

SCIENTIFIC INVESTIGATIONS

Perceived challenges in pediatric narcolepsy: a survey of parents, youth, and sleep physicians

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Study Objectives: To clarify the most common and problematic symptoms, psychosocial challenges, and comorbidities among youth with narcolepsy based on input from key stakeholders.

Methods: A nationwide cross-sectional survey of youth with narcolepsy, parents, and sleep physicians.

Results: Overall, 116 parents, 35 youth, and 30 providers completed the entire survey. Symptoms that were rated as most common and problematic by both parents and youth were (in descending order) as follows: daytime sleepiness, disturbed nighttime sleep, mood challenges, cataplexy, sleep-related hallucinations, and sleep paralysis. Most of the 18 queried psychosocial concerns were identified as substantial challenges by both adults and youth, including difficulty focusing and memory, school, worry and anxiety, diet and nutrition, lack of motivation, mood problems, and relationship problems. In contrast, while physicians did recognize some of these challenges, they rated medication side effects and driver's license issues as relatively greater challenges.

Conclusions: These data highlight the high prevalence of psychosocial challenges, discordance between physician and family ratings of challenges, and a high rate of medical comorbidities in youth with narcolepsy and support the application of a biopsychosocial framework in the assessment and treatment of pediatric narcolepsy.

Keywords: pediatric narcolepsy, biopsychosocial model, quality of life, sleep

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BRIEF SUMMARY

Current Knowledge/Study Rationale: Pediatric narcolepsy is a complex neurologic disorder in youth that can affect many domains of functioning. This study explored the most common and challenging aspects of life with narcolepsy per parent, youth, and physician report.

Study Impact: Our survey of key stakeholders demonstrates a high prevalence of psychosocial challenges in youth with narcolepsy and their families and bolsters the existing literature in support of a biopsychosocial framework for treating pediatric narcolepsy. Based on our results, we present a simple pre-visit worksheet for use in the sleep clinic as a tool to better identify a range of patient needs.

INTRODUCTION

Narcolepsy is a lifelong neurological disorder characterized by excessive daytime sleepiness and variable presence of rapid eye movement–related symptoms, including cataplexy (in type 1 but not type 2 narcolepsy), sleep-related hallucinations, and sleep paralysis.¹ Symptom onset is typically in the first or second decade of life, and while there has been a historical delay between symptom onset and diagnosis, an awareness of narcolepsy in youth is increasing. While medications that effectively improve symptom management are available, there is currently no known cure for narcolepsy. In addition, while these medications are typically helpful in decreasing symptom burden, some degree of residual sleepiness almost always remains.² Individuals with narcolepsy may have substantial medical and psychological comorbidities, as recently reported by Cohen and colleagues,³ with more than half experiencing psychiatric comorbidity, one-third endocrinopathies, and one-third obesity. Similar findings were reported by Black and colleagues,⁴ who also found the greatest excess of prevalence (increased

prevalence compared with a control group of patients without narcolepsy) of medical comorbidities within psychiatric disorders, followed by diseases of the digestive system. With respect to the pediatric population, Plazzi and colleagues⁵ reported associated comorbidities including rapid weight gain, precocious puberty, and significant burden of illness related to mental health and school performance. An analysis of claims data of over 1,000 pediatric patients with narcolepsy also identified significant comorbidity and health care utilization.⁶

Given the significant medical and psychiatric comorbidity associated with narcolepsy, optimal management might be best achieved through an interdisciplinary approach. A recent survey of adult patients with hypersomnia suggests that the symptoms for a sizable proportion of patients are not sufficiently managed by medications alone and that over 90% of patients used nonpharmacologic therapies.^{7,8} Likewise, an interdisciplinary approach within a biopsychosocial framework was recently advocated for by Graef and colleagues,⁹ who suggested several areas of future investigation, including obtaining key stakeholder input regarding important factors to consider in a

biopsychosocial model. While the previous work cited above has initiated a valuable line of inquiry, it has been limited by a variety of factors such as single respondent type, focus on adult patients, and lack of input from physicians and parents. In the current study we surveyed youth with narcolepsy, parents of youth with narcolepsy, and sleep physicians as a first step to better elucidate the most problematic narcolepsy symptoms, psychosocial challenges, and comorbidities.

METHODS

Participants

An invitation to participate in this research study was distributed via the WakeUpNarcolepsy website and social media platforms (ie Facebook, Twitter) for parents and youth, and via the PedSleep listserv for pediatric sleep physicians. The PedSleep listserv contains approximately 300 participants (including physicians, psychologists, and other pediatric sleep providers/researchers). Individuals were eligible to participate in the survey if they were the parent of a youth with narcolepsy aged 22 years or younger, a patient with narcolepsy between the ages of 12 and 22 years, or a sleep physician who provides care to youth with narcolepsy. The survey was housed in a REDCAP (Research Electronic Data Capture) database and was anonymous, so individual respondents would not be identified. This study was approved by the institutional review board at Children's Mercy Hospital.

