

Publications based on patient surveys that quantify and qualify patient experiences (PEs) fill a critical information void, thus improving regulatory decision making and patient-centered care.



Background

- First person PEs are often missing from medical literature.
 - When shared, PEs are usually shared as patient-reported outcomes using instruments that do not capture disease or treatment-specific impacts as experienced by the patient.¹
- In rare diseases where there is a general paucity of information, PEs are even more scarce.²
- The United States Food and Drug Administration encourages patient experience data as part of its approval process, especially in rare diseases.
- In rare and other orphan diseases, PEs can fill knowledge gaps and exert significant influence, offering invaluable insights that help in trial design, shape clinical practice, and foster patient-first healthcare.
- We examine the potential reach and impact of PEs derived from surveys on the understanding and management of rare diseases, specifically focusing on neuromyelitis optica spectrum disorder (NMOSD), amyloidosis, and thyroid eye disease (TED).

Methods

- We designed disease-specific surveys with the input of patients with NMOSD, amyloidosis, and TED.
- The surveys included unrestricted, open text options to capture patients’ perspectives including experiences and narratives.
- Surveys were fielded via SurveyMonkey.
- The blinded data were aggregated and analyzed. The results were published in peer-reviewed journals or presented at medical congresses.³⁻⁵
- Patients’ permission was obtained to use their direct quotes.
- Article metrics from the journals were used to assess impact of each publication.

Results

Table. The Impact of PE Publications (Data as of April 15, 2024)

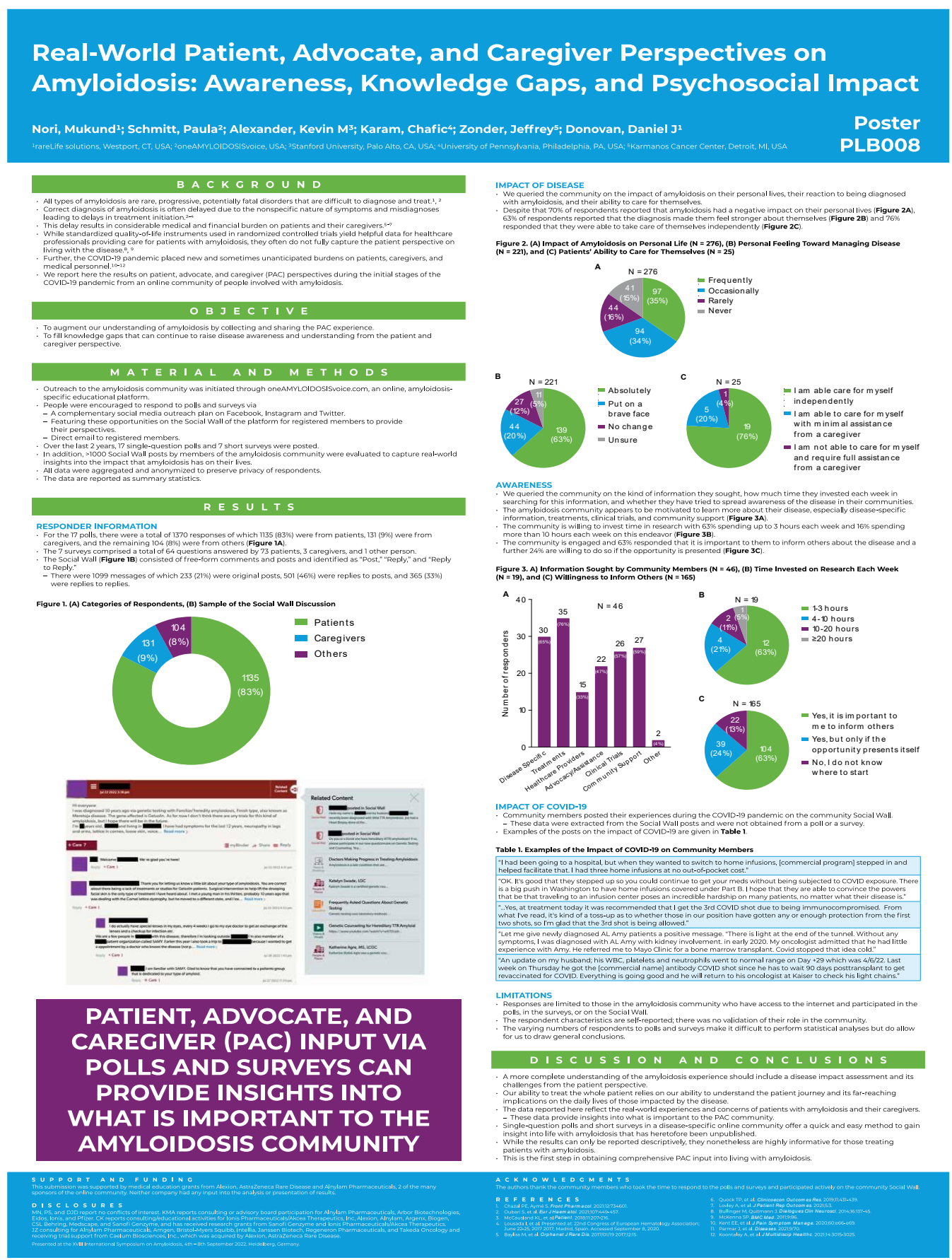
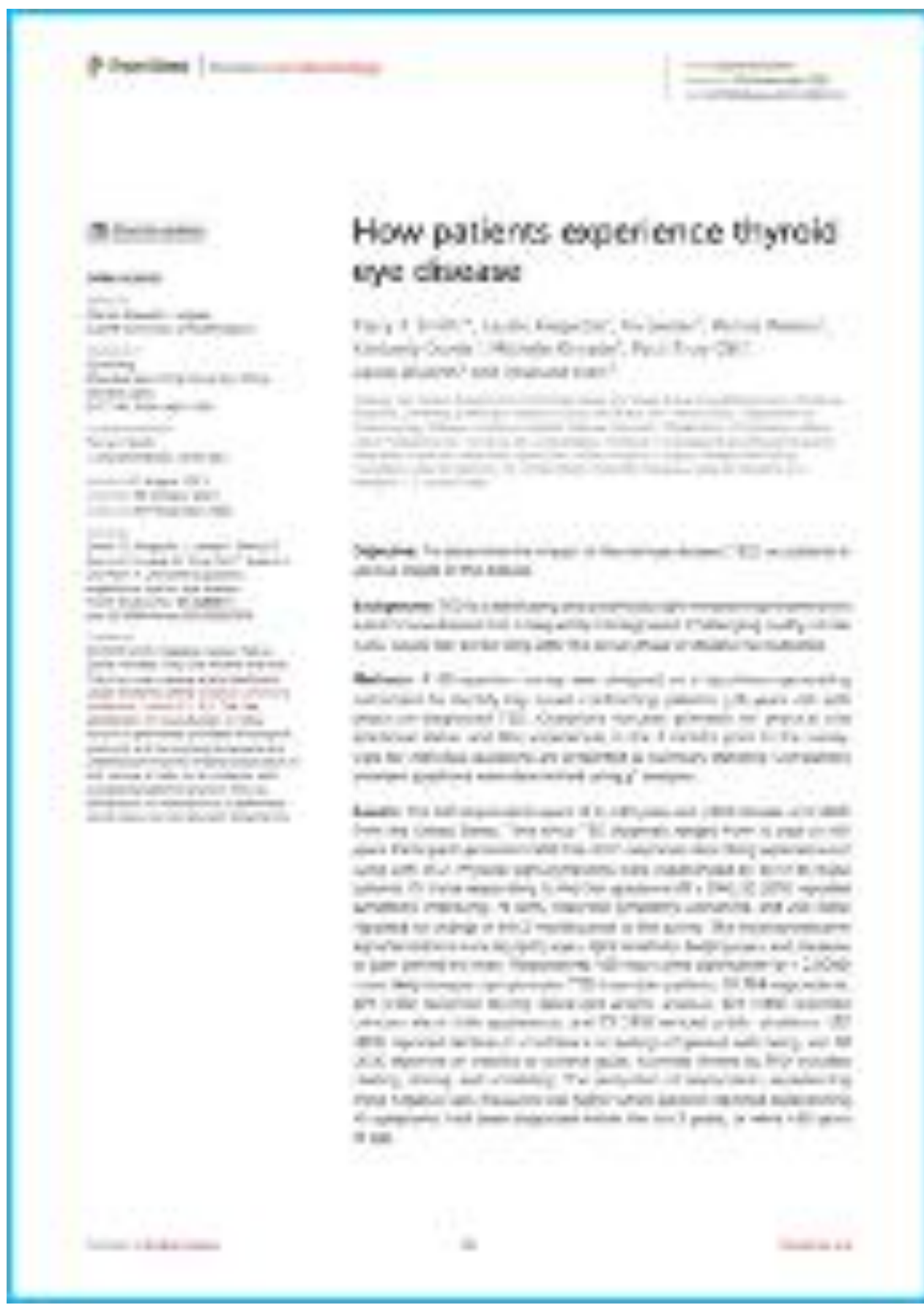
Article topic	Publication date	Views (rank ^a)	Downloads (rank ^a)	Citations	Altmetric
Patient roles	Apr 2022	NA	4028 ^b	3	3
NMOSD	Sep 2022	4870 (67%)	1869 (83%)	5	11
TED	Nov 2023	2441 (48%)	621 (39%)	0	9

