"I want them to hear me. I want them to help me fix the problem so I can move on."
– Patient with NF1

Challenges of Managing NF1-Related Plexiform Neurofibromas (NF1-PN):

Understanding Perspectives of Patients and HCPs to Bridge Gaps and Optimize Care

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Introduction

Neurofibromatosis type 1 (NF1), also known as von Recklinghausen's disease, is a common autosomal dominant genetic condition that affects approximately 1 in 2500 births.[1] NF1 leads to a broad spectrum of clinical manifestations affecting many organs, including pigmentary abnormalities like café-au-lait macules and freckling, as well as the development of benign and malignant tumors.[2-4] A hallmark feature of NF1 is the formation of neurofibromas (nerve sheath tumors), including cutaneous neurofibromas and plexiform neurofibromas (PNs), also referred to as plexiform tumors. PNs develop in more than 50% of patients with NF1 and are considered significant complications because they can invade deep tissues. PNs may result in physical deformity and neurological dysfunction and can cause significant pain. They grow along internal nerve plexuses or peripheral nerve branches and have a 13% lifetime risk of transforming into malignant peripheral nerve sheath tumors.[2,4,5]

Understanding the experiences and challenges from the perspectives of patients, caregivers, and healthcare professionals (HCPs) is crucial for optimizing care. While there is currently no cure for NF1 or its associated tumors, FDA- and EC-approved treatments like mirdametinib and selumetinib are intended to stop growth and reduce the size of PNs. Despite these advancements, treatment is not always appropriate or successful, and the management of PNs can still be challenging for both patients and providers due to the substantial physical and emotional burden the PNs impose. Increased awareness of these manifestations and fostering interdisciplinary collaboration among medical specialists are essential for providing comprehensive and effective care for patients with NF1 and NF1-PN.[2,5]

The findings from the surveys administered to patients with NF1 and NF1-PN, their caregivers, and HCPs who manage these conditions uncovered the shared and divergent perspectives of each group. Commonalities and discrepancies were identified through assessment of the survey results. This project aims to raise awareness of patient, caregiver, and HCP perspectives, unmet needs, and challenges in managing PNs in patients with NF1. The authors provide solutions to help bridge gaps and facilitate better communication between patients, their caregivers, and HCPs to ultimately improve patient care.

Methods

The patient- and caregiver-reported survey was developed in partnership with NF Network, NF Midwest, NF Northeast, and the Texas NF Foundation. The survey that resulted from this collaboration served as the scaffold for generating the HCP survey.

Surveys were distributed to email lists from seven patient advocacy groups, as well as a distribution list of HCPs who treat patients with NF1.

Participants were anonymized; confidential information was not analyzed for use in the survey results.

Survey question types included yes/no, multiple choice, and open-ended response. Formal statistical analysis of the responses was not conducted.

While all patients have NF1, not all patients have NF1-PN. Therefore, the survey contained questions about NF1 and NF1-PN. Thus, the results described herein include a mixed use of the terms NF1 and NF1-PN.

