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THIRD IN A MEDICAL OBSERVER THREE-PART SERIES

COSTING LIVES

the price of orphan drugs



Are we generous enough in the way we assess and fund orphan drugs for rare diseases? *Byron Kaye* reports.

SARAH Walker needs her own bedroom. For her sister's sake, not hers. See, Sarah, aged three, was born with pulmonary interstitial glycogenosis, a rare lung disease, and needs a noisy BiPAP breathing regulator to sleep.

The only problem is, the huge cost of giving Sarah some chance at a normal life has driven her parents deep into debt. For now the room, and her sister's sleep, will have to wait.

"The cost of living in hospital while trying to raise the rest of my family at home – petrol, parking, food, specialist appointments – it just all adds up," Sarah's mum Susanna tells *MO*.

"We're still in the red now, we still live week-to-week, we haven't been able to save money."

It's not just those calculable hand-in-pocket items. The true cost of raising a child with a rare disease – which means it affects fewer than 2000 Australians – is insidious.

Aside from logistics and medications and doctor appointments, there are opportunity costs. Most often at least one parent of an afflicted child is forced to give up work and become a full-time carer, unpaid.

"Most [rare diseases] are chronic diseases and therefore they're very expensive," says Professor Elizabeth Elliott, who is founder of the Australian Paediatric Surveillance Unit and professor of paediatrics and child health at the University of Sydney, and also a paediatrician at the Children's Hospital at Westmead.

"If you have a child with a

rare congenital syndrome, they might have heart problems and bone problems and kidney problems and liver problems and brain problems, so they need to see all of those different specialists. It's one syndrome with five different specialists involved. And you need multiple visits because it's a chronic problem."

Less palatable an issue, adds Professor Elliott, who is currently conducting Australia's first "audit" of which resources are going into rare disease research and funding, is the fact that affected children are living longer thanks to advances in treatment. Good for loving parents, not so good for bank accounts – and there's a flow-on effect to the wider health budget.

"We've got this increasing, accumulative burden on the health system," she says.

Professor Elliott complains that while rare diseases will affect

up to 8% of Australians, directly or otherwise, there is no aggregated data on how much is spent researching them or subsidising their treatment. Nor is there data quantifying Australia's international standing when it comes to funding orphan drugs.

Australia is, statistically speaking, among the least willing to categorise a disease as 'rare' and thus the least likely to grant the waivers and bonuses typically accorded orphan drugs. While the Pharmaceutical Benefits Advisory Committee's (PBAC) definition of 'rare disease' is one that affects 2000 people, or about one in 10,000 Australians, the US definition is one that affects 200,000 people or one in 1500 Americans. In Europe it is five in 10,000 or one per 2000.

How does the PBAC decide which orphan drugs to recommend for PBS listing? It's unclear. The body declined an interview request but a spokesperson said in an email it "considers applications from companies for PBS listing having regard to the clinical effectiveness and cost effectiveness [value-for-money] of medicines in comparison with other available treatments".

Much the same as for any other listing application, in other words. What's different is rare treatments can be 'orphan designated' from the outset, meaning they are exempt from the usual fees to get on the Australian Register of Therapeutic Goods (ARTG) and, from there, the PBS. Orphan drugs also receive 'priority evaluation'.

The PBAC email added, without elaboration, that to achieve orphan designation a product

"must not be commercially viable to supply to treat, prevent or diagnose another disease or condition". Registration must be submitted by an Australian entity.

According to the TGA website, there are currently 196 designated orphan drugs compared with 81 in 2004. The PBAC did not give numbers of orphan drugs on the ARTG and PBS, although historically about a quarter of orphan-designated drugs are PBS-listed. This works out to roughly 50.

There is a total of 7000 rare disorders, including those that are rare complications of common disorders, according to Professor Elliott.

So, is this enough?

Perhaps not, says John Christodoulou, professor of paediatrics, child health and genetic medicine at Sydney Medical School. "We need more orphan drugs to be listed on the PBS as this will ensure equity of access at a national level," he says.

But he acknowledges that, while drugs that save lives should be subsidised, deciding how to proceed with treatments that offer more complex benefits may be a trickier proposition – financially, anyway.

"Where a drug is clearly revolutionary and is life-saving, [subsidising] it should be a no-brainer," he says. "But whether it ends up being listed through the PBS or administered through another scheme will depend on the cost of the treatment and the number of people who would be likely to benefit from the drug.

"The waters become even murkier where the therapeutic agent has the potential to improve the quality of life – a lesser but

nonetheless very important achievement – because this is difficult to quantify from a health economics perspective."

It's a conundrum shared by the pharmaceutical industry – different though its motives may be. That is, while the default setting of big medicine would seem to be arguing for more subsidies to boost sales, the relative demand for any single orphan drug in Australia is so low that it is not deemed worth campaigning for. It is best left up to the PBAC.

"There's not a lot of money in it," says Rob Koster, sales and marketing manager at Aspen Pharmacare, parent company of rare drug supplier Orphan Australia.

"The reality is that it's usually the patients and the doctors that are driving the availability for patient subsidies or [for products] to be covered on the PBS.

"The Government is being put in a position where they have to say to you: 'This drug will give you five more months of life, but it's going to cost us \$250,000 a year and yet we don't have enough money to provide mammograms for all the women.' Realistically, the Government's in a hard place."

A 2005 submission by pre-Aspen-owned Orphan Australia to a House of Representatives standing committee on health and ageing suggests a less patient outlook.

"As a class, orphan drugs have a high failure rate in PBAC applications," the submission said. It suggested creating "specific PBAC-effectiveness criteria or dispensations for orphan drugs" – presumably on top of the fee


waiver and registration fast-tracking. One Orphan Australia product, Agrylin, which is subsidised in the US as treatment for certain thrombocythaemia disorders, remains PBAC-rejected.

A 2006 pharma-funded report (by IMS Consulting) comparing US and Australian drug subsidies found that if the "centralised, mandatory" approach of the PBAC were implemented in the US, thousands of patients using orphan drugs would be unable to afford their medications.

South Australian GP Dr Peter R. Mansfield, director of advocate Healthy Skepticism, says while his organisation did not have a formal position, patent monopoly protection "should be abolished and the savings used to fund drug research and medical education etc via competitive grants".

He adds: "This would include massive increased funding for the NHMRC and similar bodies in other countries. They would fund grant winners to develop new drugs and devices that any manufacturer capable of achieving adequate quality could sell. Price competition would keep the prices down."

He also believes it would be better to subsidise the patients rather than the drugs so families could decide which treatments they wanted.

Meanwhile, Susanna Walker makes do on a \$110-a-fortnight carer allowance instead of pursuing her career as an environmental chemist. But she believes she's one of the lucky ones. "Our situation is bad," she says, "but there must be people out there whose situations are so much worse." 

"Where a drug is clearly revolutionary and is life-saving, [subsidising] it should be a no-brainer" Professor John Christodoulou