# **Narcolepsy & Idiopathic Hypersomnia:** FDA Patient-Led Listening Session Summary Report

Timely Patient Perspectives from the
Narcolepsy and Idiopathic Hypersomnia Community:
Unmet Needs, Real-Life Impacts, and Current Treatments

Session Date: August 8, 2022
Report Published: December 2022

PREPARED BY

projectsleep

# TABLE OF CONTENTS



.

*To be awake is to be alive.*

— HENRY DAVID THOREAU

## INTRODUCTION

On August 8th, 2022, from 2:00 - 3:30 p.m. EDT, a patient-led Listening Session took place online between the Food and Drug Administration (FDA), Project Sleep, and patient advocates in the narcolepsy and idiopathic hypersomnia community.

This summary report, created by Project Sleep, highlights key takeaways from the meeting.

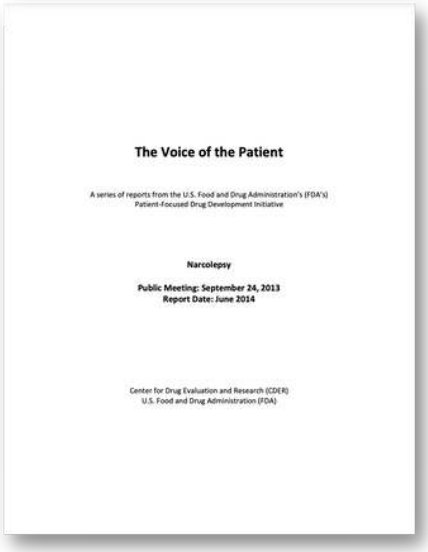
### OBJECTIVES OF THE LISTENING SESSION

- Build upon and update FDA regarding the topics covered in the 2013 Patient-Focused Drug Development (PFDD) Meeting on Narcolepsy
- Focus on the first-hand experience of patients and caregivers managing narcolepsy and idiopathic hypersomnia
- Convey a sense of urgency and enthusiasm for partnering with FDA to advance new treatment options

### SUMMARY OF TOPICS DISCUSSED

- Impacts of symptoms on daily functioning and quality of life
- Underrecognized and invisible aspects of living with narcolepsy or idiopathic hypersomnia
- Challenges with current therapies
- Patient and caregiver concerns (including symptom severity, quality of life, disruption of daily activities, impacts on employment and parenting, and related considerations that are self-reported and often difficult to track clinically)

## BUILDING ON FDA'S 2014 REPORT


The Voice of the Patient

A series of reports from the U.S. Food and Drug Administration's (FDA's) Patient-Focused Drug Development Initiative

Narcolepsy

Public Meeting: September 24, 2013
Report Date: June 2014

Center for Drug Evaluation and Research (CDER)
U.S. Food and Drug Administration (FDA)

On Sept. 24, 2013, the FDA hosted a five-hour Patient-Focused Drug Development (PFDD) Meeting on Narcolepsy to hear directly from patients, caregivers, and advocates about their experiences with narcolepsy and its treatments.

Approximately 70 narcolepsy patients or representatives attended in-person, and over 50 provided input via a live webcast. This participation rate was considered high for a rare disease community.

In June 2014, the FDA published an in-depth Report summarizing the PFDD Narcolepsy Meeting, highlighting key findings:

- Excessive Daytime Sleepiness (EDS) significantly affects people's daily lives, but narcolepsy also involves much more than EDS.
- Narcolepsy symptoms often change over time and the condition can exert a significant social, emotional, and financial toll on patients and their families.
- Almost all participants used prescription medications to treat their condition, but saw a continued need to enhance treatment options.
- People with narcolepsy face additional challenges to get proper diagnosis, treatment, and support.

In hosting a 90-minute Patient Listening Session with the FDA in 2022, Project Sleep aims to build upon the 2014 Report by highlighting additional underrecognized impacts while also providing new perspectives on current therapeutic options. Read the FDA's 2014 "The Voice of the Patient" Narcolepsy Report.