Survey

The survey was developed for the current study with the input of a sleep medicine physician (D.G.I.), sleep psychologist (S.L.S.), and 2 representatives of WakeUpNarcolepsy (C.C. and L.J.). Separate parent, youth, and provider versions of the questionnaire included both multiple-choice and open-ended questions assessing respondent characteristics, narcolepsy symptoms, psychosocial challenges, comorbidities, treatment options, specialist care, and sleep clinic care. Items and responses were developed based on a review of the literature and were then reviewed and revised by the research team. For multiple-choice questions, frequency of queried symptoms/challenges were assessed (choices were 0, 1–3, 4–6, 7 days per week, or “I don't know”) as well as perceived severity (choices were “not a problem,” “a small problem,” “a medium problem,” or “a big problem”). Physicians were asked to rate frequency of challenges as well (“frequently,” “sometimes,” “rarely,” “I don't know”). In order to help preserve anonymity, we did not query or attempt to correlate individual youth and parent survey responses.

Data analysis

Descriptive statistics were used to examine the distribution of responses and are reported as percentages or means and standard deviations. Analyses were performed in IBM SPSS Statistics (IBM Corporation, Armonk, NY). When indicated, comparisons between categorical variables were performed with chi-square test and continuous variables with Student's *t* test. $P < .05$ was considered statistically significant. Responses to open-ended questions were coded and categorized

into representative themes using a grounded-theory approach. Grounded theory stipulates that the collected data are systematically examined line by line and key phrases are identified and coded into categories in order to identify overarching themes.¹⁰

RESULTS

Survey participation

The survey remained open for 2 months. During that time, 251 individuals opened the survey link and 230 proceeded to the survey questions. Of the parents, 116 (77%) out of 150 completed the entire survey. Of the youth, 35 (76%) out of 46 completed the survey. Finally, of the providers, 30 (88%) out of 34 completed the entire survey.

Respondent characteristics

Demographic characteristics of youth and parents are presented in **Table 1**. Most youth respondents were female (74%), and the age at the time of survey completion was a mean of 19.0 (2.6) years, 11.0 (4.2) years at the time of symptom onset, and 14.6 (4.4) years at the time of narcolepsy diagnosis. Similarly, parent respondents reported that their youth were predominantly female (58%), although their ages at the time of survey completion (15.4 [3.8]), symptom onset (9.7 [3.7]), and diagnosis (12.1 [3.5]) were slightly younger compared with youth respondents (likely reflecting the older minimum age for inclusion of youth respondents). Most youth respondents (94%) and parent respondents were White (88%), and the majority of patients had type 1 narcolepsy (80% youth report and 65% parent report). Physician respondents were 93% board-certified in sleep medicine, 80% in pediatrics, 36% in pulmonology, 7% in family medicine, and 6% in neurology. Physicians were generally very experienced, with 33% having more than 15 years of sleep medicine experience, 30% having 11–15 years, 23% having 5–10 years, and 13% having fewer than 5 years. Most (73%) practiced in an academic medical center, with 20% in private practice and 7% in solo practice. Almost all (90%) practiced in a setting where 75–100% of patients were pediatric patients. Sixty percent of physicians saw fewer than 5 patients with narcolepsy per month, 33% saw 5–10 per month, and 7% saw more than 15 per month.

Narcolepsy symptoms

Core narcolepsy symptoms are presented in **Figure 1** by respondent. Respondents were asked how frequently each symptom occurred per week and how much of a problem the symptom was for them. Both youth and parents reported excessive daytime sleepiness, disturbed nighttime sleep, and depression/mood challenges as the most frequent and problematic symptoms associated with narcolepsy. In contrast, cataplexy, sleep-related hallucinations, and sleep paralysis were less common or problematic. Sleep paralysis was more frequently reported by parents compared with youth (16% vs 3%; $P = .039$), and sleep-related hallucinations were more often rated a medium/big problem by parents compared with youth (30% vs 9%; $P = .010$). Otherwise, frequencies of reported

Table 1—Respondent characteristics.

| | Youth Report (n = 35) | Parent Report (n = 116) | P* |
|------------------------------------|-----------------------|-------------------------|-------|
| Sex, % | | | .198 |
| Male | 26 | 41 | |
| Female | 74 | 58 | |
| Other | 0 | 1 | |
| Current age of child, years | 19.0 (2.6) | 15.4 (3.8) | <.001 |
| Age of symptom onset, years | 11.0 (4.2) | 9.7 (3.7) | .080 |
| Age of narcolepsy diagnosis, years | 14.6 (4.4) | 12.1 (3.5) | .001 |
| Race, % | | | .830 |
| White | 94 | 88 | |
| Black | 3 | 5 | |
| Asian | 0 | 2 | |
| American Indian | 0 | 1 | |
| Prefer not to respond | 3 | 4 | |
| Narcolepsy type, % | | | .088 |
| Type 1 | 80 | 65 | |
| Type 2 | 20 | 35 | |

*P values were calculated from chi-square or t tests for frequency and continuous data, respectively.

narcolepsy symptoms between parents and youth were not significantly different. Reported sex was assessed in the combined youth/parent group, and there were no statistically significant differences noted in rated severity (medium/big problem) of core narcolepsy symptoms, although females rated sleep paralysis as more frequently occurring at least 3 days per week (17% vs 5%; $P = .004$). Finally, type 1 and 2 narcolepsy groups were compared and, unsurprisingly, participants with type 1 reported significantly more problematic (medium/big problem) cataplexy (50% vs 0%; $P < .001$) as well as more frequent cataplexy at least 3 days per week (42% vs 2%; $P < .001$), but no other statistically significant differences were noted.

