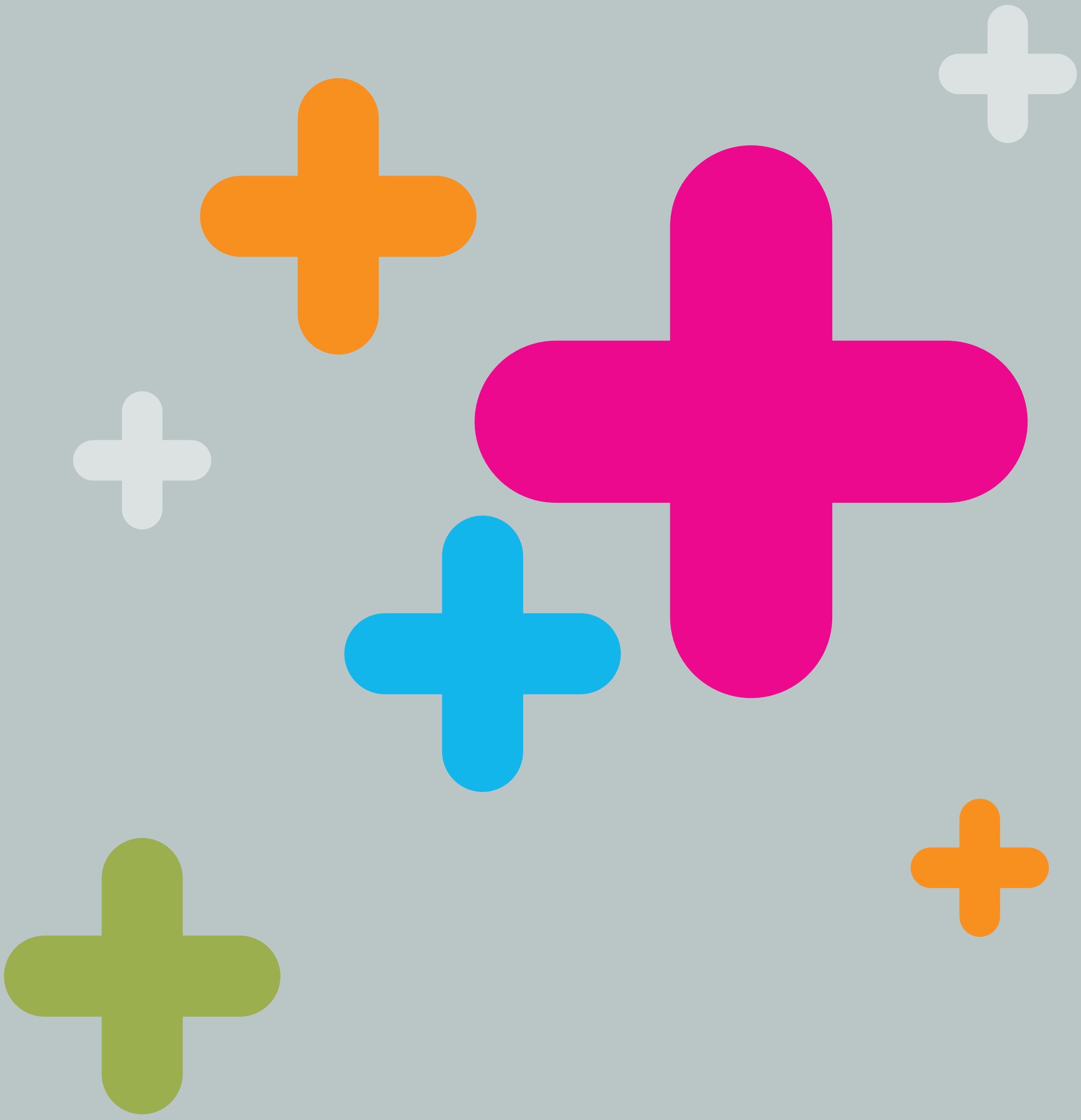
Mapping the Journey of Australian Paediatric Low-Grade Glioma (pLGG) Patients



A summarised report of work by Layla Carmeli-Wolski, Sienna Stuart-Williams, and Thomas Wakeling

Acknowledgement

This report is a summarised group Honours thesis authored by Layla Carmeli-Wolski, Sienna Stuart-Williams and Thomas Wakeling from Monash University, undertaken as part of Monash University's Bachelor of Science Advanced — Global Challenges (Honours) degree, with academic supervision from Dr Rose Herbert and Associate Professor Djuke Veldhuis. Industry supervisors were Simon Higgins, Warren Brooks and Christina Xinos of Ipsen Pharmaceuticals.

Developed by Biointelect

Level 4, 143 Macquarie Street,
Sydney NSW
Australia 2000

T 1300 800 984
E info@biointelect.com
www.biointelect.com

Foreword from Dr Kim Wark,

Head of Research, Robert Connor Dawes Foundation

A diagnosis of paediatric low-grade glioma (pLGG) changes life profoundly, not only for the child affected, but for the families and caregivers who must navigate a complex and often uncertain journey over many years. Although pLGG is classified as a rare disease, it is the most common childhood brain tumour, and its impact extends well beyond treatment, affecting everyday life, education, wellbeing and long-term care.

To improve outcomes meaningfully, we must look beyond clinical survival alone and better understand the lived experiences of children and their families. This report provides a clear and accessible view of the Australian pLGG journey, drawing on patient, caregiver and clinician perspectives. By mapping key stages from early symptoms and diagnosis through to treatment, survivorship and transition, it captures both the diversity of individual experiences and the shared challenges faced across the community.

At the Robert Connor Dawes Foundation, we are committed to amplifying the voices of children with brain cancer and those who care for them. Patient centred resources such as this Patient Journey Map are vital in translating lived experience into practical insight. They help inform research, advocacy and policy, while also offering families reassurance, understanding and a sense of preparedness for the path ahead.

I commend the authors for developing a thoughtful and compassionate resource that reflects the realities of living with pLGG in Australia. It is my hope that this report will support families, inform stakeholders and encourage collaboration across the pLGG community, ultimately contributing to more holistic, equitable and person-centred care.

K. L Wark

Dr Kim Wark
Head of Research, Robert Connor Dawes Foundation

Liz Dawes

Liz Dawes, OAM
Founder & CEO, Robert Connor Dawes Foundation

> *"Our experience has been a journey full of unknowns, we are still learning as we go. Our greatest challenge has been accessing support for siblings, a diagnosis affects them deeply too. We hope in time that siblings needs are better understood, helping families in the future who end up on this same journey."*
>
> – Parent of a pLGG survivor

Executive Summary

While still being deemed a rare disease, paediatric low-grade gliomas (pLGGs) are the most common childhood central nervous system (CNS) tumours. Despite its relative prevalence, no research has comprehensively assessed how pLGG is treated in Australia, and how patients and their families experience their treatment journey. This report outlines the journey of Australian pLGG patients, from initial symptom recognition through to post-treatment care, capturing the lived experience of Australian pLGG families.

Using semi-structured interviews with five pLGG families and three pLGG-treating clinicians, an Australian-first pLGG patient journey map (PJM) was developed. A comparison between Australian and international standards for pLGG was undertaken using a systematic review of international treatment guidelines. The review and patient journey map revealed key themes in pLGG care and treatment, including the importance of biopsies for genetic testing, systemic health system challenges, increased availability of targeted therapies, and the need to prioritise quality of life in pLGG management.

Despite the small sample size, the PJM establishes the foundation for patient-centred policy development and research. It is the first resource of its kind, presenting the journey of pLGG patients in a visually engaging and succinct manner, designed as both a support resource for families and an advocacy tool on their behalf to convey their experiences to their broader community, to ultimately improve outcomes for pLGG families.

Table of contents

Background and Literature Context

What is pLGG

Paediatric low-grade gliomas (pLGGs) are the most common childhood central nervous system (CNS) tumours[1,2,3]. The diagnosis encompasses a heterogeneous group of childhood tumours that start in the glial cells of the brain or spinal cord[1]. The tumours are classified as 'low-grade', reflecting their typical slow growth and limited tendency to spread[1,4]. Each patient's experience of pLGG can vary significantly[3,5], determined by the age at diagnosis and the location, size, and genetic composition of the tumour[1,5,6].