## DISCLAIMER

Discussions in FDA Patient Listening Sessions are informal. All opinions, recommendations, and proposals are unofficial and non-binding on FDA and all other participants. This report reflects Project Sleep's account of the perspectives of the patients, caregivers, and speakers who participated in the Patient Listening Session with the FDA. To the extent possible, the terms used in this summary to describe specific manifestations of narcolepsy and idiopathic hypersomnia, health effects and impacts, and treatment experiences reflect those of the participants. This report is not meant to be representative of the views and experiences of the entire narcolepsy and idiopathic hypersomnia patient population or any specific group of individuals or entities. There may be experiences that are not mentioned in this report.

## LISTENING SESSION PARTICIPANTS

PARTNER ORGANIZATION, PROJECT SLEEP

Project Sleep is a 501(c)(3) non-profit organization dedicated to raising awareness about sleep health, sleep equity, and sleep disorders. Believing in the value of sleep, Project Sleep aims to improve public health by educating the public about the importance of sleep. The organization educates and empowers individuals and communities using events, campaigns, and programs to bring people together and talk about sleep as a pillar of health. Project Sleep organized this session and recruited the speaker participants.

SPEAKER PARTICIPANTS

The meeting featured seven patient or caregiver advocate speakers, along with a Project Sleep representative and a clinician. Speakers included:

- Amy – a person living with idiopathic hypersomnia
- Chelsea – a person living with type 1 narcolepsy with cataplexy
- Kenya – a person living with type 1 narcolepsy with cataplexy
- Kristyn – a person living with type 2 narcolepsy without cataplexy
- Lindsay – a parent of a child living with type 1 narcolepsy with cataplexy
- Matt – a person living with type 1 narcolepsy with cataplexy
- Michael – a person living with type 1 narcolepsy with cataplexy
- Julie Flygare, JD – President & CEO of Project Sleep
- Anne Marie Morse, DO – hypersomnia clinical expert

LISTENING PARTICIPANTS

- Dale Dirks, Health & Medicine Counsel
- Dane Christiansen, Health & Medicine Counsel
- Lauren Oglesby, MPH, Programs Manager, Project Sleep
- Rachel Aubrey, Digital Media Specialist, Project Sleep

FDA DIVISIONS REPRESENTED

The following FDA offices and divisions were represented at the Listening Session:

Office of the Commissioner (OC) – 4 offices

- OC/OCPP/PAS – Office of Clinical Policy and Programs/Patient Affairs (organizer)
- OC/OCPP/OCliP – Office of Clinical Policy and Programs/Office of Clinical Policy
- OC/OCPP/OCP – Office of Clinical Policy and Programs/Office of Combination Products
- OC/OCPP/OOPD – Office of Clinical Policy and Programs/Office of Orphan Products Development

Center for Biologics Evaluation and Research (CBER) – 5 offices/divisions

- CBER/OCD – Office of the Center Director
- CBER/OCD/PS – Office of the Center Director/Policy Staff
- CBER/OCBQ/DIS/PSB – Office of Compliance and Biologics Quality/Division of Inspections and Surveillance/Program Surveillance Branch
- CBER/ORO – Office of Regulatory Operations
- CBER/OVRR/DVRPA/CRB2 – Office of Vaccines Research and Review/ Division of Vaccines and Related Product Applications/Clinical Review Branch 2

Center for Drug Evaluation and Research (CDER) – 7 offices/divisions

- CDER/OCD – Office of the Center Director
- CDER/OND/ODES/DCOA – Office of New Drugs/Office of Drug Evaluation Science/Division of Clinical Outcome Assessment
- CDER/OND/ON – Office of New Drugs/Office of Neuroscience
- CDER/OND/ON/DNI – Office of New Drugs/Office of Neuroscience/Division of Neurology I
- CDER/OND/ON/DP – Office of New Drugs/Office of Neuroscience/Division of Psychiatry
- CDER/OND/ORDPURM/DRDMG – Office of New Drugs/Office of Rare Diseases, Pediatrics, Urology, & Reproductive Medicine/Division of Rare Diseases & Medical Genetics
- CDER/OCD/PFDD – Office of the Center Director/Patient-Focused Drug Development