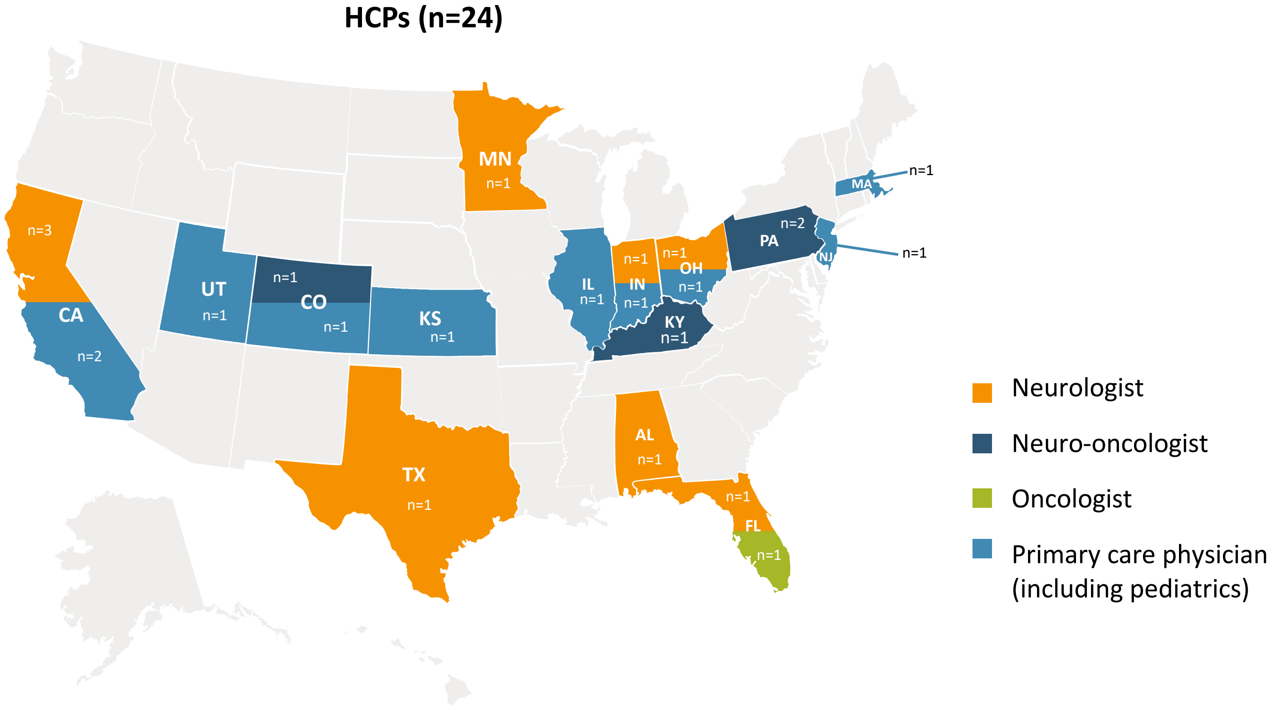
Demographics of Survey Participants

Twenty-four HCPs and 61 patients with NF1 or NF1-PN and their caregivers (n=24 patients, n=37 caregivers) completed the surveys (**Figure 1, A and B**).

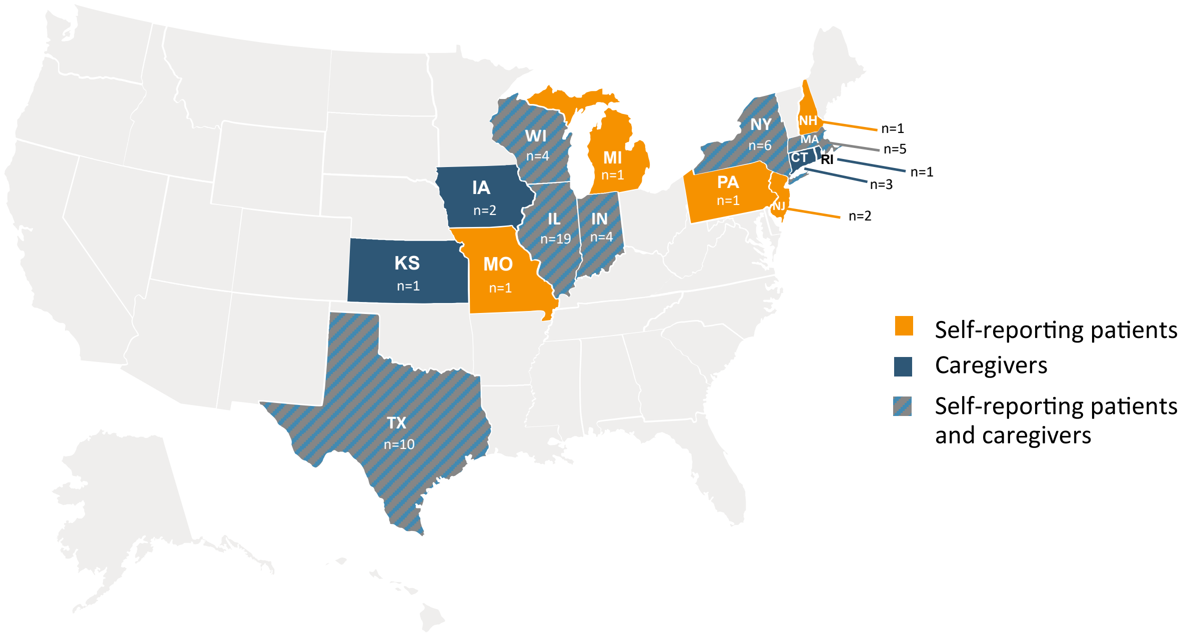
HCP respondents were affiliated with a Neurofibromatosis Clinical Network (NFCN) either in an academic setting (n=5) or community setting (n=5) or practiced in non-NFCN community settings (n=14). The most common co-specialties were neurology, neuro-oncology, and pediatrics (primary care). Overall, the clinicians were very experienced in managing patients with NF1. At the time of survey completion, HCPs managed 1 to 10 (n=11), 11 to 20 (n=7), or more than 20 (n=6) patients with NF1, and each had between 5 and 30 years of experience.

Figure 1. Geographic location of **(A)** HCPs (n=24) and **(B)** patients with NF1 (n=24) or their caregivers (n=37) who completed the survey.

A.



B.



Self-reporting Patients With NF1 (n=24) and Caregivers (n=37)

The age of patients ranged from 22 to 74 years in the self-reporting group, while the majority in the caregiver-reported group were aged <18 years. This difference in age groups may have impacted the answers to specific questions. Overall, diagnosis was generally made before the age of 20, and most patients received care at an academic medical center. If patients had insurance, they typically met with at least one provider (e.g., neurologist, oncologist, and primary care provider [PCP]). Overall, 65% of patients reported having had, or currently having, a PN. Adult patients (the majority in the self-reporting group) had a higher frequency of PNs (79%, n=19, self-reported; 51%, n=19, caregiver-reported; **Table 1**).

Table 1. Patient demographics and characteristics.

