



PATIENT-FOCUSED NARCOLEPSY SURVEY

Interim Analysis as of September 16, 2013

For Distribution at September 24, 2013 FDA Meeting on Drug Development for Narcolepsy

On September 24, 2013, patients with narcolepsy, family members and caregivers will gather at the Silver Spring, Maryland, campus of the U.S. Food & Drug Administration (FDA) to provide direct input on drug development issues affecting nearly 200,000 Americans with narcolepsy. The meeting, the fourth in the FDA’s Patient-Focused Drug Development Initiative series mandated under a 2012 law, will be the first session dedicated to a rare disease.

To prepare the patient community for this opportunity to speak directly with FDA regulators, Wake Up Narcolepsy, Inc., a nonprofit working to speed diagnosis of narcolepsy and help in the search for a cure, launched a special patient education and engagement initiative, Unite Narcolepsy. A comprehensive survey based on questions about narcolepsy posed by FDA was initiated on August 26, 2013 and has since generated response from more than 1,350 individuals, including more than 1,000 people diagnosed with narcolepsy by a physician. This is the largest survey ever conducted by a narcolepsy patient organization.

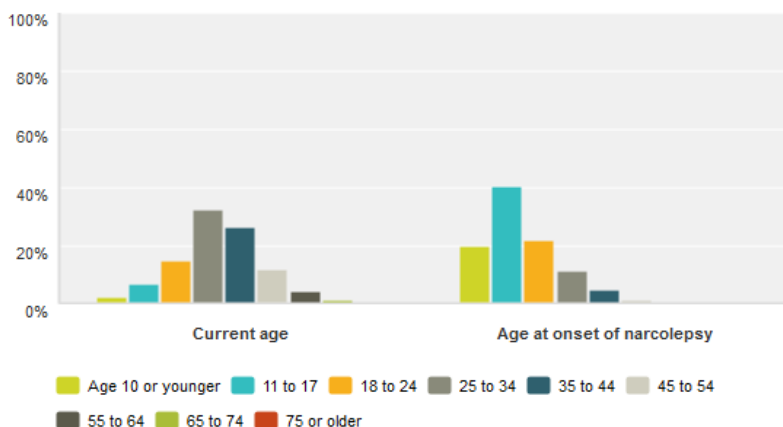
The survey will remain open until November 15, 2013 and a final analysis will be presented to the FDA through a public docket established for the purpose of hearing from the narcolepsy patient community. A summary of interim survey results is presented here:

Participant Profile:

- Of the 1,350 total respondents, 74% were individuals diagnosed with narcolepsy by a physician. 17% were the parent, spouse or other family caregiver for a person diagnosed with narcolepsy. 6% had been diagnosed with idiopathic hypersomnia or had a condition resembling narcolepsy but had not received a diagnosis from a physician.

What is your age (or the age of the person with narcolepsy with whom you are close)?

Answered: 1,128 Skipped: 222



- More than half of respondents, 55%, have been affected by narcolepsy for more than 10 years.
- Nearly 51% of survey respondents reported that it took 6 years or longer to get properly diagnosed.
- 40% reported being between the ages of 11 and 17 when symptoms first appeared. There are currently no medications approved for use by children or teens with narcolepsy, adding challenges to management of the condition for younger patients.
- There is a high rate of other comorbid conditions reported through the survey. Two-thirds of respondents had one or more other medical conditions and one-fifth had three or more conditions in addition to narcolepsy. Anxiety, sleep apnea, migraines, fibromyalgia and other chronic pain conditions were among the most frequent diagnoses reported by respondents.

- Here are representative excerpts from comments about diagnosis submitted by respondents:
 - “It took me many years to realize that something was wrong – this was my ‘normal’ but I didn’t consider that it wasn’t normal at all. Once I decided to see a doctor when I was lucky enough to have insurance, things moved quickly although I’ve had an assortment of diagnoses over the years.”

- “I have spent thousands of dollars on tests, prescription medicines, and doctors that did not help me. I have been made to feel crazy, like a hypochondriac, that I am really just lazy and have no self-control. I had severe onset at age 17, and since then have been on scores of antidepressants and mood stabilizers. I went from being a high achieving student to a college dropout with multiple suicide attempts and a dead-end blue collar job. I don’t see a future for myself.”

- “As doctors and even a university hospital weren’t able to diagnose anything, I searched the Internet and found a possible diagnosis which was confirmed in the sleep laboratory soon afterwards.”