Presenting symptoms depend on the tumour location and include headaches, nausea, vomiting, impaired vision and significant fatigue[1,5,6]. The manifestation of symptoms can be mild in some and life-threatening for others, resulting in premature death in some cases[5].

While 90% of patients treated for pLGG survive at least 10 years[7,8], long-term survival data remains limited. Although traditionally considered a benign condition, many advocate for the consideration of the chronic nature of pLGG as this better reflects the lasting burden on young patients and their families[5,9,10]. Disease onset can be as early as birth[6], and patients who survive will often require ongoing, and often lifelong, management of side effects of both the disease and its treatment[5,9,10]. Therefore, evaluation of the success of a patient's overall treatment and ongoing survivorship must involve a holistic understanding of the burden of pLGG, not just an assessment of tumour progression[11].

pLGG Burden in Australia

In 2021, 194 children between the ages of 0 and 14 were diagnosed with a CNS tumour in Australia[12]. Based on the assumption that pLGG represents 30-40% of paediatric CNS tumours[1-3], it can be estimated that 68 children aged 0-14 years were diagnosed with pLGG in Australia in 2021. However, this estimate is likely an underrepresentation of the true incidence. Australian cancer registries do not capture pLGG as a discrete disease entity, and in some cases, stratify their data using outdated classification systems that do not reflect contemporary molecularly defined tumour subtypes[14,15]. Publicly available registry data are fragmented across jurisdictions, lack consistent age stratification, and are subject to multi-year reporting delays[13,14,15]. In addition, pLGG may be diagnosed beyond early childhood, as such, cases captured in the registry data are not fully representative[13].

The personal burden of pLGG spans multiple quality-of-life (QoL) domains. Physical symptoms, such as impaired motor function, speech, swallowing, vision, hearing, and seizures, can limit participation in everyday activities, including eating, play, schooling, and driving later in life[16]. Physical limitations negatively affect the patient's emotional well-being, adding to the neurocognitive deficits caused by the tumour and/or treatment[16]. Additionally, the patient's symptoms may lead to challenges in social relationships[17] and increased depression and anxiety[16].

As pLGG patients are children, caregivers play a central role in managing symptoms and long-term side effects, often limiting their ability to work at pre-diagnosis capacity[5,18,19]. As disease management falls largely to caregivers, most often mothers[20], the experiences of caregivers and family members must be considered to capture the full burden of pLGG.

Treatment of Paediatric Low-Grade Glioma: Literature and Clinical Context

Paediatric low-grade glioma treatment in Australia is not standardised under a single national guideline, resulting in variation between hospitals and individual patient experiences. Treatment is guided by tumour location, progression and symptom burden[6]. Management options include surgery, chemotherapy, radiation therapy, targeted therapy, or a watch-and-wait approach where immediate treatment is not required[1,5,6].

Surgery	Where feasible, surgery is the preferred initial management approach, as consistently recommended across the literature[21-28]. Complete resection is preferred and may be curative, though it is frequently not feasible due to tumour location, yet, even when complete resection is not possible, biopsy is often pursued to inform diagnosis[21,29]. Surgery may result in post-operative deficits or side effects, including endocrine dysfunction and fatigue[22,30].
Chemotherapy	When surgery is not possible or incomplete, particularly if the residual tumour remains symptomatic, chemotherapy is pursued[31]. Chemotherapy is frequently used as a post-surgical treatment for pLGG[7,21] but can cause significant short-term and, less commonly, longer-term toxicities, including myelosuppression, neuropathy, allergic and anaphylactic reactions, and renal and hepatic dysfunction, depending on the regimen received[1]. Chemotherapy schedules vary and may last up to 85 weeks[32].
Radiation therapy	The use of radiation therapy in pLGG has declined due to its association with long-term endocrine, neurocognitive, neurologic, secondary cancer and vascular complications[21,33]. Given the high survival rate of pLGG[9] and the potential for lifelong side effects, radiation therapy is generally avoided, and is considered only when all other treatment options have been exhausted and there is a risk of death due to uncontrolled tumour growth[22,23].
Targeted therapy	Advances in molecular research have demonstrated that pLGG is largely driven by abnormal activation of the MAPK/ERK pathway[9,34]. This distinguishes paediatric-type LGGs from adult-type LGGs, which are more commonly driven by isocitrate dehydrogenase (IDH) mutations[2,11]. These scientific discoveries have enabled the evolution of targeted therapies, aimed at stabilising or reducing tumour growth. Several targeted therapies have demonstrated a reduction in tumour growth in clinical trials, indicating their potential as first- or second-line treatments[35,36]. The role of targeted therapies in pLGG treatment is rapidly evolving. In Australia, patients whose disease is not amenable to surgical excision, or who experience disease progression following surgery, may be eligible for first line targeted therapy if they carry a BRAF V600 mutation, in accordance with PBS eligibility criteria[37]. As access to PBS listed targeted therapy is contingent on identification of a qualifying mutation, molecular testing, where feasible, is mandated to inform treatment selection. This reflects a broader shift toward molecularly informed treatment strategies. Management of other pLGG subtypes remains dependent on tumour biology, treatment response, and access considerations[1,34].
Watch and wait	Where the potential harms of treatment outweigh the symptoms of a non-life-threatening tumour, a watch-and-wait approach may be adopted[6]. This strategy involves active clinical surveillance rather than immediate treatment[6].
Quality-of-life considerations	90% of patients treated for pLGG survive at least 10 years[7,22]. Given these high survival rates[9] and the potential for lifelong treatment-related morbidity, treatment decisions are guided by careful assessment of risk versus benefit. Therefore, quality-of-life considerations are central to treatment selection and sequencing across the pLGG care pathway. Treatment guidelines emphasise prioritising QoL and minimising morbidity, rather than focusing solely on survival, when determining pLGG treatment strategies[21,23-26,28]. This approach reflects the expected high overall survival of pLGG patients, with tumour boards often assuming long-term survival when weighing treatment options[21,23,28]. As a result, radiation therapy is frequently avoided, as its potential benefits often do not outweigh the significant long-term morbidities that patients would need to manage across their relatively long lives[22,26,28]. Ultimately, treatment decisions for pLGG are guided by an assessment of risk versus benefit. Where the potential harms of the treatment outweigh the symptoms of a non-life-threatening tumour, a 'watch-and-wait' approach may be adopted[6].

Methodology

To date, no research has comprehensively assessed how pLGG is treated in Australia and how patients and their families experience this journey, representing a significant gap.

This study used a qualitative mixed-methods approach to map the Australian pLGG patient journey. A systematic review of international pLGG treatment guidelines was undertaken to provide contextual comparison in the absence of an Australian-specific guidance. For the purposes of this report, pLGG was defined according to paediatric-type low-grade glioma classification, as distinct from adult-type low-grade glioma and based on tumour biology. This definition allowed inclusion of individuals diagnosed in adolescence or early adulthood whose tumours met recognised paediatric-type pLGG criteria, rather than adult-type LGG classification. This approach recognises that paediatric age definitions and tumour classification practices vary across Australian registries and health system frameworks.

Semi-structured interviews with Australian pLGG families and treating clinicians captured their lived experiences across diagnosis, treatment and survivorship. Interview guide data were analysed using reflexive thematic analysis and used to develop an Australian-specific Patient Journey Map (PJM), which was subsequently validated by key clinical, research and patient stakeholders.

Interviews were conducted with two key groups: pLGG families and pLGG-treating clinicians. pLGG families included: parents and/or caregivers of pLGG patients, adult family members of pLGG patients, and adults who were treated for pLGG as children. pLGG-treating clinicians included: two doctors and a nurse. All participants were over the age of 18.

Clinician Interviews

A total of three clinician interviews were conducted, as presented in Table 1.

All three clinicians who were interviewed were based at the same public Victorian paediatric hospital; all of them were directly involved in the care of pLGG patients. The sample provided a firsthand experience of managing pLGG patient care and treatment in Australia's public healthcare system (Table 1).

Table 1: Clinical interview characteristics

Interviewee	Role	Direct involvement with pLGG patients (YES/NO)	Institution type	State
1	Doctor	YES	Public paediatric hospital	VIC
2	Nurse	YES	Public paediatric hospital	VIC
3	Doctor	YES	Public paediatric hospital	VIC

pLGG Families

A total of five participants were interviewed for the pLGG family participant group, as presented in Table 2.