	Patient-reported (n=24)	Caregiver-reported (n=37)
Male, n/total n (%)	3/23 (13)[a]	–
Female, n (%)	20/23 (87)[a]	–
Median age, years (range)	52 (22 to 74)	–
<18 years of age, n/total n (%)	0	15/22 (68)[a]
≥18 years of age, n/total n (%)	24 (100)	7/22 (32)[a]
Age at NF1 diagnosis, n (%)		
≤2 years	7 (29)	30 (81)
3 to 10 years	9 (38)	6 (16)
11 to 18 years	6 (25)[b]	1 (3)
>18 years	2 (8)	0
Prior or current PN, n (%)	19 (79)	19 (51)
Prior treatment for PN (surgery or oral medication), n/total n (%)	8/16 (50)[a]	5/18 (28)[a]

[a]Missing survey responses. [b]n=1 reported as a self-diagnosis. PN, plexiform neurofibroma.

Understanding the Challenges of Managing NF1 and NF1-PN: HCP Versus Patient Experience

The management of NF1 and NF1-PN, both as an HCP and a patient, is complex. In addition, HCPs and patients face an array of medical and logistical concerns and challenges in a healthcare environment that includes coordinating with specialists and negotiating with insurance carriers.

HCPs highlighted that the complexity of illness, comorbidities, and the need for the involvement of multiple specialists make treatment selection a challenge. They specifically mentioned that learning difficulties are prevalent and noted that many patients also have comorbid mental health conditions like attention-deficit/hyperactivity disorder, which can impact their daily lives.

> *"[There are] so many organ systems that can be affected, resulting in multiple comorbidities, many of which I cannot resolve." – HCP*
>
> *"Pain, discomfort, and mental health issues interfere with the ability to care for oneself and function in everyday activities. Psychological and emotional disturbances are significant." – HCP*

HCPs pointed out the lack of effective treatments that can reverse or slow progression, and the absence of innovative therapies to achieve a cure. Even with the medicines currently available for NF1-PN, HCPs noted several potential barriers to effective management, including coordination of care among subspecialists, patient compliance and adherence to treatment, as well as issues with insurance coverage.

> *"Trying to remember what kind of monitoring they need regularly and making sure everything gets done [is challenging]." – HCP*

The NF1 community perspective focused on how NF1 can affect patients' daily lives: chronic pain and associated limitations on activities, the influence of visible lesions and tumors on self-consciousness and social interactions, and visual and other sensory impairments that negatively impact self-esteem and interpersonal relationships (**Figure 2, A and B**).

> *"PNs affect every part of my daily life—from the pain, to how people look at me." – Patient*
>
> *"[PNs] can make me feel self-conscious about my appearance and can be difficult to talk about if [people] ask questions or notice my tumors." – Patient*

Some of these issues lead to psychological challenges like anxiety and depression. In children especially, learning difficulties and educational impacts were common, including missed school days due to frequent medical appointments and treatments.

> *"Constant awareness that she is 'different' and [she] has social and emotional challenges." – Caregiver*
>
> *"Lack of [possibility of participating in] many sports, learning challenges, communication, and social challenges." – Caregiver*

Both patients and their caregivers, as well as HCPs, recognized that NF1 has a major impact on a patient's daily life (**Figure 2C**). While HCPs focused on the challenges associated with the coordination of care, patients and caregivers listed the emotional burden, as well as medical care issues, as being most significant.

Figure 2. Challenges to activities of daily life and management of NF1 as reported by **(A)** HCPs, **(B)** patients, and **(C)** caregivers.

The Impact of PNs: HCP Understanding Versus Patient-Lived Experience

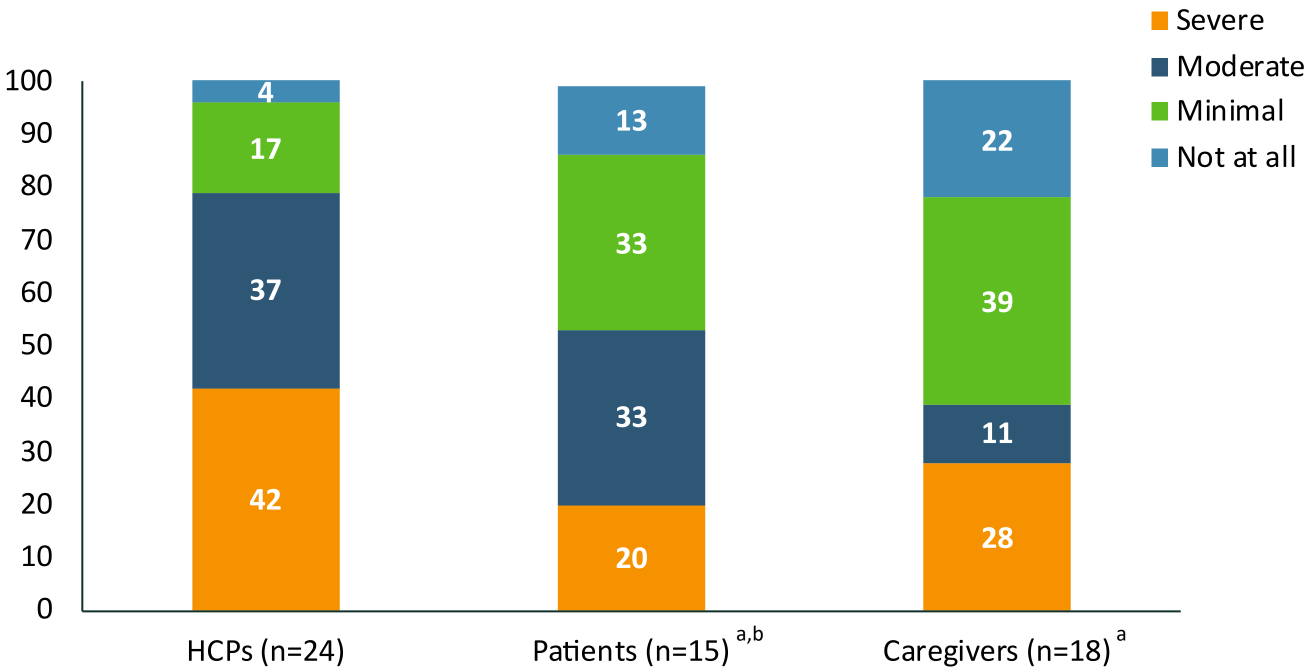
When patients develop PNs, treatment and management needs can become very challenging and complex. The majority of HCPs surveyed (79%) perceived that PNs moderately or severely affect patients' lives (**Figure 3**). The size and location of the PNs vary, and HCPs report that a small percentage of patients are only minimally affected by them.

