BRAIN TUMOUR
ALLIANCE AUSTRALIA Inc.

Christine Whittall
Executive Officer, Brain Tumour Alliance Australia
Pearce Community Centre
Collett Place Pearce, ACT 2607
christine@btaa.org.au | 0411 898 289

19 January 2026

PBAC Secretariat
Pharmaceutical Evaluation Branch
Department of Health and Aged Care
Email: pbac@health.gov.au

Dear Members of the Pharmaceutical Benefits Advisory Committee,

Re: Strong support for listing of vorasidenib on the Pharmaceutical Benefits Scheme

On behalf of the Brain Tumour Alliance Australia (BTAA) and the brain cancer community we represent, I am writing to reaffirm our strong support for the listing of vorasidenib on the Pharmaceutical Benefits Scheme (PBS) for the treatment of IDH-mutant glioma.

Following its consideration in July 2025, we were disappointed that vorasidenib was not recommended for PBS listing. However, we welcomed the Committee's recognition of the high unmet clinical need, demonstrated clinical benefit, and meaningful improvement to quality of life.

Since that time, BTAA has sought further input from patients and carers to help the Committee better understand the real-world impact of vorasidenib, as well as the impact of limitations to its accessibility. In addition to BTAA's original submission (attached), please consider this update, summarising the feedback we have received.

These comments illustrate the tangible benefits of vorasidenib, the challenges faced when access is limited, and the ways PBS subsidisation could meaningfully improve outcomes for the community. We encourage the Committee to consider these perspectives alongside the clinical and economic evidence in making its recommendation.

Impact of lack of accessibility

The majority of Australian patients who have had access to vorasidenib have done so through the compassionate access scheme offered by Servier, which has since been terminated. Patients and their families express anxiety about losing access to the *only* treatment available to them that stops tumour growth.

"I am accessing Voranigo on the compassionate access scheme. If this drug is not approved for PBS I will not be able to afford to take it"

"I'm on compassionate access right now, I wouldn't be able to afford it otherwise! Please put it on the PBS"

“The cost of accessing Voranigo is a significant barrier. At this stage we have committed to the medication for a year or so ($58,000 pa) to provide some breathing space to reassess our future, but it will not be a long-term option given our financial position unless approved by PBS.”

“We got approval to access superannuation to pay the medication costs but as I’m only 58 years old this then counts as taxable income hence further increasing the cost.”

“It is hard enough living with the mental and physical torment of having a Brain Tumour but knowing there is a drug available that can help but is so unaffordable for the average person is just devastating.”

“I am anxious about whether it will be approved by PBS or not and the difficult decision we will need to make if not approved which is likely to shorten my quality and length of life.”

“If I cannot access this medication on the PBS I will have to stop taking it due to the high cost of Voranigo. That feels like a death sentence to me with no hope for the future.”

Impact on quality of life

‘The Australian Brain Cancer Landscape 2025’ presented to the Australian Government in November 2025 details the significant burden of this disease belied by incidence statistics alone. People living with IDH-mutant glioma face significant, cumulative impacts on quality of life – from disease progression, but also due to the long-term impacts of existing treatment options.

Vorasidenib gives these patients and their families a real improvement in quality of life – from seizure control to improved mental health, and protection from the life-long cognitive impacts that are a risk of current treatment options.

See the Australian Brain Cancer Landscape 2025 for more on the real impacts of living with brain cancer: https://btaa.org.au/news/latest-news/new-research-brain-cancers-impact-society

“As a healthy 37 year old, working mother and wife this has given me quality of life and longevity”

“Knowing Voranigo is managing her IDH-glioma, her mental stresses are significantly reduced, she has avoided chemo/radiation, is a happier person and can spend time with our son. Voranigo is also extremely well tolerated, means her MRIs are less frequent and we need less frequent appointments - helping to reduce the burden on the hospital and doctors.”

“By not having to undergo radiation, I won’t experience early cognitive decline given where my tumour is.”

"I am more alert... I have energy to do rehab which is improving my quality of life”

“I don't suffer from as many headaches. I also have experienced lessened mobility issues and brain fog/fatigue.”

“Having an alternative treatment that is working with little to no side effects without the consequences that radiation and chemotherapy cause.”

“My underlying anxiety is much reduced as I know this is so effective. I am able to put a lot of difficult questions out of my mind. I am appreciative that every day on (vorasidenib) hopefully pushes any negative news out further and further”

“Consider myself fortunate to access Voranigo compared to reported impacts of more invasive chemotherapy and radiation”

Impact on productivity

‘The Australian Brain Cancer Landscape 2025’ quantified the significant national economic impact of brain cancer in Australia. The incredible toll on productivity is due to the burden of brain cancer on both patients and their care givers.

