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RARE PATIENT Voice LLC®
Also Non-Rare!

Rare Caregivers Report: Experiences Supporting Loved Ones Living with Rare Diseases

March 21, 2024

Rare Disease Summit

About Rare Patient Voice and Wes Michael

- **Wes Michael** founded Rare Patient Voice in 2013 to empower patients and family caregivers to share their voices through participation in research studies. The RPV community has since grown to 145,000+.

- The company has rewarded patients and family caregivers over $13 million for taking part in research. Many have been recruited at in-person patient events and through a referral program with patient advocacy and support groups.

- RPV now covers non-rare as well as rare diseases in the U.S., Canada, U.K., France, Germany, Italy, Spain, Australia, and New Zealand.

Rare Patient Voice Caregiver Survey

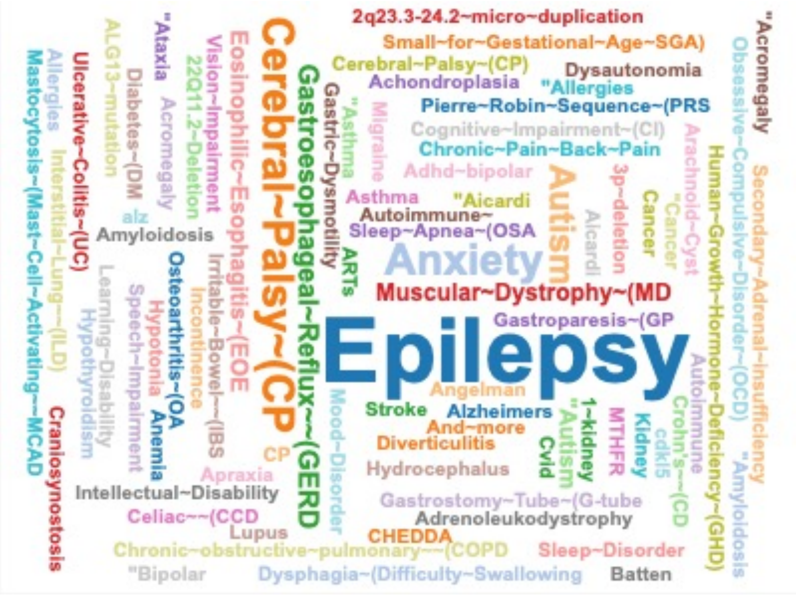
- In December 2023, Rare Patient Voice conducted a survey of 735 family caregivers (loved ones helping with patient care) of people living with rare diseases in the United States.

- Questions pertained to how being a caregiver has impacted them emotionally, financially, and in terms of their career, education, family life, and relationship with medical professionals.

- Also covered were their interest in clinical trials, the sources turned to for information about rare diseases, and the tasks they perform.

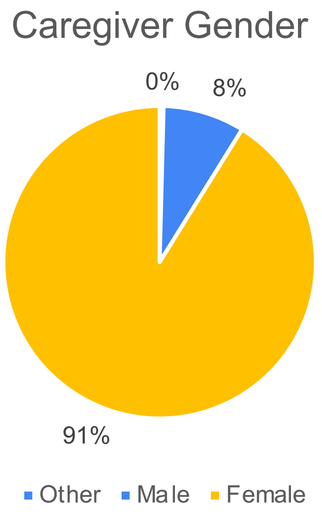
Rare Diseases Represented

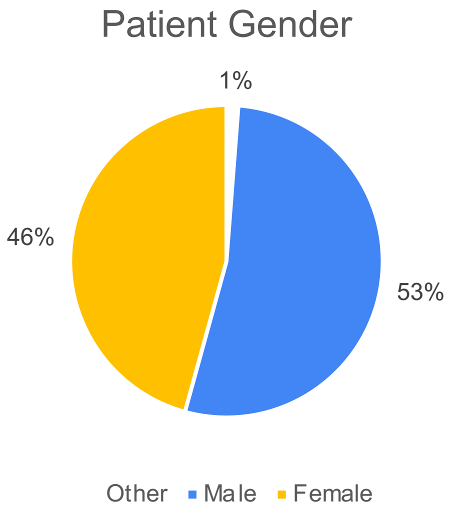
- Dozens of rare diseases were represented.
- Many rare disease patients also suffer from non-rare conditions, which they also listed.