Center for Devices and Radiological Health (CDRH) – 7 offices/divisions

- CDRH/OPEQ/OHTI – Office of Product Evaluation and Quality/Office of Health Technology I
- CDRH/OPEQ/OHTI/DHTIA – Office of Product Evaluation and Quality/Office of Health Technology I/Division of Health Technology IA
- CDRH/OPEQ/OHTI/DHTIC – Office of Product Evaluation and Quality/Office of Health Technology I/ Division of Health Technology IC
- CDRH/OPEQ/OHTIII – Office of Product Evaluation and Quality/Office of Health Technology III
- CDRH/OPEQ/OHTIII/DHTIIIB – Office of Product Evaluation and Quality/Office of Health Technology III/ Division of Health Technology IIIB
- CDRH/OPEQ/OHTIII/DHTIIIC – Office of Product Evaluation and Quality/Office of Health Technology III/ Division of Health Technology IIIC
- CDRH/OSPTI/DAHRSSP – Office of Strategic Partnership and Technology Innovation/Division of All Hazards Response Science and Strategic Partnership

Office of Regulatory Affairs (ORA) – 1 office

- ORA/OMPTO/OPQO/DPQOIII/PQIB – Office of Medical Products and Tobacco Operations/Office of Pharmaceutical Quality Operations/Division of Pharmaceutical Quality Operations III/Pharmaceutical Quality Investigation Branch

**KEY MEETING THEMES SUMMARY**

# SPECTRUM OF SYMPTOMS AND VARIABLE EXPERIENCES

People living with type 1 narcolepsy with cataplexy (NT1), type 2 narcolepsy without cataplexy (NT2), and idiopathic hypersomnia (IH) share a common symptom of excessive daytime sleepiness, but experiences vary widely based on factors like additional symptoms, severity, and age of onset. Sharing first-hand perspectives shows both the commonalities and differences between individuals and underscores the need for patient-reported outcome measures and increased awareness amongst physicians and the public.

Age at symptom onset: Across seven patient experiences represented by the speakers, symptoms began as early as age 4 and as late as age 32.

- Lindsay's son was 4 years old when his symptoms began, and he was diagnosed with NT1 that year.
- Kenya was 9 years old when she was diagnosed with NT1.
- Amy experienced symptoms for most of her life. She was initially diagnosed with NT2 at age 22. Her diagnosis changed to IH three years later.
- Matt's symptoms started when he was 12 years old, and he searched for a diagnosis for 13 years before being diagnosed with NT1 at age 25.
- Kristyn started experiencing NT2 symptoms at age 14, but she wasn't diagnosed until age 23.
- Michael was initially diagnosed with NT2 at age 19. His diagnosis recently changed to NT1.
- Chelsea's NT1 symptoms began when she was 32 years old, and she was diagnosed that year.

Cataplexy: For people with NT1, cataplexy (striking, sudden episodes of muscle weakness usually triggered by strong emotions) can range from severe to subtle and impacts vary.

- Lindsay shared that her son, diagnosed with NT1 at age 4, experienced cataplexy over 100 times per day and had to be carried or put in a stroller to go anywhere. His body would melt to the ground when he experienced joy, which severely impacted his quality of life.
- Kenya experienced a cataplexy episode at an IMAX theater on a school field trip. Nobody around her knew what was happening or how to help. This experience ultimately led to her diagnosis with NT1.
- Matt fears walking down stairs due to his cataplexy. He has broken ribs, his leg, and incurred concussions from falling. His partial cataplexy includes slurred speech and dropping things. Matt believes his symptoms are not isolated; they work in tandem. Sleepiness increases cataplexy, which in turn increases sleepiness.
- When laughing with co-workers, Chelsea felt overwhelming weakness and started dropping her coffee mug. She broke two mugs in one week; once collapsing to floor with hot coffee. Chelsea has avoided happy and sad events because her emotions exacerbate her symptoms. It is extremely isolating.

Idiopathic hypersomnia: Idiopathic hypersomnia was not included in the 2013 Narcolepsy PFDD meeting or 2014 Report, but over time the community has come to recognize a spectrum of central disorders of hypersomnolence. More research is needed to better understand the causes of IH and NT2 and to develop improved diagnostic tools.

