SUMMARY

Paediatric Data from the PH Global Patient Survey

The Pulmonary Vascular Research Institute (PVRI) published The Pulmonary Hypertension Global Patient Survey: Understanding the Invisible Burden of Paediatric Pulmonary Hypertension, presenting data from 136 caregivers of children with pulmonary hypertension in 32 countries. This is the first research to look at pediatric pulmonary hypertension patients internationally.

Children with pulmonary hypertension (PH) have different needs than adults. They need full support as they develop, they have more hidden challenges, and safety is more of a concern when they participate in research.

The data had four main themes.

1. Hidden challenges

Families talked about the burden of managing an invisible disease. Pulmonary hypertension isn't always a visible disability, so schools and communities sometimes doubt its severity and impact. This leaves families feeling isolated and unsupported.

Nearly 40% of families reported that their child's PH medication had side effects, but only 1/3 talked to their medical team about changing medications. The researchers felt this was because families know there aren't many other options.

What can be done?

The researchers suggest educational initiatives targeting schools and communities about how people can have significant limitations without looking disabled. Tools for looking at side effects could help care teams discuss medication adjustments.

2. Deep impacts on families

Pulmonary hypertension is a rare disease, so it's nearly always unexpected: 90% of families didn't know anything about PH before their child was diagnosed.

40% of families provide around-the-clock care for their child with pulmonary hypertension, so there can be significant disruption to employment or education for family members. Caregivers had to stop working, reduce their working hours, or change careers. Children with pulmonary hypertension had their education disrupted. Sometimes, their siblings did as well.

What can be done?

Researchers suggest that pulmonary hypertension care teams integrate family supports, including having social workers on the team to help with things like navigating benefit systems.

3. Challenges accessing specialist care

Four in five families do have specialist care for their child with pulmonary hypertension, but there are still access issues. For 41%, it took longer than 6 months to get a PH diagnosis for their child. Families might have to travel to see their specialist, and this can be difficult and expensive. A third of families travel more than 2 hours to see their specialist and more than a quarter of families don't have any of their travel costs reimbursed.

What can be done?

Researchers suggest telemedicine might help reduce these challenges.

4. Barriers to participating in research

Only one in five families (19%) participate in pulmonary hypertension research, and almost three quarter (71%) of these families would be willing to participate again. In families who did not participate in research before, almost half wouldn't consider participating in future clinical trials as well. Barriers to participating in clinical trials are quite different from those adults experience. While adults mostly worry about the logistics of participating, caregivers of children with pulmonary hypertension reported that they weren't aware of clinical trials, were worried about safety for their child, or were concerned that the clinical trial would affect their child's development.

What can be done?

Researchers suggest that there needs to be age-appropriate information about research and that research study protocols need to address specific family-related concerns, not just be slight adaptations to adult protocols.

What's next?

Actions for future surveys include adding targeted questions on research barriers, increasing engagement with low-income countries, thinking about developing paediatric-specific patient reported outcome measures that address things like peer relationships, play, and development; and looking more deeply into the "invisible disease" concept and school interventions.