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RARE DISEASE CAREGIVING IN AMERICA

National Alliance for Caregiving

IN PARTNERSHIP WITH

Global Genes®
Allies in Rare Disease

RARE DISEASE CAREGIVING IN AMERICA

Acknowledgments

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I. Executive Summary

This report outlines findings from the research study *Rare Disease Caregiving in America.* This is a study of 1,406 caregivers ages 18 or older living in the United States who provide care to a child or adult with a rare disease or condition, from data collected during fall 2017. Rare diseases or conditions are those that affect fewer than 200,000 people.[2] The purpose of this study is to describe the experiences and challenges of this targeted group of *caregivers* in a holistic way, across many diseases. Although understanding the impact of rare disease on the actual person with the rare disease is important and worthy of research, the goal of this study is to look at the impact of rare disease on the caregiver. It is important to note that all data presented herein are from the perspective and experience of the rare disease caregiver.

It is estimated that 25 to 30 million Americans currently have a rare disease or condition.[3] This deep dive research into the lives of rare caregivers[4] – that is, American adults who provide care to an adult or child with a rare disease or condition – suggests rare disease has a broad and lasting impact on caregivers, in both daily life and long-term well-being.

Most rare caregivers are providing care to a child under the age of 18 (62%), with the average age of the care recipient – that is, the person who had a rare disease, condition, or illness – being 20.9 years old. Most rare caregivers are immediate relatives, with 59% caring for their own child under 18, 17% caring for their own adult child, and 14% caring for a spouse or partner. Nearly all rare caregivers live in the same household as their care recipient (89%).

Rare caregivers tend to provide care to someone whose rare condition or disease is genetic in nature (71%), though the list of conditions for which care is provided in this study is over 400 unique conditions long (see Appendix B for full list of conditions, available online at www.caregiving.org/rare). Only 43% report there is a treatment available for the underlying disease or disorder and most (82%) report that treatments or therapies are used for symptom management rather than actual disease treatment.

Rare caregivers rely on doctors and medical professionals to help them and their care recipient through the experience of rare disease. More than eight in ten rely on medical professionals for information (82%). However, adequate medical and support services

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[2] National Institutes for Health: https://rarediseases.info.nih.gov/diseases/pages/31/faqs-about-rare-diseases

[3] According to the NIH Genetic and Rare Diseases Information Center (GARD), which is component of the National Center for Advancing Translational Sciences (NCATS), the total number of Americans living with a rare disease is estimated at between 25-30 million. https://rarediseases.info.nih.gov/diseases/pages/31/faqs-about-rare-diseases

[4] Rare caregivers is the term used hereafter to refer to American adults who provide unpaid care to a care recipient with a rare disease, condition, or illness.

can be difficult to access. Fewer than two in five rare caregivers feel their care recipient's local hospital can handle the underlying rare disease or condition (38%). Roughly one out of every three rare caregivers report having difficulty in accessing treatments or therapies for symptom management (34%) or treatment(s) for the underlying disease (31%).

Rare caregivers may need more support from the doctors and care professionals they interact with. Fewer than half (48%) have had a doctor, nurse, or social worker ask what was needed to provide care to the recipient, and just one in four have had these discussions about their *own* care needs.

This lack of outreach about rare caregiver needs may be a missed opportunity, in that caregiving can become a role that fundamentally alters daily life for many years to come. Most rare caregivers have been providing care for a significant period of time, 8.9 years, on average – more than double the caregiving commitment found among caregivers more generally.[5] Rare caregiving is also time-intensive. Rare caregivers of adults spend about 37 hours a week providing care on average – about 12 more hours a week than general caregivers.[6] Even more striking is the amount of care provided by rare caregivers of a child: 53 hours a week, on average, compared to 30 hours for general child caregivers.[7]

Rare caregivers spend their time taking on a variety of tasks for their care recipient, which translates into a high-burden[8] caregiving situation. Nearly all rare caregivers help their care recipient with at least one Instrumental Activity of Daily Living (IADL) task such as housework, shopping, or transportation. Three in five rare caregivers (62%) help with at least one Activity of Daily Living (ADL). Two out of every three rare caregivers have a high caregiver burden on the Burden of Care Index (67%) – far higher than general caregivers.[9]

A defining feature of rare caregiving seems to be one of expertise. Rare caregivers have to function as nurses, performing medical/nursing tasks:[10] 84% help their care

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[5] Caregiving in the U.S. 2015," National Alliance for Caregiving and AARP Public Policy Institute, 2015. http://www.caregiving.org/caregiving2015/ found caregivers of adults care for 4.0 years on average. "Caregiving in the U.S. 2009," National Alliance for Caregiving and AARP, 2009. http://www.caregiving.org/research/general-caregiving/ found that caregivers of children under age 18 care for 4.2 years on average.