Abbreviations: NA, not available; NMOSD, neuromyelitis optica spectrum disorder; TED, thyroid eye disease.

^aRank indicates the percentage of all published articles in *Frontiers* journals viewed/downloaded less than the article being evaluated.

^bTop 6 in the history of the journal. NA, not available

- Patients contributed to the development of each survey and served as coauthors.
- Inclusion of PEs was identified as a valuable feature by reviewers.
- The article on the global role of patients is among the top 6 most read articles in *Future Rare Diseases*.
- The articles had relatively high numbers of views and downloads within a short period and continue to be downloaded daily.



Conclusions

- Publications based on patient surveys that quantify and qualify PEs fill a critical information void, thus improving regulatory decision making and patient-centered care.
- Inclusion of patient experiences and narratives can
 - have a positive impact on reviewers and
 - amplify the value of a publication.
- Inclusion of patients as authors can
 - increase the robustness of information,
 - add authenticity and relatability, and
 - impact a broader audience.
- A more complete understanding of PEs will ultimately help improve care and treatment.

References

1. Uslar V, et al. *Endocrinol Diabetes Metab.* 2022;5:e357.
2. Walewski J, et al. *Expert Opin Orphan Drugs*, 2019;7:513-519.
3. Nori M, et al. *Future Rare Dis.* 2022;2:2.
4. Delgado-Garcia G, et al. *Front Neurol.* 2022;13:966428.
5. Smith TJ, et al. *Front Endocrinol.* 2023;14:1283374.