Gender of Caregivers and Patients

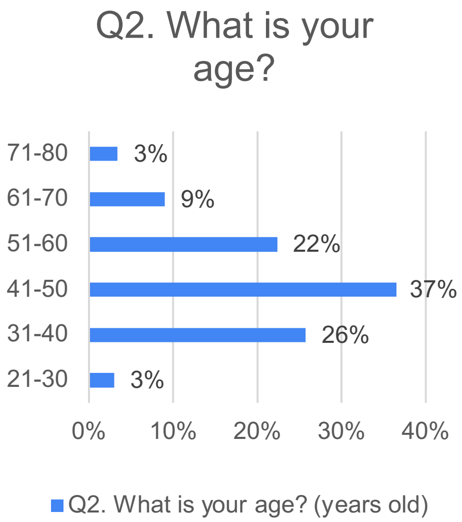
- Caregivers were overwhelmingly female.
- The patients they care for are fairly evenly split between male and female.

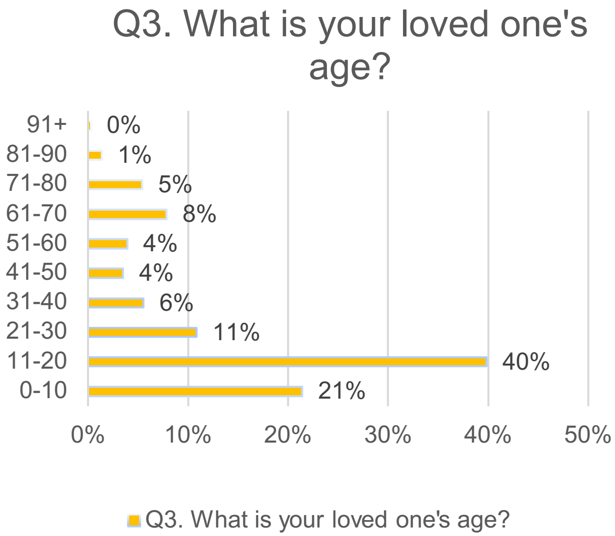

Caregiver Gender


Patient Gender

Ages of Caregivers and Patients

