clear first choice.”

“Death is the number one problem. That was kind of what I was going to say for the answer for the last question [about] what is always on [my] mind... I know where this disease heads, so... [the thought is] always there.”

Hospitalization due to worsening PAH (#2 most important outcome to avoid)

“And I’ve seen in so many people, once you have [a hospitalization], it’s really hard to come back from. If you lose that momentum you have ... you’ve lost a lot of ground in your PAH. And so to me, that’s a big divot that you cannot sometimes come back from.”

Lung transplant (#2 most important outcome to avoid)

“It was first mentioned to me when I was 25 [that] 50% of all lung transplant patients die within the first 5 years. That means I could have died before I was 30. As of right now, it’s something that I find scary.”

“I’ve lost so many people who have had lung transplants. I can’t do that. I just can’t. I’d rather die with my own lungs.”

Worsening quality of life (#3 most important outcome to avoid)

“When I chose my treatment for PAH, my doctor said, ‘Do you want quality or quantity?’ I said I'd rather have 5 years of ‘wow’ than 10 years of ‘ick’. So, yeah, I want to feel normal and have energy and be able to be myself ...”

Decrease in WHO functional class (#4 most important outcome to avoid)

“As scary and as horrific as [a WHO decrease] is ... I go into the doctor’s appointment and we’re constantly reassessing [that outcome]. So, I’m prepared mentally for it to get worse or for it to get better.”

Treatment decisions

- Participants were divided about how much they wanted to be involved in treatment decisions:
 - Preferring to be very involved (5 participants)
 - Somewhat involved (5 participants)
 - Not very involved (1 participant)
- Quality of life was mentioned most often as influencing treatment selection

Participant recommendations for educating people diagnosed with PAH

- Improved disease education and advocacy are most helpful for new patients to learn about outcomes
- Experienced patients will be more empowered to advocate for themselves with support to better understand outcomes
- Informative educational materials about outcomes written in patient-friendly language are considered the most helpful support manufacturers can provide

Summary: insights of people diagnosed with PAH

- Education about outcomes does not typically occur at diagnosis
- Mortality, hospitalization due to worsening PAH, lung transplant, and worsening quality of life were rated as the most important outcomes to avoid
- Educational materials on outcomes are most helpful but should be in patient-friendly language with additional language options available
- Engaging with patients to understand their priorities and preferences is essential to inform clinical research and to focus on improving the outcomes that matter most to patients

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Disclosures

- MGM is a consultant for Acceleron/Merck, Aerami (ended), Allrock, Bayer (ended), Janssen, JucaBio, Keros, Respira, United Therapeutics; George Washington University receives/received grant support to allow MGM to be a Principal Investigator from: Acceleron/Merck, Aerovate (ended), Altavant (ended), Gossamer; and MGM's husband is an employee of Pathos Therapeutics
- CV has participated in past advisory boards for Janssen and Merck and is a consultant for Regeneron
- AA, JY, and MC are employees of Actelion Pharmaceuticals US, Inc., a Johnson & Johnson Company
- SP was an employee of Actelion Pharmaceuticals US, Inc., a Johnson & Johnson Company
- JJR has served as researcher, advisor, and speaker for Johnson & Johnson, Kiniksa Pharmaceuticals, Liquidia, Merck, and United Therapeutics
- DL has served on the advisory board for Gossamer, Liquidia, and Merck Roivant, and has been a speaker for Janssen and United Therapeutics
- RS has served as an advisor or consultant for United Therapeutics, Gossamer Bio, and Johnson & Johnson Innovative Medicine

Thank you!

<https://www.janssescience.com/media/attestation/congresses/pulmonary-hypertension/2024/team-phenomenal-hope/pulmonary-arterial-hypertension-clinical-trial-endpoints-and-outcomes-patient-perspectives-and-prefe.pdf>

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