Living with IDH-mutant glioma impacts not only the patient’s ability to work but has repercussions on those around them. Vorasidenib provides patients with the quality of life and stability that enables them to return to work, and to give their care givers the confidence to do so as well.

“With not having to undergo chemotherapy, I’ve been able to spend time healthy with my family and return to work!”

“As a result of access to Voranigo, my wife is able to return to work and contribute to society.”

“I can continue working 5 days a week and contribute to society.”

“It is allowing some time to reassess how we face an uncertain future and despite some fatigue it is allowing me to continue use to work and contribute to my family and society”

“The side effects from treatment with radiation and chemotherapy have meant I could not commit to work. During treatment I was made redundant, in part because I could not commit to my full-time role. Access to vorasidenib would have increased my capacity to work, providing me with purpose and my family with financial stability.”

Impact on families

IDH-mutant gliomas disproportionately affect people in the prime of their lives, access to this treatment will not only impact patients, but will also provide an immense benefit to young families.

“Access to vorasidenib would give me meaningful time with my children (5 and 7 years old) – time in which I can actually be present as a mother, be active in their lives, and be ‘well’ enough to share their lives. It would mean a real shot at being there when they reach adulthood”

“I feel anxious awaiting this drug to be put onto the PBS and am fearful if it does not get put on. This is a real chance for me to have quality years with my children”

“(Vorasidenib) enables me to watch my 2-year-old daughter grow up and have quality of life.”

“As a mother, it breaks my heart every day knowing she has this tumour. The only things that I want in life for her is to have a long happy life, a long happy marriage. To see her 2-year-old son and 4-year-old daughter grow up to be as amazing as their Mum and to see them become amazing adults like she is.”

Impact on disease progression

While we acknowledge there is limited evidence on the long-term impact of vorasidenib on disease progression and survival, our patients are seeing early signs of arrested disease progression. Our patient community cannot wait five-, ten-, or twenty years for long term data to be collected in a clinical trial setting while access to this drug is limited.

“Voranigo has kept my wife's IDH-mutant glioma stable, when it was looking like it would progress “

“My daughter has a grade 2 oligodendroglioma and has had stable MRI scans. No growth”

“I have had two scans since which show no progression!”

“My cancer has gone to being stable or growing very slowly (MRI results aren't clear), which means I am still alive”

Hope

A common theme in the feedback we received is that this new treatment option has given patients and their families hope, for the first time since diagnosis.

While hope itself is not a PBAC criterion, it reflects meaningful gains in quality of life, engagement in daily activities, and adherence to ongoing treatment. These comments provide insight into the broader benefits of access to vorasidenib, complementing the clinical and economic evidence under consideration.

“I’ve been able to truly live my life, and this has given me and my family hope against the horrible disease of brain cancer. “

“The biggest one is hope. Hope that my wife will be ok. And trust that this will give her more time.”

“It gives me great confidence and much greater hope of a longer future with family and friends.”

“This medication is a game changer, and I would encourage public funding of this. There hasn't been a more significant development in IDH mutant glioma treatment in decades. Finally, there is some hope.”

“My future was very unclear, my tumour cannot be removed, but with Voranigo I have hope that it could possibly prolong my life by slowing the growth process”

“My family member has been very stable on Voranigo with no side effects so far and lots of hope for the future”

BTAA strongly supports PBS listing for vorasidenib

The combined factors of few alternative treatment options, the significant burden of living with brain cancer, and the high cost of private prescription highlight why PBS subsidisation of vorasidenib is urgently needed.

Patients with IDH-mutant glioma often face years of uncertainty, repeated surgeries, and progressive loss of function. Many continue working, raising families, and contributing to their communities while managing the physical and cognitive impacts of treatment. Access to vorasidenib offers these patients meaningful time. Time with family, stability in employment, and preservation of independence.

Without PBS access, patients face costs of hundreds of thousands of dollars in treatment costs, or must forgo treatment altogether, creating inequity based purely on financial means. The PBAC’s framework recognises both clinical effectiveness and the broader social value of reducing disease burden. In this case, PBS listing would align with both: improving survival and quality of life, while reducing the long-term costs of more invasive care.

Once again, BTAA strongly urges the PBAC to recognise the extraordinary burden borne by people living with lower-grade glioma, the proven clinical benefit of vorasidenib, and the transformative impact PBS listing would have on equity and quality of life.

We thank the Committee for its consideration and welcome the opportunity to provide further consumer insight or testimony in support of this submission.

Yours sincerely,
Christine

Christine Whittall
Executive Officer
Brain Tumour Alliance Australia