SEPT 22, 2022 'WORLD NARCOLEPSY DAY' CAMPAIGN

NARCOLEPSY + SODIUM OXYBATE

Response to **Bedtime Reading**: **Inquiry into Sleep Health Awareness in Australia,** by the *House of Representatives Standing Committee on Health, Aged Care and Sport* and the **National Strategic Action Plan for Rare Diseases** by the *Australian Government Department of Health*.

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FOR NARCOLEPSY SUPPORT AUSTRALIA, SEPTEMBER 2022

INTRODUCTION

Narcolepsy is a rare, lifelong and disabling neurological and central nervous system disorder having devastating physical, emotional, social and financial impacts on the health and wellbeing of an estimated 6 000 -10 000 Australians and their families.

Living with Narcolepsy requires chronic care, creating a substantial medical and economic burden. This comes at a significant cost to both individuals and the wider community. Inadequate treatment options, insufficient access to support and a lack of national research, all exacerbate the problem. The detrimental effects on professional and personal development often result in mental health issues such as depression and anxiety.

> If not properly diagnosed and treated, narcolepsy may have a devastating impact on the life of the affected individual, causing social, educational, psychological, and financial difficulties.
>
> Brain Foundation, 2022

The Parliamentary Inquiry into Sleep Health Awareness in Australia's subsequent report 'Bedtime Reading - Inquiry into Sleep Health Awareness in Australia' was published in April 2019. This Inquiry states, "We have known the importance of sleep for decades yet for many reasons, sleep health has not received the attention it deserves within our community and in the health programs run by state and federal governments."
https://www.aph.gov.au/Parliamentary_Business/Committees/House/Health_Aged_Care_and_Sport/SleepHealthAwareness/Report

The 'National Strategic Action Plan for Rare Diseases', led by *Rare Voices Australia* with funding from the *Australian Government Department of Health,* received bipartisan support for the resulting Action Plan in November 2018. This plan acknowledges areas of significant unmet need and the unacceptably high burden of rare diseases resulting from equity and access issues.
https://www.health.gov.au/sites/default/files/documents/2020/03/national-strategic-action-plan-for-rare-diseases.pdf

Narcolepsy Support Australia, on behalf of people with Narcolepsy (PWN), implore members of the 47th Parliament to act upon recommendations that support the health and quality of life of our members. Given this Government's commitment to health, recently affirmed by the Hon Anthony Albanese MP, we hope to seize this opportunity to remove inequitable access barriers.

> ‘No country can claim to have achieved universal healthcare if it has not adequately and equitably met the needs of those with rare diseases.’
>
> Helen Clark, United Nations Development Programme
> © Commonwealth of Australia (Department of Health) 2020 p.10

REPORT & RECOMMENDATIONS

'Bedtime Reading' broadly describes the impact and cost of inadequate sleep and sleep disorders on the Australian community and recommends that "the Australian Government prioritise sleep health as a national priority and recognise its importance to health and wellbeing alongside fitness and nutrition."

Narcolepsy Support Australia supports the inquiry across the entire Terms of Reference and acknowledges the need for improvements across the three pillars presented in the National Strategic Action Plan for Rare Diseases; Awareness and Education, Care and Support plus Research and Data. However, in the urgent interests of our members, we would like to immediately focus on Recommendation 7.

Recommendation 7
The Committee recommends that if there is no distributor willing to put forward a submission, the Australian Government work with patient advocacy groups such as Narcolepsy Support Australia and the Sleep Health Foundation to make a submission for the listing or registration of Sodium Oxybate under the Orphan Drug Program.

Recommendation 7 is informed by two key chapters

3. Sleep Disorders which outlines the causes, symptoms and prevalence of sleep disorders; potential links between sleep disorders and other health conditions; and personal experiences of living with these conditions.

5. Diagnosis, Management and Treatment of Sleep Disorders which includes the accessibility and affordability of sleep medicine and the role of primary care and other healthcare workers in the provision of sleep health services.

Why Sodium Oxybate?
International guidelines (from Europe and the USA) recommend the use of sodium oxybate as an important first-line therapeutic option for PWN. The current Australian treatments of psychostimulants and antidepressant agents are often inadequate and fail to regulate sleepiness, cataplexy and/or sleep fragmentation that contribute to common functional limitations and comorbidities faced by many PWN. Given the weight of evidence, the Australasian Sleep Association argues that it should be available to Australian patients as a prescriber permit-controlled Schedule 8 medication.

ACCESS BARRIERS

The National Strategic Action Plan for Rare Diseases addresses three core, interrelated pillars. PWN access to Sodium Oxybate requires change across all three, as identified by the Bedtime Reading report and across the Inquiry submissions.

1 Awareness and Access: Lack of recognition of Narcolepsy symptoms among General Practitioners and Sleep Specialists results in delayed diagnosis and inconsistent treatment that can increase comorbidities. Many PWN feel disempowered and unsupported. The inability to access existing, effective treatments leaves many PWN feeling frustrated and abandoned. Lack of institutional recognition can leave PWN dismissed, overwhelmed and even humiliated.