The chronic nature of PNs, difficulty in managing large tumors, and concern about overlooking newly developing PNs were cited as the greatest challenges for HCPs. In addition, HCPs noted the uncertainty around how best to treat PNs, citing the lack of established treatment guidelines, the morbidity associated with available surgical options, and the challenges in managing the pain often linked to PNs as major sources of concern.

For patients living with PNs, daily activities and social interactions can be impacted. Spinal tumors often result in mobility issues and are noted as particularly painful. Facial PNs have a considerable impact on body image and self-esteem. Generalized pain and itching are also commonly cited symptoms.

> *"'It's [PNs] around my spine. It hurts tremendously 24/7.'" – Caregiver, quoting patient*
>
> *"A plexiform tumor is the reason my whole left side is smaller than the right." – Patient*
>
> *"One causes almost daily pain in my right [side] neck and shoulder. The pain sometimes wakes me up at night, which is part of why I only get 4 to 5 hours of sleep a night." – Patient*

Figure 3. How plexiform tumors affect daily life: comparing perspectives of patients, caregivers, and HCPs.



[a]Missing responses. [b]Due to rounding, values add to 99%.

Although both HCPs and patients listed pain as a major issue, they sometimes use different terms, which may impact optimal understanding of one another. For example, some patients wish doctors would take symptoms potentially related to PNs, like itching or abdominal pain, more seriously. Patients and caregivers used terms that emphasize the emotional impact, including "discouraging," "frustrated," "stressful," and "scared." Similarly, HCPs reported patients using words that convey their emotional distress, such as "alone," "confused," "isolation," "misunderstood," "defeated," and "distressing." Patients may also describe symptoms that are difficult to classify clinically.

> *"I say that they [PNs] feel weird, and I can feel them more when I have headaches." – Patient*
>
> *"I can't put it in words. It's all kinds of pain twisted together and stretched out for what seems like forever." – Patient*
>
> *"They [PNs] cause her a lot of pain. They're gelatinous and jiggly, itchy, lumpy, red, and irritated." – Caregiver*

