

PHenomenal Hope 2024

Knowledge, Research & Advocacy in PH

This material is distributed for scientific purposes on Janssen Science, and is not for promotional use

PHenomenal Hope 2024

Knowledge, Research & Advocacy in PH

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

Mardi Gomberg-Maitland,¹ Corey Ventetuolo,² Ankita Adhia,³ Joseph Yen,³ John J. Ryan,⁴ Denise Lewis,¹ Michelle Cho,³ Sumeet Panjabi,³ Rajan Saggar⁵

¹George Washington University School of Medicine and Health Sciences, Washington, DC, USA;

²Brown University, Providence, RI, USA; ³Actelion Pharmaceuticals US, Inc., a Johnson & Johnson Company, Titusville, NJ, USA; ⁴University of Utah Health, Salt Lake City, UT, USA; ⁵UCLA Health, Los Angeles, CA, USA



The QR code is intended to provide scientific information for individual reference, and the information should not be altered or reproduced in any way.

Introduction

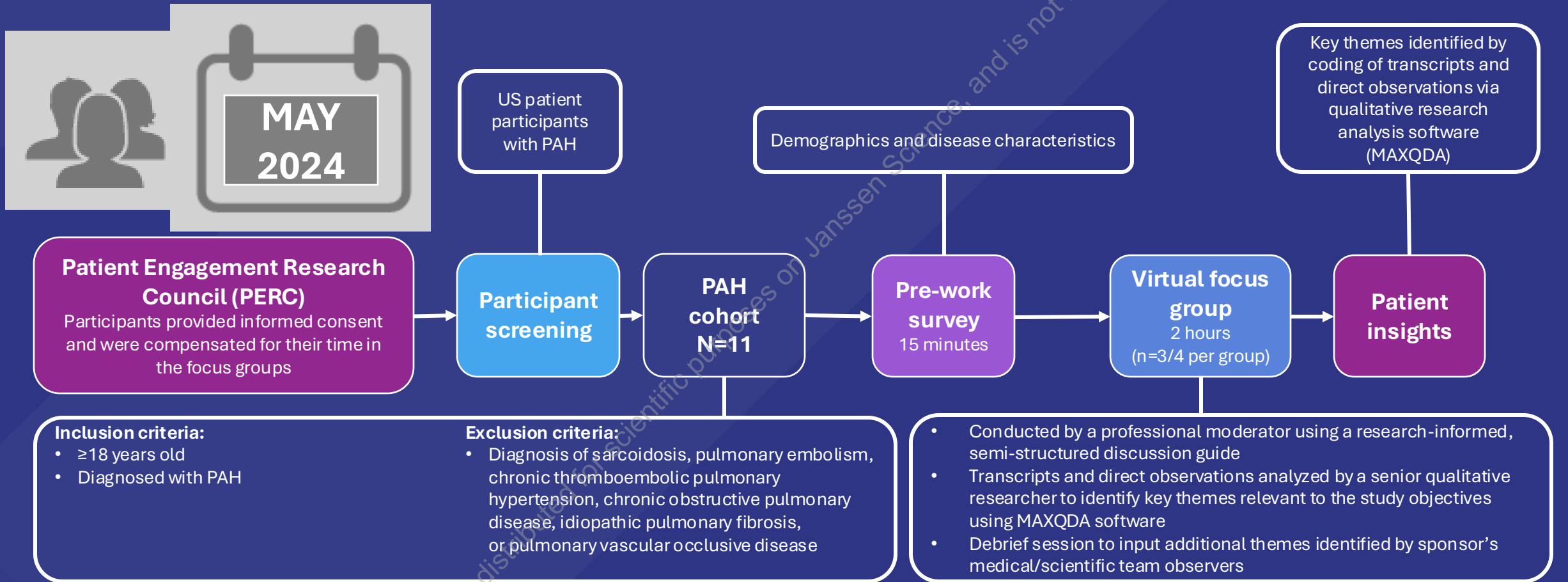
- Pulmonary arterial hypertension (PAH) is a complex, progressive, and fatal disease with a profound impact on the quality of life of affected individuals¹
- Recently, greater focus has been placed on the patient perspective in PAH, specifically as it relates to disease management
- Insights from the 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