Table 2: pLGG family interviewee and patient characteristics

Interviewee	Relationship to pLGG patient	Histology	Location of tumour	Age at diagnosis	Current age	Treatment undergone	Most disruptive symptoms
1	Parent	Pilocytic astrocytoma	Cerebellum and brain stem	7	10	Surgery, watch and wait	Fatigue
2	Patient (18+)	Pilocytic astrocytoma	Brain stem	19	32	Surgery, RT, watch and wait	Fatigue
3	Patient (18+)	Pilocytic astrocytoma	Spinal cord	13 months	21	Surgery, chemotherapy, watch and wait	Migraines, nerve damage
4	Parent	Ganglioglioma	Brain stem	5	11	Watch and wait, chemotherapy, surgery, targeted therapy	Physical disabilities (left side), fatigue, swallow difficulties
5	Patient (18+)	Paediatric low-grade glioma	Cerebellum	10	19	Watch and wait, surgery	Chronic pain

The PJM was informed by interview data from five pLGG families and three clinicians. No external references were used in the creation of the PJM to ensure the map authentically reflects the lived experience of participants within an Australian context. Therefore, this PJM is the first of its kind to summarise the patient experience in this manner, support families as they prepare for the journey ahead and guiding their communities in how best to support their journey.

Novel Research Findings: Australian pLGG Patient Journey Map

Key Insights to the pLGG Patient Journey in Australia

This section presents novel qualitative research findings, synthesised through the Australian pLGG PJM and informed by primary interviews with pLGG families and clinicians, supported, where appropriate, by literature.

Across the interviews conducted for this study, families frequently reported experiencing dismissal during the pre-diagnosis stage, as healthcare professionals failed to attribute their child's symptoms to a potential brain tumour. These delays highlight how the rarity of pLGG, compounded by regional isolation, exacerbates inequities in achieving a timely diagnosis.

The PJM highlights MRI as central to identifying brain lesions in pLGG; however, MRI alone was often insufficient in the current era of molecular diagnostics. In some cases, a definitive pLGG diagnosis required genetic testing of tumour tissue obtained via biopsy or tumour resection.

Patients unable to undergo a successful biopsy, often due to tumour location, do not always receive a conclusive pLGG diagnosis, limiting access to targeted therapy. Families and clinicians reported that access to targeted therapy is restricted to patients meeting strict eligibility criteria, either through clinical trials or for the limited therapies approved by the TGA and listed on the PBS.

During treatment, families also faced limited therapeutic options. Although new targeted therapies have emerged in recent years, relatively few are approved and reimbursed in Australia[37,38]. Australian patients are further disadvantaged by government-funded medicine reimbursement timelines taking approximately three to four times longer than in other leading countries[39]. This challenge is amplified in pLGG due to the rarity of the disease and the small size of the eligible patient population, which limit the availability of robust health economic data[40,41]. Since cost-effectiveness assessments are a core requirement of Health Technology Assessment (HTA) submissions for PBS listings, rare diseases such as pLGG are disproportionately affected by evidentiary gaps, contributing to prolonged listing timelines[42,43] and reinforcing inequities in timely access to treatment.

The PJM highlights the heterogeneity of pLGG experiences, underscoring the need to treat each child as an individual. The PJM patient timelines illustrate this variability, showing that pLGG journeys differ widely across pre-diagnostic symptoms, treatment approaches, relapse patterns and overall timelines. As a result, defining a "typical" pLGG patient journey would be inherently reductive. This complexity is reflected in the absence of national treatment guidelines.

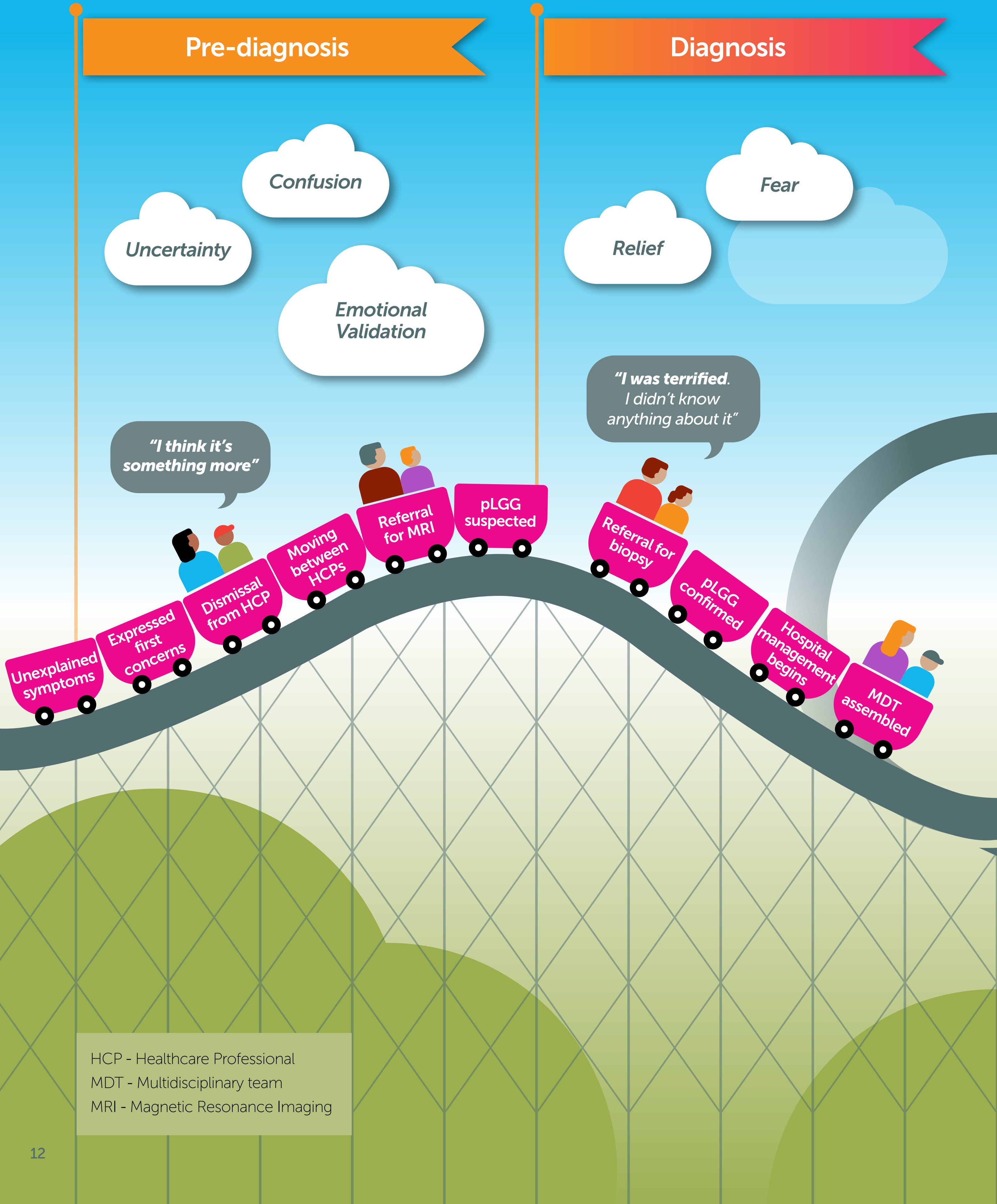
The PJM's 'Living with pLGG' page highlights that survivorship is largely shaped by managing ongoing side effects from the tumour or its treatment. These include fatigue, speech, hearing and functional deficits, as well as readjustment to social challenges following isolating, high-intensity treatment phases.

Given that 90% of patients treated for pLGG survive at least 10 years[7,9], pLGG is largely characterised by extended non-treatment periods, underscoring the importance of survivorship support alongside treatment. Support varies markedly between intensive treatment phases, when hospitals coordinate allied health, education and social services and post-discharge periods. While some Australian paediatric hospitals offer extensive in-hospital supports[44-46], families reported limited support once home, including challenges with school reintegration, educational accommodations and ongoing care coordination, alongside poor school awareness of pLGG. This disparity reflects a system focused on acute care rather than long-term management of a chronic condition.

