

Real-World Patient, Advocate, and Caregiver Perspectives on Amyloidosis: Awareness, Knowledge Gaps, and Psychosocial Impact

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BACKGROUND

- All types of amyloidosis are rare, progressive, potentially fatal disorders that are difficult to diagnose and treat.^{1, 2}
- Correct diagnosis of amyloidosis is often delayed due to the nonspecific nature of symptoms and misdiagnoses leading to delays in treatment initiation.²⁻⁴
- This delay results in considerable medical and financial burden on patients and their caregivers.⁵⁻⁷
- While standardized quality-of-life instruments used in randomized controlled trials yield helpful data for healthcare professionals providing care for patients with amyloidosis, they often do not fully capture the patient perspective on living with the disease.^{8, 9}
- Further, the COVID-19 pandemic placed new and sometimes unanticipated burdens on patients, caregivers, and medical personnel.¹⁰⁻¹²
- We report here the results on patient, advocate, and caregiver (PAC) perspectives during the initial stages of the COVID-19 pandemic from an online community of people involved with amyloidosis.

OBJECTIVE

- To augment our understanding of amyloidosis by collecting and sharing the PAC experience.
- To fill knowledge gaps that can continue to raise disease awareness and understanding from the patient and caregiver perspective.

MATERIAL AND METHODS

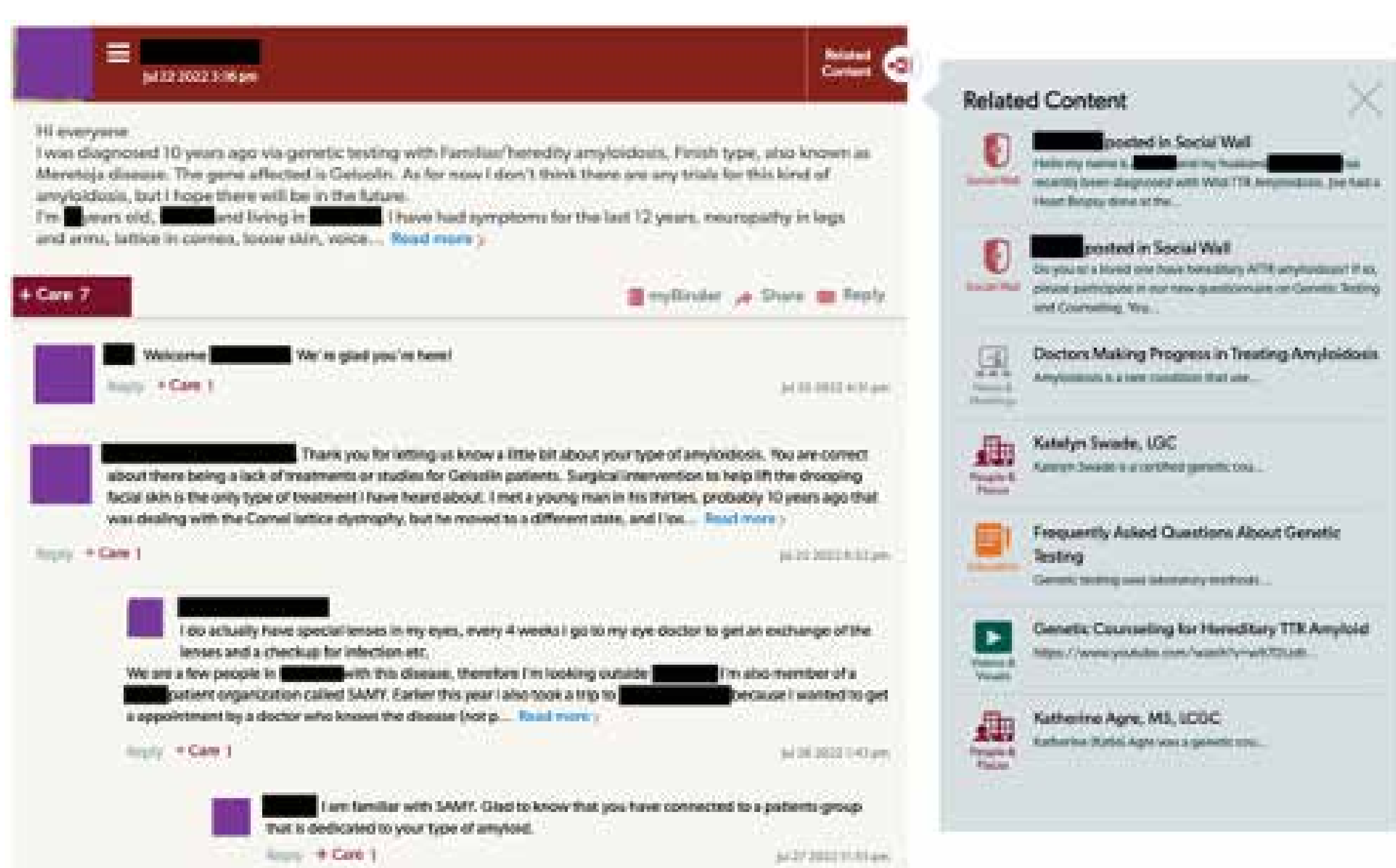
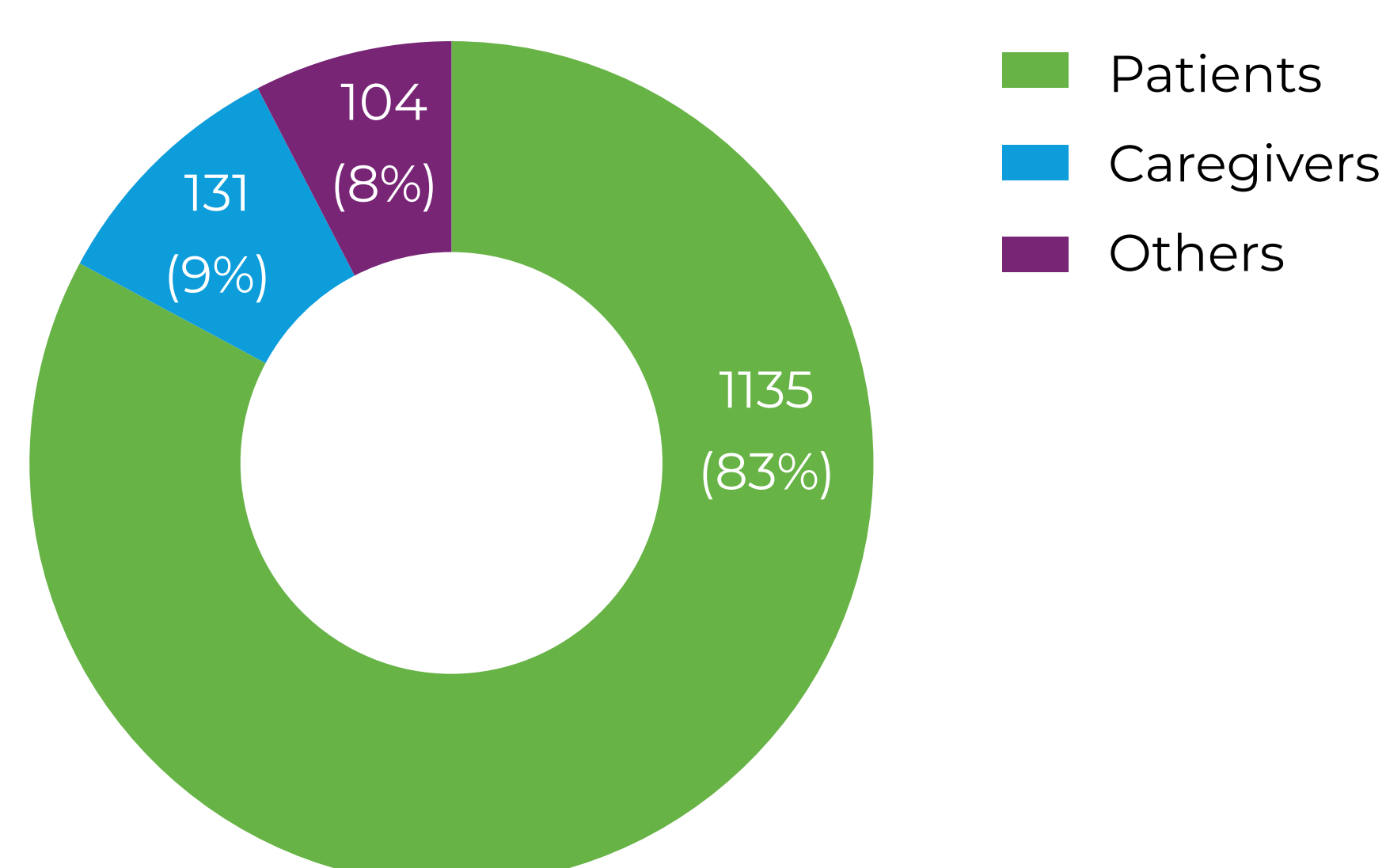
- Outreach to the amyloidosis community was initiated through oneAMYLOIDOSISvoice.com, an online, amyloidosis-specific educational platform.
- People were encouraged to respond to polls and surveys via
 - A complementary social media outreach plan on Facebook, Instagram and Twitter.
 - Featuring these opportunities on the Social Wall of the platform for registered members to provide their perspectives.
 - Direct email to registered members.
- Over the last 2 years, 17 single-question polls and 7 short surveys were posted.
- In addition, >1000 Social Wall posts by members of the amyloidosis community were evaluated to capture real-world insights into the impact that amyloidosis has on their lives.
- All data were aggregated and anonymized to preserve privacy of respondents.
- The data are reported as summary statistics.