Psychosocial challenges

Parents and youth were asked to rate potential psychosocial issues in terms of how challenging they felt they were in their lives (not a problem, small problem, medium problem, or big problem). **Figure 2A** shows the frequency of challenges rated as either a medium or big problem by youth or parents. The top 5 most frequently rated challenges by youth were difficulty focusing/memory, worry about the future, schoolwork, getting easily upset, and diet/nutrition. The top 5 rated challenges by parents were difficulty focusing/memory, schoolwork, getting easily upset, worry about the future, and diet/nutrition (tied with lack of motivation). Most of the queried psychosocial concerns were identified as substantial challenges by both adults and youth, with no statistically significant differences in how often individual problems were rated as “medium/big” challenges. We assessed for any differences by reported sex, and the only statistically significant difference found was that males reported a slightly higher prevalence of concern for injury (16% vs 13%; $P = .046$). Finally, comparing type 1 and 2 narcolepsy, we found that participants with type 2 narcolepsy had more problems with

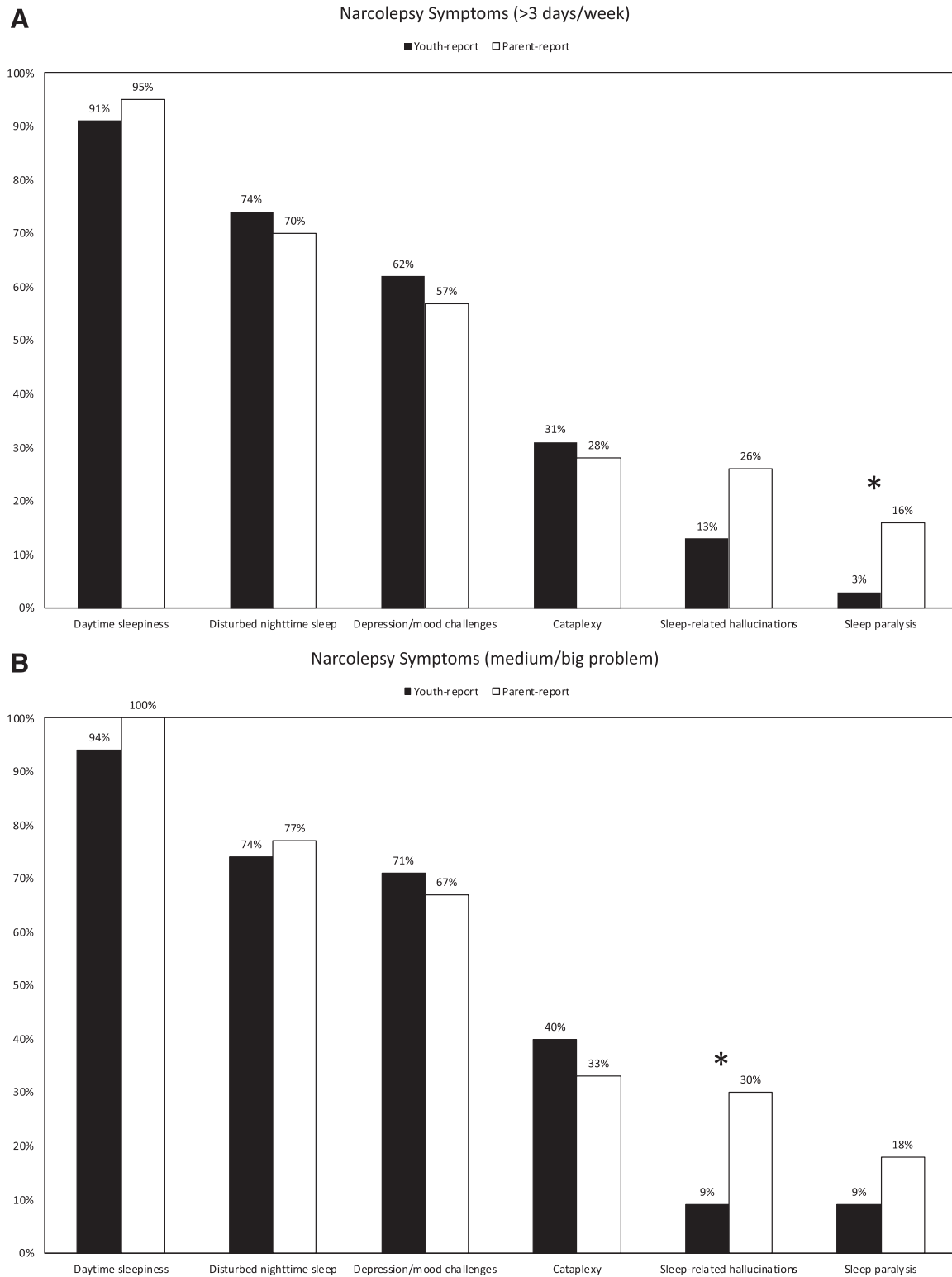
“lack of desire to do anything” (67% vs 62%; $P = .037$), whereas participants with type 1 narcolepsy had more concern for injury (20% vs 2%; $P = .003$); no other statistically significant differences were found.