Long-term quality of life is further shaped by the transition from paediatric to adult care. Clinicians emphasised the need for coordinated transitions to ensure continuity of care, consistent with the literature[47,48]. This transition is often challenging[49,50], particularly due to limited disease awareness in adult services and difficulties forming new care relationships[51-53], making it a critical phase for patients with this chronic condition.

The PJM is a concise, visual summary of key insights derived from interviews with Australian pLGG families and clinicians. It captures the core stages of the patient experience and highlights practical opportunities to improve outcomes and involvement. While it can inform stakeholders such as regulators, sponsors, and researchers, it is primarily designed to support pLGG families. The PJM functions as a standalone resource that patients can use to understand, explain, and share their journey with their wider community.

Patient Journey Map



Pre-diagnosis

Uncertainty

Confusion

Emotional Validation

“I think it’s something more”

Unexplained symptoms

Expressed first concerns

Dismissal from HCP

Moving between HCPs

Referral for MRI

pLGG suspected

Diagnosis

Relief

Fear

*“**I was terrified**. I didn’t know anything about it”*

Referral for biopsy

pLGG confirmed

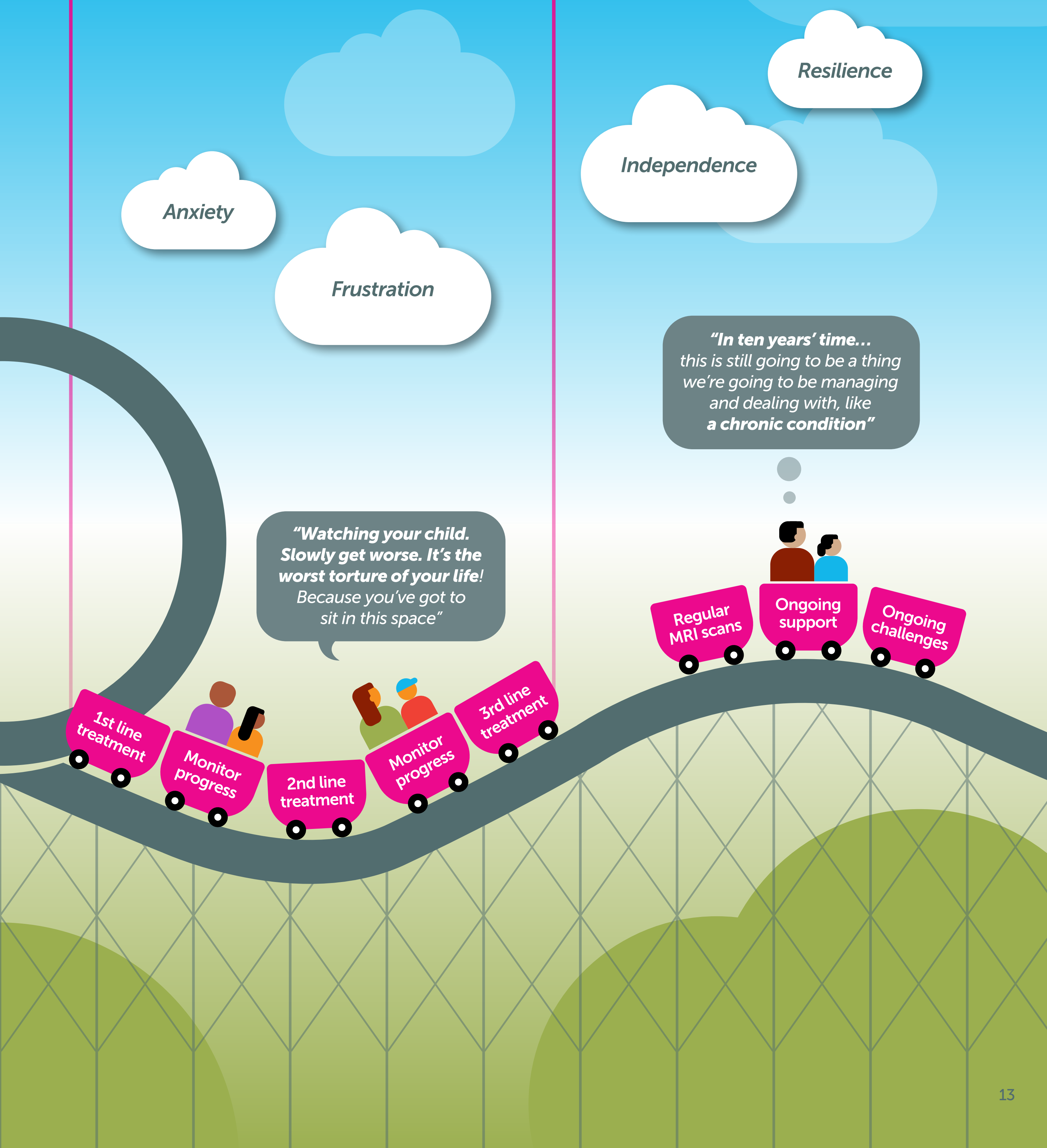
Hospital management begins

MDT assembled

HCP - Healthcare Professional
MDT - Multidisciplinary team
MRI - Magnetic Resonance Imaging

Treatment

Living with pLGG



"Watching your child. Slowly get worse. It's the worst torture of your life! *Because you've got to sit in this space"*

"In ten years' time… *this is still going to be a thing we're going to be managing and dealing with, like* ***a chronic condition"***

Patient Timelines

No journey is the same

Diagnosis

"[Patient] just kept getting colds"

Initial symptom recognition		Surgery	Watch and wait
Age: 7	**3 months later**	**3 days later**	**Ongoing**
Nightly vomiting Headaches Dizziness	**Pilocytic astrocytoma** BRAF mutation Cerebellar & brain stem	Near total removal of tumour	6-monthly MRIs

Age: 10

Initial symptom recognition		Watch and wait		Chemotherapy	Biopsy	Chemotherapy	Biopsy	Targeted therapy
Age: Newborn	**Age: 2**	**Age: 5**	**4 months later**	**11 months later**	**2 months later**	**2 weeks later**	**3.5 years later**	**Ongoing**
Collapsed lung	Delayed speech development Left side palsy Paralysed palette	MRI revealed a non-specific brain lesion	**Ganglioglioma** suspected Brain stem	Weekly vinblastine for 52 weeks	Tissue sample obtained Ganglioglioma diagnosis confirmed	Monthly carboplatin for 11 months (incomplete schedule due to anaphylaxis)	Insufficient tissue mass removed for genetic profiling	Trametinib (MEK inhibitor) via compassionate access

Age: 11

"I was a very keen netballer, but I would fall over all the time"

Initial symptom recognition		Watch and wait	Surgery	Surgery	Surgery	Watch and wait
Age: 6	**4 years later**	**2 days later**	**3 years later**	**4 days later**	**5 years later**	**Ongoing**
Falling Headaches Poor vision	**Paediatric-type low grade glioma** Cerebellum	6-monthly MRI scans, followed by 3-monthly as the tumour size grew	Insufficient tissue mass removed	More tumour mass removed	More tumour mass removed Genetic profiling confirmed pLGG	Regular 6-monthly MRI scans

Age: 19

Initial symptom recognition		Surgery	Chemotherapy	Surgery	Watch and wait
Age: 5 months	**8 months later**	**2 months later**	**1 week later**	**6 years later**	**Ongoing**
Slower development Delayed crawling and communication	**Pilocytic astrocytoma** Cerebellum	Tumour mass removed	Monthly carboplatin for 18 months	Tumour mass removed	Regular MRI scans (originally 6-monthly, now yearly)

Age: 21

"I thought I just needed glasses"

Initial symptom recognition		Biopsy	Radiation therapy*	Watch and wait
Age: 19	**3 weeks later**	**5 days later**	**4 months later**	**Ongoing**
Double vision	**Pilocytic astrocytoma** Brain stem	Tumour deemed inoperable Tissue sample obtained	Treated for 3 months	Regular MRI scans (originally 3-monthly, now 2-yearly)

Age: 32

"My tumour hasn't grown in 13, 14 years"

**This patient was treated several years ago. Currently, radiation therapy is strongly discouraged in pLGG as its significant and lasting morbidities (inc. fertility concerns and the risk of secondary malignancy) outweigh its potential benefits.*

Pre-diagnosis

What's going on?