[6] "Caregiving in the U.S. 2015"

[7] "Caregiving in the U.S. 2009"

[8] Refers to Burden of Care Index, which aims to measure the intensity or demands of the caregiving situation.

[9] 40% high burden among general adult caregivers in "Caregiving in the U.S. 2015" and 38% among general child caregivers in "Caregiving in the U.S. 2009".

[10] Medical/nursing tasks are a body of caregiving tasks that are highly skilled in nature and are things typically handled by someone like a health-care aide, nurse, or other care professional. See: Reinhard, S.C., Levine, C., & Samis, S. "Home Alone: Family Caregivers Providing Complex Chronic Care," AARP Public Policy Institute & United Hospital Fund, 2012.

recipient with medical/nursing tasks. These tasks are a *defining feature* of rare caregiving: as rare caregivers face barriers to service support and care access, they are being called upon to do these complex medical/nursing tasks for the duration of their role as caregiver. The majority "become" teachers, educating health-care professionals about their care recipient's rare disease or condition (89%). Rare caregivers can also find themselves researching and accessing clinical trials. One in four report their care recipient has participated in a clinical trial and when that occurs, the rare caregiver often has to help with things like paperwork (77%), transportation (65%), trial response documentation (62%), and care coordination (59%).

Despite rare caregivers' higher Burden of Care score versus general caregivers, they are no more likely to rely on paid help, with just one in three reporting use of paid help or aides (33%). Support service use is also not common, with just 22% having used respite and 14% relying on an outside transportation service. This day-to-day responsibility means that other immediate and extended family members may be called upon to help provide care, with 59% reporting help from at least one other unpaid caregiver. This domino-effect of care can extend to youth in the family, with 24% reporting their care recipient receives unpaid care from at least one youth under age 18.

Rare caregivers note the ripple effect of rare disease on their family, with 74% struggling with a sense of loss for what their care recipient's life could have been and fewer than half feeling their role has had a positive impact on their family (44%). This ripple effect does have some positive results, as most rare caregivers report feelings of pride in improving the understanding of their recipient's condition (94%) and feeling close to their care recipient (85%). More than half (56%) feel a sense of purpose due to their caregiving role.

The day-to-day impact for rare caregivers is pervasive in all aspects of their life, with no areas untouched: the effects are seen in the workplace, school, and on their physical and emotional health. Two out of three rare caregivers have worked while providing care (65%) and nearly all report a bleed over into their work life, more so than general caregivers who work.[11] Nearly all have gone in late or left work early to provide care (91%). One in ten rare caregivers are also students and, as with work, they often struggle to fulfill their school responsibilities.

Rare caregivers say providing care to their care recipient is emotionally stressful (67%), twice as high as that of general caregivers.[12] Four in ten report having fair or poor emotional or mental health (41%). To a lesser degree, rare caregiving can be a physical strain to caregivers, with 37% reporting high physical strain. Those who do more hands-on care, like medical/nursing tasks and Activities of Daily Living, experience greater physical strain. Rare caregiving takes a clear toll on the physical health of caregivers,

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[11] "Caregiving in the U.S. 2015"

[12] 34% high stress among general caregivers of a child from "Caregiving in the U.S. 2009" and 39% among general caregivers of an adult from "Caregiving in the U.S. 2015".

with 30% rating their physical health as fair or poor, and 58% finding it difficult to take care of their own health.

