

AWaRDS Study

Adults with **Rare Disorders** Support Study



Research Summary: Health-Related Quality of Life Among Adults with Diverse Rare Disorders

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The Adults with Rare Disorders Support project aims to improve the psychological and social support needs of people with a variety of rare diseases and disorders. This multi-part project involves surveys and focus groups, and records types of information and support available from rare disease organizations. We are reporting results from the first survey, which was recently published in Orphanet Journal of Rare Diseases.

Background

Approximately 350 million people worldwide live with a rare disease. Although there are about 7,000 different rare diseases, people with diverse rare disorders share similar challenges. Many RDs are chronic, have no effective treatment, and require complex care. Rare disorders may impact quality of life because of poor access to information, treatment, and support as well as high levels of stigma and isolation.

Methodology

We completed the first large-scale survey of adults living in the U.S. diagnosed with any rare disease. We reached people through rare disease organizations, including National Organization for Rare Diseases (NORD), Coordination of Rare Diseases at Sanford (CORDS), as well as social media. 1218 participants were included in this study.

Quality of life was determined by having adults rate their anxiety, depression, fatigue, pain, physical function, and ability to participate in society. These survey questions are from the standardized Patient-Reported Outcomes Measurement Information System (PROMIS), which let us compare quality of life between adults with rare diseases to that of the U.S. population and Americans with common chronic diseases.

Findings

Participants waited on average 9 years to receive a correct diagnosis. A total of 232 rare disorders were represented, and 13% of participants had more than one rare disorder.

Participants with rare disorders had poorer quality of life than the U.S. population and even people with common chronic diseases like hypertension, arthritis, and asthma. On average, participants with rare diseases experienced worse anxiety than 75% of Americans, worse depression than 70% of Americans, worse fatigue than 85% of Americans, worse pain than 75% of Americans, worse physical functioning than 85% of Americans, and worse ability to participate in society than 80% of Americans.

Quality of life was compared across categories of rare diseases. Participants with systematic, rheumatic, and neurological diseases reported very poor quality of life. Participants with developmental anomalies experienced fewer problems with quality of life than the other groups.

Conclusions

The quality of life needs of people with rare diseases are not being met. Americans with rare disorders had higher anxiety, depression, pain, fatigue, and poorer physical function and ability to participate in society than the general U.S. population. People with rare diseases are at greater risk of poor quality of life than people with other chronic health conditions.

Recommendations

- **Rare disease organizations and funding agencies should include quality of life in their mission statements and funding priorities.** The goal of many rare disorder organizations and funding organizations is to find new treatments and cures. However, few focus on improving quality of life issues like psychological and social support. Supporting quality of life is especially important for people living with one of the 95% of rare disorders that have no effective treatments.
- **Rare disease organizations should increase psychosocial support for people with rare diseases.** Our previous research has found that support groups or conferences are effective for promoting social support and reducing stigma and isolation. Future research will examine effectiveness of a variety of support methods.
- **People with rare disorders who are experiencing poor quality of life should seek support from a mental health professional, rare disease groups, and peer support from others with rare disorders.**

Next Steps

This study was the first of many planned in the AWARDS project. We are currently analyzing responses to other survey questions, including short answer comments, and responses from people outside of the United States. This work will help us understand the reasons behind poor quality of life and find ways to improve support for people with rare disorders. Focus groups are the next stage in the AWARDS project. Participants who indicated that they were interested in joining focus groups will be contacted in the next year. The long-term goal of this project is to develop recommendations for providing psychological and social support to people with rare disorders.

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Acknowledgements

Most importantly, we thank the participants with rare disorders for their time, effort, and generosity in sharing their experiences. This research was funded in part by an Oregon State University College of Liberal Arts Research Award. We thank NORD and the Coordination of Rare Diseases at Sanford for assistance with recruitment. We thank NORD and its member organizations who consulted with the authors regarding this research. We thank Makenzie Atwood for assistance with data collection and management.



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Reference

Bogart, K. R., & Irvin, V.L. (2017). Health-related quality of life among adults with diverse rare disorders. *Orphanet Journal of Rare Diseases*. 12(177). doi: 10.1186/s13023-017-0730-1
Weblink: <http://rdcu.be/AFfO>