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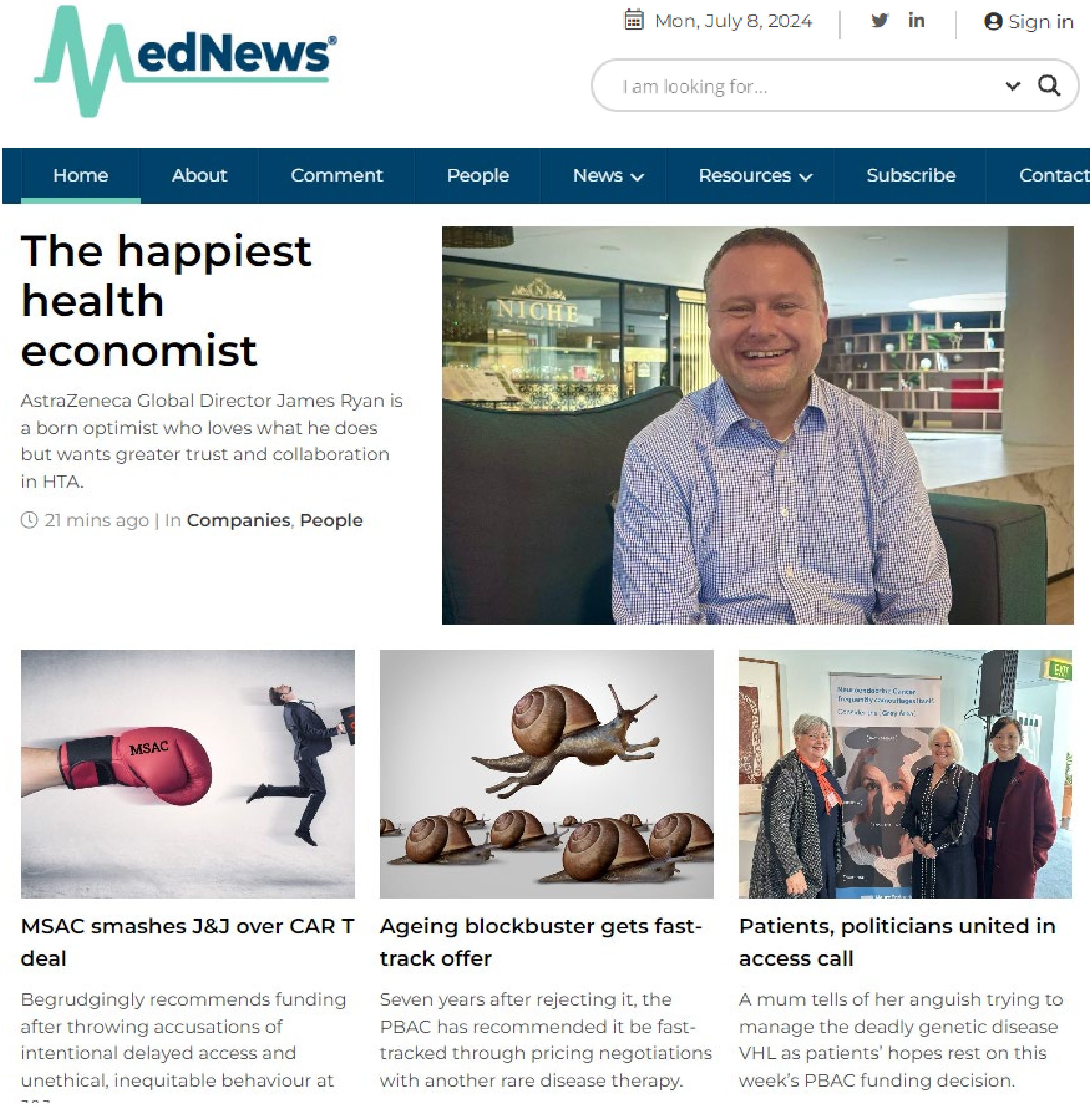

The happiest health economist

AstraZeneca Global Director James Ryan is a born optimist who loves what he does but wants greater trust and collaboration in HTA.

