

# Rare disease journey in snapshots: real-world insight from online rare disease communities

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## Background

- Patient- and caregiver-provided journey information is sorely lacking in rare diseases
- A recent article revealed that only 0.2% of the medical literature database is devoted to rare disease, and there was nothing—zero—from the patient or caregiver perspective<sup>1</sup>
- We think that needs to change
- A primary objective of our onevoice educational communities is to publish helpful insights into the patient and caregiver experience\*
- One of the techniques we use are single-question polls to gather real-world data that advance disease understanding from the perspective of those impacted most
- Results are shared with those who participate on the platforms, and we actively seek to publish helpful insights in vehicles that will extend the reach of those insights

## Objective

- To understand and share the rare patient journey as collected through single-question polls in online rare disease communities

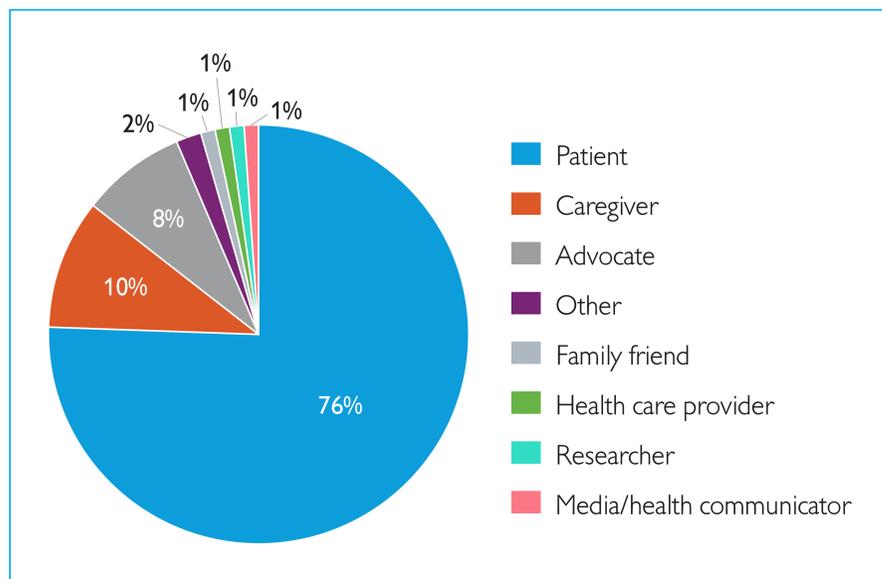
## Methods

- A single-question polling technique was employed to engage digital community members with quick, relevant questions, providing users with immediate feedback
- We designed and fielded polls in 5 online communities devoted to each of the following diseases: sickle cell disease, amyloidosis, familial adenomatous polyposis, Graves' disease, and X-linked hypophosphatemia
- Polls appear on the home page, which is accessible to members only
- Members respond via a simple radio button
- We reviewed selected anonymized responses within and across communities

## Results

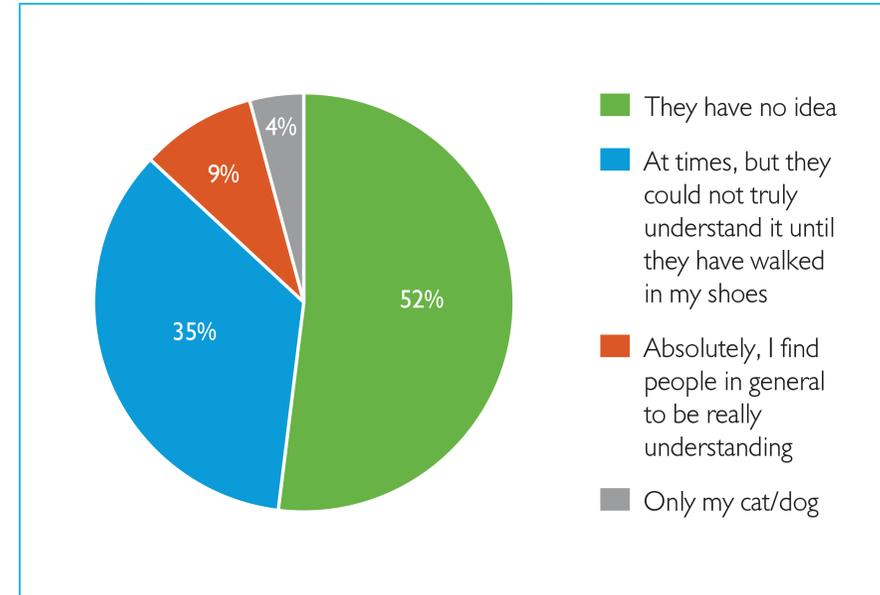
- There were 5211 accumulated responses across all polls
- We analyzed 3 poll questions that ran in all 5 rare disease communities
- A total of 826 responses are included in this assessment
- Patients and caregivers constituted over 85% of total respondents (Figure 1)