Patient experience	• Early symptoms were often **confusing** and **minimised** • **Unexplained**, worrying **changes** included dropping objects, double vision, stumbling or sudden, frightening events such as falls, collapsing and seizures • Symptoms often **disrupted daily life**	***"I thought I just needed glasses... [cancer] didn't cross my mind"*** ***"I would probably be picked up at least once a month [from school]"***
Caregiver experience	• Persistent **worry, exhausted** nights in ED, juggling other children and work, and deep frustration when concerns were dismissed • Feeling **dismissed** or told it was likely something else • Parents were hand-balled between several specialists	***"I felt ghastly because I was going, I think it's something more"*** ***"You're just being... a helicopter parent"***
Clinical pathway	• Most diagnoses hinged on **MRIs**, but routes to MRI were highly variable • Where non-GP HCPs (e.g. optometrist, ENT or ophthalmologist) insisted on imaging, diagnosis was often reached faster • Frequent **attribution to other causes**	***"I think we were in a place of very blissful ignorance, because whilst we had concerns, and whilst we knew something was wrong. They had presented a very plausible explanation for us"***
Signs and symptoms	• Persistent headaches • Unexplained nightly vomiting • Visual disturbances • Frequent falls, unsteady gait • Developmental delay or regression • Palate paralysis / speech /swallowing challenges	

UNMET NEEDS

- HCPs unable to attribute symptoms to a potential brain tumour
- Earlier referrals to MRIs could have saved critical time

"There was this really extended period of time where we felt like doctors and people dismissed us. They told us there was nothing wrong with our child. And I know it's not an uncommon story"

"If ED had more understanding of what signs to look out for in potential brain tumour kids, that would have been helpful... by the time they diagnosed him it was considered life-threatening"

Diagnosis and Treatment

Finally some answers…

	Diagnosis	Treatment
Patient experience	• Children demonstrated an awareness of their circumstances, reflecting a maturity as they engaged actively by asking questions. They appreciated **honest explanations** from parents and HCPs • Adolescents described **terror** on hearing the word "tumour," while some younger children initially accepted the news without much need for detail	• Across modalities, patients experienced **fear, frustration**, and **loss of normal childhood/ adolescence**. Many felt *"different"* from peers as they missed school, sport and grieved missed social milestones • Treatments often meant dependence on parents, aides, or medical staff. Older adolescents highlighted frustration at being overprotected, while **younger children more readily adapted**
	"Mum! I decided to name the thing in my brainstem… I'm going to call it Rainbow"	
Caregiver experience	• Diagnosis was marked by **acute shock**, and **fear of what's to come**, paired with the relief of **finally knowing what was wrong** • Several parents described an intense 24-hour period after hearing a "lesion" existed in their child's brain. For some, treatment was immediate, but others described an extended waiting period with **limited answers** (*"we will see you in three months"*). This prolonged anxiety and fear of the unknown • **Support services**, such as advocacy groups and therapy, were available not just for the patient, but also **for families**, acknowledging that family members were also affected by the news of diagnosis	• **Disruption** to daily routine, siblings missed out on attention, parents missed work, and **routines were reshaped around hospital visits** and the child's energy levels • Families reported at least one caregiver needing to **alter their regular work schedule** in order to look after the patient • Parents carried **anxiety** about survival, long-term side effects, and the future • Parents are offered **choices** by the hospital team when appropriate: *"The choice between vinblastine and carboplatin… depends on the family's preference"*
	"We know that if we've ever got any concerns, his team at [the hospital] are there down the line anytime, so we can call them if we've got any concerns"	

UNMET NEEDS

- Insensitive explanation of the diagnosis to parents
- Those unable to have a successful biopsy did not always have a conclusive pLGG diagnosis, often leading to more uncertainty
- Diagnostic delays meant that patients suffered through their symptoms for longer and risked tumour growth and symptoms worsening

"Isn't it unfair that you can't just do surgery and be cured?"

"well-meaning friends [would say]… 'oh, but I looked [pLGG] up on the internet; if they can just remove it she's cured'. And I'm sitting there going, 'yes, you're right, if it was in her frontal lobe, we would have some of those options, but we don't"

Parental Advocacy

The responsibility of advocacy fell heavily on parents, with one parent reflecting that **persistence** with ED staff "saved" their child. Parents repeatedly described being the coordinators of their child's care at this stage, chasing results, insisting on further tests, and not accepting dismissals. Their persistence often determined whether diagnosis and treatment happened promptly

It's a balancing act...

Clinical pathway

Diagnosis

- **MRI was the pivotal diagnostic tool**, signalling seriousness and often triggering urgent escalation. For some, MRI and surgical consultation happened within days and for others, interpretation delays stretched into weeks or months
- While MRI revealed abnormalities, it did not always provide a conclusive pLGG diagnosis (i.e. which specific subtype of pLGG), which required **biopsy** for histology and grading. Biopsies clarified these details but were not always possible due to tumour location or insufficient tissue mass. **Genetic testing**, when feasible, provided important insights

Treatment

- There are no Australia-wide pLGG treatment guidelines, since **available treatments are constantly evolving. Treatment can and does vary across different hospitals**
- Determining treatment is a balancing act, **weighing up the burden** of symptoms, the side effects of treatment, and the risks of tumour growth if untreated
- Operable tumours were treated with **surgery** when risks were considered acceptable and the tumour location dictated the extent of resection
- Inoperable or incompletely resected tumours required alternate approaches such as **chemotherapy, targeted therapy** or **watch-and-wait**
- Surgical care was managed by **neurosurgeons**, with ongoing treatment transferred to a **neuro-oncological team** if resection was incomplete
- **Targeted therapy** is an **emerging option**, but **access is limited** when genetic testing cannot be performed due to unsuccessful or absent biopsy
- Treatment involved complex multi-disciplinary care from various medical fields

Signs and symptoms

Diagnosis

- The immediate triggers for MRI and further workup were **neurological or functional red flags**. These symptoms represented a **tipping point** where other explanations were no longer accepted

"We're lucky that he's still here, that we did keep pestering them ... if we didn't question it, he wouldn't be here"

UNMET NEEDS

- Regional hospitals often lacked experience with pLGG, leaving parents to educate staff themselves
- Medication access gaps forced families to bring in their own supplies to regional hospitals
- Parking, travel, and out-of-pocket costs for medications and supplies were heavy
- Some families felt overlooked due to "low-grade" diagnosis: *"If she had [a more common childhood cancer] ... there would have been a team of social workers. But it's like, oh, it's just low-grade glioma ... That's been really impactful"*

Cost

Treatment often meant spending a lot of time in the hospital. Although formal support services were available and the cost of primary care was covered while in hospital, the significant associated costs of treatment were often overlooked (e.g. parking, missing work, organising meals, and complementary medicines etc.)

"It just progressively adds up"

Treatment Pathway

Treatment	Patient experience	Side effects	Family/Caregiver experience
SURGERY			
If the tumour is safely operable and symptoms are significant, surgery is often pursued. Complete resection is preferred, though partial resection may still relieve symptoms by reducing tumour size. Surgery may be encouraged early to improve neurological function.	• Initially frightening, eased by clear explanations • Recovery varies widely • Some experience lasting deficits (e.g. nerve damage, fatigue, headaches) • Hospital stays range from days to weeks	• Short- and long-term physical, cognitive and emotional effects • Risks include pain, infection, bleeding, brain and nerve damage • Surgery may relieve some tumour-related symptoms	• Often felt there was no real choice • Consent process described as traumatic • Fear of surgical outcomes and survival • Distress for siblings seeing hospitalisation *"The stress of knowing that his brother was going to be stuck and may not be coming out... it was a lot"*
CHEMOTHERAPY			
Used when surgery is unsafe or incomplete. Common regimens include, combination carboplatin and vincristine (weekly hospital visits), and monotherapies carboplatin (every 4 weeks for approx. 12 months) and vinblastine (weekly for approx. 52–74 weeks). Other agents may be used once options are exhausted.	• Physically exhausting and emotionally taxing • Significant side effects; ports sometimes required • Visible changes affect body image • Increased anxiety, social isolation and loss of independence • Long treatment duration with uncertain necessity	• Nausea, vomiting, fatigue, allergic reactions • Risk of carboplatin intolerance and anaphylaxis • More stable health reported with vinblastine	• Emotional burden of witnessing child's suffering • Long hospital hours and disruption to family life • Impact on schooling and siblings • Ongoing uncertainty balancing benefit vs harm • Monthly carboplatin preferred to reduce travel • Hospital-at-home used where possible