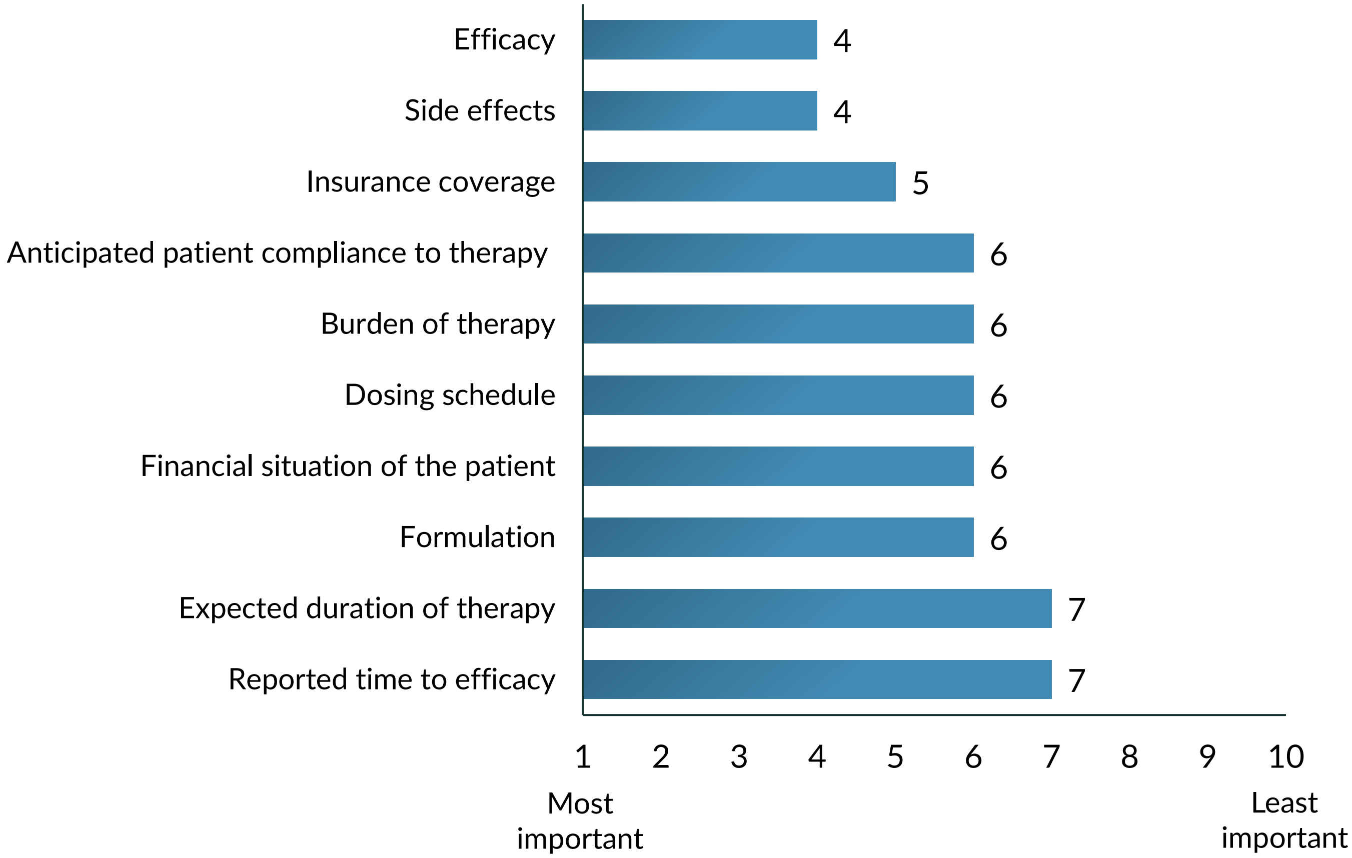
Perspectives on Treatment for NF1 and PNs

When asked whether their patients with NF1-PN received surgery, systemic therapy, or pain management, many HCPs responded that they utilize all modalities. Several HCPs indicated they primarily use pain management for most of their patients, while surgical therapy is used in ≤50% of cases. The most important considerations when choosing a pharmacologic treatment were efficacy and side-effect profile, although insurance coverage and cost also played a significant role (**Figure 4**).

Forty percent (n=13) of patients reported that they had undergone treatment (e.g., surgery, oral medication) to manage their plexiform tumors. The main reasons for undergoing treatment were to manage pain or to manage challenges related to the specific location of the tumor. Current treatments can stop growth and reduce the size of plexiform tumors, although some patients may not be able to receive surgery, either due to access or the potential for permanent nerve damage. Patients are hopeful that in the future, a medical treatment that can remove the entire tumor could be possible. They stressed the importance of pain control and symptom management. Compared with HCPs, patients described more concerns with managing their PNs.

> *"I feel that more needs to be done to remove tumors, no matter how small, when the patient wants them removed. It shouldn't be a matter of insurance coverage." – Patient*
>
> *"[The PN] is on the bottom of my foot, [and I am] worried about pain and shoes." – Patient*

Figure 4. Average ranking (1 being most important, 10 being least important) of HCPs' pharmacologic treatment consideration factors for patients with NF1-PN.[a]



[a] Question was posed only to HCPs.

The Importance and Challenges of Communication Between HCPs and Patients

Some HCPs (n=6) wished patients and caregivers would take specific symptoms more seriously, such as early pain, neuropathy, subtle neurologic signs of weakness associated with PNs, and developmental delays observed with NF1 in general. Approximately half of HCPs felt their patients adequately express the impact of their symptoms during consultations, but felt that some patients may feel embarrassed while doing so. Other HCPs felt that patients may underestimate the seriousness of symptoms, avoid mentioning the emotional impact, or have unrealistic expectations of treatment outcomes.

> *"We see both patients misunderstanding the seriousness of their issues, but also sometimes catastrophic world views about their long-term outlook." – HCP*
>
> *"They may not tell me about their emotional state as often as they could. They normalize their isolation/depression/anxiety." – HCP*

Overall, physicians prefer when patients are engaged at appointments and offered several questions that should be addressed to develop a collaborative care plan and set expectations.

> *"How do we measure success with treatment? What are their [patients'] treatment priorities, and how can I help?" – HCP*
>
> *"How can we manage this as a team? How can they [patients] be more engaged and keep updated with treatments and follow-up?" – HCP*

Sixty-seven percent (n=16) of patients who self-reported felt adequately heard by their doctors. Notably, this finding was numerically higher when caregivers completed the survey (89%, n=33). However, caregivers acknowledged that patients can sometimes encounter communication challenges when attempting to express their experiences to their doctor. Patients and caregivers agreed that fully expressing the challenges they face can sometimes be difficult, with potential reasons being fear of bad news, limited appointment time, and simply being used to their problems (normalization of symptoms).