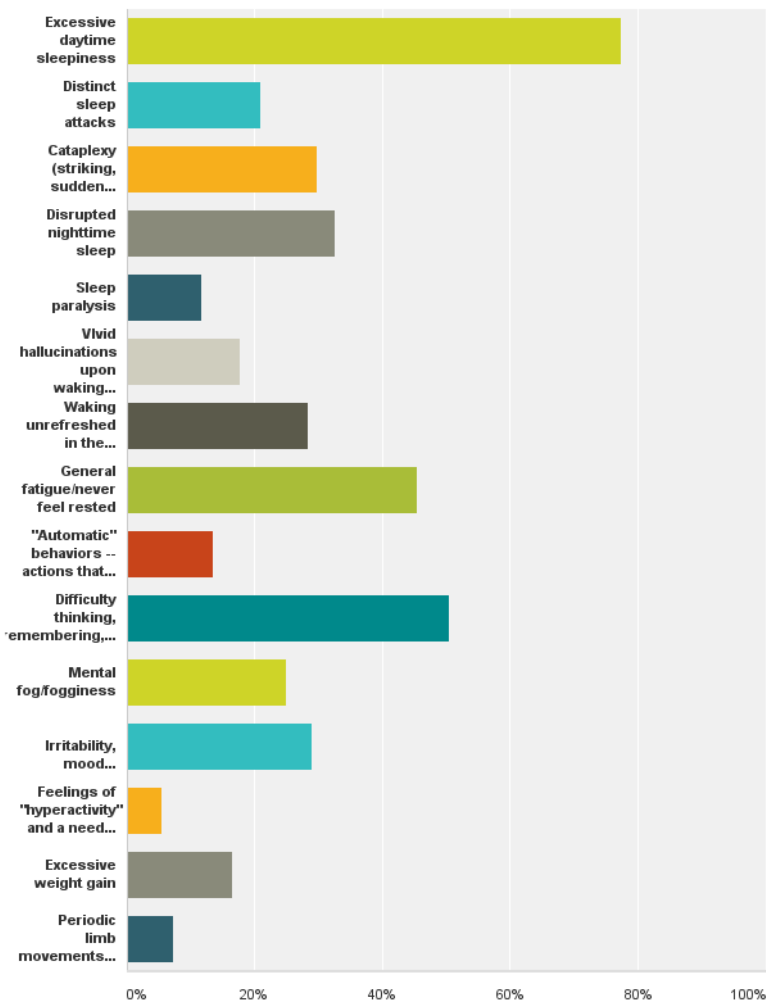
- “My daughter has seen more than 15 specialists over the past 5 years and they couldn’t find anything wrong. It took the right doctor asking the right questions to pick up on something that prompted a sleep study.”

Symptoms:

- Cataplexy, a striking and sudden episode of muscle weakness often triggered by strong emotion, was reported by 64% of

Q11 Of all the symptoms that you experience because of your condition, which one to three symptoms have the MOST SIGNIFICANT impact on your life?

Answered: 1,087 Skipped: 263

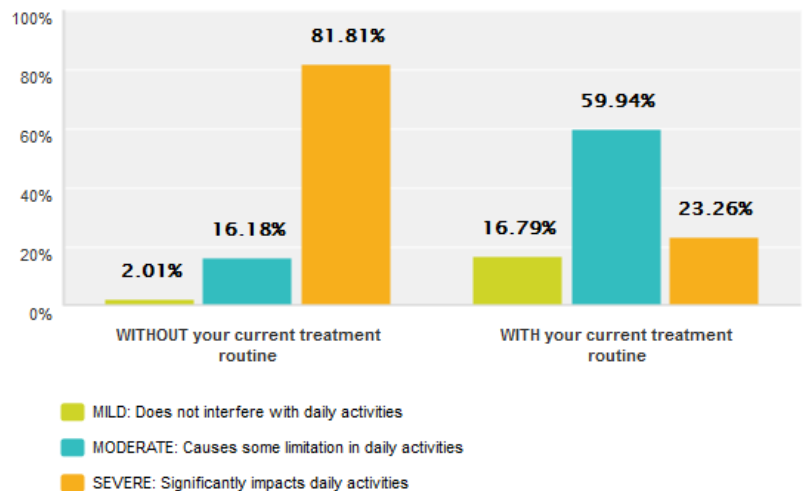


respondents. For those who experience cataplexy, it was most often triggered by laughing excitedly (43%), being angry (42%) and/or being startled (39%).