Physicians were asked how frequently the same set of psychosocial concerns arise in their patients. The percentage rated as “frequently” (vs “sometimes” or “rarely”) is illustrated in **Figure 2B**. With the exceptions of driver’s license concerns, schoolwork, college/workforce, difficulty focusing/memory, and medication side effects, less than one-third of physicians rated the queried psychosocial concerns as “frequently” encountered.

Comorbidities

Comorbidities, either current or previously diagnosed, are represented in **Figure 3**. The most frequent comorbidities, reported by at least 20% of youth and parents, were vision problems, anxiety, depression, nasal allergies, overweight/obesity, eczema, asthma, chronic pain, gastroesophageal reflux disease/reflux, and restless legs syndrome/periodic limb movement disorder. Note that, in this section of the survey, responses represented a current or previous diagnosis rather than a participant-report-alone concern. Parents reported obstructive sleep apnea as a more frequent comorbidity of their children compared with youth (30% vs 3%; $P = .001$); otherwise, there were no statistically significant differences by respondent type. Female participants more frequently reported anemia or a bleeding problem (20% vs 5%; $P = .035$) and early-onset puberty (27% vs 14%; $P = .033$); otherwise, there were no statistically significant sex differences. Finally, participants with type 1 narcolepsy were less likely to have depression (40% vs 65%; $P = .005$), anxiety (51% vs 75%; $P = .004$), chronic pain (33% vs 52%; $P = .025$), nasal allergies (40%

Figure 1—Core narcolepsy symptoms rated by youth and parents.



Core narcolepsy symptoms rated by youth and parents by frequency (A) and severity (B). * $P < .05$.

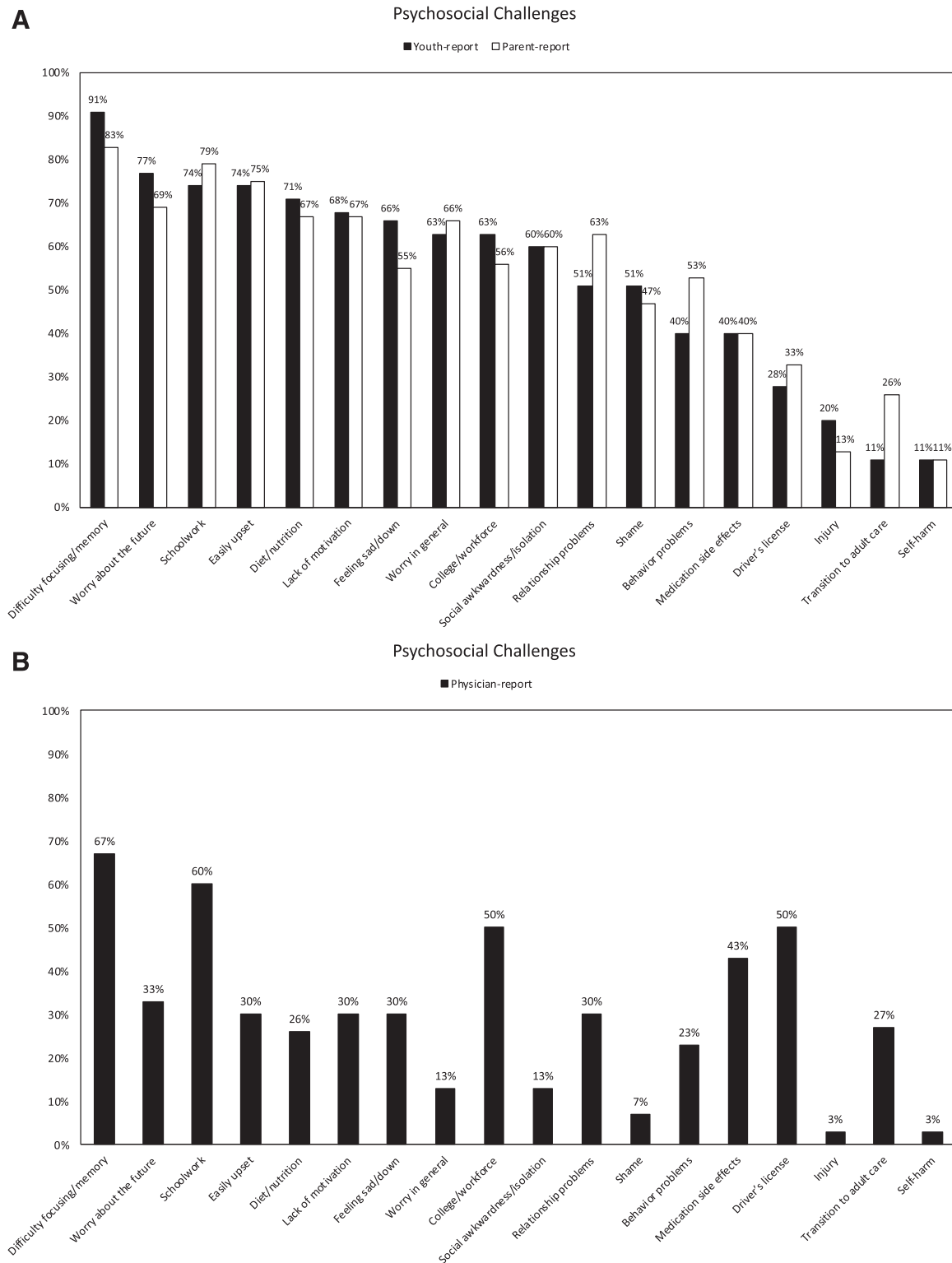
vs 63%; $P = .009$), problems with vision (50% vs 67%; $P = .049$), and anemia or a bleeding problem (11% vs 23%; $P = .047$) but were more likely to have overweight/obesity (47% vs 25%; $P = .012$).