Treatment	Patient experience	Side effects	Family/Caregiver experience
TARGETED THERAPY			
A newer option, accessed via clinical trials, PBS or compassionate use, with strict eligibility. Patients without clear molecular drivers may struggle to access testing or treatment.	• Improved quality of life from symptom and tumour reduction • Fear of rapid tumour regrowth after stopping treatment • Side effects generally tolerable	• Rash, paronychia (infection of the nail fold), fatigue • Skin conditions and infections	• Financial stress due to limited PBS coverage • Dependence on compassionate access, which may be withdrawn • Anxiety about relapse due to limited long-term evidence
WATCH & WAIT			
Close monitoring through regular scans and reviews, used when treatment risks outweigh the risks of observation.	• Ongoing surveillance creates uncertainty and anxiety • Symptoms may worsen during observation • Disease progression prompts reconsideration of treatment	• Symptoms reflect underlying disease stage	• Emotionally exhausting, particularly during deterioration • Feelings of helplessness while waiting *"The uncertainty... Watching get worse. It's the worst torture of your life. Because you've got to sit in this space"*

Living with pLGG

A cycle of peaks and troughs…

Patient experience

- **Ongoing anxiety** around regular scans
- Long-term social and 'life-progression' challenges (e.g. inability to work while studying, concerns about fertility, etc.)
- **Younger children adapted more easily**, while teens/young adults carried heavier psychosocial burdens
- Once patients turn 18, treatment is transferred to an adult hospital: *"a lot of these kids we've known since they were little, and so, to trust a new clinician and a new team is such a foreign thing"*
- Many drew resilience from their experience and developed a motivation to 'seize the day'

Caregiver experience

- Parents balanced hope with fear, often **finding strength in their child's survival and positive disposition**
- Siblings and family dynamics were often disrupted or altered, although parents described strong resilience and unity and a strong ability to adapt
- It can take a while for anxiety to reduce and for a feeling of normalcy to return
- **Ongoing anxiety** around regular scans

"We don't know what the future looks like…we try and put a positive turn onto all of what is"

Clinical pathway

- Survivorship always involves **ongoing surveillance** of the tumour in the form of **regular MRIs** after treatment, tapering from 3-monthly to annual scans if stable. Sometimes clinicians have to look at more than just imaging to assess disease progression (e.g. side effects)
- Ongoing allied health services are often required, including occupational therapy, physiotherapy, psychology, and fertility specialists
- Finding the right **therapist/supportive care** was often challenging, but transformational when successful. During the **'off' periods**, when patients are not receiving active treatment, hospital support is limited. However, the introduction of a 'survivorship' clinical nurse consultant role in some hospitals has been transformative for patients

Tumour/ treatment deficits

- Fatigue
- Speech and hearing deficits
- Functional deficits

"I think there's a difference between surviving and living …surviving you're just waking up each day, but living you're actually making meaning"

Supportive care

- Therapy had a transformative impact on both patients and other family members. Music therapy was highlighted as especially powerful for patients.
- Advocacy group events fostered community and purpose
- Support from the NDIS and schools, in the form of aides, therapy and allied health, was valued

"The [hospital school was] involved in helping with the transition back to school in the community. Without them it would have been much harder"

UNMET NEEDS

- Insensitive explanation of the diagnosis to parents
- Those unable to have a successful biopsy did not always have a conclusive pLGG diagnosis, often leading to more uncertainty
- Diagnostic delays meant that patients suffered through their symptoms for longer and risked tumour growth and symptoms worsening

pLGG is a chronic condition. It's a lifelong journey marked by periods of intense treatment mixed with stretches of relative normalcy

How to meet the needs

	Families	Healthcare system
PRE-DIAGNOSIS	• **Trust your instincts**, you know your child best. • Don't be afraid to advocate and ask for more tests if symptoms persist. • Keep notes of symptoms and changes. This helps your care team. • Seek advice or second opinions early if you feel dismissed. • **Connect** with other families for reassurance and advocacy tips. • Use community and advocacy group resources to learn red flags and referral options.	• Recognise and escalate neurological red flags early. • Provide clear next steps when symptoms remain unexplained. • **Avoid minimising parental concerns** by listening actively and validating them.
DIAGNOSIS	• Ask your clinical team to explain results in plain language. • Encourage **open, age-appropriate conversations** with your child. • **Reach out** for psychosocial, emotional, and financial support. It's available and there to support you. • Use advocacy networks to find practical guides and peer connections Remember: your persistence matters! Your voice helps shape your child's care.	• Deliver results with **empathy**, clarity, and time for questions. • Immediately **connect families** to psychosocial and financial support. • Provide written or digital summaries that families can refer back to. • Communicate diagnostic updates back to GPs to improve brain tumour symptom recognition awareness.
TREATMENT	• Stay involved: you are an essential part of the care team. • Ask about treatment options, side effects, and available supports. • Reach out to advocacy groups and charities for travel and cost assistance. • Look after **your wellbeing**. Caring for yourself helps your child. • Keep school, friends, and siblings connected through updates and inclusion. • Share your experience with advocacy groups to help other families.	• Provide **clear explanations** of treatment goals and side effects. • Coordinate between tertiary and regional centres to avoid gaps in care. • **Normalise psychosocial support** as part of routine treatment. • Advocate for equitable access to therapies and medications. • Check in regularly about caregiver wellbeing and practical challenges.
LIVING WITH PLGG	• Continue and value the regular follow-ups to encourage stability and confidence. • **Encourage your child's independence** while recognising ongoing challenges. • Don't hesitate to **ask for school or workplace accommodations**. • Seek peer or mentorship programs for long-term support. • **Celebrate milestones:** focus on living fully, not just surviving. • When it becomes age appropriate, educate your child on their diagnosis and pathway. • Keep advocating: your lived experience helps drive system change.	• Provide structured survivorship and **transition-to-adult-care plans**. • Offer ongoing allied health and psychological support. • Ensure **clear collaboration** between paediatric and adult services. • Support education and workplace reintegration. • Recognise pLGG as a **chronic, lifelong condition requiring ongoing care and monitoring**.

Summary of support services

Redkite For families facing childhood cancer	Support call line where you can talk to them about anything and everything. They have all sorts of practical help, information and support to help you plan for the long term and stay on top of things when new challenges pop up. Additionally, they can work with you to help relieve some of the financial pressure.
Camp Quality	Camp Quality offers a range of services for families with kids aged 0-15, including FREE camps, getaways, fun days, and special experiences designed to help you relax, recharge, and rediscover laughter. Resources to help children understand cancer in a child friendly way. Additionally, they provide counselling and peer support services.
Robert Connor Dawes **RCD** Foundation Helping kids with brain cancer	Supporting families by coordinating and funding emotionally invaluable in-home music therapy and music-based resources through the RCD Foundation's Music Matters Program. The program provides families with access to blocks of 10 private, in-home music therapy sessions for children and young people under 25 with a brain tumour diagnosis across Australia, or a Music Resource Grant that enables recipients to access a musical instrument, a music experience, or funding for music lessons.
CHILDREN'S CANCER FOUNDATION	Provides a wide range of practical supports and resources such as counselling services to assist families in navigating their unique challenges. The CCF has partnered with My Room Children's Cancer Charity to fund a new national program that will provide fertility preservation services.
canteen	Canteen provides a wide range of free support services to help them overcome the specific challenges they're dealing with. They also provide services for parents dealing with cancer in their family.
peace of mind	Provides vital support for brain tumour patients and families through advocacy, navigation, financial aid, counselling, and community connection.
my room CHILDREN'S CANCER CHARITY	Providing funding for household bills, funerals, and other immediate needs, as well as providing uplifting experiences to brighten spirits along the way.
Ronald McDonald House Charities Australia	Through our accommodation and support services, we strive to keep families together when life gets turned upside down due to a child's illness. Provides a home away from home for families with children in hospital and education support for school-aged children who medically cannot go to school.
Challenge supporting kids with cancer	Challenge aims to manage the impact of a cancer diagnosis by addressing the emotional, social, and practical needs of your family. Whether it is help at home or a weekend away, we tailor our services to meet the requirements of each individual within the family unit.
BRAIN TUMOUR ALLIANCE AUSTRALIA inc 1800 857 221	BTAA seeks to provide peer support to people living with a brain tumour. They extend this to their carers, families and friends. BTAA provides information resources for newly diagnosed patients and can assist with referrals to the most appropriate support services in their area.