> *"I don't tell the doctor how much it inhibits and depresses me." – Patient*
>
> *"You can't explain nerve pain. It hurts so much. It brings you to tears. It is a pain that lasts a very long time." – Patient*
>
> *"It's their normal. So many times, they don't realize they are impacted so much." – Caregiver*

Patients want their symptoms to be taken seriously by their doctors, even if they are not "typical" symptoms, such as itching, gastrointestinal complaints, and dizziness. They mentioned that it is often hard to remember to mention symptoms that only occur sporadically.

> *"Because NF is so different for everyone, he just doesn't know what to say or ask or even know anyone who is like him." – Caregiver*
>
> *"He feels like the answer he always gets is, 'That's what NF does.' (Try to cope with it.)" – Caregiver*
>
> *"It's hard for me to convey or ask for that consideration, given that my concerns are almost entirely aesthetic, and my case of NF is so much less detrimental/damaging than other NF patients who face much more serious challenges." – Patient*

Fragmented care and the need for repetition of previously discussed topics were also cited as problems.

> *"It is hard sometimes as a parent managing and advocating when you have to be the intermediary between the local primary care or local ER and experts." – Caregiver*
>
> *"Have the entire [care] team together at one time versus individual appointments." – Caregiver*
>
> *"As a child, my parents were the ones the doctors were speaking to and not me, the patient, so I have a lot of gaps in knowledge and a lot of questions that might not have answers." – Patient*

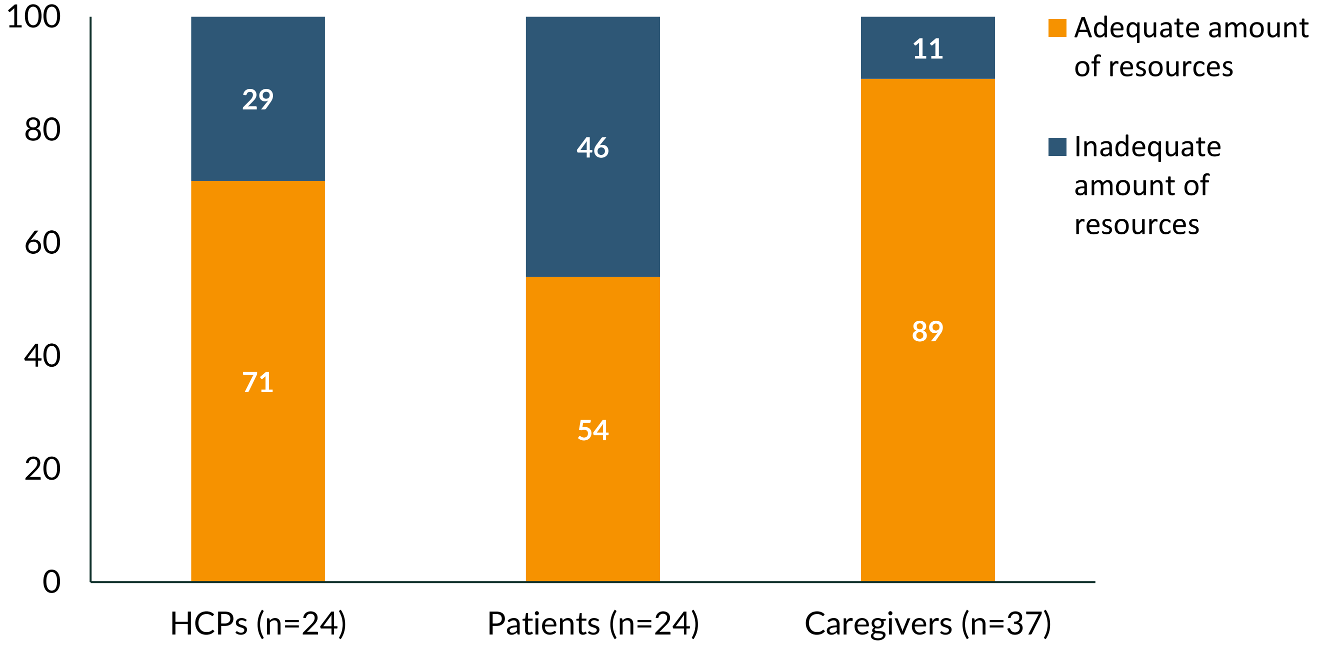
Access to more members involved in the care team, such as nurses, physician assistants, social workers, and PCPs, helps patients and their caregivers feel supported. A checklist with potential questions that could be sent to patients ahead of visits and continued access to HCPs via email or a patient portal were also proposed by patients as helpful tools. Both HCPs and patients mentioned support groups (or camps for children with NF1) and access to psychologists and other mental health therapists who understand the burden of NF1 as potentially helpful resources.

Disconnect on Education and Available Resources for NF1 and NF1-PN

Several HCPs expressed that they have limited exposure to information regarding treatments and limited access to clinical trials, which can complicate their ability to care for patients with NF1. HCPs were asked whether they felt they had adequate resources and information about NF1 to share with their patients. Most HCPs reported that they have adequate resources and information about NF1, but they also acknowledged that specific information about treatments and clinical trials from reliable, noncommercial academic medical centers was insufficient (**Figure 5**).