RESULTS

RESPONDER INFORMATION

- For the 17 polls, there were a total of 1370 responses of which 1135 (83%) were from patients, 131 (9%) were from caregivers, and the remaining 104 (8%) were from others (Figure 1A).
- The 7 surveys comprised a total of 64 questions answered by 73 patients, 3 caregivers, and 1 other person.
- The Social Wall (Figure 1B) consisted of free-form comments and posts and identified as "Post," "Reply," and "Reply to Reply."
 - There were 1099 messages of which 233 (21%) were original posts, 501 (46%) were replies to posts, and 365 (33%) were replies to replies.

Figure 1. (A) Categories of Respondents, (B) Sample of the Social Wall Discussion



PATIENT, ADVOCATE, AND CAREGIVER (PAC) INPUT VIA POLLS AND SURVEYS CAN PROVIDE INSIGHTS INTO WHAT IS IMPORTANT TO THE AMYLOIDOSIS COMMUNITY

SUPPORT AND FUNDING

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DISCLOSURES

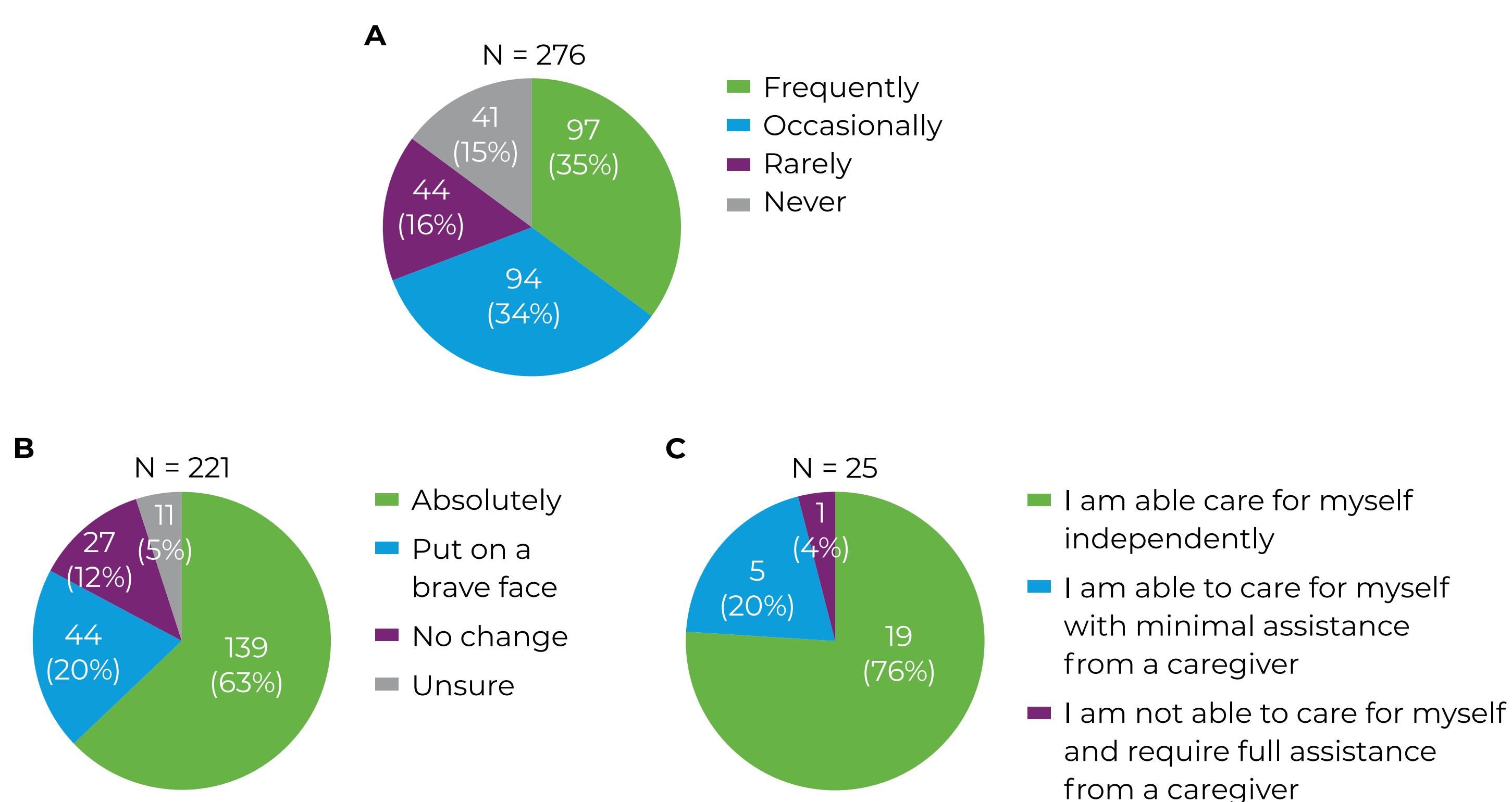
MIN, PS, and DJD report no conflicts of interest. KMA reports consulting or advisory board participation for Alnylam Pharmaceuticals, Arbor Biotechnologies, Eisai, Ionis, and Pfizer. CK reports consulting/educational activities for Ionis Pharmaceuticals/Akcea Therapeutics, Inc, Alexion, Alnylam, Arganx, Biogen, CSL Behring, Medscape, and Sanofi Genzyme, and has received research grants from Sanofi Genzyme and Ionis Pharmaceuticals/Akcea Therapeutics. JZ consulting for Alnylam Pharmaceuticals, Amgen, Bristol-Myers Squibb, Intellia, Janssen Biotech, Regeneron Pharmaceuticals, and Takeda Oncology and receiving trial support from Caelum Biosciences, Inc, which was acquired by Alexion, AstraZeneca Rare Disease.

