Sotatercept for treating pulmonary arterial hypertension [ID6163]

A formal representation to NICE

PHA UK | March 2026

The Pulmonary Hypertension Association UK (PHA UK) is the patient advocacy charity for people living with pulmonary hypertension in the United Kingdom. We exist to support, inform, and represent the PH community at every level - from individual patient support to national policy and drug access advocacy.

Over the last 25 years, the PHA UK has sustained campaigns for access to every significant new PH therapy. These campaigns have been conducted without public funding and without the resources of multinational pharmaceutical companies. We carry this burden because our community has no one else to carry it for them.

The PHA UK is engaged across the full landscape of sotatercept access and we make this representation as part of that sustained, long-term commitment.

We make this representation not as adversaries of NICE, but as the organised, evidenced voice of a patient community that is being failed by a process that claims to serve them. Our singular goal is commissioning access to sotatercept for UK PH patients. Everything in this paper is directed at that goal.

1. The central argument: A knowledge problem, not a data problem

This is the heart of the PHA UK's representation. The appraisal process has followed its own procedures. The problem is that those procedures are structurally inadequate for rare disease, and the inadequacy has a specific, identifiable cause: the lived experience of patients has not been genuinely central to this appraisal.

NICE's standard appraisal methodology was built for common diseases with large patient populations and abundant randomised trial data. In rare disease, where populations are small, trial data is limited, and the individual burden of disease is profound, this methodology generates uncertainty as a structural artefact. It is not that the evidence of

sotatercept's value is insufficient. It is that the framework for weighing evidence is not designed to receive the most important evidence of all: what it means, in real lives, to live with this disease - and what it would mean to have a treatment that addresses it differently.

The PHA UK understands and appreciates that NICE faces a genuine challenge in its work. Balancing different kinds of evidence using standardised methods is essential to ensuring consistency and fairness across decisions - and we recognise how difficult it can be to incorporate more qualitative inputs within that framework. Our representation is not a criticism of NICE's commitment to rigour. It is an invitation to consider how that rigour might evolve to better reflect the full range of evidence that matters in rare disease appraisal.

The philosopher Miranda Fricker identifies epistemic injustice as the systematic undervaluing of a person's knowledge because of who they are. In healthcare appraisal, this takes a specific form: the people with the most direct, irreplaceable knowledge of disease burden and treatment value are patients themselves - yet the processes designed to make decisions about their care routinely treat patient knowledge as supplementary rather than foundational.

This is not a peripheral concern. It is developed in academic work by Armstrong and Winter (submitted to Lancet Digital Health, 2025), which argues that patient-reported outcome tools such as EmPHasis-10 - now a mandatory data point in the National Audit of PH and the most widely used PH-specific health-related quality of life tool globally - function not merely as measurement instruments but as epistemic tools: microphones for lived experience that create space for patient knowledge in clinical and policy encounters. When those tools and the knowledge they carry are not genuinely central to an appraisal, the outcome is structurally compromised.

Lived experience is the 'yeast' in any rare disease appraisal. Leave it out, and what you produce cannot be the right result - however rigorous everything else has been.

The NHS 10 Year Plan commits the NHS to being a system genuinely underpinned by patient empowerment. NICE has itself publicly committed to reforming its methods and processes to deliver on that ambition. We welcome those commitments wholeheartedly. But commitment and delivery are not the same thing. The test of whether patient empowerment is real is not whether patients are invited into the room. It is whether what they say changes what happens.

True patient empowerment is not when a patient is given a voice. It is when a patient knows that their voice matters.

The uncertainty NICE has expressed about the value of sotatercept is, in significant part, a consequence of the absence of that kind of empowerment. The remedy is not more trial data. It is a process redesigned to genuinely centre patient knowledge - and a willingness to

recognise that the evidence the PHA UK has already produced is not soft evidence. It is knowledge, and it belongs at the heart of this appraisal.

Of course, we understand that this drug comes at a very high cost and the NHS has finite resources. But value for money can only be properly judged with a true understanding of what is at stake for the patients who need the treatment.

2. The PH service in the UK: A foundation for safe commissioning

One dimension of NICE's appraisal uncertainty concerns appropriate use: will an expensive therapy be prescribed to the right patients, monitored effectively, and withdrawn if it is not working? In most disease areas this is a legitimate and important question. In pulmonary hypertension in the UK, it is not an uncertainty. It is answered by the structure of the service itself.

PH in the UK is managed through a small number of designated specialist centres. Every patient with a confirmed PH diagnosis is under the care of one of these centres, ensuring that therapeutic decisions are made by clinicians with deep disease expertise. All patients and all therapies are captured in the National Audit of PH - a mandatory, systematic, national dataset that tracks patient outcomes, therapy use, and clinical response across the entire PH population. Expensive therapies in PH are already subject to closer, more systematic scrutiny than almost any other rare disease therapy in the NHS.