Figure 1. Demographics of Poll Respondents



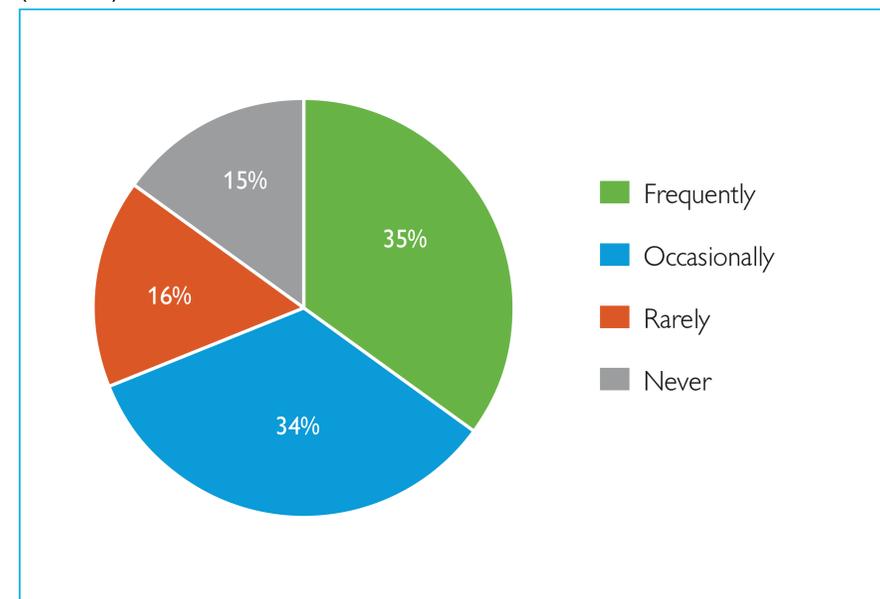
Feelings of isolation are often associated with living with rare disease, particularly among patients and caregivers. Indeed, nearly 90% of respondents with a rare disease shared that others do not understand the challenges of their disease, which contributes to feelings of loneliness (Figure 2).

Figure 2. Do You Think Others Outside of Your Close Friends and Family Understand the Challenges You Face in Your Rare Disease Journey? (N = 329)



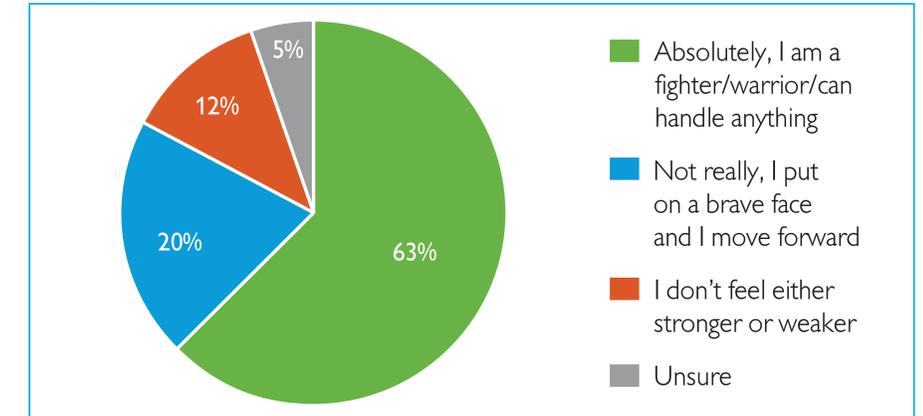
Those emotions can have a severe impact on a patient's ability to forge meaningful relationships. Nearly 70% of patients with a rare disease reported that their personal relationships were impacted by their disease. Only 15% indicated that they were not impacted (Figure 3).

Figure 3. Has Your Rare Disease Ever Interfered in Your Personal Relationships? (N = 276)



On the flipside, oftentimes with adversity comes strength, and our poll results reflected that belief as well. More than 60% of patients with a rare disease felt that they were stronger because of and/or as a result of their disease (Figure 4). This was particularly true in the sickle cell community, where 81% of respondents identified as a sickle cell warrior.

Figure 4. Do You Feel That Managing Your Rare Disease Experience Has Made You a Stronger Person? (N = 221)



## Limitations

- Respondents identify their role in the community when they join and we accepted them at their word without requiring validation
- Responses were not evenly distributed across all disease communities, which could skew the results

## Discussion

These results only begin to scratch the surface of the psychosocial impact a rare disease can have on patients, caregivers, and other stakeholders. It is only by first asking questions that we can obtain, compile, and analyze responses and begin to understand the rare disease journey. These results need to be shared with all stakeholders, including physicians, to obtain a holistic understanding of the patient. The onus is then on health care professionals (HCPs) to treat all aspects of the disease affecting the patient to better manage and address their needs. Accordingly, it is the authors' fervent wish that we all publish this information to ensure that we can continuously improve our current understanding of these complex diseases.

## Conclusions

- Snapshots of rare disease online communities via single-question polls, though individually narrow in scope, offer a real-world, real-time look into the lives and psyches of rare disease community members
- When snapshots are aggregated within a rare disease, the story of patients with that rare disease begins to evolve
- When snapshots are aggregated across multiple rare diseases, the traits that are common begin to emerge
- A simple question helps us to understand the challenges, needs, emotions, and psychosocial health of community members
- This understanding can then be shared with the community, so they recognize that they are not alone
- With the knowledge that polls provide, the responses should be acted upon through the development of support and educational programs that address the everyday burden faced by these patients, their families, and their caregivers
- The authors also believe these data should be shared with HCPs; doing so will allow HCPs to better understand the emotional and physical burdens patients face on a daily basis
- Publication of these data and insights in a medical journal is recommended to reach HCPs and other critical stakeholders

## References

1. JL Walewski, D Donovan, MNori. How many zebras are there, and where are they hiding in medical literature? A literature review of publications on rare diseases. *Expert Opin Orphan Drugs*. 2019;7(11):513-519.

## Note

\*onevoice digital educational rare disease communities are a product of rareLife solutions, Inc., Norwalk, CT.