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IMPACT OF DISEASE

- We queried the community on the impact of amyloidosis on their personal lives, their reaction to being diagnosed with amyloidosis, and their ability to care for themselves.
- Despite that 70% of respondents reported that amyloidosis had a negative impact on their personal lives (Figure 2A), 63% of respondents reported that the diagnosis made them feel stronger about themselves (Figure 2B) and 76% responded that they were able to take care of themselves independently (Figure 2C).

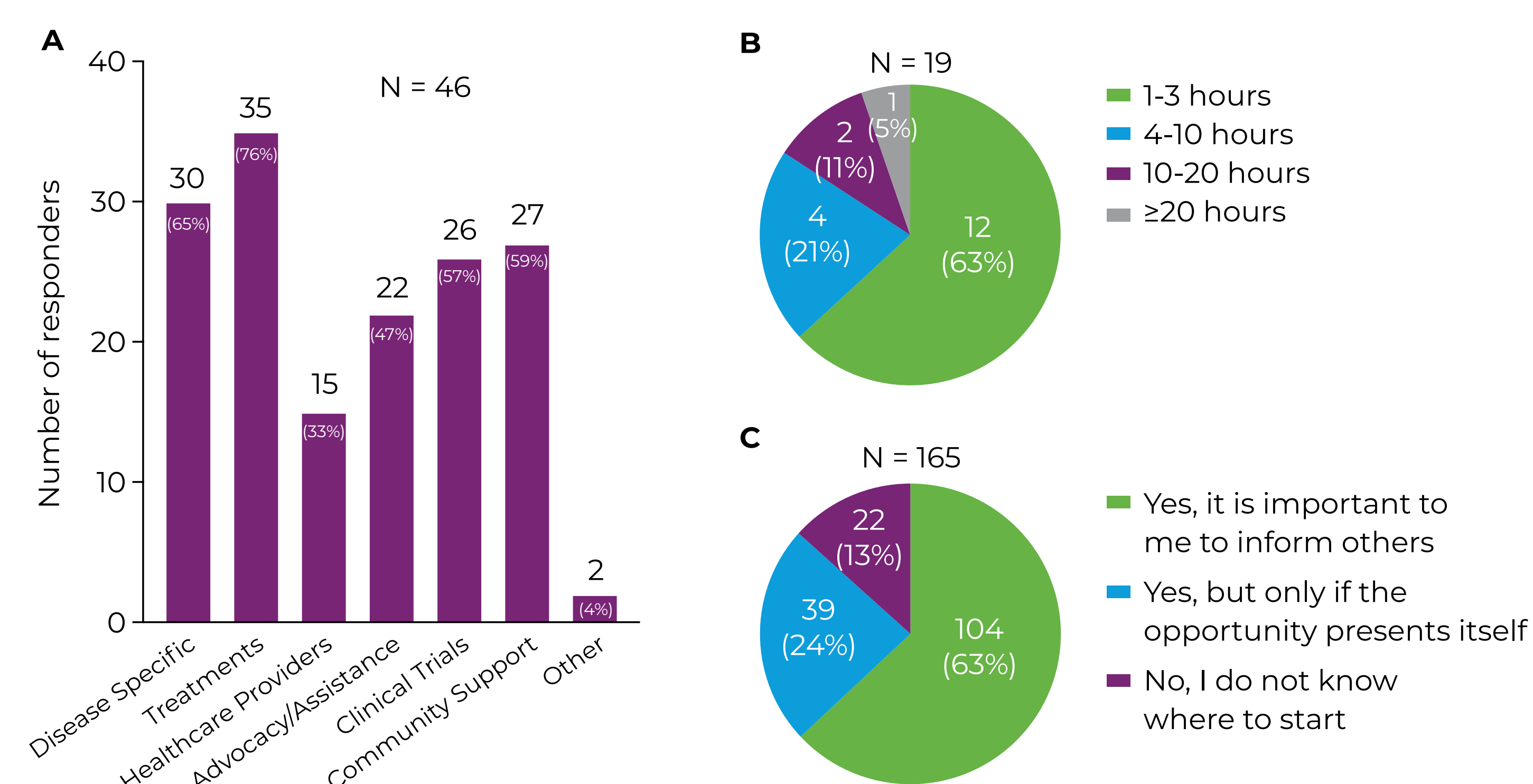
Figure 2. (A) Impact of Amyloidosis on Personal Life (N = 276), (B) Personal Feeling Toward Managing Disease (N = 221), and (C) Patients' Ability to Care for Themselves (N = 25)