- Amy had symptoms of IH most of her life, but the impacts became more noticeable in her early 20s. While in graduate school, she was unable to stay awake in class or while studying for the CPA exam. She got angry when people tried to wake her, but wouldn't remember these interactions.
- Amy and those around her did not see her symptoms as something wrong for many years. Diagnosed with NT2 at age 22, her diagnosis then changed to IH at age 25.
- Julie explained that recent data demonstrate poor repeatability of the Multiple Sleep Latency Test (MSLT) in NT2 and IH. Clinical diagnostic criteria for NT2 and IH can be difficult to replicate via MSLT and patients' diagnosis may be switched. In one retrospective study, when MSLT was repeated, NT2 cases changed to IH 26% of the time, or to a negative MSLT 57% of time [Ruoff 2018]. Changing diagnosis is confusing and disruptive.

## SLEEPINESS IS SERIOUS

Excessive daytime sleepiness has significant impacts on functioning and everyday activities.

- Chelsea describes, "This is not normal tired. Everyone experiences sleepiness - this is different. Normal is like a wave washing over you. My sleepiness is like a tsunami, it takes me down. Unstoppable."
- Michael's family supports him with many daily tasks. He must ration his energy carefully. Sometimes he needs to nap while cooking. He can't always brush his teeth or shower without using too much energy.

> *It feels like I've been hit by a freight train. I wake up feeling worse than when I went to bed.*
>
> — MICHAEL

- Michael can only drive in the afternoons. He naps beforehand and allows extra time to nap if needed on the way. He drives for a maximum of 30 minutes, with an extra 30-minute "buffer" for napping both ways.
- After starting treatment, Chelsea tried to work part-time but was often late regardless of when she took medications or whether she showered or skipped showering. She was too sleepy to drive 15 minutes safely. She was fired from her job and felt devastated. Chelsea still must pull over to nap while driving (even with medications). She has fallen asleep in all kinds of places, leaving herself "painfully vulnerable."
- Matt struggled to work full-time, as his sleepiness left his body feeling physically ill, and he had to rest after work, taking a toll on his role as a father of three children.
- Amy describes IH as more of a "wakefulness disorder" rather than "sleep disorder" because she is always struggling to be awake. Her grad school friend said, "Amy is a really cool girl. It's too bad we can never talk to her because she's always sleeping."

## BRAIN FOG AND COGNITIVE IMPACTS

The FDA's 2014 Narcolepsy Report highlighted brain fog as a significant part of many people's experiences with narcolepsy, thus elevating brain fog as an important and underrecognized aspect of the condition.

- Despite taking treatments, Michael took a year and a half off of work due to challenging brain fog. He recently returned part-time, still losing focus quickly and crashing each day.
- Before diagnosis, Chelsea was typing one day at work and noticed she had written “vanilla ice cream" into a military operating procedure document. After diagnosis, Chelsea was evaluated by a career specialist while filing for disability. The specialist reported that Chelsea was "not capable of skilled work due to impaired executive function." Hearing those words was heartbreaking, even though she knew it was true. It still took Chelsea over eight years to be granted disability benefits.
- To this day, Kristyn experiences times when her eyes are open, but her memory and cognition are gone. She can’t remember names, passcodes, or work decisions.
- Kenya has experienced worsening brain fog and memory loss recently. She is having trouble articulating her thoughts and spelling simple words.
- Brain fog has impacted Matt's role parenting his three children. He cuts family activities short, and sometimes is unable to help his son with his third grade math.

## WAKEFULNESS IS NOT ONE STATE

Being half-awake is not fully living. People with narcolepsy or IH can appear awake while experiencing extreme sleepiness and struggling to stay alert. Many also experience automatic behavior (continuing activities with little conscious awareness and often no memory).

- Michael experiences automatic behavior: cutting his finger while cutting carrots, burning eggs, zoning out and stubbing his toes - he broke a toe recently. He carries a leakproof water bottle because of clumsiness, wears shoes inside to protect himself, and sets timers for everything.