The ripple effect of rare caregiving can extend past day-to-day tasks of providing care, the need for others in the family to pitch in, and the emotional and physical well-being of the caregiver. Rare caregiving can ultimately impact the finances of the rare caregiver and their family unit, and these financial impacts loom large. Half of rare caregivers face high levels of financial strain (51%) and three out of four worry about the ability of their own family or their care recipient’s family to pay for care. Nearly all rare caregivers have experienced financial hardship because of their caregiving role (86%). Common areas of impact include cutting back on household spending (81%), not saving for long-term goals (62%), and using up personal savings (59%). Nearly one in ten have filed for bankruptcy (9%). Future planning is not the norm among rare caregivers: just 35% have a contingency plan in place in case they are unable to provide care and only 29% have plans in place for their care recipient’s future care (i.e. financial, health, and living arrangements).

Not only does rare caregiving impact the caregiver’s daily life, the family unit, their well-being, and their long-term finances, but the role of rare caregiver changes their social lives. About half of rare caregivers have difficulty maintaining friendships (51%) and 53% report feeling alone.

Policy Recommendations

Rare caregivers play a critical role in the health of those for whom they provide care. The purpose of this study is to showcase the enormity of the changes in caregivers’ lives, as a result of taking on this important role. Rare disease management comes with little support and guidance. It comes with expanded responsibilities that include having to become activists and advocates for their care recipient. The challenges are great, and we hope that this important information and insight will help create a community of support for those in the rare disease fight.

With these unique challenges facing rare caregivers, we recommend the following:

- **Enhance the health of family caregivers through access to respite and counseling.**
  Only 22% of rare caregivers report having used a respite service to get a break. As the burden of care increases, rare caregivers experience increasing levels of stress and strain, reduced emotional and physical health, and increased feelings of isolation and personal neglect. This is compounded by the length of time the caregiver serves in this role—nearly 10 years on average. Two out of three rare caregivers find it difficult to maintain their own health, one out of three rate their physical health as only fair or poor. overall, rare caregivers’ health is worse than United States adults overall, where just 10% report fair or poor health. Nearly seventy percent of rare caregivers say providing care is emotionally stressful, with a majority reporting that they are highly stressed. Policymakers should fund

lifespan respite care services and incorporate plans for respite care and options to receive counseling into policy proposals intended to serve caregivers of those with rare diseases.

- **Achieve financial security and workplace protections for family caregivers.** The largest hurdle for many rare caregivers is balancing work and caregiving responsibilities--on average rare caregivers of adults spend between 40 and 50 hours a week providing care. Nearly all rare caregivers have gone in late or left work early to provide care (91%) – a significantly more common occurrence than general caregivers. Significant proportions also have cut back on their hours (45%), taken a leave of absence (39%), or turned down a promotion (26%). Of note, half of employed rare caregivers of a child went from working full-time to part-time or otherwise cut back their work hours to provide care (52%). Policymakers and employers should work together to foster a workplace environment that offers paid family and medical leave. Paid family and medical leave policies should: 1) Be available for family caregivers, not just new parents. 2) Offer compensation for time off at or around sixty percent of full pay. 3) Ensure caregivers are not discriminated against for taking time off for caregiving responsibilities.

- **Ensure family caregivers receive comprehensive training and information.** Rare caregivers often find that they become the expert on their care recipient's condition. They become nurses, performing medical/nursing tasks; they are the voice of care, doing Caregiving Support Activities like coordinating care and advocating; they become teachers, educating health care professionals about their care recipient's condition; they become researchers, finding and accessing clinical trials for their care recipient. Among all rare caregivers, half indicate that an experienced medical professional is needed and difficult to find. Often, complex tasks are performed without having any prior training: Half of rare caregivers do medical/nursing tasks without any preparation. More than eight in ten rare caregivers rely on medical professionals for information, while 68% turn to condition-specific websites or organizations. Health systems, medical professional societies, patient advocate organizations, and policymakers should find innovative ways to provide rare caregivers with accessible and understandable training and information on the condition of the person receiving care.

- **Improve care coordination between skilled home health providers, direct care workers, and family caregivers.**
  While many rare caregivers report feeling that their care recipient was adequately diagnosed, there is not necessarily always a next step toward treatment. While over eighty percent report there is some sort of treatment or therapy available for managing their care recipient's symptoms, only about forty percent report that there is a treatment available for the underlying rare disease or condition. Many rare caregivers report there is no treatment for their care recipient's condition,

and rather, that treatments or medications are used for therapy for symptoms, rather than actual treatment. In addition, fewer than half have had a doctor, nurse or social worker ever ask what they need to care for their care recipient. Even fewer have had a medical professional or social worker ask themselves, the rare caregiver, what he/she needs to take care of themselves. Policymakers and health systems should ensure care models are patient- and family-centered. For instances where treatment isn't available, health care professionals and students should be better educated on symptom management techniques such as high-quality palliative or hospice care as a measure to improve the quality of life, prolong survival, and increase family satisfaction, where applicable. In addition, policymakers should consider broadening criteria for patients to qualify for skilled home health nursing and private duty nursing so training could be ordered and overseen by a health care professional but executed in the home.