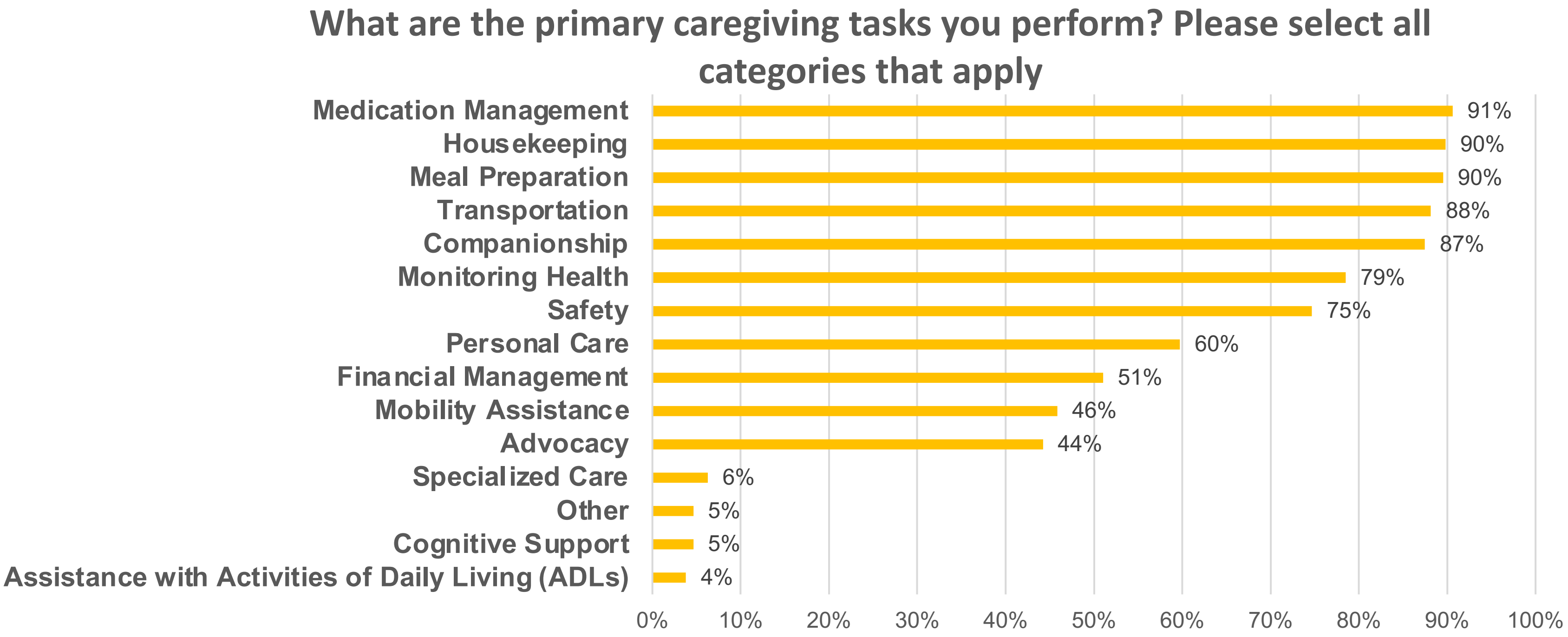
- Most caregivers in our study were middle-aged.
- The majority of patients were children, but there were middle-aged and elderly as well.





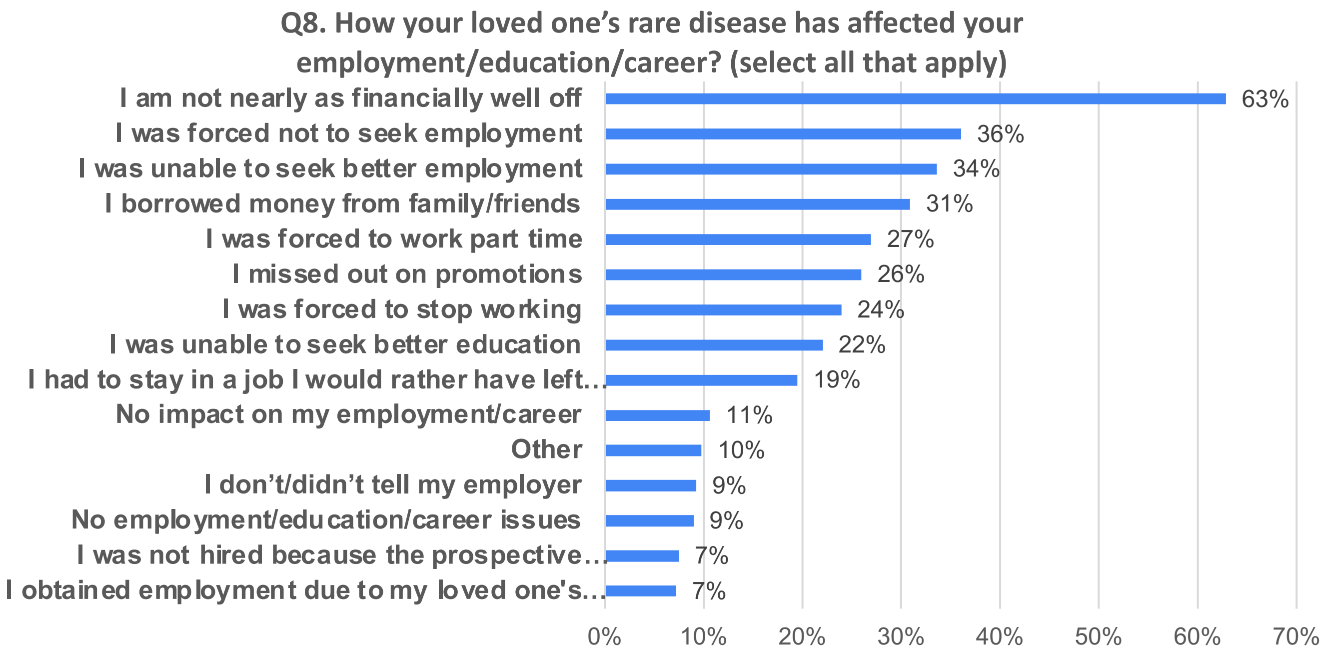
Caregiver Tasks

- Most caregivers perform many tasks.