Open-ended responses

Parents were asked, “If your child did not have narcolepsy, how would his/her life be different?” Major themes with representative quotes are presented in **Table 2**. Parents commonly felt

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Figure 2—Psychosocial challenges rated by youth, parents, and physicians.

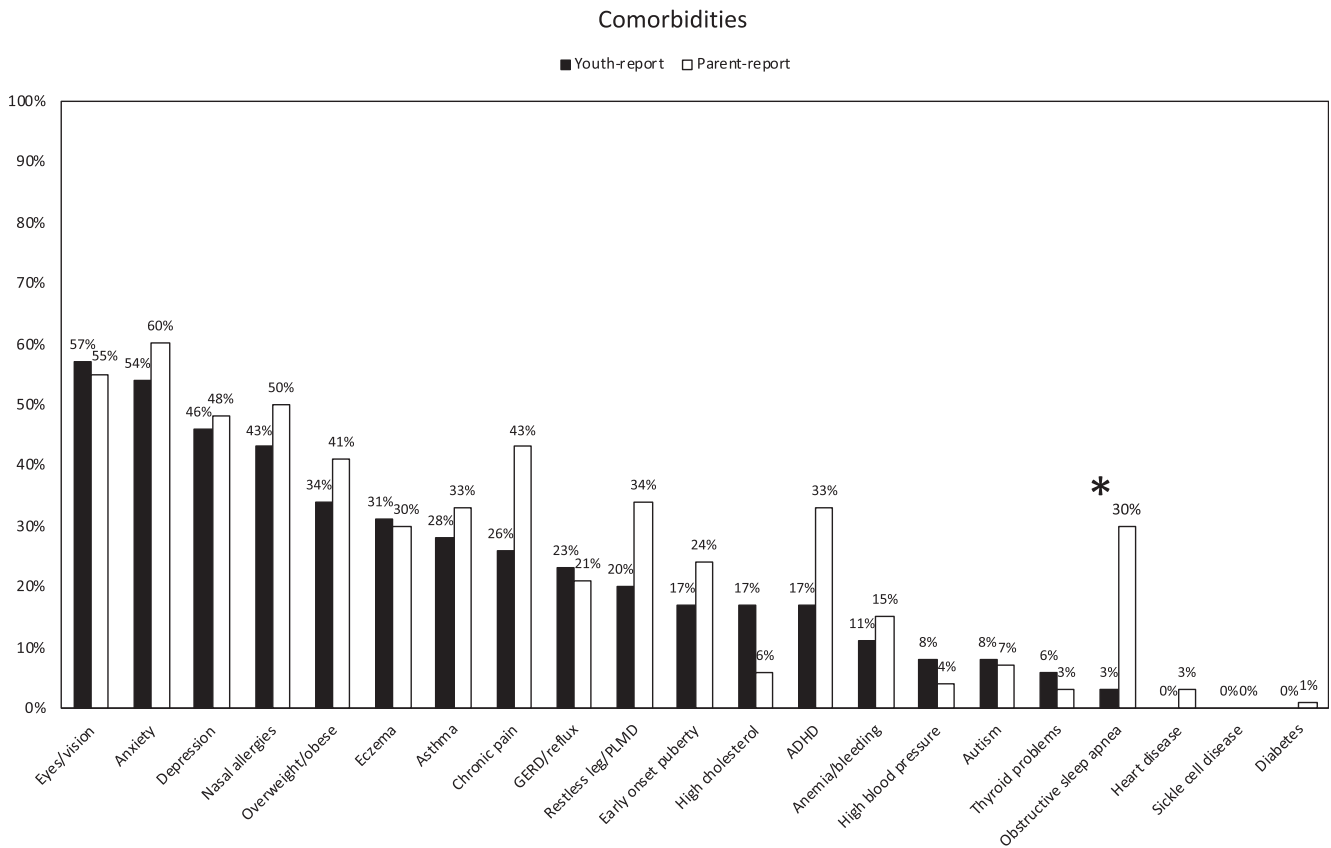


Frequencies represent challenges rated as either a “medium” or “big problem” by youth or parents (A) or challenges rated as “frequent” by physicians (B). There were no statistically significant differences between youth and parent responses in panel A.

that narcolepsy impeded their children’s ability to have positive social lives, friendships, and other relationships. Parents also reported that narcolepsy diminished their child’s ability to excel

in life and achieve their full potential. Parents expressed that their children struggled with low energy and activity levels and experienced depression, anxiety, and frustration. Parents felt

Figure 3—Comorbidities (either previous or current) reported by youth and parents.