When patients and caregivers were asked about the adequacy of resources and information provided by HCPs, responses were mixed. Among self-reporting patients, 54% (n=13) felt it was adequate, while 89% (n=33) of caregivers felt it was adequate. Missing resources identified by patients and caregivers included up-to-date information on treatments and research, reliable, noncommercial information sources, mental health support, and information about local NF1 support groups.

Figure 5. Perception of information availability about NF1.



Requests for enhanced educational materials may align with the findings that most HCPs surveyed believe their patients are only moderately educated about PNs (**Figure 6**). Overall, nearly 50% of patients and caregivers (n=61) find their HCPs to be extremely well educated about NF1. However, some mentioned the HCPs' gaps in current understandings about NF1 and ongoing clinical trials, as well as a lack of connection with other knowledgeable specialists (**Figure 7**).

Figure 6. HCPs' perception of patients' knowledge regarding PNs.

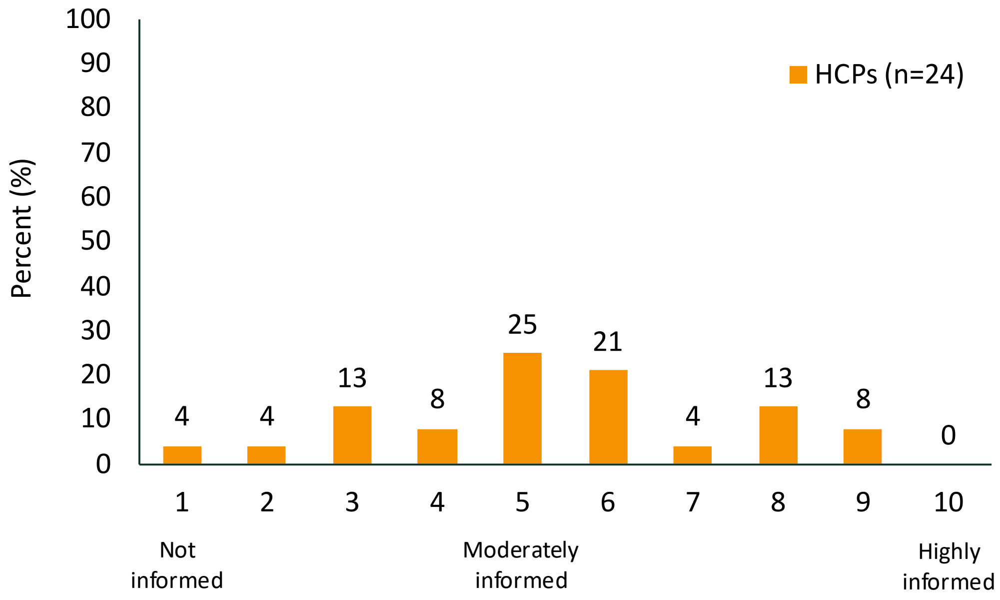
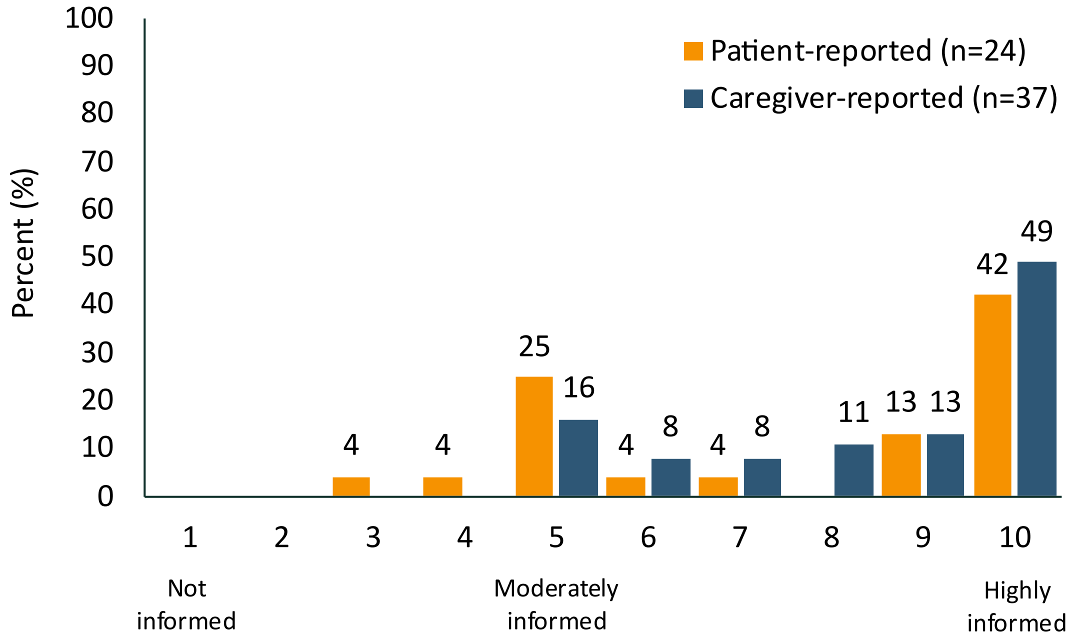


Figure 7. Patient and caregiver perception of HCPs' knowledge regarding NF1.