- The three symptoms rated as having the most significant impact on patients' lives were excessive daytime sleepiness (77%), difficulty thinking, remembering, concentrating or paying attention (50%) and general fatigue/never feeling rested (45%). For those with narcolepsy plus cataplexy, cataplexy was also rated as having a significant life impact.
- When describing their symptom patterns, 74% indicated that symptoms are present on a daily basis, with 29% reporting that they can vary from day-to-day and week-to-week. More than one-third (35%) noted that the level of symptoms varies according to other factors in their lives.
- Here are representative excerpts from comments about symptoms submitted by respondents:
 - "It is very hard to limit to only three symptoms, as the severity of the 11 of the symptoms contribute to destroying my quality of life."
 - "I have a condition that forces me to sleep and yet I am absolutely terrified of what sleep brings. Imagine being so sleepy you can't keep talking to your fiancé on the phone and nod off mid-sentence. I have hallucinations consisting of strange men at my door or thousands of bees coming of my fan, dreams so vivid it takes 10 minutes to recite them."
 - "Sleep paralysis is very scary and I have almost every one of the symptoms listed. Periodic limb movements affect my sleep too."
 - "So unfair to limit us to three! They are all connected. You sleep badly and it makes you feel more fatigued. More fatigue = more irritability and worse concentration. In 16 years I would answer this question differently depending on immediate circumstances. Today, memory loss is #1."
 - "Hallucinating with sleep attacks in the classroom is really disturbing."
 - "In all honesty, I can't quite pinpoint which symptom has 'the MOST SIGNIFICANT IMPACT' because they all do. It is a symptomatic concoction of misery."

How would you describe the severity of your condition?

Answered: 1,120 Skipped: 230



Daily Life Impacts:

- 84% report not being able to perform as they wish at work or in school. 77% indicate that they have difficulty interacting with family or friends and 71% can't get through the day without falling asleep.
- In describing how their condition has changed over time, 37% responded that they are better informed and prepared to manage their conditions, but the symptoms and severity are about the same as when narcolepsy first started. A quarter of respondents indicated that the condition is more stable and manageable now; another quarter stated that it is worse and more unpredictable than when it first started.
- Here are representative excerpts from comments about daily life impacts submitted by respondents:
 - "I am afraid to hold my infant daughter for fear of losing muscle control at any moment."
 - "I don't cook alone anymore because I almost started a fire when cataplexy hit while I was at the stove one night."

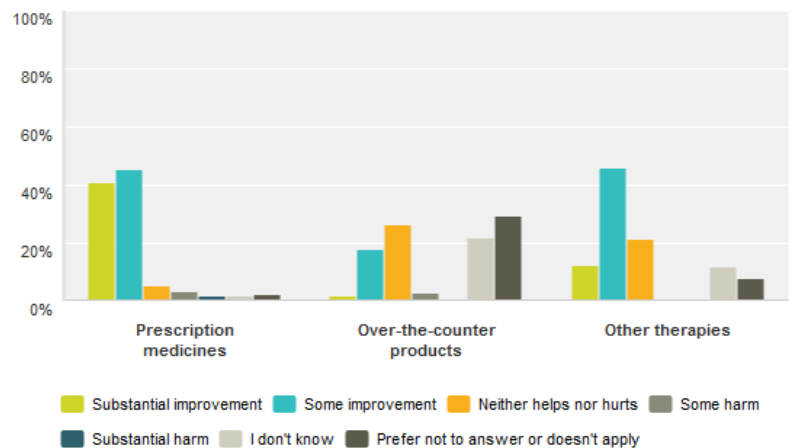
- “My daughter hardly goes out and when she does, she is so exhausted during the activity and the following day. She is afraid to drive. She is unable to work and gets tired just taking a walk. I do her laundry, shopping and cleaning.”
- “I am trapped and can’t contribute to society anymore. Even my own home is filled with perils because I am so prone to many cataplexy attacks. I miss going out with friends and loved ones, but I have no energy anymore.”
- “We held off on a family and it’s caused major issues in our relationship over the years.”
- “I no longer recognize my own life because everything has changed.”
- “I want to be/feel part of the family again.”
- “I want to have full trust of my body again, not feel like I’m going to fall over and hurt myself or damage something.”