*P < .05. ADHD = attention-deficit/hyperactivity disorder; GERD = gastroesophageal reflux disease; PLMD = periodic limb movement disorder.

that narcolepsy negatively impacted their children’s ability to lead a full, easy, or “normal” life. Finally, parents reflected on how narcolepsy altered their child’s life path, affecting their ambition and anticipated life course.

DISCUSSION

Narcolepsy is a lifelong disorder associated with substantial medical and psychosocial burdens, but the perspective of key stakeholders has not previously been examined directly. The current study helped elucidate the most frequent and bothersome aspects of disease in youth from the perspectives of parents, youth, and physicians. Our data highlight the high prevalence of psychosocial challenges, discordance between physician and family ratings of challenges, and high rate of medical comorbidities. Overall, our findings support the need for a biopsychosocial approach for the assessment and management of narcolepsy in youth.

Core symptoms of narcolepsy were assessed in the current study. Unsurprisingly, daytime sleepiness was the most frequent and problematic symptom. Notably, both disturbed nighttime sleep and mood challenges were identified as the second and third most frequent/problematic aspects of the illness by both youth and adults, respectively, ahead of cataplexy, sleep-related hallucinations, and sleep paralysis. Disrupted

nighttime sleep is a known feature of narcolepsy, with both self-reported and objective findings from studies demonstrating poor sleep quality and frequent, brief nightly awakenings.¹¹ In adolescents with narcolepsy, Parmar and colleagues found that poor sleep quality was associated with greater symptoms of depression, suggesting that improved sleep could be a target to help improve mood.¹² The high prevalence of mood challenges in our sample is not surprising, given the known association between psychiatric disorders and narcolepsy,^{13,14} with 1 recent study of youth with narcolepsy demonstrating higher rates of mood disorders along with greater inpatient and outpatient psychiatric visits.⁶

This study assessed psychosocial comorbidities beyond the core symptoms of narcolepsy. Both parents and youth rated a multitude of major challenges, with the majority (>50%) of both parents and youth rating 11 out of 18 possible as medium/big challenges. These findings are congruent with previous work demonstrating increased incidence of mood problems, social difficulties, attention problems, and behavioral problems.¹⁵ Difficulty focusing and attention problems were the most common problems in our cohort and reflect previous data demonstrating high levels of treatment-resistant attention-deficit/hyperactivity disorder symptoms in youth with narcolepsy.¹⁶ Problems with schoolwork and difficulty adjusting to college after high school were commonly reported in our sample. A prior case-control study demonstrated that youth with central

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Table 2—Themes and representative quotations from parents.

| Theme | Representative Response |
|----------------------------------------------------------------------------------|------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| Excelling/Successful— <u>Success</u> (n = 39) | "My son would have been able to achieve the goals he has wanted from the age of 6 on. He could have played Division 1 sports and joined the military. His family has a tradition of service and he wanted to follow in their footsteps. Although recruited for football, he was not able to play because he could not maintain the energy level necessary to play at that level." |
| | "If my daughter didn't have narcolepsy, she would be unstoppable. As it is, at 20, she's barely able to complete her career training (traditional college classes put her to sleep) and keep herself clothed and fed. She doesn't have the energy for any hobbies or the extracurricular activities she so enjoyed before this developed." |
| Easier Life/ <u>Full Life</u> /Normal Life/Not Missing Out on Life (n = 31) | "She would lead a full and vibrant life. I feel like her social awkwardness would not be like it is." |
| | "He would be able to attend school more frequently, he would be engaged more in sports, and he would be able to participate in normal teenage activities." |
| | "Hugely different. She does not do what normal teenagers get to do. She has no friends. Very isolated. We do not see her true abilities and capabilities. No one understands her struggle. Her life would be 'normal' she has no idea what 'normal' is." |
| Changed Life's Course/Lost Ambition— <u>Altered Life's Path</u> (n = 21) | "Narcolepsy changed his dreams, his hobbies, his circle of friends at a critical time in his teen life. His life is very different now." |
| | "He would have done a different line of work. He might be more active and spend more time with friends doing activities." |
| | "Narcolepsy changed everything. There is a distinct before and after. His personality changed, his physical body changed, he is less confident socially, and he is always struggling against time as he sleeps more at night and naps during the day. He's had to compromise so many things. And still, he manages his illness with grace and perseverance, and functions quite well overall." |
| Better Social Life/Friends/Relationships— <u>Positive Relationships</u> (n = 43) | "I believe he's had it since he was in 1st grade or so. He doesn't like to go anywhere, participate in sports, attend events, he's antisocial, dislikes school, has severe anxiety... He has a fraternal twin brother who is polar opposite. It's hard to say honestly because they could just be different." |
| | "Interact more with family/friends. Not be overweight and depressed. Not have to worry so much about her future like driving, relationships, college, supporting herself." |
| More Energy/Active/Less Tired— <u>Energy</u> (n = 35) | "He would be on the move consistently. When he didn't have narcolepsy he was always moving and never tired. Also the brain fog makes things harder for him." |
| | "It would be better because she would have more energy and the ability to focus and complete her work." |
| Happier/No Anxiety, Depression, Frustrations— <u>Happy</u> (n = 35) | "My daughter was recently diagnosed while exploring her college options. She then had a 6-month time frame where she didn't want to even think about college and quit school. Her anxiety levels would be lower and she wouldn't feel like she has to hide her diagnosis." |
| | "So much different she would be happy involved with her friends going on play dates outside playing. Doing sport. Just like she used to be before this horrible illness took over." |