- **Expand services and supports available to family caregivers on the community level.**
  Not only does rare caregiving impact the family unit, the caregiver's daily life, their health, and finances, but their role can impact their social and family lives. Many rare caregivers have difficulty maintaining friendships and face feelings of isolation. Yet, some find comfort in creating a sense of community with other rare caregivers, finding support online and through reaching out to share information. Doctors and medical specialists are trusted and relied upon heavily by rare caregivers to help them through rare disease, but are often difficult to access. Rare caregivers whose care recipient lives in a rural area express more doubts about the local hospital: only 27% agree that their care recipient's local hospital can handle their condition. Policymakers, providers, health systems, and community living programs should expand to serve family caregivers in greater capacity. Community living programs should take into consideration the needs of those across the lifespan—including those who are aging, physically or mentally disabled, and those with rare health conditions—when developing home and community-based services and supports. Community support programs should include funding to patient advocacy groups to back and expand peer support services, and ensure peer support interventions are well trained and monitored so they can achieve improved health outcomes.

- **Include family caregiver as a vital member of the team in health care settings.**
  Nearly 90% of rare caregivers are the primary caregiver. In addition to helping their care recipient with IADLs and ADLs, the vast majority of rare caregivers take on "medical/nursing tasks": a body of caregiving tasks that are highly skilled in nature – things that may have typically been handled by someone like a health care aide, nurse, or another health care professional. More than eight in ten rare caregivers help their care recipient with medical/nursing tasks. Nearly all rare caregivers monitor the severity of their care recipient's condition (94%), advocate for them (94%), and communicate with care providers (94%); and 89% find

themselves educating health care professionals about their care recipient's rare condition. Despite rare caregivers being in a higher burden care situation than caregivers generally, they are no more likely to rely on paid help than caregivers generally. Any entity responsible for interacting with a caregiver of those with rare diseases should incorporate an evidence-based methodology into their practice to assess the needs of the *caregiver* and their ability to provide proper care. The paid care team should identify the primary caregiver and ensure they are documented and included in plans for treatment. Policymakers could also work with HRSA agencies to design grant opportunities or collaborative agreements for the rare disease community that translates the medical home model into this space; helping disease states become more educated on the medical home model, tools for constructing and maintaining, and how to partner with medical teams.

- **Recognize the family caregiver's role in clinical trial research and innovation.**
  Rare caregivers are often advocates within the health care system, educating professionals about their care recipient's rare disease or condition. Due to the lack of available therapies to support a person with a rare disease or condition, caregivers are also key catalysts in researching and accessing clinical trials. One in four report their care recipient has participated in a clinical trial and when that occurs, the rare caregiver often has to help with things like paperwork, transportation, trial response documentation, and care coordination. When managing existing medications or therapies, many report there is not always a next step toward treatment. Even though more than 80% of caregivers say that there is some sort of treatment or therapy available for managing their care recipient's symptoms, less than half report that there is a treatment available for the underlying rare disease or condition. Drug and device manufacturers should consider how the presence or absence of a family caregiver impacts the development process and consider caregivers as partners in clinical trial recruitment. As patient-reported outcome measurements (PROs) are developed, innovators should identify ways to capture the perspective of the caregiver, including both the role of the caregiver in medication or device management and the impact of disease on unpaid caregivers.

Rare caregivers face challenges that are multifaceted. Pressure points on this group are significantly compounded by the duration of time spent living as a caregiver and lack of available options to treat the care recipient's condition. This means that not only are rare caregivers marginalized in their ability to contract with qualified paid direct care workers, but that they are also struggling to find the time away from work and daily responsibilities to serve as a caregiver without facing financial consequences. It is for these reasons that this report recommends to policymakers that rare caregivers require empowerment to remain in their role as caregiver, not to replace them with potentially more costly services.