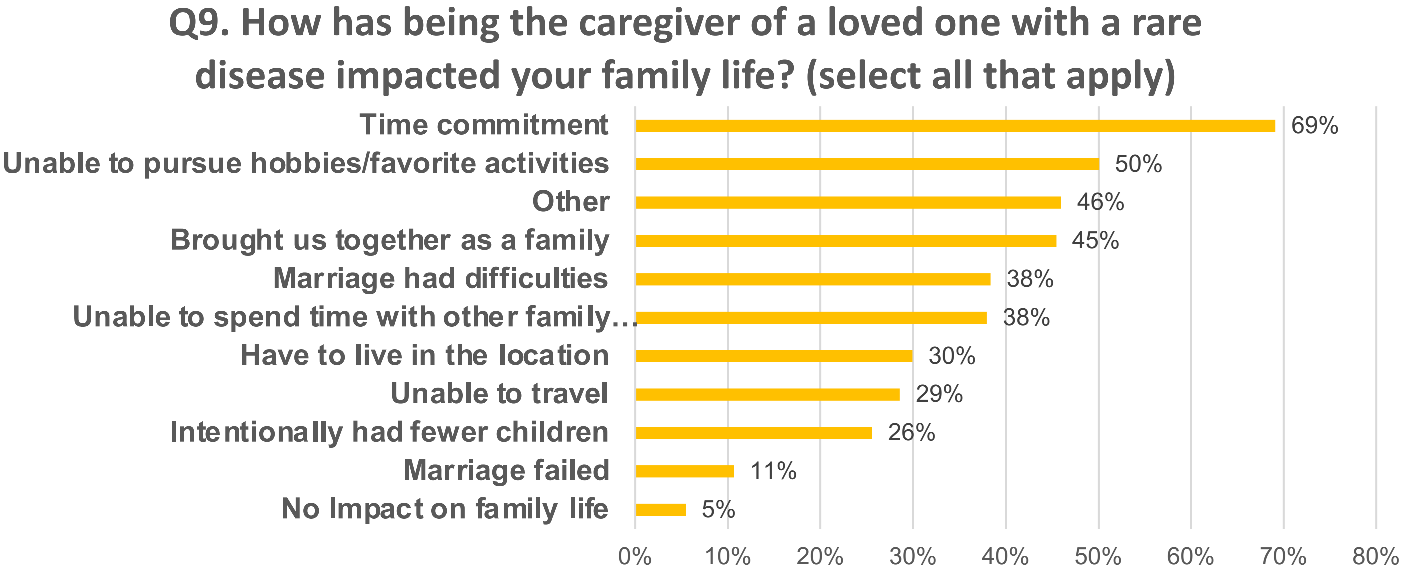
Impact of Caregiving on Finances/Career/Education

- Nearly two-thirds of caregivers have suffered a negative financial impact due to their loved one’s rare disease.



Impact on Family Life

- The time commitment is most often mentioned as having an impact on family life.