Please note that the availability of services may vary depending on the state or territory

Limitations

Recruitment of pLGG-treating clinicians was constrained by the lengthy hospital ethics approval timelines, which extended beyond the project timeframe. As a result, the clinical voice captured by the interviews reflects the perspectives of clinicians from a single Victorian hospital, though participating clinicians were able to draw on collaboration and communication with colleagues across other states and territories.

Recruitment of pLGG family participants was also challenging due to the rarity of the condition and the substantial burden placed on families managing ongoing care. Furthermore, ethics approval for this component of the project did not allow for the recruitment of families through hospitals, further limiting participation. While this means the PJM may not encompass the full breadth of clinical practice and lived experience across Australia, the insights captured provide valuable depth and authenticity. Together, these considerations highlight opportunities for future work to broaden participation and representation.

This patient journey map represents the first resource of its kind in Australia, offering a meaningful contribution that lays a strong foundation for future work to build a more comprehensive national understanding of the pLGG patient journey.

These limitations highlight clear opportunities for future research and system action, reinforcing the need for coordinated, nationally-informed approaches to pLGG care.

Conclusion

Paediatric low-grade gliomas comprise 30-40% of paediatric CNS tumours, making them the most common childhood CNS tumour. To date, there has been no comprehensive assessment of how pLGG is treated in Australia. This study aimed to outline the journey of pLGG patients, from initial symptom recognition through to post-treatment care, capturing the lived experiences of Australian pLGG families. The PJM presented in this report is the first resource of its kind, presenting the journey of Australian pLGG patients in a visually engaging and digestible manner. This resource may be used by Australian pLGG families to prepare for the journey ahead and patient advocates. The PJM represents a step towards more holistic and experience-informed care for Australian pLGG families.

This study reflects the experiences of a small, exploratory sample, with clinician perspectives drawn from a single paediatric hospital. Future work should build on this work by including larger and more diverse cohorts across Australian society, including multi-site studies that explore variation across states and metropolitan and regional settings, to develop a more nationally representative understanding of the pLGG patient journey.

Call to Action: Implications for Australian pLGG Care

1. **STRENGTHEN SUPPORT NETWORKS ONCE PLGG PATIENTS ARE DISCHARGED FROM HOSPITAL.**

   Following discharge, hospital-based support reduces significantly, shifting long-term care coordination and navigation responsibilities onto caregivers, creating sustained burden without adequate system support.

2. **ESTABLISH BETTER TRANSITION PATHWAYS FROM PAEDIATRIC TO ADULT PLGG CARE.**

   Due to pLGG's chronic nature, many patients will experience both paediatric and adult care for pLGG. Families experience this shift as abrupt and overwhelming, while clinicians emphasise the need for coordinated handover to maintain continuity of care.

3. **ENCOURAGE FURTHER COLLABORATION BETWEEN HOSPITALS.**

   pLGG's rarity limits familiarity in smaller or non-specialist centres. Leveraging expertise across hospitals is critical to delivering consistent, high-quality care regardless of geography or socioeconomic status.

4. **AMPLIFY PLGG FAMILY VOICES IN HEALTH, PLGG POLICY AND RESEARCH.**

   pLGG families possess critical insight into lived experience but are rarely engaged beyond clinical trials. Meaningful inclusion strengthens the relevance, quality, and impact of research and policy decisions.

5. **ENCOURAGE GREATER RECOGNITION AND INTEGRATION OF PATIENT EXPERIENCE DATA IN RARE DISEASE REGULATORY AND REIMBURSEMENT APPLICATIONS.**

   In rare diseases where clinical evidence is limited, structured patient experience data should be recognised as complementary evidence to ensure decisions reflect real-world needs and outcomes.

References

1. Fangusaro J, Jones DT, Packer RJ, et al. Pediatric low-grade glioma: State-of-the-art and ongoing challenges. Neuro-Oncol. 2023;26(1):25-37. doi:10.1093/neuonc/noad195
2. Bale TA, Rosenblum MK. The 2021 WHO Classification of Tumors of the Central Nervous System: An update on pediatric low-grade gliomas and glioneuronal tumors. Brain Pathol. 2022;32(4):e13060. doi:10.1111/bpa.13060
3. Ryall S, Zapotocky M, Fukuoka K, et al. Integrated molecular and clinical analysis of 1,000 pediatric low-grade gliomas. Cancer Cell. 2020;37(4):569-583.e5. doi:10.1016/j.ccell.2020.03.011
4. Stages of cancer. Cancer Council Australia. Accessed August 28, 2025. https://www.cancer.org.au/cancer-information/stages-of-cancer
5. Pediatric Low-Grade Glioma (PLGG)- Voice of the Patient Report. Pediatric Brain Tumor Foundation; 2024. Accessed August 27, 2025. https://curethekids.org/research/patientfocused/
6. Zelt S, Cooney T, Yu S, et al. Disease burden and healthcare utilization in pediatric low-grade glioma: A United States retrospective study of linked claims and electronic health records. Neuro-Oncol Pract. 2024;11(5):583-592. doi:10.1093/nop/npae037
7. Sait SF, Giantini-Larsen AM, Tringale KR, Souweidane MM, Karajannis MA. Treatment of pediatric low-grade gliomas. Curr Neurol Neurosci Rep. 2023;23(4):185-199. doi:10.1007/s11910-023-01257-3
8. Gnekow AK, Walker DA, Kandels D, et al. A European randomised controlled trial of the addition of etoposide to standard vincristine and carboplatin induction as part of an 18-month treatment programme for childhood (≤16 years) low grade glioma – A final report. Eur J Cancer. 2017;81:206-225. doi:10.1016/j.ejca.2017.04.01
9. Ryall S, Tabori U, Hawkins C. Pediatric low-grade glioma in the era of molecular diagnostics. Acta Neuropathol Commun. 2020;8:30. doi:10.1186/s40478-020-00902-z
10. Moreira DC, Lam CG, Bhakta N, et al. Tackling Pediatric Low-Grade Gliomas: A Global Perspective. JCO Glob Oncol. 2023;9:e2300017. doi:10.1200/GO.23.00017
11. Jones DTW, Kieran MW, Bouffet E, et al. Pediatric low-grade gliomas: next biologically driven steps. Neuro-Oncol. 2018;20(2):160-173. doi:10.1093/neuonc/nox141
12. Australian Childhood Cancer Statistics Online. Cancer Council Queensland. October 2024. Accessed June 9, 2025. https://cancerqld.org.au/research/explore-our-projects/australian-childhood-cancer-statistics-online/
13. Carmeli-Wolski L. Classification and Data Capture of Paediatric Low-Grade Glioma (pLGG) - Laying the Foundation for Emerging Treatment Options in Australia. Honours Thesis [unpublished]. Monash University; 2025.
14. Helwani F, Ruseckaite R, Ahern S. Recommendations for a National Approach to Rare Disease Data. Rare Voices Australia; 2023. Accessed May 8, 2025. https://rarevoices.org.au/launched-recommendations-for-a-national-approach-to-rare-disease-data/
15. Cancer data in Australia. Australian Institute of Health and Welfare. December 9, 2024. Accessed February 25, 2025. https://www.aihw.gov.au/reports/cancer/cancer-data-in-australia/contents/cancer-incidence-and-survival-by-histology-selecte
16. de Bont JM, Schouten-van Meeteren AYN. Long-term quality of survival after pediatric low-grade glioma. Childs Nerv Syst. 2024;40:3341-3355. doi:10.1007/s00381-024-06631-1
17. Desjardins L, Barrera M, Chung J, et al. Are we friends? Best friend nominations in pediatric brain tumor survivors and associated factors. Support Care Cancer. 2019;27(11):4237-4244. doi:10.1007/s00520-019-04706-3
18. Bates CR, Fairclough D, Noll RB, et al. Psychosocial Functioning of Caregivers of Pediatric Brain Tumor Survivors. Pediatr Blood Cancer. 2022;69(4):e29565. doi:10.1002/pbc.29565
19. Heller N, Melnikov S. Factors affecting caregiver burden among parents of children with cancer: A path analysis. J Clin Nurs. 2025;34(1):128-138. doi:10.1111/jocn.17202
20. Amin SM, Khedr MA, Mansy AMA, El-Monshed AH, Malek MGN, El-Ashry AM. Assessing caregiver stress and resource needs in pediatric cancer care. BMC Nurs. 2024;23:911. doi:10.1186/s12912-024-02483-x
21. Roka K, Meeteren ASV, Scheinemann K, Sehested A. Standard Clinical Practice Recommendations for Low Grade Gliomas. Published online 2022.
22. Gnekow AK, Kandels D, van Tilburg C, et al. SIOP-E-BTG and GPOH Guidelines for Diagnosis and Treatment of Children and Adolescents with Low Grade Glioma. Klin Pädiatr. 2019;231(3):107-135.
23. Roka K, Scheinemann K, Avula S, et al. European standard clinical practice recommendations for primary pediatric low-grade gliomas. EJC Paediatr Oncol. 2024;4. doi:10.1016/j.ejcped.2024.100169
24. Multi-Stakeholder Dialogue: Optimizing the Use of Real-World Evidence for Decision-Making for Pediatric Low-Grade Glioma in Canada. Published online October 27, 2022.
25. Erker C, Vanan MI, Larouche V, et al. Canadian Consensus for Treatment of BRAF V600E Mutated Pediatric and AYA Gliomas. Curr Oncol. 2024;31(7):4022-4029. doi:10.3390/curroncol31070299
26. Bennett J, Erker C, Lafay-Cousin L, et al. Canadian Pediatric Neuro-Oncology Standards of Practice. Front Oncol. 2020;10. doi:10.3389/fonc.2020.593192
27. Fawzy MA, El-Hemaly AI, Awad M, et al. Multidisciplinary Treatment of Pediatric Low-Grade Glioma: Experience of Children Cancer Hospital of Egypt; 2007-2012. Indian J Med Paediatr Oncol. 2018;39:488-492. doi:10.4103/ijmpo.ijmpo_79_17