II. Introduction

This report – *Rare Disease Caregiving in America* – is based on quantitative online surveys of 1,406 caregivers in the United States, ages 18 years or older, who were currently providing care, or had done so in the year prior to the time of the survey, for someone with a rare disease or condition. A rare disease occurs in fewer than 200,000 people nationally.[13]

The purpose of this study is to provide an in-depth look at the unique issues and challenges facing those who provide unpaid care to someone with a rare disease, disorder, or condition. Caregivers had to self-identify as providing care in the past 12 months to at least one person who had a rare disease, disorder, or condition. Respondents then indicated their care recipient's condition from a database of 6,552 diseases as provided by the Genetic and Rare Diseases Information Center (GARD).[14] Caregivers who cared for more than one person with a rare disease were asked to answer relative to their experience with one person only. For more details about how caregivers qualified for the survey, see *Section III: Detailed Methodology.*

This study was commissioned by the National Alliance for Caregiving, in partnership with Global Genes, and conducted by Greenwald & Associates, an independent research firm, in fall 2017. The survey instrument, initial findings, and final report were reviewed by an independent Advisory Panel of subject-matter experts and family caregivers.

The core areas examined in this study include the following:

- Basics of the Caregiving Situation
- Diagnosis Process, Genetic Cause, and Specific Rare Condition(s)
- Treatment, Medication, and Clinical Trials
- Information Sources
- Use of Services and Local Availability
- Financial, Work, and School Impacts
- Caregiver Stress and Well-being

This report, *Rare Disease Caregiving in America*, outlines the overall findings from this study – that is, to describe the experiences and challenges of this targeted subset of

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[13] Genetic and Rare Diseases Information Center; National Center for Advancing Translational Services; National Institutes of Health. https://rarediseases.info.nih.gov/diseases

[14] List generated by GARD on August 1, 2017.

caregivers – caregivers of someone with a rare disease or condition. We also present key comparisons to other caregivers when appropriate.[15]

The research design of this study is based on conducting online surveys with caregivers of someone with a rare disease or condition. *Rare Disease Caregiving in America* utilized an online survey, with targeted convenience and snowball samples, in order to maximize the ability to conduct this survey with these difficult-to-find caregivers. Due to this sampling strategy, the findings in *Rare Disease Caregiving in America* must be interpreted as a targeted, in-depth look at a pre-identified population rather than a nationally-representative study. For more detail on the study methodology, including the questionnaire and sample, data cleaning, and response rate please see *Section III: Detailed Methodology*.

A. Reading This Report

This report – *Rare Disease Caregiving in America* – aims to provide insight into the experiences and perspectives of the unpaid rare caregiver. It is not intended to represent clinical evaluations of health-care providers or professionals, nor is it intended to capture the experiences of the person with a rare disease or condition (the "care recipient"). This study fills the gaps in existing knowledge by gathering information from the perspective of the rare caregiver, to help further the existing research and to advocate for policy and support on behalf of the caregiver.

The main figures present results for all 1,406 rare caregivers who responded to the survey. Each chart or graph includes, on the right-hand side, a small table comparing results for caregivers of children and caregivers of adults. Statistically significant differences (95% level) between these two groups are indicated with ^ shown next to the number that is significantly higher. All numbers have been rounded to the nearest whole number. In addition, "don't know" or "refused" responses are not always presented in figures. For these reasons, data in some figures will not add to 100 percent. The results for multiple response questions may also add to greater than 100 percent.

The *n* sizes shown in each table or graphic represent the unweighted number of respondents who answered each question. To indicate statistically significant differences between subgroup findings for two-group comparisons, shown in table or chart format, the report uses ^ shown next to the number that is significantly higher. For three-or-more group comparisons, a superscript letter (e.g., ^{ABC}) next to a numerical result indicates that it is significantly higher than the numerical result in the column designated by that letter. All significance testing is at a 95% confidence level.

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[15] Comparisons are made to both "Caregiving in the U.S. 2009" (for comparisons to caregivers of a child, for some medical, behavioral, mental, or other special need) and "Caregiving in the U.S. 2015" (for comparisons to caregivers of an adult). All comparisons are footnoted where appropriate.