Other Impacts on Family Life

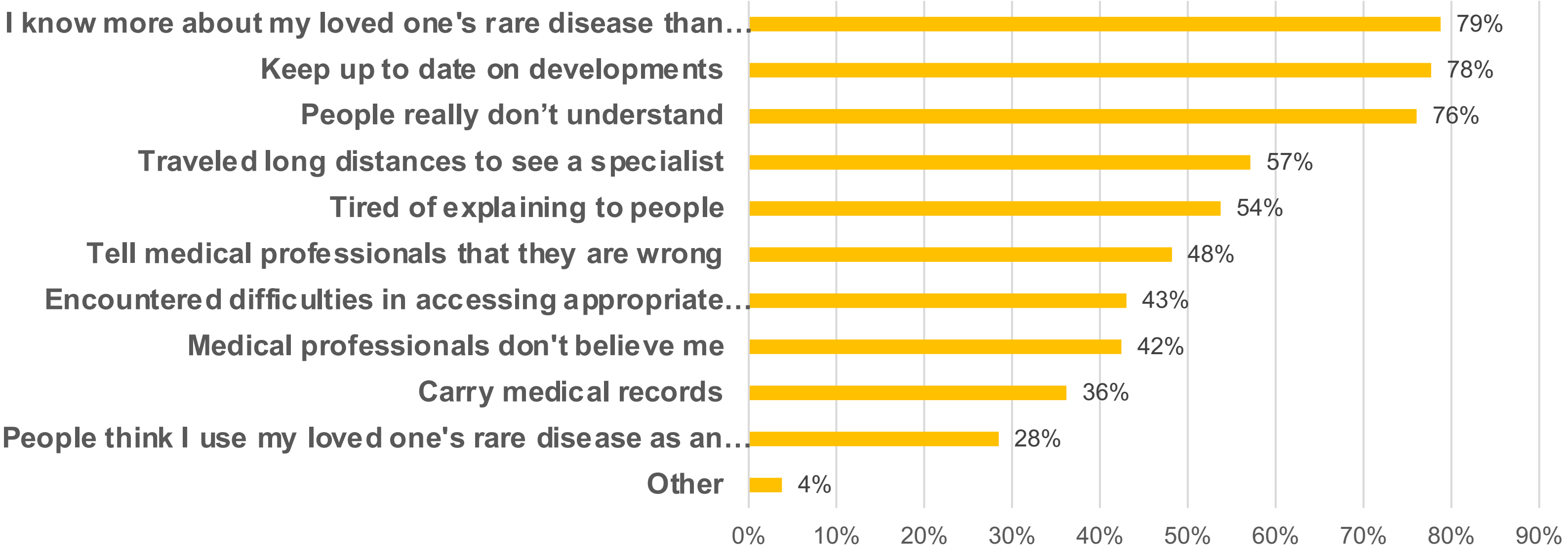
- “Because my husband's multiple myeloma makes him immune-compromised, I have been isolated since the beginning of the covid pandemic. We are still living in a ‘bubble’.”
- “Can't eat out at restaurants, difficult to travel or take vacations.”
- “My daughters schedule and needs are the first to go into our schedule, and all over family and siblings needs trickle in. Sometimes it's hard to manage.”
- “My first marriage failed because my ex-husband could not/would not help.”
- “My husband and I have had to ‘divide and conquer’ parent so one parent could be with our other children and one could be with my child with rare disease.”

Other Impacts on Family Life *(Continued)*

- "My own health has suffered due to taking care of my loved one.”
- “We usually say ‘no’ to family events because of my loved one's rare disease.”
- “When we got married I thought I was going to have a different life with a partner, not become like a mother to a 45 year-old.”
- “Caring for my mom has negatively affected my friendships, especially with people who have never had caregiving experiences. On the other hand, I have developed new friendships with people who are caregivers.”
- “We adapt and make it work, but we are limited in our travel, food choices, friends we keep, and time we have available.”

