

# PHenomenal Hope 2024

*Knowledge, Research & Advocacy in PH*

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*Knowledge, Research & Advocacy in PH*

## Perceptions of the prostacyclin pathway: insights from a pulmonary arterial hypertension patient engagement research council

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# Introduction

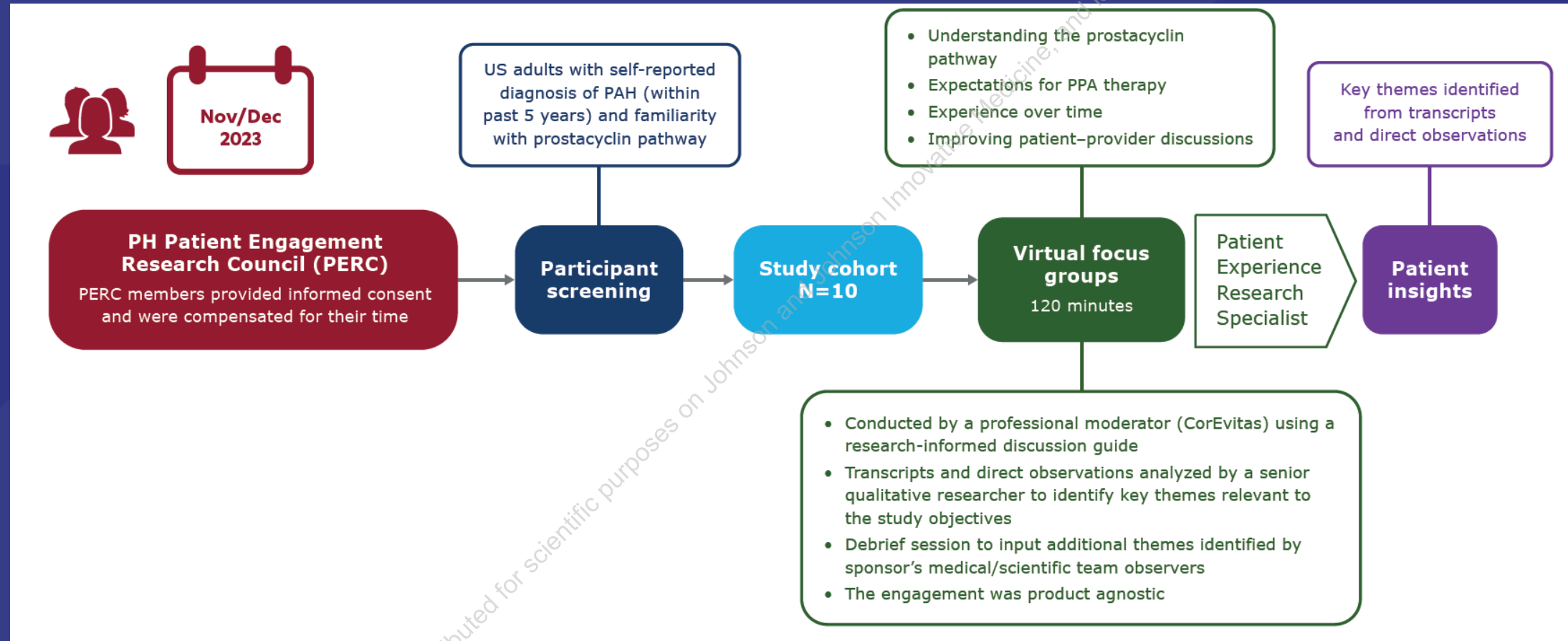
- The prostacyclin pathway represents a key foundational pathway in treating pulmonary arterial hypertension (PAH); however, targeting the pathway comes with challenges related to expected side effects and – for some therapies – a burden of administration<sup>1,2</sup>
- As a result of these challenges, patient engagement often plays a factor in therapeutic persistence
- Currently, there is limited knowledge about patients' understanding of the prostacyclin pathway and how they experience treatment with prostacyclin pathway agents (PPAs)

1. Burger CD, et al. *Am J Manag Care*. 2016;22(1 Suppl):S3–15. 2. El Yafawi R, Wirth JA. *Curr Hypertens Rep*. 2017;19:97.

# Objective

- To understand patient perspectives on PPAs, including their expectations and understanding of how the prostacyclin pathway and PPAs work
- To explore patient perspectives on ways to improve patient–provider shared decision making around the prostacyclin pathway

# Study design



PAH, pulmonary arterial hypertension; PH, pulmonary hypertension; PPA, prostacyclin pathway agent; US, United States.