21 mins ago | In **Companies**, **People**

MSAC smashes J&J over CAR T deal

Begrudgingly recommends funding after throwing accusations of intentional delayed access and unethical, inequitable behaviour at

Ageing blockbuster gets fast-track offer

Seven years after rejecting it, the PBAC has recommended it be fast-tracked through pricing negotiations with another rare disease therapy.

Patients, politicians united in access call

A mum tells of her anguish trying to manage the deadly genetic disease VHL as patients' hopes rest on this week's PBAC funding decision.

Patients, politicians united in access call

By Megan Brodie

3 hours ago | In **Patients**, **Reimbursement**

8 July 2024

Patients and politicians gathered at Parliament House in Canberra last Thursday in an urgent call for the Federal Government to fund a treatment for patients with Von Hippel Lindau (VHL).

MSD's WELIREG (belzutifan) is the first treatment approved for the rare genetic condition and, while the PBAC will consider a funding submission from MSD when it meets this week, patients are concerned about the delay in access.

NeuroEndocrine Cancer Australia (NECA) held an inaugural event in Parliament House hosted by Teal MP Zali Steggall that attracted around 60 politicians, patients, clinicians and advocates united in their support for the listing of Welireg, including Lana Hallowes who has battled VHL since she was 11.

Now aged 43, Hallowes is legally blind due to retinal tumours, almost deaf due to other tumours, has had four brain surgeries and last year learned to walk again for the fifth time after a tumour was removed from her spinal cord, now using a walking stick.

"VHL is a debilitating and devastating disease," says Hallowes.

"It is a troll which attacks me again and again – there is never really a break because even when I don't have imminent surgery on the horizon, I am dealing with deficits from previous ones and the underlying anxiety that comes with having an uncertain future."

Hallowes said Welireg offered her hope she would not lose the resto of her sight and hearing, would avoid further surgeries, and could finally be optimistic about a future where her children no longer worried about their mum getting sick or dying.

"Right now, I have a pancreatic neuroendocrine tumour which will need surgery next if I can't get belzutifan to shrink it," she said.

Jamie Snashall, Senator Wendy Askew, Meredith Cummins, Lana Hallowes, Zali Steggall and Liz de Somer at the VHL event at APH.

"This scares me as it's another major surgery which could see me become diabetic. I also have more brain, spinal and retina lesions. If I don't get belzutifan, the only option is to go under the knife – again and again. There is a build-up of medical and mental trauma which is hard to live with.

> ***"Belzutifan can't give me back what I've lost, but it can stop me losing more and, most importantly, give the next generation of VHL patients a brighter and better life. That's really who this medication is for."***

More than 17,800 people signed a petition to Health Minister Mark Butler supporting the listing while it is understood more than 500 people wrote to the PBAC through the consumer portal. NECA CEO Meredith Cummins says the Senate Inquiry into rare and less common cancers also recognised the need for specialised neuroendocrine cancer care.

"VHL patients do not have equitable or affordable access to medications such as belzutifan to control their tumours," she told *MedNews*. "Without this medication, their lives are plagued with ongoing surgery, reduced quality of life, reduced hope, and the opportunity to have a life with their family. Time is running out for many."

Zali Steggall said patients "should not have to choose between available treatment or suffering without care because that treatment is completely cost prohibitive".

"I urge the government to act on the recommendations from the recent Inquiry into Rare Cancers, including Neuroendocrine Cancer, and list belzutifan on the PBS," she said.

VHL is marked by the development of recurrent tumours in various parts of the body including the central nervous system, kidneys, pancreas, eyes, adrenal glands and inner ears.

Many patients have parents, siblings and children impacted by the same condition, Cummins saying the cost of treating VHL patients was negligible in comparison to health costs borne elsewhere.

She says better genomic screening is also needed, while people with VHL are not covered by Medicare for reproductive costs such as IVF and embryo selection.

Patients and clinicians shared their stories at the APH event.

Welireg is among 10 first-time listing submissions being considered by the PBAC when it meets this week, the cycle meeting to be chaired by consumer representative and Deputy Chair Jo Watson.

Other therapies up for consideration and targeting rare conditions include Ipsen's BILVAY (odevixibat) for progressive familial intrahepatic cholestasis and UCB's ZILBRYSQ (zilucoplan) for generalised myasthenia gravis.