The infrastructure for appropriate commissioning, monitoring, and audit of sotatercept already exists and is already functioning.

3. The evidence: Unmet need is real, severe, and documented

3.1 The best treatment in the world is not enough

UK patients with pulmonary hypertension receive what the PHA UK and the clinical community regard as the best available PH treatments in the world. Despite this, they continue to live with a severe and debilitating symptom burden. This is not an assumption - it is the central finding of PHA UK's 2025 research, conducted specifically to understand the lived reality of PH in the current treatment landscape.

3.2 Breathless and Beyond (PHA UK, 2025)

In March 2025, the PHA UK conducted the UK's first real-world study of patient-defined symptom burden in PH. Its findings, from 232 patients and 35 caregivers, are unambiguous.

- 92% of patients experience breathlessness on exertion.
- 85% experience fatigue and tiredness.
- 64% identify breathlessness on exertion as one of their three most impactful symptoms.
- 59% identify fatigue and tiredness as one of their three most impactful symptoms.
- 61% scored fatigue at 6 or 7 out of 7 on the Fatigue Severity Scale - strongly agreeing it is among their most disabling symptoms.
- 83% of respondents identify as female.

The study's conclusion states explicitly: UK patients receive the best treatments in the world, but are still severely impacted. Fatigue - almost as burdensome as breathlessness - is not currently treated with anywhere near the same clinical priority.

The patient voice in this data is not abstract. People describe being a shadow of their former lives. Unable to care for their children. Unable to walk from bedroom to bathroom without stopping to rest. Feeling useless and like a burden to those they love. These are not outliers - they are the mainstream experience of PH in the UK in 2025, on optimal treatment.

3.3 The wider economic and social burden

The PHA UK's 2022 survey, Bearing the Burden, documented the economic reality of living with PH. 78% of respondents are not working, 76% of those due to health reasons. 75% are in receipt of benefits. These are people whose entire lives have been reshaped by their disease.

NICE's standard cost-effectiveness framework, built around clinical metrics and QALYs, cannot capture what improved functional capacity would mean for a population living this reality. The value of sotatercept is not fully visible through a narrow clinical lens. It requires a framework that treats patient knowledge as data.

3.4 What sotatercept offers

Sotatercept is the first PAH therapy to act through the activin signalling pathway - making it genuinely first in class. It does not replicate the mechanism of existing therapies. For patients who remain severely symptomatic despite optimal current treatment, it represents a qualitatively different therapeutic opportunity. The question of whether its value justifies commissioning cannot be answered by trial data alone. It requires the evidence of lived experience - which PHA UK has produced, and which this appraisal has not adequately used.

4. The process: How patient knowledge has been underweighted

4.1 What happened at the first NICE committee meeting, August 2025

At the first NICE committee meeting for the sotatercept appraisal, PHA UK arranged for two patients - Josh and Paula - to attend and contribute their lived experience. The meeting ran for approximately three and a half hours. Josh and Paula were given fewer than ten minutes between them to speak.

The PHA UK wrote formally to NICE following that meeting to express serious concern about the adequacy of patient engagement. Paula subsequently provided her own direct feedback to NICE, describing her experience as deeply unsatisfactory. To our knowledge, no structural change to the process resulted from either communication.

4.2 Why this Is a structural issue, not an incidental one

The time given to patients in that committee meeting is not a scheduling oversight. It reflects how patient knowledge is currently weighted within the process. The people who carry the most direct knowledge - what it actually means to live with severe fatigue, breathlessness, and loss of function on optimal treatment - were present in that meeting. The process did not adequately receive what they had to offer.

The PHA UK's own 2025 research documents that 85% of PH patients experience fatigue as a severely disabling symptom - a never-ending drain that sleep does not resolve, that shuts the body down, that makes even the simplest tasks an effort. NICE then designed a three and a half hour online meeting and expected patients carrying this burden to participate meaningfully within it. The gap between the stated commitment to patient empowerment in the NHS 10 Year Plan and the reality of how that involvement is designed and delivered is the gap at the heart of this appraisal's current limitations.

Josh and Paula came to that meeting with knowledge that was irreplaceable. A process genuinely designed around patient empowerment would have ensured that knowledge shaped the outcome.

4.3 The consequence: How process gaps produce uncertainty

When lived experience is inadequately weighted in an appraisal process, the result is a specific kind of gap: the underrepresentation of the value of treatment for the people who most need it. The uncertainty NICE has expressed about sotatercept's value is, in significant

part, a consequence of a process that has not adequately received the evidence that would help resolve it.