Current Treatment:

- Overall, 82% of respondents describe their untreated condition as “severe” – significantly impacts daily activities. With treatment, only 22% report the same severe impact on function, with 61% describing their treated condition as “moderate” – causing some limitation on daily activities.
- Nearly 95% of survey respondents reported having been prescribed one or more of the four medications approved by FDA for treatment of narcolepsy or its key symptoms (Adderall, Nuvigil, Provigil and Xyrem). 79% use one or more of those medications currently. Other prescription medications (including stimulants, antiepileptics and hypnotics/sedatives) have been used by 70% of respondents; 90% use other therapies (including lifestyle modification, vitamins, nutritional supplements, diet and/or yoga) to help manage their condition. Over-the-counter products were currently used by just 23% of respondents. Fewer than 7% reported that they currently pursue no form of treatment for the condition.
- Without treatment, 77% of patients report experiencing 6 or more episodes of excessive daytime sleepiness each day. With treatment, the frequency of such symptoms falls dramatically to 1-2 times each day for 38% and 3-5 times each day for 41%. Episodes of cataplexy can be reduced as well; 41% report 3 or more daily cataplexy events without treatment, but just 13% experience as many episodes with currently available treatments.
- 41% of respondents credit prescription medicines with providing “substantial improvement” in their ability to do important activities of daily living and another 45% indicated that prescription medications provide some improvement in their function. By contrast, just 2% rated over-the-counter (OTC) products as providing “substantial improvement” and 18% rated OTC products as providing “some improvement.” Other therapies (lifestyle modification, vitamins, etc.) were also an important factor in managing the condition, with 12% reporting “substantial improvement” with use of these approaches and 46% reporting “some improvement.”
- The main drawbacks of currently available therapies reported by respondents include bothersome side effects (such as headaches, dry mouth, racing heart, nausea), dosing schedule, concerns about long-term use, cost and insurance reimbursement issues. To ensure safe and effective use of the four

How well do these therapies improve your ability to do specific activities that are important in your daily life?

Answered: 988 Skipped: 362

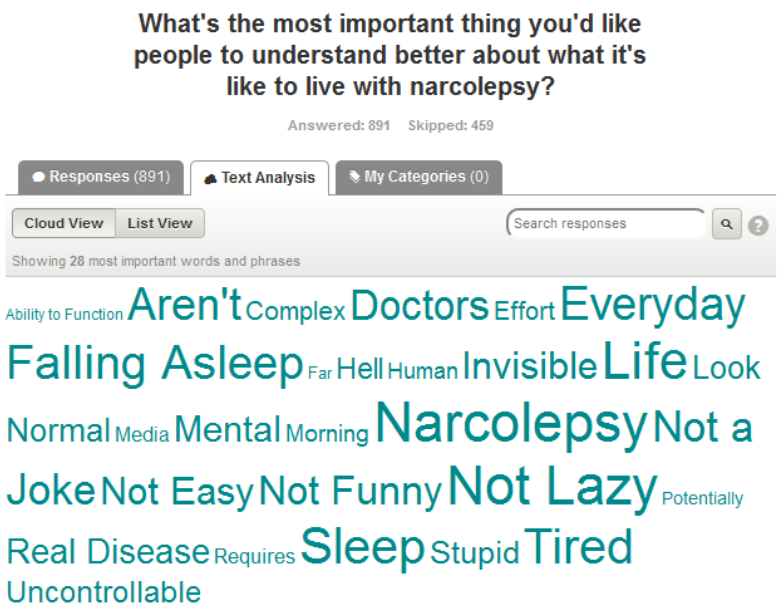


medications approved by FDA to be marketed for narcolepsy, FDA requires them to be distributed as controlled substances.

- Here are representative excerpts from comments about treatment submitted by respondents:
 - “Nothing is fool-proof. It is trial and error with every technique and/or medicine that is used.”
 - “Medications are not widely available. They’re too costly. Docs prefer not to prescribe them and pharmacies cannot or will not order them due to scheduled nature.”
 - “Caffeine and naps are my lifestyle changes.”
 - “Trying to find the right dosing schedule is a definite challenge so that I am effective but minimize my intake as much as possible and so I don’t hit the brick wall of meds not working.”
 - “All medications I take are not safe while pregnant and I would like to start a family soon. The medications are not safe for my heart and I worry about the side effects years down the road.”
 - “I am constantly afraid of taking my medication in public. Almost all narcolepsy drugs are heavily stigmatized.”
 - “The most significant downside to my medication is cost. I am concerned that I will lose coverage or no longer be approved for patient assistance. If I lose my medication, I will not be able to function and I’ll lose my job.”
 - “Inconvenient dosing and eating schedules make it hard to eat and sleep when my family does. I’m willing to do it because I don’t ever want to go back to how my life was before treatment.”
 - “Side effects can be annoying but the alternative is not functioning at all. I don’t like the fact that my heart has to work extra hard and I don’t know the damage it’s doing.”
 - “I tried dietary changes and lifestyle modifications while I was waiting for specialist referral. They helped even more than I thought they would, but my condition improved even more substantially after the addition of a prescription medication.”