> *Just because my eyes are open, it doesn't mean I'm fully awake.*
>
> — AMY

- When Matt was working full-time, he walked into a closet at work and didn’t remember why he was in there or for how long. One day, he remembered finishing a job fixing a water fountain and then getting a call from his boss later asking why it wasn’t finished. In a hazy state, he'd left the job site a mess with tools although he thought he'd cleaned up.
- Kristyn said, "At diagnosis, I had a misperception that medication would make me as wakeful as others. I've had to reestablish my 'normal' baseline."

## NIGHTTIME SYMPTOMS AND MISTIMING OF SLEEP

Terrifying hallucinations around sleep, sleep paralysis, and interrupted sleep blur the lines between sleeping and waking, and are underappreciated symptoms.

- After experiencing disrupted nighttime sleep, Michael is too sleepy in the morning to do anything, and stimulants don't help him early in the day.
- While living in Japan, Chelsea had terrifying hallucinations that someone was breaking into her house. She was paralyzed and felt her husband stroking her hair. When she finally turned over, she found he was actually fast asleep. She thought her house was haunted.
- Lindsay's son experienced scary nightmares and vivid conversations in the middle of the night starting at age 4. One night, he was trying to save people from a burning plane crash. It was heartbreaking for Lindsay to witness his distress.

## 24-HOUR NATURE OF HYPERSOMNIAS

Narcolepsy and IH symptoms often impact all 24 hours of the day. Many people organize their lives around symptoms, medications, side effects, naps, bedtimes, and other considerations.

- Kristyn has struggled with the timing of sleep and medication. Trying to figure out a schedule of medication, food, sleep, and work took "a ridiculous amount of mental gymnastics." Kristyn tried strategies like taking her stimulants while still in bed to overcome sleep inertia to get up, but still couldn't find a medication routine that allowed her to maintain wakefulness throughout work. She left her news production job and took a year off from medications.
- Lindsay shared, "Our family had to start living our lives based on a very strict schedule that revolved around my son's medications, wakefulness, nap times, and bedtimes. We often cancel plans or don't make plans because our days are ruled by my son's narcolepsy."
- Amy skips basic daily tasks and self-care if she wants to do something as simple as go out to dinner with her parents. She estimates that she is managing on about four hours of wakefulness per day.

> *All my choices revolve around narcolepsy… from monumental ones like not having children, to small ones like scheduling appointments and errands so that I'm not driving in peak traffic.*
>
> — CHELSEA

## NO "ONE SIZE FITS ALL" APPROACH TO MANAGEMENT

Given the range of symptoms and severity across individuals and additional complexities and comorbidities, there is no “one size fits all” approach to treating narcolepsy or IH. Finding a medication regimen that helps to adequately manage symptoms can be challenging. Some therapies may work well for certain people while others experience less benefit, continue to struggle, or experience side effects that outweigh the benefits.

- Many medications affect mood and/or mental health.
- Stimulants help with excessive daytime sleepiness, but don’t get to the root of the problem and have many potential side effects. e.g., tachycardia, insomnia, anxiety, nausea, high blood pressure, headache, and dizziness.
- FDA-approved treatment options for cataplexy are limited, especially in children.
- Lindsay has been willing to accept the potential risks of medications not FDA-approved for pediatric use, because her son’s quality of life was so poor. She said, "We would travel to the end of the world for a medication that would help improve my son's symptoms even a little bit."
- With a nighttime medication, Lindsay saw a significant improvement in her son’s cataplexy and excessive daytime sleepiness, although it wasn't enough. They added another medication and saw more improvement in his cataplexy, however, this was short-lived and had to increase the dose and eventually add a second dose to keep him from having cataplexy at school. Unfortunately, his cataplexy again increased in frequency. Lindsay's family felt out of options until a new medication was approved (for adults with narcolepsy) in 2019.

> *Although we saw significant symptom improvement, I do not want you to think this medication was easy, nor that it fixed or cured my son's narcolepsy.*
>
> — LINDSAY

- Without the additional new medication, Lindsay’s son’s cataplexy increases to a level that makes attending school a safety issue. If the school feels he is at risk of injury, they will want him to have a homebound placement, which means he would be secluded from the social interaction school provides. This would be unacceptable, as Lindsay said, "He has already lost so much in his lifetime."
- Lindsay noted that although the nighttime medication decreased her son's cataplexy and excessive daytime sleepiness, there are difficulties not to be minimized. These include fighting an upset child for more than an hour to take medication in the middle of the night, and sleeping in his room to monitor his safety.