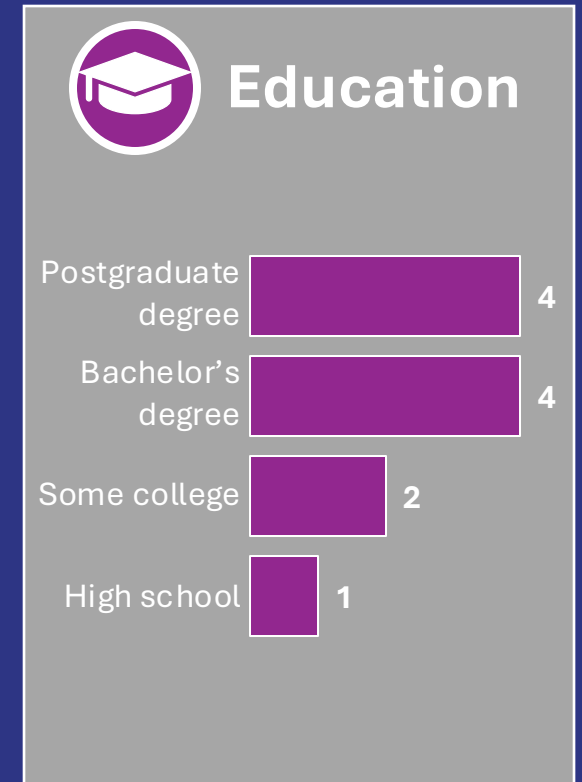
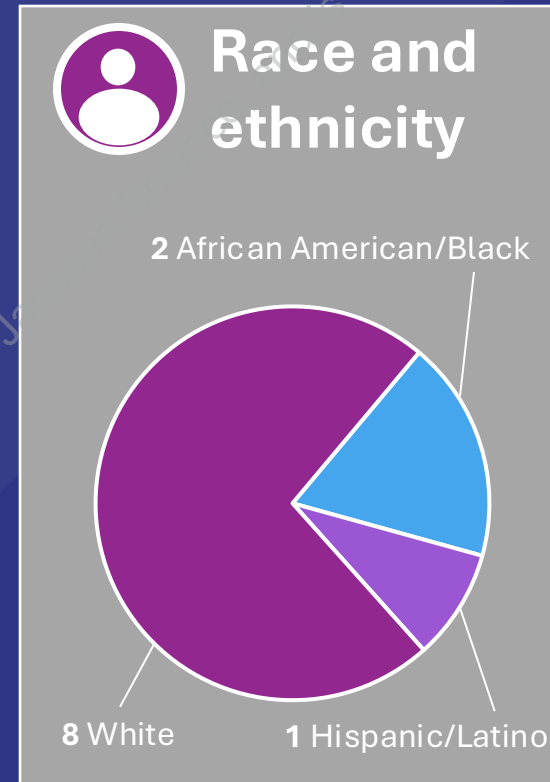
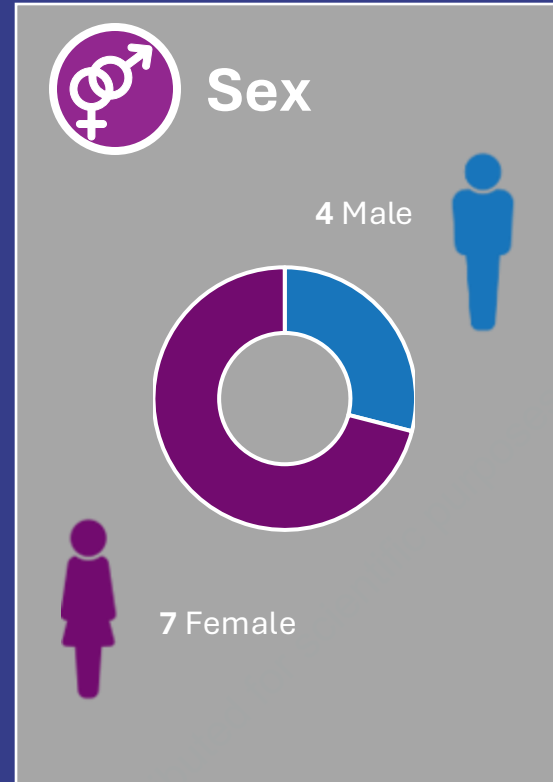
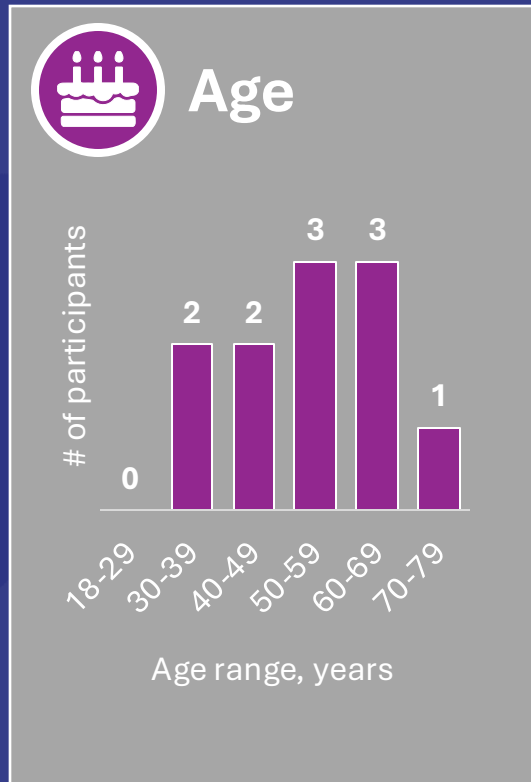
- To explore how participants in the PAH Patient Engagement Research Council (PERC) understand and assign importance to clinical trial endpoints and outcomes related to PAH
- To understand the extent to which these outcomes are discussed between patients with PAH and healthcare providers and whether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

Methods

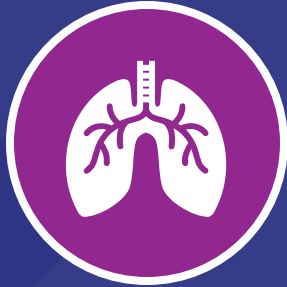


PAH, pulmonary arterial hypertension; US, United States.

Self-reported participant characteristics



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

Acknowledgments

- The authors thank the patients who participated in Johnson & Johnson's PERC activities for their engagement and insightful feedback. They also thank Wesley Peters and Jessica Kerns of Evidera, Wilmington, NC, USA, for their support
- Medical writing support was provided by Kelsey Hodge-Hanson, PhD, and Steven F Merkel, PhD, on behalf of Twist Medical, LLC, and was funded by Actelion Pharmaceuticals US, Inc., a Johnson & Johnson Company

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

The QR code is intended to provide scientific information for individual reference, and the information should not be altered or reproduced in any way.