Patients are experts. Not in the same way as clinicians or health economists, but in a way that is irreplaceable: they are the only people who know what it is to live with this disease, on these treatments, in this body. That knowledge is data. When it is absent or underweighted, the evidence base is incomplete - and conclusions drawn from it are structurally limited.

5. The policy context: Government commitments and current contradiction

5.1 Patient empowerment and the NHS 10 year plan

The NHS 10 Year Plan, published in July 2025, establishes patient empowerment and health equity as the two foundational principles underpinning the entire direction of NHS reform. NICE has itself responded to this commitment, publicly stating its intention to reform its methods and processes to deliver on the plan's ambitions - to get better care to people, faster.

The PHA UK welcomes this direction of travel. We are asking NICE to ensure it applies here. The sotatercept appraisal is not a legacy case from a previous era. It is happening now, in the context of these commitments. If patient empowerment means anything in practice, it must mean that the lived experience of PH patients - documented, evidenced, and submitted - is treated as foundational to this appraisal, not peripheral to it.

5.2 The England Rare Diseases Action Plan 2026

The England Rare Diseases Action Plan 2026, published in March 2026, formally recognises rare diseases as a health inequality and commits to developing specific plans to address disparities in healthcare access for people affected by rare conditions. The current situation — UK PH patients unable to access a licensed, first-in-class therapy available in multiple other countries — is, by the government's own newly stated definition, a health inequality. PHA UK asks NICE to acknowledge this contradiction directly.

5.3 The women's health strategy

Pulmonary arterial hypertension disproportionately affects women at approximately 3:1. The PHA UK's 2025 research found 83% of respondents identify as female. The government's Women's Health Strategy makes explicit commitments to addressing health inequalities for women in conditions that disproportionately affect them. Blocking access to

a first-in-class drug for a predominantly female rare disease on grounds of uncertainty sits in direct contradiction to those commitments.

5.4 The MHRA alignment commitment and NICE's own quality standard

By April 2026 MHRA and NICE are committed to aligning a pathway for parallel decision-making on licensing and value. Sotatercept is already licensed. The value question is what remains. The PHA UK asks that the new alignment pathway be used to expedite resolution of this appraisal, with lived experience evidence genuinely central to that process. Additionally, NICE's own quality standard for rare diseases, published in January 2026, commits to improving care for all people living with rare conditions. The sotatercept appraisal is an immediate test of whether that commitment is substantive.

6. The PHA UK's formal requests

The PHA UK makes the following formal requests to NICE, in a spirit of genuine collaboration and with the singular aim of achieving commissioning access for UK PH patients.

Immediate priorities

1. A clear and realistic pathway to commissioning sotatercept for UK PH patients, with named accountability and a defined timeline. There has been no progress since the second appraisal meeting in December 2025, and we had to make a formal plea for the confidentiality clause on the commercial discussions to be lifted, to ensure patients were not kept in the dark.
2. A written account of how the PHA UK's evidence submissions - including the Breathless and Beyond 2025 symptom burden study - have been considered and weighted in the appraisal process, and what role patient-reported and qualitative evidence played in the committee's assessment.

Less urgent, but still important

3. A formal meeting with NICE at a senior level to discuss how lived experience evidence has been weighted in the sotatercept appraisal, and how the process can be developed to better honour the patient empowerment commitments of the NHS 10 Year Plan.

4. A formal review of the patient engagement process for this appraisal, with written response to the concerns raised by the PHA UK and by patients directly, and a commitment to structural improvement.
5. Engagement with the PHA UK on practical ways in which NICE's rare disease appraisal methodology might evolve to better incorporate patient experience alongside clinical data. We recognise NICE must balance different kinds of evidence using standardised methods to ensure consistency. We are offering to work with NICE on how that balance might be recalibrated for rare disease - for example through better integration of patient-reported outcomes, structured inclusion of qualitative evidence alongside quantitative data, and greater transparency in how experiential evidence is considered and weighted in decisions.

The PHA UK is committed to this process for the long haul. We want to work with NICE constructively and collaboratively. We believe that if the lived experience of PH patients is genuinely centred in this appraisal - their symptom burden, their functional limitation, what sotatercept would mean in their daily lives - the uncertainty that currently blocks access will resolve. Not because the data changes, but because the right data is finally in the room.

It is our duty to advocate for our members, and the wider PH community, so the asks in this document come not only from the PHA UK. They come from our 4,500 members, and their family and friends too. They put their trust in us, as they put their trust in the NHS. We cannot fail them.

The people we represent participated in the clinical trials that brought sotatercept into existence. They contributed their time, their data, and in some cases their health to its development. They are now waiting - some of them deteriorating - while a commercial and appraisal process runs its course. They deserve better. This representation is our commitment to making sure they get it.

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