> *I want to have better medications, so that I am not avoiding life and just surviving. We all deserve to thrive.*
>
> — MATT

- For Michael, night eating and morning nausea are big challenges while taking nighttime medications. He had to install padlocks on his cabinets and refrigerator to prevent himself from binge-eating at night, and must lock his phone and other devices in a time-delayed safe to avoid ordering food while on his nighttime medication.
- Stimulants do not help Michael with morning fatigue. He suffers through the first two to three hours of the day until he can take stimulants, and then struggles with brain fog and sleepiness the rest of the day.
- Amy is not functional on stimulants and had scary side effects. For years, she used a medicated compound cream that she rubbed on her arms (an off-label use of a drug used to reverse the effects of benzodiazepines) to wake up in the morning and get out of bed. She is looking for better medications to treat her IH and hopes to qualify for a clinical trial.
- Chelsea is thankful for stimulants, but they affect her mood and inhibit her creative process. She said, "I have to choose whether to be alert or creative."
- Kenya and Kristyn are both coping without medications currently, due to severe side effects from the treatment options they have tried.

## TREATMENT OVER A LIFETIME

Narcolepsy and IH symptoms can shift throughout life due to changes of circumstances, family planning, aging, and other factors. No treatment will work for every person at every stage of their life, and individuals may quickly cycle through available medications and be left without options.

- Kristyn said, "Once I was diagnosed with NT2, I thought medication would help me get to the same level of wakefulness as others." After trying different medications, she hasn't been able to find one that helps maintain wakefulness without severe side effects.
- A nighttime medication worked well to manage Michael's symptoms for the first few years, but lost effectiveness over the past five years. He now has a harder time falling asleep and staying asleep, getting a maximum of five to six hours of sleep per night.
- Amy's symptoms improved while she was pregnant, but returned about one year postpartum. Now she is unmedicated while breastfeeding.
- Chelsea said, "If improved treatments were available, maybe I could have my dreams and goals again. Maybe I could become a nurse and help other people."

## ALIGNING CLINICAL MEASURES WITH PATIENT GOALS

Dr. Morse spoke about the need for validated clinical measures that focus on meaningful improvements in areas that are important to patients, such as life tasks, relationships, and goals. Current outcome measures, e.g. numeric reduction of the Epworth Sleepiness Scale (ESS) score, are insufficient to capture the scope of living with narcolepsy or IH.

- Current tests and metrics measure excessive daytime sleepiness, but they don't measure quality of wakefulness. Amy said, "As the FDA is looking at metrics and ways to measure outcomes, I would love to see a way to measure levels of functioning while awake, as this has the highest impact on my quality of life."
- Patients and physicians aim to engage in shared decision-making regarding treatments and lifestyle adjustments, but available clinical data can be difficult to translate to life impact in real-life terms.
- Validated measures for patient-reported outcomes (PROs) are needed along with additional clinical diagnostic tools, especially for differentiating between NT2 and IH.
- Looking forward, the patient community is eager to be consulted and engaged in shaping future developments of outcome measures and clinical trial designs.

> *Narcolepsy has taken my dreams, my goals, and my self-worth. It has not taken my voice.*
>
> — CHELSEA

