

MSD leads PBS Christmas gift list

By Megan Brodie

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MSD has secured an important new listing on the PBS for patients with von Hippel-Lindau (VHL) disease, a rare genetic condition for which there were no treatments listed on the PBS, the company securing a cost utility listing while Bayer achieved listing for the first therapy for endometriosis in 30 years and Takeda secured an important expansion for patients.

WELIREG (belzutifan) listed on the PBS yesterday (Sunday) in what is nothing short of an extraordinary Christmas gift for patients and a demonstration of the PBAC working with a sponsor to manage uncertainty, and listening to the people with the most to benefit.

While the patient access gap (PAG) between when Welireg was registered by the TGA and listed on the PBS is 693 days, the delay was due to MSD gathering sufficient evidence to help PBAC deal with the high level of uncertainty, the PBAC consequently playing its part in recommending Welireg at first appearance, even offering MSD a Pathway A facilitated pricing track.

While at five months the pathway proved no faster than the standard pricing track (as previously noted by BioMarin Managing Director Kathryn Evans), the listing is a demonstration of how stakeholders can successfully work together.

"Words cannot express how fabulous this is for the VHL Community – there is now HOPE for so many generations and this is no longer just a dream we wanted to come true!" said Meredith Cummins, CEO of patient group Neuroendocrine Cancer Australia (NECA), which campaigned heavily for the listing through a parliamentary petition in April and parliamentary event in July.

"The VHL community and so many others have done so much to make this happen. We feel so privileged to have worked alongside the patients and their families to lobby and advocate for Welireg to be available on the PBS."

PRICING PATHWAY A SUBMISSIONS					
Product	Indication	Sponsor	Meeting	PBS listed	GAP
Welireg	Von Hippel-Lindau (VHL) disease	MSD	Jul-24	Dec-24	5mths
Humira	vision-threatening non-infectious uveitis	AbbVie	Mar-24	Aug-24	5 mths
Koselugo	symptomatic, inoperable plexiform neurofibroma	Alexion	Mar-24	Aug-24	5 mths
Onpatro	hereditary transthyretin-mediated amyloidosis	Alnylam	Dec-23	Aug-24	8 mths
Revolade	severe aplastic anaemia (sAA)	HSANZ	Nov-23	May-24	6 mths
Vyndamax	ATTR cardiomyopathy	Pfizer	Jul-23	May-24	10 mths
Kimmtrak	Uveal melanoma	Synevi	Sep-23	Feb-24	5 mths
Lynparza	Epithelial ovarian, fallopian tube or primary peritoneal cancer	AZ	Jul-23	Jan-24	6 mths
Keytruda	Triple negative breast cancer (early stage)	MSD	Jul-23	Dec-23	5 mths
Enhertu	HER2+ metastatic breast cancer (3L)	AZ	Jul-23	Nov-23	4 mths
Enhertu	HER2+ metastatic breast cancer	AZ	Mar-23	Nov-23	8 mths
Keytruda	Triple negative breast cancer	MSD	Mar-23	Sep-23	6 mths
Keytruda	Cervical cancer	MSD	Nov-22	Oct-23	11 mths
Voxzogo	Achondroplasia	BioMarin	Nov-22	May-23	6 mths
Nucala	Chronic rhinosinusitis with nasal polyps	GSK	Nov-22	Apr-23	5 mths
Jakavi	Chronic Graft vs Host Disease	Novartis	Nov-22	Nov-22	5 mths
Onureg	Acute myeloid leukemia	BMS	Sep-22	Sep-23	12 mths
Tecentriq	NSCLC adjuvant	Roche	Jul-22	May-23	4 mths
Kuvan	Phenylketonuria (adults)	BioMarin	Jul-22	Apr-23	9 mths
Keytruda+Lenvima	Endometrial cancer	MSD/Eisai	Mar-22	Jun-23	14 mths
Opdivo	Gastro-oesophageal cancers	BMS	Mar-22	Oct-22	7 mths
Spinraza	Spinal Muscular Atrophy	Biogen	Jul-21	Sep-22	14 mths
Trikafta	Cystic fibrosis	Vertex	Jul-21	Apr-22	9 mths
Braftovi	metastatic colorectal cancer	Pierre Fabre	May-21	Jan-22	8 mths
Adcetris	Peripheral T-cell lymphoma	Takeda	Mar-21	Sep-21	6 mths
Opdivo+Yervoy	malignant pleural mesothelioma	BMS	Mar-21	Jul-21	4 mths

Source: MedNews©

“The reality of this being a PBS medication has so many benefits not only for the patients and their families to have control of tumours and shrink tumours. It also has a huge cost saving benefit to the health system.

“VHL patients can now plan for their futures, be active members of the community, in the workforce and able to have a much better quality of life – a life they deserve. We are just so relieved and happy!”

The listing has a net cost to the government of more than \$20 million a year, so required Cabinet approval, but is now available for the 500 eligible patients with VHL suffering associated conditions. On average, VHL typically manifests around the age of 26, and nearly 97 per cent of individuals carrying a VHL gene mutation will experience symptoms by the time they reach 65. It is passed on to children, meaning generations of families suffer. Welireg is the first and only treatment for the condition.



NECA led the charge for the PBS listing of Welireg, including CEO Meredith Cummins (centre).