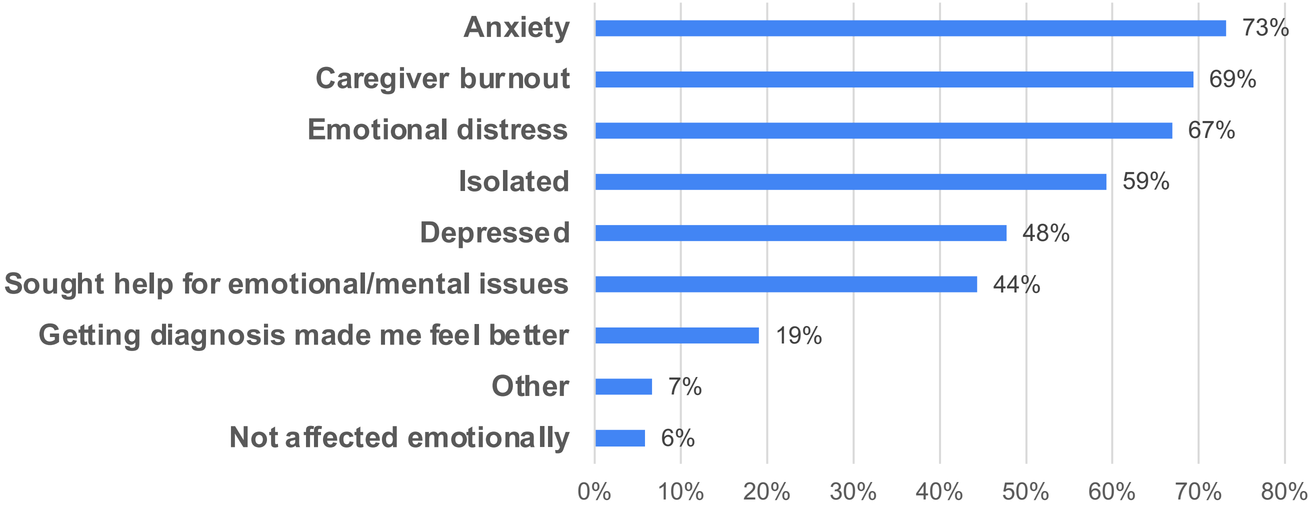
Dealing with Medical Professionals

Q11. How has being the caregiver of a loved one with a rare disease affected how you deal with medical professionals and others? (Select all that apply)



Emotional Impact

Q10. How has being the caregiver of a loved one with a rare disease affected you emotionally? (Select all that apply)



Dealing with Medical Professionals

- “This was a horrible experience and highlighted that when you really need it most, doctors and insurance companies are not there to help. You are left alone to figure out a solution.”

- “I was accused of MSBP [Munchausen syndrome by proxy, a form of child abuse that describes children whose parents or caregivers invent illness stories and substantiate the stories by fabricating false physical signs] when my daughter was younger.”

- “I have felt as though I am threatened by medical providers if we do not provide care the way that they want us to, even if the care they are suggesting is not the best care for our loved one.”

- “I have been rejected many times for appointments just for having Medicaid.”