# Participants

10 participants



## Gender

6 Female  
4 Male

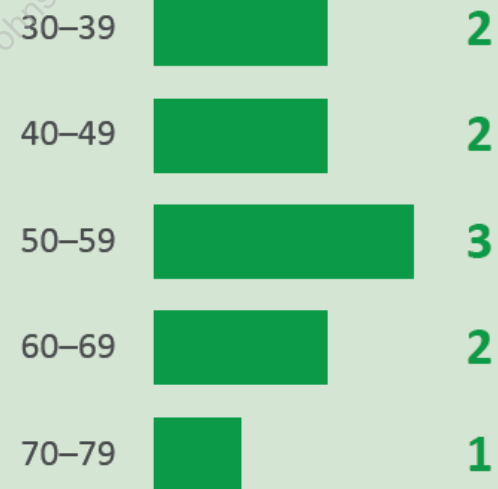


## Race/ ethnicity

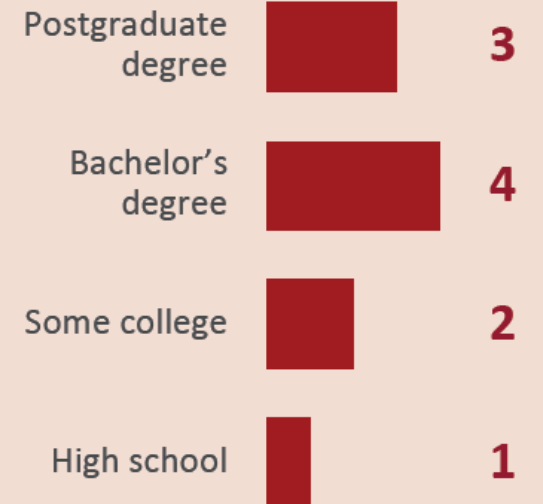
7 White  
2 African American/Black  
1 Hispanic/Latino



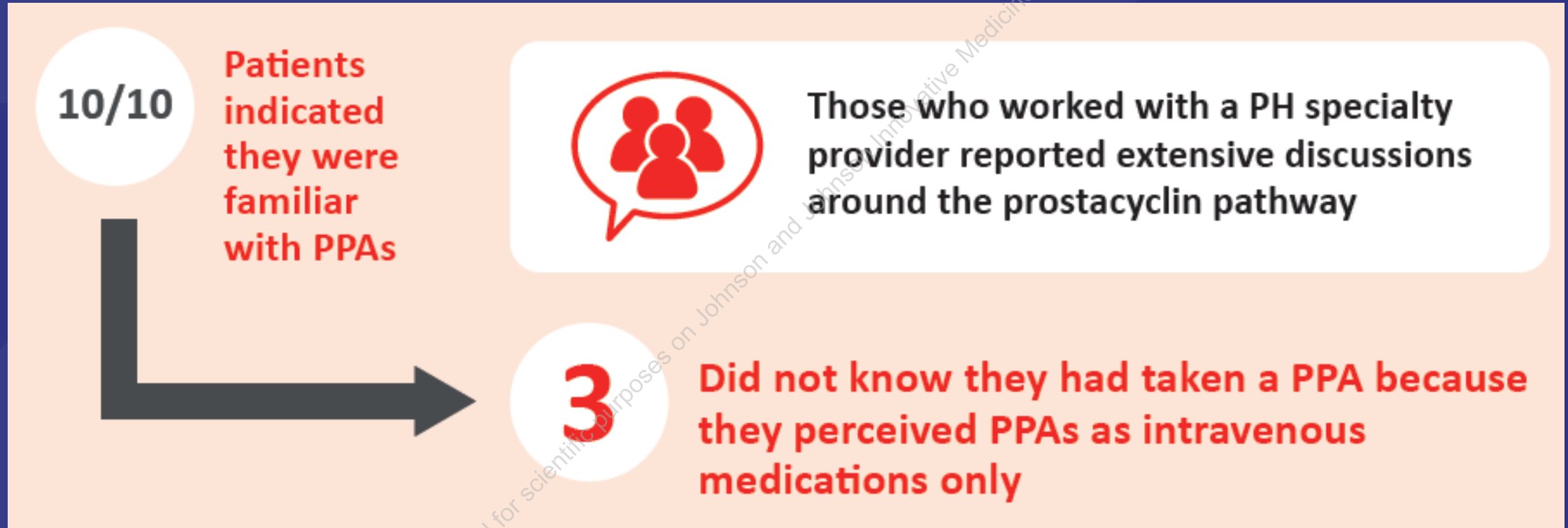
## Age, years



## Education



# Results: Understanding the prostacyclin pathway



PH, pulmonary hypertension; PPA, prostacyclin pathway agent.

