Perceptions of the prostacyclin pathway: Insights from a pulmonary arterial hypertension Patient Engagement Research Council

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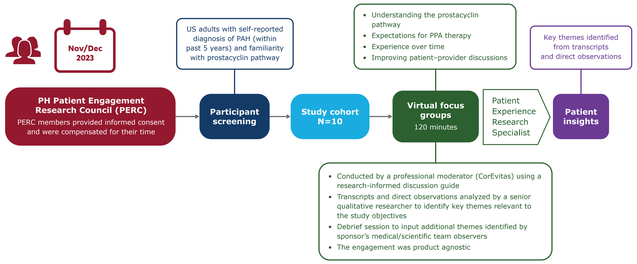
Introduction

- The prostacyclin pathway represents a key foundational pathway in treating pulmonary arterial hypertension (PAH); therapies that target the prostacyclin pathway are associated with improved World Health Organization (WHO) functional class, delayed disease progression, decreased mortality, improved cardiovascular hemodynamics and fewer hospitalizations[1,2]
  - However, targeting the prostacyclin pathway comes with challenges related to expected side effects and - for some therapies - a burden of administration[3,4]
- 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)
- This exploratory, qualitative focus group set out to understand patient perspectives on PPAs, including their expectations and understanding of how the prostacyclin pathway and PPAs work, and to explore patients' thoughts on ways in which patient–provider discussions around the prostacyclin pathway could be improved

Methods

- Adult patients with a self-reported diagnosis of PAH were recruited through Johnson & Johnson's Pulmonary Hypertension Patient Engagement Research Council (PERC) to participate in a virtual, 2-hour focus group. Patients were provided general statements about prostacyclin therapies and then screened for inclusion in the PERC based on their responses

Study design and participants

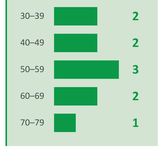


10 participants

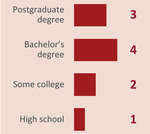
Gender
- **6** Female
- **4** Male

Race/ethnicity
- **7** White
- **2** African American/Black
- **1** Hispanic/Latino

Age, years



Education



Results

Understanding the prostacyclin pathway

- Patients were typically introduced to the prostacyclin pathway and PPAs when providers were considering additional therapy at a time of deterioration and disease progression
- Patients felt insufficient information was provided about the value of the prostacyclin pathway, PPAs, and the different PPA formulations, leading to confusion when engaging with clinicians during treatment discussions

10/10 Patients indicated they were familiar with PPAs

Those who worked with a PH specialty provider reported extensive discussions around the prostacyclin pathway

3 Did not know they had taken a PPA because they perceived PPAs as intravenous medications only

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'"

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]"

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 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"

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"

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"

Experience over time

- Most patients considered side effects a necessary trade-off, preferable to disease worsening or sudden death
- Long-term benefits of PPAs discussed with providers usually included improvement to heart function and pressures, including:
  - Slow/stop disease progression
  - Improved pulmonary function
  - Increased 6MWD

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"

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"

"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

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'"

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"

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"

Expectations for therapy

- Several patients felt their treatment experiences did not reflect what was discussed with their providers, and commented that providers may not fully appreciate the intensity of side effects (particularly during titration) and the impact of side effects on daily life
- Specifically, patients 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

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"

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"

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 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"

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"

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"

Patient recommendations for improved provider discussions around 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

Presented at the PHA 2024 International PH Conference and Scientific Sessions; Indianapolis, IN, USA; August 15–18, 2024

ABBREVIATIONS

6MWD, 6-Minute Walk Distance; **GI**, gastrointestinal; **IV**, intravenous; **PAH**, pulmonary arterial hypertension; **PH**, pulmonary hypertension; **PPA**, prostacyclin pathway agent; **SubQ**, subcutaneous.

REFERENCES

1. Barnes H, et al. *Cochrane Database Syst Rev.* 2019;5:CD012785. **2.** McConnell JW, et al. *Pulm Circ.* 2020;10:2045894020911831. **3.** Burger CD, et al. *Am J Manag Care.* 2016;22(1 Suppl):S3–15. **4.** El Yafawi R, Wirth JA. *Curr Hypertens Rep.* 2017;19:97.

Poster Number 1044

Key takeaways

Patient-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

Limitations

- The small number of patients interviewed may not be representative of the entire PAH patient population on prostacyclin pathway therapies

Conclusions

- Insights provided by this small group of patients with PAH provided a unique perspective into their experience with their providers and with different formulations of PPAs
- Patients conveyed a desire to be more informed and engaged in treatment decisions
- Patients would like providers to emphasize the benefits of taking PPAs over time, as well as explaining side effects, how long they are expected to last, and ways to manage them
- A better understanding of gaps in communication and education can inform strategies for better shared decision-making and patient engagement to optimize outcomes

Acknowledgments

Medical writing support was provided by Melanie Jones, BSc on behalf of Twist Medical and was funded by Actelion Pharmaceuticals US, Inc., a Johnson & Johnson Company, Titusville, NJ, USA.

Disclosures

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