"n" represents the frequency of responses coded to the theme.

hypersomnia had lower grades, stayed home more days from school, and scored lower on school-related quality-of-life measures.¹⁷ In contrast to physician ratings, ratings by parents and youth commonly reported diet and nutrition challenges. Youth with narcolepsy have known increased risk of obesity,¹⁸ and notably a study in adult patients with narcolepsy demonstrated improvements in daytime sleepiness with a low-carbohydrate diet.¹⁹ While physicians widely acknowledged frequent problems with attention and schoolwork, they disproportionately rated medication side effects and driver's license issues (delaying or not being allowed to get a driver's license) as more problematic compared with youth and parents. The issue of driving readiness is certainly of interest to sleep physicians, likely due to a desire to optimize driver safety for both the driver and public.²⁰ Physicians understandably may focus more on medication side effects as they typically work to titrate medication doses by balancing efficacy and side effects for an individual patient with narcolepsy.²¹

In terms of medical comorbidities, our survey identified several frequently co-occurring conditions. Over 30% of both youth and parents reported visual problems, anxiety/depression, nasal allergies, overweight/obesity, and eczema. Additionally, over 30% of parents also reported asthma, chronic pain, restless legs syndrome/periodic limb movement disorder, attention-deficit/hyperactivity disorder, and obstructive sleep apnea. Ocular manifestations of narcolepsy were first recognized in the 1920s, with diplopia and blurring being common findings, likely related to excessive sleepiness.²²⁻²⁴ Similarly, in a recent analysis of medical claims data, diseases of the nervous system and sense organs (eyes/ears) exhibited some of the largest excess prevalence compared with a control group.⁶ Claims data also showed excess prevalence of mood disorders, allergy disorders, obesity, and obstructive sleep apnea. Our finding of an increased risk of comorbid depression, anxiety, and chronic pain in children with type 2 vs type 1 narcolepsy is interesting and also reflected in the previously published claims

Figure 4—Proposed 1-page pre-visit worksheet for families and youth with narcolepsy.

PEDIATRIC NARCOLEPSY PRE-VISIT WORKSHEET

Diagnosis: *Do you have type 1 or 2 narcolepsy?*

- Type 1 narcolepsy (with cataplexy) Type 2 narcolepsy (without cataplexy)

Core Narcolepsy Symptoms: *Which of the following are currently medium/big challenges?*

- Daytime sleepiness Disturbed nighttime sleep Depression/mood challenges
 Cataplexy Sleep-related hallucinations Sleep paralysis

Other Challenges: *Which of the following are currently medium/big challenges?*

- | | | |
|-----------------------------------------------------|-------------------------------------------------|------------------------------------------------------|
| <input type="checkbox"/> Difficulty focusing/memory | <input type="checkbox"/> Worry about the future | <input type="checkbox"/> Schoolwork |
| <input type="checkbox"/> Easily upset | <input type="checkbox"/> Diet/nutrition | <input type="checkbox"/> Lack of motivation |
| <input type="checkbox"/> Feeling sad/down | <input type="checkbox"/> College/work | <input type="checkbox"/> Worry in general |
| <input type="checkbox"/> Social difficulties | <input type="checkbox"/> Shame | <input type="checkbox"/> Relationship problems |
| <input type="checkbox"/> Medication side effects | <input type="checkbox"/> Behavior problems | <input type="checkbox"/> Driving/Driver’s license |
| <input type="checkbox"/> Injury | <input type="checkbox"/> Self-harm | <input type="checkbox"/> Transitioning to adult care |
| <input type="checkbox"/> Weight/weight management | <input type="checkbox"/> Other _____ | |

Comorbidities: *Which of the following have you been diagnosed with?*

- | | | |
|--------------------------------------------------|-----------------------------------------------|----------------------------------------------|
| <input type="checkbox"/> Anxiety | <input type="checkbox"/> Eyes/vision problems | <input type="checkbox"/> Nasal allergies |
| <input type="checkbox"/> Depression | <input type="checkbox"/> Chronic pain | <input type="checkbox"/> Overweight/obese |
| <input type="checkbox"/> Restless leg/PLMD | <input type="checkbox"/> Asthma | <input type="checkbox"/> ADHD |
| <input type="checkbox"/> Obstructive sleep apnea | <input type="checkbox"/> Eczema | <input type="checkbox"/> Early onset puberty |
| <input type="checkbox"/> Other _____ | | |

Narcolepsy Medications: *What medications are you currently taking for narcolepsy?*

Behavioral Therapies: *What lifestyle or behavioral strategies are you using for narcolepsy?*

Visit Priority: *What is the most important thing you want to talk to your doctor about today?*

ADHD = attention-deficit/hyperactivity disorder; PLMD = periodic limb movement disorder.

data. Cohen and colleagues³ examined comorbidities in adults and youth with narcolepsy among an Olmsted County cohort in Minnesota, and found a high prevalence of anxiety, obstructive sleep apnea, depression, and obesity. Unlike these findings, however, endocrine (diabetes/thyroid) and cardiovascular (hyperlipidemia/hypertension) were less commonly reported in our pediatric cohort. Our results suggest that clinicians caring for youth with narcolepsy should remain vigilant for these

medical comorbidities in patients with either type 1 or 2 narcolepsy, beyond just classical weight gain and early-onset puberty.