Hopes For the Future:

- FDA asked, “Assuming there is no complete cure for your condition, what specific things would you look for in an ideal therapy for your condition?” Here are representative excerpts from comments submitted by survey respondents:
 - “A drug that would provide consistent and adequate control of the daytime sleepiness without the hard crash and one that would require one dose taken at bedtime resulting in 8 hours of restorative sleep.”
 - “Better sleep without acting out dreams and injuring myself. Being able to say awake for several hours without feeling like I need to sleep.”
 - “My ideal therapy would be education of others. Having an invisible illness that is misunderstood takes a huge emotional toll. I am neither lazy, incompetent or unfocused. I am living impaired in an unforgiving world.”
 - “Ability to start a family while on medication. Ability to drive a long distance.”
 - “Something that would let me be awake without having my life ruled by pills.”



- “Longer-acting medications. Fewer side effects.”
- “Something that ‘directly’ addresses the hypocretin/orexin deficiency that causes narcolepsy and corrects that deficiency.”
- “Having more than two hours a day with my mind and body functioning at the same time.”
- Unite Narcolepsy asked, “What’s the most important thing you’d like people to understand better about what it’s like to live with narcolepsy?” Here are representative excerpts from comments submitted:
 - “We’re not lazy. It’s not a joke. These conditions are real. Most of us would give anything to be like an average person again.”
 - “Narcolepsy robs you of your life’s goals and dreams and is also humiliating.”
 - “That even if she looks ‘normal’ on the outside, she is struggling with symptoms all the time which makes everyday things a battle.”
 - “I don’t want to sleep all the time. I want to be awake and doing things with my family and children.”
 - “We are still humans with emotions.”
 - “It’s a life-changing disease that has slowly taken everything away I used to love doing.”
 - “It’s uncomfortable and we hate it. Please be patient with us. We need support from our family and friends. We need you to understand, even if you don’t.”
 - “For people to understand there is no ‘magic pill’ that completely controls narcolepsy. Even with the proper dose of my medications and lifestyle modifications, I still have to work hard to function anywhere close to normal.”
 - “One more cup of coffee will not make me more alert. When my body wants to sleep, I am at its mercy, no matter what plans I thought I had for the day.”
 - “That we have a disability like Parkinson’s or MS or lupus. We have a form of brain damage. We cannot be expected to do everything a healthy person can do and yet we are ignored, denied help and treatment, and medically mistreated because health care professionals don’t know about our condition.”
 - “MY tired is not YOUR tired.”
 - “You don’t LIVE with narcolepsy. You EXIST with it. Not DEAD but not really ALIVE either.”

Wake Up Narcolepsy co-founder and executive director Monica Gow is gratified by the community’s candor in sharing their experiences and perspectives. “Our family has seen first-hand through my son’s experience all the ways in which narcolepsy robs a person of his or her life, but the survey results underscore the breadth and depth of losses patients and their loved ones bear. The thousands of comments submitted tell a heart-breaking story of dreams deferred, career and educational plans put on hold, independence lost, and families torn apart. Simple things we take for granted are lost to people combatting narcolepsy,” said Gow.

Life with a rare disease that’s often the butt of jokes can be very isolating. Unite Narcolepsy organizers took care to reach far and wide across the community to educate patients about this unique invitation from the FDA. They created a new website and hosted a series of webinars to provide ample resources explaining the FDA program and a growing role for patients in drug development. A popular narcolepsy blogger, author and spokesperson, Julie Flygare, J.D., helped launch and sustain the effort via social media. Other organizations including Global Genes Project and the Patient Advocate Foundation helped spread the word through rare disease and disability communities.

Narcolepsy is a neurological sleep disorder that affects some 200,000 Americans and 3 million adults and children worldwide. To learn more about narcolepsy, the Unite Narcolepsy initiative and the upcoming FDA meeting, please visit www.UniteNarcolepsy.org or www.WakeUpNarcolepsy.org. To complete the survey, please visit <https://www.surveymonkey.com/s/unitenarcolepsy>.