

Attention: Sandra Titus  
FDA Center for Drug Evaluation and Research (HFD-1)  
Peripheral and Central Nervous System Advisory Committee  
5600 Fishers Lane  
Rockville MD 20857  
NDA 21-196, Xyrem (sodium oxybate, Orphan Medical Inc.)

Presenter: Brian A. Hunter  
Young Adults With Narcolepsy – YAWN

Dear Sandra:

I am requesting time to make a formal oral presentation on my views regarding the NDA 21-196 application being held before the Peripheral and Central Nervous System Advisory Committee meeting on June 6, 2001. I will require approximately four (4) minutes to present my views for consideration by the committee concerning risk management issues pertaining to the safety and efficacy of Xyrem (sodium oxybate, Orphan Medical Inc.) I have attached my intended comments for your review.

Young Adults With Narcolepsy, YAWN is an online organization working to support, advocate, and advance public awareness of narcolepsy, on behalf of young adults their families, peers, coworkers, employers, teachers, and others whose lives are affect by this often debilitating sleep disorder. By working at the grassroots level, YAWN is able to make an immediate impact on the lives of our younger generation by coordinating local support groups, involving other non-profit services in providing access to rehabilitative services and social service agencies and by educating teaching professionals from junior high school counselors to University professors.

Thank you for your kind consideration of this request.

Sincerely,

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I feel it is important to preface my comments today by disclosing that my organization, Young Adults with Narcolepsy (YAWN) has received a \$5000 grant from Orphan Medical to underwrite the developmental expenses for our website and have provided a scholarship for my travel and accommodations to attend this meeting.

As founder of YAWN, the first online youth-focused patient support and advocacy organization, and a person with narcolepsy and cataplexy, I believe that I am in a unique position to comment on the issue currently under consideration by this committee. I do not and have not used Xyrem for treatment of my cataplexy, but as the representative of a large number of young adults with narcolepsy who have, or would like to have, participated in clinical trials for Xyrem, I am compelled to present my views on risk management issues pertaining to the safety and efficacy of Xyrem (sodium oxybate, Orphan Medical Inc.).

YAWN works to support, advocate, and advance public awareness of narcolepsy on behalf of young adults, their families, employers, teachers, and others whose lives are affected by this often-debilitating sleep disorder. By working at the grassroots level, YAWN is able to make an immediate impact on the lives of these young adults by coordinating local support groups and involving other nonprofit services in providing access to rehabilitative services and social service agencies.

Narcolepsy is most commonly diagnosed by the middle of the third decade of life often 5-15 years after the onset of symptoms, the most dramatic of which is cataplexy. Excessive daytime sleepiness combined with the impact of sudden attacks of cataplexy that may last from a few seconds to hours can be profoundly damaging to the social, interpersonal, and educational development of these young adults at a critical point in their development. It has been well documented that the cataplexy and excessive sleepiness of narcolepsy has multiple effects on these individuals and their families. This disease has a significant negative impact on education, interpersonal relationships, gainful employment, motivation, and marital life. I submit that the risk for experiencing the negative impact of untreated cataplexy on the potential of young adults with narcolepsy is a serious issue that must be included in any discussion of risk management of Xyrem.

Xyrem offers a singularly important therapy for the 65%-70% young adults with narcolepsy who suffer with cataplexy. Other therapies including tricyclic antidepressants such as Prozac are only minimally effective in controlling symptoms of cataplexy in this patient population. Xyrem has been shown to be an effective therapy in limiting the cataplexy episodes that result from sudden surges of emotion, including surprise, anger and happiness.

We must recognize the consequences of failing to approve Xyrem to treat the 1:1000 people suffering with narcolepsy, an incidence equal to multiple sclerosis. For example, three months after founding YAWN, I was contacted by the parents of a sixteen-year-old boy living in a small town three hours away from the nearest city. This young man was bright, did well in school, and was active in his community until his twelfth birthday

when he began experiencing severe episodes of cataplexy that lasted for hours. When I first spoke to him on the phone, he told me that his condition was so severe that he is forced to spend five days a week in a nursing home. What are the costs of providing nursing home care in a public institution for a sixteen-year-old for the next 60-70 years? By not adequately controlling his cataplexy, what are his chances for becoming a contributing member of society?

Unfortunately, this young man's story is all too common. Unless something is done about the current environment of limited access to inadequate pharmaceutical therapies, the future of young adults suffering with cataplexy will remain bleak.

This, however, doesn't have to be the case. In fact, a brighter future has been achieved by the lucky few who have participated in GHB clinical trials. They have become success stories. To these young adults with narcolepsy, GHB has meant the difference between a life within an institution and having the opportunity to achieve their goals free from the physical constraints of their disease, by earning their PhDs, by becoming successful artists, entrepreneurs, lawyers, teachers, doctors, politicians, Olympic athletes, or simply by being good parents.

These and the thousands of other talented and capable young adults who have not yet had a chance to fulfill their dreams are the reason I formed YAWN and why I am here testifying before you today. It is my responsibility to protect their right to pursue a happy and productive life by having access to medications that will effectively treat their disease. We can no longer afford to neglect the potential of so many young adults by failing to provide them with the only medication known to be safe and effective.

Thank you for allowing me to present these remarks to you today. I urge you to approve the NDA 21-196 for Xyrem. There are lives at stake.