Our findings have important clinical implications for those caring for youth with narcolepsy. These data bolster the growing literature showing that psychosocial challenges are commonplace and bothersome in youth with narcolepsy, and that clinicians may underestimate their importance to families. Furthermore, the fact that residual hypersomnia was commonly

reported in our sample suggests that physicians may benefit from additional education regarding residual symptom-management strategies. Clinicians may be better aligned with the goals of care by considering a structured assessment of impairments in various quality-of-life domains, including school, emotional functioning, and diet/nutrition. One model that encapsulates this approach is the biopsychosocial framework. Graef and colleagues⁹ recently reviewed currently available research and concluded that evidence supported the application of the biopsychosocial model in pediatric narcolepsy. They advocate for assessment across multiple domains, including biological (eg, pubertal development), psychological (eg, stress, self-efficacy, etc), social/contextual (eg, relationships, life events, etc), and sleep (eg, disturbed nighttime sleep, sleep schedule) in order to optimize outcomes. An interdisciplinary team for youth with narcolepsy may be helpful to guide the goals of care and address the common concerns highlighted in the current survey results.

We developed a pre-visit worksheet based on the results of the current survey (Figure 4). This low-tech and free tool is simple for families and youth to complete and may help identify challenges to be addressed during a visit from a range of possibilities across the biopsychosocial spectrum. Youth identify as having either type 1 or 2 narcolepsy, which allows the physician to also assess if they know the difference. The survey then queries the core narcolepsy symptoms, psychosocial challenges, and possible associated comorbidities. Respondents then list the medication and behavioral therapies they are utilizing to manage their narcolepsy. Finally, we ask them to identify the most important issue they want discuss during the visit (visit priority). Our hope is that this worksheet will serve as a tool to empower families and physicians to discuss the most pertinent issues in a more comprehensive fashion.

The current study has several strengths. First, our sample represents viewpoints from the perspectives of parents, youth with narcolepsy, and physicians. While, in many areas, all 3 groups were in agreement, in other areas important differences emerged, such as the relative importance of psychosocial challenges. Second, in order to preserve anonymity, we did not query respondents regarding geographic location, but the fact that the survey was distributed via a worldwide patient advocacy organization (WakeUpNarcolepsy) suggests that our findings are likely generalizable. Third, our qualitative approach to free-text answers allowed for a deeper and richer understanding of the patient and family experiences. Fourth, although not a validated instrument, the pre-visit worksheet we developed provides a practical and free tool that can be readily applied in clinical settings.

Despite its strengths, our study does have several important limitations. First, the survey nature of our study may lead to imprecise measurements for metrics such as disease comorbidity prevalence. That said, our findings are largely in line with previous studies demonstrating substantial psychosocial and medical comorbidities in youth with narcolepsy. Second, we did not have a control group to compare survey responses with those of our participants; this would have allowed for better assessment of excess risk associated with narcolepsy itself (as opposed

to being at risk for a mood disorder as a teenager without narcolepsy, for example). However, in a clinical context, an overall high rate of particular comorbidities or psychosocial difficulties suggests that these topics are significant and worth assessing. Third, while the current investigation included viewpoints from multiple groups (youth with narcolepsy, parents, and physicians), we did not link multi-respondent reports to the same individual patients so direct comparison within families was not possible. Fourth, while our utilization of WakeUpNarcolepsy as a distribution hub was a strength for geographic diversity, we cannot rule out the possibility of sampling and/or nonresponse bias. Individuals who chose to participate in survey research studies may differ in important ways that can limit the generalizability of results, such as socioeconomic status, educational level, age, and online social media engagement. In the current study, we are unsure of the effect of bias on the results; we speculate that individuals may have been more likely to participate if they had more frequent disease-management challenges. Fifth, we did not directly ask youth and parents if their challenges and concerns were being adequately addressed by their treating sleep physicians; inclusion of this would have strengthened the case for a more structured assessment. Sixth, while youth and parent respondents were similar in several characteristics, youth respondents were significantly older (Table 1). While we cannot rule out that this age difference in groups may have affected any differences in response patterns, we speculate that this age difference simply reflects the necessarily older minimum age for inclusion of youth participants. Seventh, in retrospect, it would have been optimal for the language of the question prompts between physician and youth/parent surveys to have been identical in terms of frequency categories for psychosocial challenges; this would have allowed direct statistical comparison rather than qualitative contrasts.

In conclusion, the current study helps to elucidate the most common and problematic symptoms and comorbidities among youth with narcolepsy reported by key stakeholders, including parents, patients, and physicians. Our findings strongly support a biopsychosocial approach to assessment and continued monitoring within pediatric narcolepsy, and we propose a pre-visit worksheet to help aid clinicians in this endeavor. Future studies evaluating the impact of biopsychosocial assessment and multidisciplinary treatment approaches are warranted.

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