2 Care and Support: Australia's narcolepsy management is inadequate by international standards and arguably inequitable according to the National Strategic Action Plan for Rare Diseases. Sodium Oxybate was approved as a first-line treatment option for Narcolepsy in the US in 2002. Twenty years later Australian PWN still don't have affordable access. Sodium Oxybate, which is currently only available through the Special Access Scheme, is not subsidised and the annual $15 000 to $22 000 cost is prohibitive.

3 Research and Data: a lack of accurate information on the nature and number of PWN makes it difficult to determine the market viability and/or Orphan drug status eligibility. Narcolepsy rates may be overstated as GPs may be miscoding cases of excessive daytime sleepiness and idiopathic hypersomnia as narcolepsy. This may distort the prevalence of narcolepsy in Pharmaceutical Benefits Scheme (PBS) and Medicare Benefits Schedule (MBS) data.

> People with narcolepsy are frustrated by the standard of healthcare they receive in Australia. With good reason. Although Australia boasts a first-class healthcare system, our approach to narcolepsy management is 'manifestly inadequate'.
>
> The Woolcock Institute of Medical Research

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RESEARCH INSIGHTS

WHAT IS NARCOLEPSY?

- Narcolepsy is a rare disorder with environmental and genetic origins
- Primarily known for its impact on sleep, Narcolepsy evolves into a progressive multisystem disease
- The impact on vital neurological and physiological functions contributes to an array of concerns
- Sleep effects, comorbidities and functional losses vary in severity across individuals

WHAT IS THE CAUSE?

- Type 1 Narcolepsy (NT1) is linked to auto-immune and genetic origins with environmental triggers that result in the loss of Orexinergic neurons in the hypothalamus
- Type 2 Narcolepsy (NT2) does not yet have a clear aetiology and is not accounted for by a loss of Orexin

FIVE BASIC SYMPTOMS

- The 5 main symptoms of narcolepsy may be referred to by the acronym CHESS
- All PWN experience excessive daytime sleepiness, they may not experience all 5 symptoms

Cataplexy: (NT1) Loss of muscle control, triggered by strong emotion, while conscious	Hallucinations: hypnagogic and/or hypnopompic vivid dream events while awake	Excessive daytime sleepiness: unable to stay awake or alert where sleep is irrepressible	Sleep paralysis: disturbing inability to move or speak during sleep-wake transitions	Sleep disruption: frequent waking, resulting in a lack of restorative night time sleep

INSUFFICIENT CARE

- Substantial and widespread gaps exist in understanding narcolepsy symptoms
- Only 22% of Sleep Specialists and 7% of General Practitioners can identify the 5 basic symptoms
- Diagnostic delays result in a mean time of 10-20 years from onset to diagnosis
- Delays and misdiagnoses increase impact, psychosocial effects and the socioeconomic burden
- Treatment of Narcolepsy consists of symptomatic rather than curative management, so limited understanding of the symptom spectrum undermines care for PWN

PHYSICAL IMPACT

- Narcolepsy can have an array of parasympathetic, metabolic, sudomotor, and autonomic effects
- Orexin (aka Hypocretin) is a critical component of sleep circuitry that also impacts gastrointestinal, urinary, cardiovascular, thermoregulatory, energy, motor, psychiatric and pupillomotor subdomains.
- Comorbidities include chronic pain, hypertension, obesity, diabetes, depression, anxiety & ADHD
- Increased accidents at home, while driving and in the workplace and injuries from falls and burns
- Diagnostic delays, limited understanding and insufficient treatments often exacerbate these effects

COGNITIVE IMPACT

- Disturbed sleep patterns with reduced non-REM sleep results in cognitive dysfunction
- Decreased executive control, attention and concentration, and memory encoding are all concerns
- Recently, widespread bilateral white matter abnormalities have been detected in NT1

FUNCTIONAL EFFECTS

- Before and after diagnosis, narcoleptics often experience unrelenting severe psychosocial stress
- It is often extremely incapacitating, interfering with every aspect of life, work, relationships, education, access to health care, income, relationships, social participation and cultural engagement are some of the many affected life domains

SODIUM OXYBATE

- Sodium oxybate is the sodium salt of the CNS depressant γ-hydroxybutyric acid (GHB)
- First approved in the US in 2002 for narcolepsy with cataplexy, it is now also EU approved
- Double-blind, placebo-controlled, multicenter study across the United States, Canada, the Czech Republic, France, Germany, the Netherlands, Switzerland, and the United Kingdom found Sodium Oxybate to be well tolerated and highly effective in reducing the frequency of cataplexy, improving sleep architecture and reducing EDS in patients with narcolepsy
- Systematic Review and Meta-Analysis Narcolepsy found that PWN using Sodium Oxybate had significant reductions in cataplexy and daytime sleepiness as well as improved quality of life