

Treatment Approaches for Von Hippel-Lindau Syndrome



**NeuroEndocrine
Cancer Australia**

Being diagnosed with Von Hippel-Lindau (VHL) syndrome can be a confusing and overwhelming time for you and your loved ones. Even when you have been living with VHL for some time, appointments, scans and decisions about surveillance or treatment can feel stressful.

Each person with VHL syndrome is different, so your surveillance and treatment plan will be individualised. Treatment may be recommended depending on the type, size and location of a tumour or cyst, how quickly it is changing, whether it is causing symptoms, and the potential impact on surrounding organs.

Not every tumour or cyst needs treatment straight away. Some can be safely monitored with regular scans, examinations and check-ups. When treatment is needed, your specialist team will consider the potential benefits and risks of each option and discuss the most appropriate approach with you.

Multidisciplinary Teams

VHL syndrome can affect different parts of the body, so your care may involve a range of Medical Specialists with different areas of expertise. You may need regular surveillance, investigations and, if required, treatment for different VHL-associated tumours or other manifestations.

A multidisciplinary team (MDT) brings together healthcare professionals from different specialties to discuss your care and work together on an individualised plan. This may include specialists in areas such as genetics, endocrinology, neurology, neurosurgery, ophthalmology, kidney care, urology, ear, nose and throat (ENT), oncology and nuclear medicine.

Working as a team helps your healthcare professionals consider how different aspects of VHL may affect each other and coordinate your surveillance and treatment.

Your healthcare team may also include your GP, nurses, allied health professionals and other specialists depending on your individual needs.

You may not need to see every member of an MDT. The healthcare professionals involved in your care will depend on your VHL-associated conditions, surveillance needs and treatment options.

Members of the team may include:

- Endocrinologist
- Neurologist
- Neurosurgeon
- Nephrologist
- Urologist
- ENT Specialist
- Genetic Specialist
- Medical Oncologist
- Radiation Oncologist
- Nuclear Medicine Physician
- Interventional Radiologist
- Ophthalmologist
- Genetic Counsellor
- Pancreatic Surgeon

Active Surveillance (Watch and Wait)

Not all VHL associated cysts or tumours need treatment straight away. Some can be safely monitored with surveillance - regular scans, examinations and check-ups.

Active surveillance allows your healthcare team to monitor changes over time and identify when treatment may be needed. This approach can help avoid or delay treatment and reduce the risk of long-term complications.



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Surgery

Surgery may be recommended to remove a tumour or cyst if it is:

- causing symptoms
- growing
- at risk of causing damage to an important organ.

The decision to proceed with surgery depends on several factors, including the type, size and location of the tumour or cyst, your symptoms, your overall health and the potential benefits and risks of surgery.

Surgery may be used to treat selected VHL associated tumours involving the:

- **Brain or spinal cord** – haemangioblastomas
- **Kidneys** – renal cell carcinoma (RCC)
- **Adrenal glands** – pheochromocytomas
- **Paraganglia** – paragangliomas
- **Pancreas** – pancreatic neuroendocrine tumours (pNETs)
- **Inner ear** – endolymphatic sac tumours

When surgery is recommended, it will be planned carefully to preserve as much healthy organ tissue and function as possible.

Ablation Techniques

Ablation is a minimally invasive treatment that destroys tumour tissue without the need for extensive surgery. The type of ablation used depends on the location, size and characteristics of the tumour.

Cryoablation uses extreme cold to freeze and destroy tumour tissue. It may be used for selected VHL associated kidney tumours and can help preserve healthy kidney tissue.

Radiofrequency ablation (RFA) uses heat energy to damage and destroy tumour tissue. It may be considered for selected kidney tumours and, in some circumstances, other tumours.

Radiotherapy

Radiotherapy uses carefully targeted radiation to damage and destroy tumour cells while aiming to limit the effect on surrounding healthy tissue.

It may be considered for certain VHL-associated tumours when surgery or other treatment options are not suitable or when additional local treatment is needed.

Depending on the individual circumstances, radiotherapy may be considered for selected haemangioblastomas in the brain or spinal cord and, in some circumstances, retinal haemangioblastomas.

Your specialist team can discuss whether radiotherapy is appropriate for your particular situation.

Laser Therapy and Cryotherapy

For retinal haemangioblastomas, local treatments may be used to control tumour growth and reduce the risk of complications that could affect vision.

Laser therapy uses focused light energy to treat the tumour and the blood vessels supplying it.

Cryotherapy uses extreme cold to destroy tumour tissue. It may be considered when laser treatment is not suitable, depending on the size, location and characteristics of the retinal haemangioblastoma.

Your ophthalmologist will recommend the most appropriate treatment based on the tumour and its effect on your vision.

Targeted Therapy

Belzutifan is an oral targeted treatment known as a HIF-2α inhibitor. It blocks the activity of HIF-2α, a protein involved in processes that can contribute to the growth of VHL-associated tumours.

Belzutifan may reduce the size of some VHL-associated tumours and may help delay the need for surgery or other local treatment in some people.



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It may be used for selected VHL-associated tumours, including tumours affecting the kidneys, pancreas and central nervous system, depending on the individual circumstances.

Whether Belzutifan is appropriate depends on the type and location of your tumours, previous and planned treatments, your individual circumstances and the potential benefits and risks. Your specialist team can discuss whether this treatment is suitable for you.

Other targeted or systemic treatments may be considered in specific circumstances, particularly when tumours are more advanced, difficult to treat or when other treatment options are not suitable. Treatment recommendations may change as new evidence becomes available.

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Potential treatments by tumour location

Brain and spinal cord haemangioblastomas: treatment may be recommended when tumours are causing symptoms or are likely to cause complications. Surgery is commonly used for symptomatic or larger tumours.

Pancreatic neuroendocrine tumours (pNETs): smaller tumours may be monitored, while treatment may be considered when tumours are larger, growing or have other features that increase concern.

Kidney tumours: regular monitoring is important, with treatment planned when the size, growth or other features indicate that intervention is appropriate. Kidney-sparing approaches, including surgery or ablation, may be considered to preserve kidney function.

Retinal haemangioblastomas: laser treatment or cryotherapy may be used to control tumours and help protect vision. Some tumours may be monitored depending on their location and effect on vision.

Phaeochromocytomas and paragangliomas: treatment depends on the location, size, hormone production and other characteristics of the tumour. Surgery may be recommended for selected tumours, with appropriate medical preparation where required.

Endolymphatic sac tumours: treatment depends on the tumour and its effect on hearing, balance and surrounding structures. Surgery may be considered to prevent or manage complications.

NECA gratefully acknowledges sponsorship from MSD Australia for our VHL resources.

If you need further support or information about VHL or neuroendocrine cancer, contact the **NET nurses at NeuroEndocrine Cancer Australia on 1300 287 363** Monday-Friday 9am-5pm



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