Unmet Needs: Bridging the Gap Between Providers and the NF1 Community

Despite the advances in treatment for PNs, there remains a need for disease-modifying therapies for all persons with NF1, but especially those with symptoms and disfigurement caused by PNs. Many HCPs report that their adult patients need improved pain and symptom management, as until recently there were no FDA- or EC-approved treatment options for adults with NF1-PN. They also emphasized the need for enhanced support systems (emotional, social, and educational), access to knowledgeable mental health providers, and prioritizing developmental and quality-of-life concerns. Furthermore, HCPs cited the necessity for additional NF1 clinics and dedicated centers of excellence with access to multidisciplinary teams and adequate insurance coverage.

Some of the same issues were mentioned by patients and their caregivers. They stated that better pain control and improved management of associated mental health issues were needed. Additional explanation regarding disease progression and treatment options, as well as solutions to medical challenges such as tumor growth and prognostic uncertainties are also desired. And they wished for improved coordination of care and support during transitions from pediatric to adult care settings, as transitional challenges are common and often associated with anxiety.

> *"Getting used to seeing a new doctor after being with the previous one for a long time [is difficult]." – Patient*
>
> *"My doctor wants me to transition soon [from pediatric to adult HCP]. I don't have insurance, but once I do, I want to go to him one last time." – Patient*
>
> *"It is hard to find a good neurologist who understands NF1 and the complications it could present." – Caregiver*

Conclusion and Recommendations

These surveys aimed to identify the communication gaps between HCPs and patients with NF1 and NF1-PN. It is important to consider how disconnects in patient-provider communication (e.g., language barriers, use of medical terminology, accurate symptom description) may be affecting clinical outcomes. Empathic communication methods, including patient-friendly explanations that demonstrate understanding of the patient's needs, as well as the use of friendly, nonverbal cues, have been shown to improve patient health outcomes.[6] In an effort to help understand the patient experience, specific questionnaires to assess the impact of NF1-PNs on patients' quality of life have been developed.[7]

Improved patient education resources that appeal to different age groups and treatment needs can help reinforce and motivate patients while also providing a sense of advocacy and ownership over their treatment plan. Similarly, HCP concern over lack of effective therapeutics to reverse or slow the progression of the condition highlights a potential lack of awareness or understanding about new treatment options and emphasizes the need for up-to-date HCP educational resources as well.

It is also important that access to multidisciplinary care teams and NF clinics be facilitated to help address the complexity of NF1 and NF1-PN management and improve coordination of care. This should include increased support for mental health and psychosocial needs from clinicians trained in rare diseases or chronic health conditions. Strengthening the transition from pediatric to adult care is a universal need for patients with chronic diseases as they approach adulthood. Frameworks to ensure a smooth and informed transition from pediatric to adult care have been developed but need to be more broadly implemented.[8]

The results from these surveys represent a unique resource that includes real-life HCP, patient, and caregiver perspectives. However, the surveys are not without limitations. In the instances in which caregivers answered on behalf of the individual they care for, it is not possible to know how the patient perceived those answers, which could potentially explain some of the discrepancies in communication satisfaction between HCPs and patients. Some answers might have addressed other aspects of NF1 and were not specific to PNs. Furthermore, to maintain patient anonymity, specific analyses like home location, ethnicity, and additional details about patients <18 years of age are beyond the scope of this survey. Also, the survey does not specifically explore the proximity of patients to their treatment centers, and distance may contribute to some patients having limited access to treatment resources. Additional research is essential to further understand the lived experiences of individuals with NF1 and NF1-PN and the effectiveness of various treatment approaches from the patient's perspective. This necessitates collaboration among HCPs, patients, caregivers, researchers, and patient advocacy groups to address the identified gaps to facilitate long-term outcomes and improve the lives of those affected by NF1 and NF1-PN.

HCPs may not fully appreciate the daily impact of the disease on the patient's quality of life. These disconnects point to an urgent need for strategies to improve dialogue and shared decision-making. Based on these survey insights, the authors offer the following recommendations for consideration to help bridge gaps and define unmet needs to improve patient care:

Patients/caregivers

- Utilize a checklist or tracking diary for patients, and ensure they can share and update this information with HCPs through clinic visits or a patient portal
- Strengthen educational support systems and ensure patients and caregivers have access to mental health providers experienced with NF1 or rare diseases
- Adopt broader implementation of structured transition pathways to facilitate a well-informed transition of care from pediatric to adult medical services[9]
- Foster collaboration among HCPs, patients, patient advocates, and industry partners to provide comprehensive, up-to-date resources on treatments, research opportunities, and local NF1 support groups

HCPs

- Develop comprehensive treatment guidelines for PNs, including recommendations for monitoring, pain management, surgical intervention, and use of current therapies
- Increase HCPs' utilization of specialized questionnaires to assess the impact of NF1 and NF1-PN on quality of life among patients and their caregivers
- Create an NF1 and NF1-PN data dictionary or lexicon to harmonize the way these conditions and symptoms are described in order to foster more uniform documentation of clinical symptoms

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