# Understanding the prostacyclin pathway

## Key insights



**Patients felt the basics of the prostacyclin pathway were not always discussed in sufficient detail**

“[My provider] didn't explain it as, ‘Here’s the pathways.’ She would just say, ‘Oh, well, studies find out that if you put yourself on this combination of meds, it could possibly slow down the progression of your PH’

## Opportunities for improvement



**Patients felt they were rushed into making a decision due to their worsening condition and wished there had been more time to discuss details with their provider**

“And when you go into the doctor’s office for the first time you’re told that you have PH and what the drugs are, you’re scared for your life, you do whatever it takes ... without even thinking or even knowing, or processing everything ... I was in heart failure when I got diagnosed. I was scared out of my mind. So I’m like, ‘OK,’ now, thinking back, I would not have done that”

# Understanding the prostacyclin pathway

## Key insights



**While all patients were familiar with prostacyclin, a few did not realize they had taken a PPA**

“You know what, during this conversation, I’m starting to [realize] that... when I started treatment, I was on inhaled [prostacyclin]. ... So, that’s probably what I was on, but they never told me [it was prostacyclin]”

## Opportunities for improvement



**Patients suggested concise educational materials on PPAs and connection to patient advocacy would be helpful**

“I just prefer a short, brief pamphlet saying, ‘This is what the drug is, read this’ – not ‘War and Peace.’ Just a brief 1-page thing”

# Understanding the prostacyclin pathway

## Key insights



**Patients reported limited discussion of different formulations**

“I begged for an oral option. [But] I was so inconsistent with oral.... I really just needed an infusion that would do the work for me. So, I don't even think [my doctor] wanted to discuss pathways with me because I was such an angry, depressed, resentful patient”

## Opportunities for improvement



**Patients wished they had been given more information about different formulations**

“I just wish they would have given me options ... because I would have probably chosen the pill first, and then the inhaled, and then the pump would be absolute [last] if I had to”

# Expectations for therapy

- Participants wanted more information on:
  - What to expect during the titration period
  - How long side effects would last even after titration
  - More options for pain management
  - More guidance on switching between different formulations, if necessary

# Expectations for therapy

## Key insights



**Patients felt that titration and side effects were not always discussed adequately**

“[Regarding] side effects, the doctors have no clue what you’re going to go through. ... I got home on SubQ; my doctor had not prescribed me anything for pain or diarrhea. I was in so much pain”

## Opportunities for improvement



**Patients felt they would benefit from more information about how long side effects last**

“Even years later, I would still have side effects. And that was up to the point that I transitioned off IV therapy. I would still have bumps in the road or side effects”

# Expectations for therapy

## Key insights



**Some patients felt they didn't receive enough information around pain management**

“Side effects on the [oral] were almost intolerable – just leg pain and jaw pain and headache. ... They just said, ‘Take Tylenol,’ which did nothing. It was pretty miserable”

## Opportunities for improvement



**Patients wanted more options around pain management**

“I wish they were more willing to prescribe a little bit more for pain management; it would have helped me transition. Tylenol wasn't enough.... I think there are just certain circumstances where a stronger pain medication is appropriate”

# Expectations for therapy

## Key insights



**Patients found switching to oral medication easier than expected**

“I was totally bracing for the worst because I knew the oral had messed some people up GI-wise and migraine-wise, [but] I literally was like, ‘Are these placebos?’ ... because I was having no side effects. When I was discharged, I was just like, ‘I hope it's working’ because I felt nothing”

## Opportunities for improvement







**Patients felt that outcomes, particularly functional benefits, should be highlighted clearly in a way patients understand**

“Presenting it in language that is more tangible could be also making it more beneficial to somebody who's brand-new to the PH space. Because all this [info] is like good stuff. These are the goals that they all want. Just make sure that it's [communicated] in a way that they fully understand”

# Experience over time

- Most participants considered side effects a necessary trade-off for long-term benefits, such as:



-  Slow/stop disease progression
-  Improved pulmonary function
-  Increased 6MWD