**KEY TAKEAWAYS**

- There is a spectrum of symptoms across NT1, NT2, and IH, and people's experiences vary widely.
- Sleepiness is serious and has significant impacts on daily functioning and real-life activities.
- Brain fog is an underappreciated and deeply challenging part of many people's experiences with narcolepsy and IH.
- Wakefulness is not one state. Many people with narcolepsy or IH experience automatic behavior, i.e. continuation of routine activities with little conscious awareness and often no memory of them.
- Nighttime symptoms such as terrifying hallucinations around sleep, sleep paralysis, and interrupted sleep blur the lines between sleeping and waking, and are underappreciated aspects of patient experiences.
- People must manage narcolepsy and IH symptoms 24 hours a day. Many plan their lives around symptoms, medications, side effects, napping, etc.
- There is no "one size fits all" approach to treatment or management of symptoms. Finding a medication regimen that adequately manages symptoms can be challenging. Treating narcolepsy over a lifetime requires adjustments and many options. No treatment will work for every person at every stage of life.
- Current clinical measures (e.g., the Epworth Sleepiness Scale) are inadequate to assess meaningful impacts of treatment on patients' lives. Validated patient-reported outcomes measures are needed along with additional diagnostic tools.
- The patient community is eager to be consulted and engaged in shaping future development of outcome measures and clinical trial designs.

## APPENDIX A: QUESTIONS FROM FDA REPRESENTATIVES

- Q: Can anyone comment on the transportation issue and the fear of motor vehicle accidents affecting their quality of life, job, and/or family?
    - Chelsea: Must take stimulants and have taken a nap and can't drive more than 30 mins at a time, talks to doctor about her symptoms and driving, self-limits. Michael's limitations are similar.
    - Dr. Morse: It is very hard to determine when a patient has the ability to drive, unlike epilepsy which has a very clear standard.
    - Matt: My physician and I have put a self-imposed limit of no more than 45 minutes without breaks. Also, I take medications and adjust to maximize safety.
- Q: Is getting disability benefits difficult even with documented diagnosis and continued symptoms?
    - Chelsea: Yes, I had a clear diagnosis well-documented from multiple doctors. It took eight years, multiple appeals, and I was denied benefits by two judges. I had to appeal it to the highest level, the Appeals Council, who reversed the prior judges' decisions.
    - Dr. Morse: It is very difficult to get [disability] even with clear evidence of narcolepsy.
- Q for Matt: How well is your obstructive sleep apnea controlled? (Note: Matt is living with obstructive sleep apnea [OSA] in addition to narcolepsy.)
    - Matt: 100% compliance with PAP device.
    - Dr. Morse: It is also important to note that patients with narcolepsy are at 18-25x risk for OSA.
    - Michael: I've had five sleep studies over 13 years and only now have a sleep study scheduled to check for OSA *while* taking nighttime medication. Normally sleep studies require me to stop nighttime meds.
    - Chelsea: I use a CPAP every night because nighttime medication slows my breathing.
- Q: Can you say a bit more about the stigma you face from people who might misunderstand, "So, you're sleepy... we all get sleepy"?
    - Kristyn: During overnight shifts in news production, everyone is sleepy and that made it difficult. I had to get a narcolepsy diagnosis to keep my job, and it's isolating knowing that your sleepiness is different in a way that people can't understand. In an industry where everyone complains about how tired they are all the time, I don't know how to explain that my sleepiness is different.
    - Dr. Morse: In 2014 the CDC reported that sleep deprivation is a public health crisis. We live in a culture of sleep deprivation; "Everyone is sleepy, why can't you get your act together?" This makes it harder for people with sleep disorders to gain diagnosis and understanding.
    - Chelsea: Everyone experiences sleepiness, which makes it difficult for others realize their sleepiness is in a different solar system compared to my sleepiness.
- Q: Would you like to see more patient reported outcomes?
    - Dr. Morse: We need validated measures that capture what's important to patients, e.g., life tasks, goals, and relationships. Not "down to a 9 from a 17 on the Epworth Sleepiness Scale" but meaningful changes in life activities.

## APPENDIX B: OVERVIEW OF NARCOLEPSY AND IDIOPATHIC HYPERSOMNIA

### NARCOLEPSY

Narcolepsy is a chronic neurological condition that impairs the brain's ability to regulate the sleep-wake cycle. It affects 1 in 2,000 people—200,000 Americans and 3 million people worldwide.