28. Hessissen L, Parkes J, Amayiri N, et al. SIOP PODC Adapted treatment guidelines for low grade gliomas in low and middle income settings. Pediatr Blood Cancer. 2017;64(55). doi:10.1002/pbc.26737
29. Lim YJ. Medical Treatment of Pediatric Low-Grade Glioma. Brain Tumor Res Treat. 2022;10(4):221-225. doi:10.14791/btrt.2022.0039
30. Lasso-Palomino RE, Gómez IE, Soto-Aparicio MJ, et al. Postoperative complications after central nervous system tumor resection in pediatric patients admitted to an intensive care unit in Colombia. Front Oncol. 2024;14:1491943. doi:10.3389/fonc.2024.1491943
31. Joh-Carnella N, Bauman G, Yock TI, Zelcer S, Youkhanna S, Cacciotti C. Case report: Pediatric low-grade gliomas: a fine balance between treatment options, timing of therapy, symptom management and quality of life. Front Oncol. 2024;14. doi:10.3389/fonc.2024.1366251
32. Lassaletta A, Zapotocky M, Bouffet E. Chemotherapy in pediatric low-grade gliomas (PLGG). Childs Nerv Syst. 2024;40:3229-3239. doi:10.1007/s00381-024-06458-w
33. Bansal I, Merchant TE. Radiotherapy for pediatric low-grade glioma. Childs Nerv Syst. 2024;40(10):3277-3290. doi:10.1007/s00381-024-06460-2
34. Ziegler DS, Lehmann R, Eisenstat DD. A paradigm shift in how we treat pediatric low-grade glioma—Targeting the molecular drivers. Neuro-Oncol. 2024;26(4):593-595. doi:10.1093/neuonc/noae008
35. O'Hare P, Cooney T, de Blank P, et al. Resistance, rebound, and recurrence regrowth patterns in pediatric low-grade glioma treated by MAPK inhibition: A modified Delphi approach to build international consensus-based definitions—International Pediatric Low-Grade Glioma Coalition. Neuro-Oncol. 2024;26(8):1357-1366. doi:10.1093/neuonc/noae074
36. Dhillon S. Tovorafenib: First Approval. Drugs. 2024;84(8):985-993. doi:10.1007/s40265-024-02069-6
37. Dabrafenib and Trametinib. The Pharmaceutical Benefits Scheme (PBS). April 1, 2025. Accessed April 11, 2025. https://www.pbs.gov.au:443/medicinestatus/document/1137.html
38. Trametinib and Dabrafenib. Therapeutic Goods Administration (TGA). June 21, 2022. Accessed February 28, 2025. https://www.tga.gov.au/resources/auspar/auspar-trametinib-and-dabrafenib
39. Compare: Comparison of Access and Reimbursement Environments. Published online 2019. https://www.medicinesaustralia.com.au/wp-content/uploads/sites/65/2020/11/0007_Med-Aus_Compare_5_Booklet_FA_HR.pdf
40. National Strategic Action Plan for Rare Diseases. Published online 2020. https://www.health.gov.au/resources/publications/national-strategic-action-plan-for-rare-diseases?language=en
41. Counting The Cost: How we can assess the true value of investing in cancer treatment. Published online 2023. Accessed October 9, 2025. https://www.canteen.org.au/about-us/canteen-reports/counting-the-cost-report
42. Picone D, Casey D, Single A, et al. Accelerating Access to the Best Medicines for Australians Now and into the Future
43. Stafinski T, Glennie J, Young A, Menon D. HTA decision-making for drugs for rare diseases: comparison of processes across countries. Orphanet J Rare Dis. 2022;17(1):258. doi:10.1186/s13023-022-02397-4
44. Kids Health Info : Education support for students with health conditions. The Royal Children's Hospital Melbourne. July 2020. Accessed October 9, 2025. https://www.rch.org.au/kidsinfo/fact_sheets/Health_conditions_chronic_illness_and_school/
45. Yalingbu Yirramboi – The Royal Children's Hospital School : Yalingbu Yirramboi – The Royal Children's Hospital School. Accessed October 9, 2025. https://www.rch.org.au/Yalingbu-Yirramboi/
46. Parents. Monash Children's Hospital School. Accessed October 10, 2025. https://monashchildrenshospitalschool.vic.edu.au/parents/
47. Cassidy M, Doucet S, Luke A, Goudreau A, MacNeill L. Improving the transition from paediatric to adult healthcare: a scoping review on the recommendations of young adults with lived experience. BMJ Open. 2022;12(12):e051314. doi:10.1136/bmjopen-2021-051314
48. Bray EA, Salamonson Y, Everett B, George A, Chapman IA, Ramjan L. Transitioning between paediatric and adult healthcare services: a qualitative study of the experiences of young people with spinal cord injuries and parents/caregivers. BMJ Open. 2022;12(11):e065718. doi:10.1136/bmjopen-2022-065718
49. Sandquist M, Davenport T, Monaco J, Lyon ME. The Transition to Adulthood for Youth Living with Rare Diseases. Children. 2022;9(5):710. doi:10.3390/children9050710
50. Ebel F, Greuter L, Guzman R, Soleman J. Transitional Care in Pediatric Brain Tumor Patients: A Systematic Literature Review. Children. 2022;9(4):501. doi:10.3390/children9040501
51. Padley N, Moubayed D, Lanteigne A, et al. Transition from Paediatric to adult health services: Aspirations and practices of human flourishing. Int J Qual Stud Health Well-Being. 2023;18(1). doi:10.1080/17482631.2023.2278904
52. Ebel F, Greuter L, Guzman R, Soleman J. Transitional Care in Pediatric Brain Tumor Patients: A Systematic Literature Review. Children. 2022;9(4):501. doi:10.3390/children9040501
53. Scognamiglio D, Boarini M, la Forgia MC, et al. Defining priorities in the transition from paediatric to adult healthcare for rare bone disease patients: a dialogic approach. Eur J Med Genet. 2024;67:104891. doi:10.1016/j.ejmg.2023.104891

Appendix

To review full report and methodology, interview discussion guide, reference list. The full document is housed **here**.