# Experience over time

## Key insights



**Patients had discussed long-term benefits of PPA with providers in terms of slowing or stopping progression**

“[Prostacyclin] raised my heart function in 3 weeks. And [my providers] really had hope that long-term, it would get me to a stable place, and it did. Enough to transfer off the IV and onto oral. ... I thought [IV] was a lifetime situation. I never thought I would transfer to oral”

## Opportunities for improvement



**Some patients would have welcomed more information about the benefits of PPA treatment over time**

“And unless you ask questions, they’re not going to give any answers. We just take the word of our specialist as the gold standard, and our faith in our specialists that what they’re giving us is going to work. They don’t tell us, ‘Do this, do that, lower your numbers’ ”

# Experience over time

## Key insights



**Patients considered the trade-off of side effects to be preferable to disease worsening or death**

“One day you’ll be alive, and that’s enough. ... We can manage side effects, but what we can’t manage is death. Death is final. ... I hate to be so blunt, but that’s how my doctor was with me: ‘Hey, you’re not going to wake up pretty soon’”

## Opportunities for improvement



**Patients felt that the consequences of not taking a PPA should be emphasized as a reason for treatment**

“I think what’s missing is the other side of it – what it looks like without [prostacyclin]. ... If you don’t take it, here’s what happens. ... What does it look like to have [hospitalizations] increase by 90%, or whatever. Maybe talking about the people who took the placebo – what happened with them”

# Experience over time

## Key insights



**Patients considered the trade-off of side effects to be preferable to disease worsening or death**

“I had come across so many [PH patients] in my lifetime, and they’re gone, and some could have lived longer if they had made different choices possibly. ... I was almost ready to say, ‘Forget [prostacyclin]; I’d rather die.’ I’m thankful that I went through it, and I pushed through”

## Opportunities for improvement



**Patients thought it was important to be informed about living with potential discomfort during titration**

“Some people can’t handle being uncomfortable for an extended period of time. ... Discomfort was worth the reward at the end of the tunnel. Find a good therapist and get together with your team to help you resolve these issues to make the ride as [comfortable] as possible”

# Participant-recommended talking points for providers when introducing PPAs and discussing formulations

- What is the prostacyclin pathway?
- How do PPAs work?
- Long-term benefits of taking PPAs
- Side effects, potential duration, and ways to manage

PPA, prostacyclin pathway agent.

# Participant recommendations for improved provider discussions on the prostacyclin pathway and PPA treatment



**Present outcomes data in clear language focusing on meaningful functional outcomes (e.g., daily function)**



**Emphasize the compelling benefits of PPAs over time (e.g., reduced hospitalization and disease progression)**



**Highlight the consequences of not taking a PPA (e.g., disease worsening and mortality risk)**



**Allow more time for patients to make decisions about initiating therapy**



**Provide patient-friendly information on**

- The prostacyclin pathway and different PPAs, including different formulations available
- Side effects and how long they will last
- Ways to manage side effects (particularly pain) and tips for living with discomfort



**Supply links to patient advocacy groups and foster connections to patient “mentors” with PH**

# Limitations

- The small number of participants interviewed may not be representative of the entire population of patients with PAH who are on prostacyclin pathway therapies

PAH, pulmonary arterial hypertension.

# Conclusions

- Insights provided by this group of participants with PAH provided a unique perspective into their experience on different formulations of PPAs, such as:
  - A few did not realize they had taken a PPA
  - Some found switching to oral medication easier than expected
  - Some wanted more options around pain management
  - Some felt that the consequences of not taking a PPA should be emphasized
- Participants conveyed a desire to be more informed and engaged in treatment decisions
- A better understanding of gaps in communication and education can inform strategies for better shared decision making and patient engagement to optimize outcomes

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# Disclosures

- These insights reflect participants' opinions and experiences with their medications and do not reflect Johnson & Johnson's position on this subject
- JWM received financial support, research grants and personal fees from Janssen Pharmaceutical Companies of Johnson & Johnson; personal fees and financial support from Bayer Pharmaceuticals, United Therapeutics, Reata Pharmaceuticals and Arena Pharmaceuticals; and financial support from Eiger Pharmaceuticals, and Bellerophon
- RM, CB, GD, and MH are employees of Actelion Pharmaceuticals US, Inc., a Johnson & Johnson Company, and are Johnson & Johnson stockholders

# Thank you!

<https://www.janssescience.com/media/attestation/congresses/pulmonary-hypertension/2024/team-phenomenal-hope/perceptions-of-the-prostacyclin-pathway-insights-from-a-pulmonary-arterial-hypertension-patient-engag.pdf>

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