### NARCOLEPSY SYMPTOMS

Symptoms vary by person but may include:

- Excessive daytime sleepiness: Periods of extreme sleepiness during the day that feel comparable to how someone without narcolepsy would feel after staying awake for 48-72 hours.
- Cataplexy: Striking, sudden episodes of muscle weakness usually triggered by strong emotions such as laughter, exhilaration, surprise, or anger. The severity may vary from a slackening of the jaw or buckling of the knees to falling down. The duration may be for a few seconds to several minutes and the person remains fully conscious (even if unable to speak) during the episode.
- Hypnagogic and hypnopompic hallucinations: Visual, auditory, or tactile hallucinations upon falling asleep or waking up. These can be frightening and confusing.
- Sleep paralysis: The inability to move for a few seconds or minutes upon falling asleep or waking up. It is often accompanied by hypnagogic or hypnopompic hallucinations.
- Disrupted nighttime sleep: Unlike public perceptions, people with narcolepsy do not sleep all the time. Timing of sleepiness is "off" with narcolepsy so one may fight sleepiness during the day but struggle to sleep at night.

Additional symptoms may include:

- Brain fog: Cognitive functioning issues such as difficulty thinking, remembering, concentrating, or paying attention.
- Micro-sleeps: Periods of memory lapse or blackouts caused by very short periods of sleep.
- Automatic behavior: Continuation of a routine activity with little conscious awareness and often without memory of it.
- Sleep inertia: A transitional state after waking, with an extreme desire to return to sleep and feeling "foggy."

### TYPES OF NARCOLEPSY

There are two forms of narcolepsy: type 1 narcolepsy with cataplexy (NT1) and type 2 narcolepsy without cataplexy (NT2). Recent research suggests that NT1 is caused by a lack of hypocretin (also called orexin), a key neurotransmitter that helps sustain alertness and regulate the sleep-wake cycle. Less is known about the causes of NT2.

## IDIOPATHIC HYPERSOMNIA

Idiopathic hypersomnia (IH) is a chronic neurological sleep disorder marked by an insatiable need to sleep that is not eased by a full night's slumber. IH causes excessive daytime sleepiness despite adequate, or more typically, long sleep amounts (more than 9-10 hours in a 24-hour period).

## IDIOPATHIC HYPERSOMNIA SYMPTOMS

Symptoms vary by person but may include:

- Excessive daytime sleepiness: Periods of extreme sleepiness that feel comparable to how someone might feel after staying awake for 48-72 hours
- Sleep inertia: A transitional state after waking, with an extreme desire to return to sleep and feeling "foggy"
- Sleep drunkenness: Grogginess or disorientation after waking
- Brain fog: Cognitive functioning issues such as difficulty thinking, remembering, concentrating, or paying attention

## NARCOLEPSY & IH DIAGNOSIS

Because of low awareness and misperceptions, there is an average of 8 to 15 years between narcolepsy symptom onset and diagnosis. It is estimated that the majority of people with narcolepsy or IH are currently undiagnosed or misdiagnosed (common misdiagnoses include epilepsy, depression, and schizophrenia).

Narcolepsy and IH diagnoses typically rely on a 24-hour sleep study that includes a nighttime portion and daytime portion (multiple sleep latency test, or MLST). An NT1 diagnosis is mainly based on how quickly and frequently one's brain enters rapid eye movement (REM) sleep /dream sleep during these tests. NT2 and IH are differentiated by the number of times one enters REM sleep upon falling asleep during the MSLT.

If you suspect that you or a loved one could have a sleep disorder, consult an AASM board-certified sleep medicine doctor. To find a local sleep specialist or accredited sleep center, visit www.sleepeducation.org.

## NARCOLEPSY & IH TREATMENT

There is no cure for narcolepsy or IH. Treatment varies by person and may include:

- Wake-promoting, histamine-directed, or stimulant medications to increase alertness
- Nighttime medications to decrease excessive daytime sleepiness and cataplexy
- Antidepressant medication to decrease cataplexy
- Scheduled daytime naps

Currently only one treatment is FDA-approved for IH, and other medications approved to treat narcolepsy are commonly used off-label to manage IH symptoms.

Coping strategies vary widely by person and may include:

- Social support such as meet-up groups or social media
- Improvement in general health and wellness through sleep hygiene, diet, and fitness

To learn more about Project Sleep and our efforts to raise awareness about sleep health, sleep equity, and sleep disorders, please visit:

project-sleep.com

or email us at info@project-sleep.com.