AWARENESS

- We queried the community on the kind of information they sought, how much time they invested each week in searching for this information, and whether they have tried to spread awareness of the disease in their communities.
- The amyloidosis community appears to be motivated to learn more about their disease, especially disease-specific information, treatments, clinical trials, and community support (Figure 3A).
- The community is willing to invest time in research with 63% spending up to 3 hours each week and 16% spending more than 10 hours each week on this endeavor (Figure 3B).
- The community is engaged and 63% responded that it is important to them to inform others about the disease and a further 24% are willing to do so if the opportunity is presented (Figure 3C).

Figure 3. (A) Information Sought by Community Members (N = 46), (B) Time Invested on Research Each Week (N = 19), and (C) Willingness to Inform Others (N = 165)



IMPACT OF COVID-19

- Community members posted their experiences during the COVID-19 pandemic on the community Social Wall.
- These data were extracted from the Social Wall posts and were not obtained from a poll or a survey.
- Examples of the posts on the impact of COVID-19 are given in Table 1.

Table 1. Examples of the Impact of COVID-19 on Community Members

"I had been going to a hospital, but when they wanted to switch to home infusions, [commercial program] stepped in and helped facilitate that. I had three home infusions at a no out-of-pocket cost."
"OK. It's good that they stepped up so you could continue to get your meds without being subjected to COVID exposure. There is a big push in Washington to have home infusions covered under Part B. I hope that they are able to convince the powers that be that traveling to an infusion center poses an incredible hardship on many patients, no matter what their disease is."
"...Yes, at treatment today it was recommended that I get the 3rd COVID shot due to being immunocompromised. From what I've read, it's kind of a toss-up as to whether those in our position have gotten any or enough protection from the first two shots, so I'm glad that the 3rd shot is being allowed."
"Let me give newly diagnosed AL Amy patients a positive message. "There is light at the end of the tunnel. Without any symptoms, I was diagnosed with AL Amy with kidney involvement, in early 2020. My oncologist admitted that he had little experience with Amy. He referred me to Mayo Clinic for a bone marrow transplant. Covid stopped that idea cold."
"An update on my husband; his WBC, platelets and neutrophils went to normal range on Day +29 which was 4/6/22. Last week on Thursday he got the [commercial name] antibody COVID shot since he has to wait 90 days posttransplant to get revaccinated for COVID. Everything is going good and he will return to his oncologist at Kaiser to check his light chains."

LIMITATIONS

- Responses are limited to those in the amyloidosis community who have access to the internet and participated in the polls, in the surveys, or on the Social Wall.
- The respondent characteristics are self-reported; there was no validation of their role in the community.
- The varying numbers of respondents to polls and surveys make it difficult to perform statistical analyses but do allow for us to draw general conclusions.

DISCUSSION AND CONCLUSIONS

- A more complete understanding of the amyloidosis experience should include a disease impact assessment and its challenges from the patient perspective.
- Our ability to treat the whole patient relies on our ability to understand the patient journey and its far-reaching implications on the daily lives of those impacted by the disease.
- The data reported here reflect the real-world experiences and concerns of patients with amyloidosis and their caregivers.
 - These data provide insights into what is important to the PAC community.
- Single-question polls and short surveys in a disease-specific online community offer a quick and easy method to gain insight into life with amyloidosis that has heretofore been unpublished.
- While the results can only be reported descriptively, they nonetheless are highly informative for those treating patients with amyloidosis.
- This is the first step in obtaining comprehensive PAC input into living with amyloidosis.

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