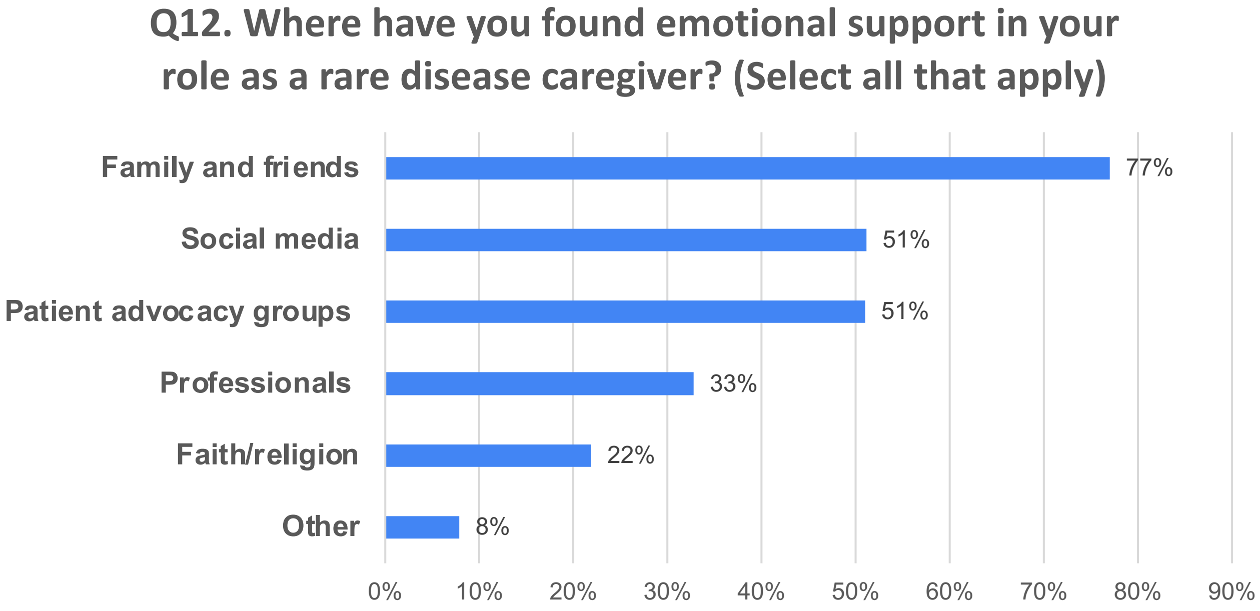
Dealing with Medical Professionals

(*Continued*)

- “I have learned to stand my ground, especially when medical professionals have argued with me.”
- “I’ve had to change healthcare plans and multiple doctors and specialists because they overlooked or disregarded my concerns for my loved ones rare disease that were validated by another provider.”
- “I have met many compassionate researchers; without their efforts, much healing would not come to pass.”
- “There have been times when the medical professionals just DON’T KNOW what to do next as far as treatment goes.”

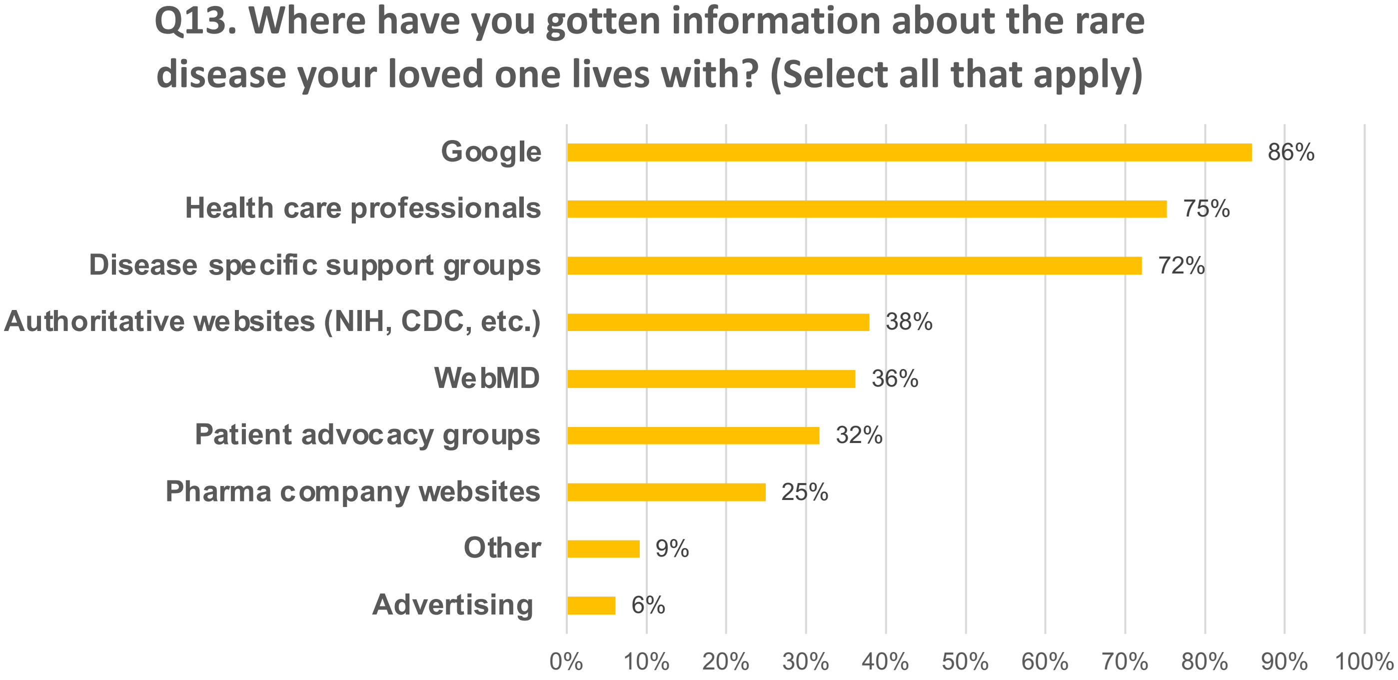
Sources of Emotional Support

- Family and friends provide emotional support for three-quarters of caregivers.



