

An Interview with Orphan Medical about Xyrem®

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Waiting for Xyrem®

Persons with narcolepsy, especially those who experience cataplexy, are anxiously awaiting the decision by the Food and Drug Administration (FDA) on the approval of Xyrem® as a treatment for the symptoms of narcolepsy. A decision is expected later this spring, as the Xyrem® New Drug Application has been given Priority Review Status.

As a result, there are a number of questions being raised by those most interested in Xyrem®. Naturally, many of these have come up in our regularly scheduled Narcolepsy Chats, so TalkAboutSleep contacted Orphan Medical, the company developing Xyrem, to get some answers. Several individuals from the Orphan Medical corporate staff were kind enough to respond, including CEO John Bullion, Chief Operating Officer William Houghton M.D., and Vice President of Marketing Patti Engel.

The interview covered a number of topics, including availability, cost, medical education, and "how it works". We hope you enjoy reading this interview and find the information helpful to you.

Availability of Xyrem®

TalkAboutSleep: Can you summarize the current status of Xyrem and the NDA? When do you expect the FDA to complete its review? With FDA approval, how long will it be before Xyrem will be available for doctors to prescribe?

Orphan Medical, John Bullion, CEO: Orphan Medical filed the New Drug Application (NDA) on October 2, 2000, and the application was granted priority review status on October 10, 2000. There will also be an advisory committee meeting on March 15, to discuss Xyrem. Based on the priority status, the FDA is expected to provide a decision regarding the NDA by April 2 of this year.

The Company expects that Xyrem will be available for patients within a few weeks following the approval with a full launch campaign and education materials for physicians coming a few months after the approval.

TalkAboutSleep: Will Xyrem be available in pharmacies? Can you describe how the distribution of Xyrem will occur? Is this part of the FDA decision process? Or are there other regulatory groups involved in this process?

Orphan Medical, Patti Engel, Vice President: Xyrem will be available through a specialty distribution system that will utilize a central pharmacy that will handle the delivery of medicine to the patients.

To order Xyrem, a physician will write a prescription and fax that to the central pharmacy. That pharmacy will process the prescription request, call the physician to verify the prescription, call the patient to assist them in gaining coverage from their insurance company, and then set up a delivery time directly to the patient so that they may receive their medicine.

This system was designed by Orphan Medical with assistance and input from State Scheduling authorities, experts in specialty distribution, drug diversion investigators, field law enforcement, narcolepsy patient groups, and pharmacists experienced in dealing with Scheduled medicines.

TalkAboutSleep: What progress is being made on the state regulations where GHB is still "outlawed"? Will people who reside in states with stricter regulation than the federal rules simply not be able to use Xyrem?

Orphan Medical, Patti Engel, Vice President: The federal law allows for patients to have Xyrem as prescribed by their doctor. As of this writing, many states have adopted this stance: a few are waiting for formal FDA approval. Once approved, patients can call 1-888-8ORPHAN to determine Xyrem's status in their particular state.

Pricing and Insurance

TalkAboutSleep: What is the estimated cost to Narcolepsy patients? Is it likely that the drug will be covered by health insurers? Will Orphan Medical provide assistance to persons with narcolepsy in getting insurance coverage? Will Xyrem be available free or at a reduced cost to low-income individuals without insurance? How will this be done?

Orphan Medical, Patti Engel, Vice President: Because the handling requirements are not yet finalized by the FDA, there is not yet a price established for Xyrem. That will be determined nearer the time of approval. If approved, Xyrem will likely be covered by many health insurance plans and our Reimbursement Services department will be available to assist patients that are working with their plans to obtain coverage.

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For patients who are uninsured or need financial assistance to obtain Xyrem, there will be a patient assistance program administered through the National Organization for Rare Disorders (NORD) and they will determine the eligibility for patients based on their financial status.

How Does Xyrem Work?

TalkAboutSleep: Originally Xyrem was expected to be a drug for the control of cataplexy for people who have Narcolepsy. We know from personal experiences that it can also help alleviate Excessive Daytime Sleepiness (EDS). Does Orphan Medical know exactly how Xyrem alleviates cataplexy? Is it a chemical effect or does it occur purely by improving sleep?

Orphan Medical, William Houghton, MD, COO: The ultimate mechanism of action is not known. Many of the biochemical changes in the brain have been identified in terms of modulation of levels of important chemicals and neurotransmitter functions in the brain. As well, primary effects of improved sleep architecture occur that may contribute to why Xyrem reaches its optimal results after 8 to 12 weeks. It stands to reason that maintained changes in sleep contribute to improve daytime functioning, but further research into the science of narcolepsy is required.

TalkAboutSleep: With the improvement in EDS that many with narcolepsy experience, has Xyrem been tested on those who don't have cataplexy or those with a diagnosis of Idiopathic Hypersomnolence? If so, have these patients also experienced improvement in their EDS?

Orphan Medical, William Houghton, MD, COO: No, we've been entirely focused on narcolepsy and entry into the clinical trials has been designed to include cataplexy as a diagnosis for narcolepsy. We've not investigated Xyrem for the potential use in other sleep disorders.

TalkAboutSleep: Over the years of testing, have any negative side effects of Xyrem been found?

Orphan Medical, William Houghton, MD, COO: The side effects seen in the trials have been generally minor in nature and tend to abate over time and have included headache, nausea, dizziness, enuresis, and sleepwalking.

Medical Education

TalkAboutSleep: Unfortunately, so many of the doctors who treat patients with Narcolepsy around the country do not keep up with current research and are unaware of the success so many with Narcolepsy have had with Xyrem. Will Orphan Medical have information available to doctors? Will there be an education program for those who may be reluctant to prescribe it?

Orphan Medical, Patti Engel, Vice President: Information on Xyrem will be available to physicians through printed material, participation at medical meetings, and through representatives of Orphan Medical that will be able to discuss the details of Xyrem.

There is also a program called the Xyrem Physician Success Program that will ensure that a physician understands the unique nature of Xyrem and how the prescription and distribution process works to provide this medicine to the patients that need it in a responsible manner.

TalkAboutSleep: Thank you, Orphan Medical, for answering these questions about Xyrem® for our audience.



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