Information Sources

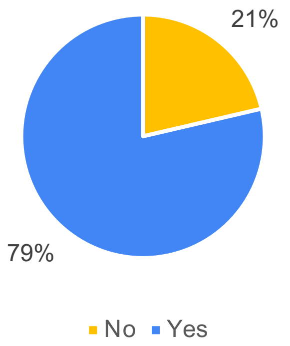
- “Dr. Google” is used as a source of information by more caregivers than health care professionals.



Adequate Information

- Most caregivers tend to feel adequately informed about their loved one's condition and available resources.

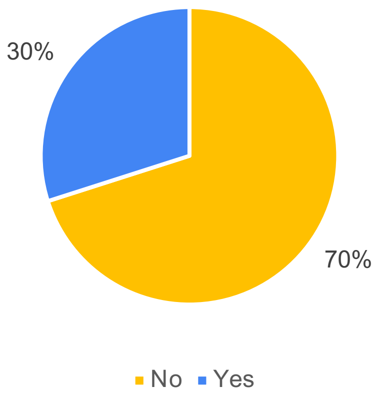
Q14. Do you feel adequately informed about your loved one's condition and available resources?



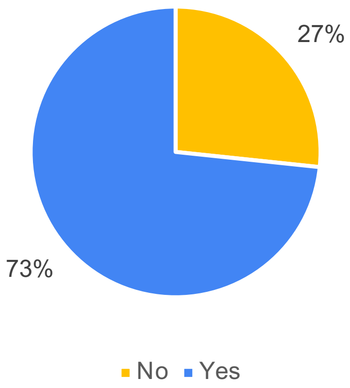
Clinical Trials

- Most patients have not taken part in clinical trials, but a majority would like to.

Q15. Has your loved one taken part in a clinical trial?



Q15a. Would you like your loved one to take part in a clinical trial in the future?



Why Not Take Part in a Clinical Trial?

- “Clinical trials are like rolling the dice: they may work, they may not. No point in getting your hopes up.”
- “His current treatment is working very well, so there is no need to be in a trial.”
- “I’m afraid of potential side effects not yet encountered by new medications (long term).”
- “It is a genetic condition. I don't see how it could be further helped.”
- “Most require needles often which he doesn’t do well with.”

What Would Make Caring for Your Loved One Easier?

- “Knowing how to set up people to care for her when I am gone.”
- “Financial support.” “More money.” “Financial assistance.”
- “School healthcare providers and regular healthcare providers be more knowledgeable.”
- “More societal support and understanding of the role of caregivers.” “More understanding from people around us.”
- “Ability to have a ‘break’ but have the patient with knowledgeable qualified individuals.”

What Would Make Caring for Your Loved One Easier? *(Continued)*

- “Being paid to be a caregiver.”
- “Less general stigma about/othering of disability in society.”
- “Easier to navigate transition to adult services.”
- “Doctors willing to collaborate with each other outside their specialty to offer the best care and learn from each other.” “More specialists knowledgable about the disease.” “Physicians that will accept they don’t know everything.”
- “Accessibility wherever we go and the ability to make our home more accessible.”

Summary

- Rare caregivers are overwhelmingly female, most middle-aged, with many caring for their children.
- Their lives are significantly impacted by their role as caregiver, largely not for the best, particularly in terms of career opportunities, finances, and the emotional impact.
- The time commitment of caregiving is noted as having the greatest effect on their family life.
- There are many ways rare caregivers' lives can be improved, with financial assistance emerging as the most commonly mentioned.

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For more information or to request the study data:

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