All demographic information about the rare caregivers is in reference to their current situation if currently caregiving, or their situation at the time they last provided care, if not a current caregiver.

For the sake of brevity, we refer to the study respondents as “rare caregivers” throughout the report. This term refers to the study population – that is, adults age 18 and older, living in the United States, who provide unpaid care to an adult or child who has a rare disease or condition. The person receiving care is referred to as a “care recipient.”

III. Detailed Methodology

Rare Disease Caregiving in America, is based on quantitative online surveys of 1,406 caregivers in the United States ages 18 or older who currently provide care, or had done so in the year prior to the time of survey, for someone with a rare disease or condition.

The survey instrument, initial findings, and final report were reviewed by an independent Advisory Panel of subject-matter experts and family caregivers. The sections below describe, in detail, the study purpose, the questionnaire and sample, data cleaning, and response rate.

A. Study Purpose

The purpose of this study is to provide an in-depth look at the unique issues and challenges facing those who provide unpaid care to someone with a rare disease or condition. This study was commissioned by the National Alliance for Caregiving. The findings are based on data collected in fall 2017.

The core areas examined in this study include the following:

- Basics of the Caregiving Situation
- Diagnosis Process, Genetic Cause, and Specific Rare Condition(s)
- Treatment, Medication, and Clinical Trials
- Information Sources
- Use of Services and Local Availability
- Financial, Work, and School Impacts
- Caregiver Stress and Well-being

It is estimated that 25 to 30 million Americans currently have a rare disease or condition.[16] Although it is important to understand the experiences of those individuals, the main goal of this report is to describe the experiences and challenges of a targeted subset of caregivers – caregivers of someone with a rare disease or condition. We also present key comparisons to other caregivers when appropriate.[17]

B. Research Design

The research design of this study is based on achieving the key goal of conducting online surveys with caregivers of someone with a rare condition or disease. In the United States, a rare disease is defined as a condition that affects fewer than 200,000 people.

Rare Disease Caregiving in America utilized an online survey, with targeted convenience and snowball samples. One of the main reasons for this study design, as opposed to a probability-based design, was the difficult nature of finding qualified rare caregivers in any general population sample. Therefore, to maximize the ability to conduct online surveys with these difficult to find caregivers, *Rare Disease Caregiving in America* utilized a targeted convenience and snowball sample design. To find caregivers of someone with rare disease or condition, the survey link was distributed to a variety of partner groups, disease organizations, advocacy groups, and at a national conference for rare illnesses. A variety of online and social media advertisements were also targeted to recruit respondents to take the online survey. Rare caregivers were also encouraged to share the link for the survey with other rare caregivers.

As with prior research,[18] the use of a targeted, non-probability based design presents the first of two key study limitations: results are not generalizable to either the general population or to the full population of rare caregivers. Rather, the results must be interpreted as a targeted, in-depth look at a pre-identified population: one which may be, by design, more active and connected to other rare caregivers or support groups.

The decision was also made to conduct the study online, which presents the second study limitation: results are biased toward those with Internet access. Existing data show that younger, more educated, and higher income[19] people more often have Internet access. Accordingly, those who completed this study had high education levels (shown in *Section IV: L: Respondent Profile*). The majority of rare caregivers have a

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[16] NIH Genetic and Rare Diseases Information Center (GARD), National Center for Advancing Translational Sciences (NCATS).
[17] Comparisons are made to both “Caregiving in the U.S. 2009”, National Alliance for Caregiving and AARP and “Caregiving in the U.S. 2015”, National Alliance for Caregiving and AARP Public Policy Institute. All comparisons are footnoted where appropriate.
[18] “On Pins and Needles: Caregivers of Adults with Mental Illness,” National Alliance for Caregiving, 2015.
[19] File, Thom and Camille Ryan, “Computer and Internet Use in the United States: 2013,” American Community Survey Reports, ACS-28, U.S. Census Bureau, Washington, DC, 2014. http://www.census.gov/content/dam/Census/library/publications/2014/acs/acs-28.pdf

bachelor's degree or higher (59%) – much higher than past research has shown for caregivers of all adults (regardless of condition; 34%[20]) or caregivers of children (regardless of condition; 32%[21]). However, this bias toward socially or virtually connected caregivers and toward higher education caregivers suggests that *Rare Disease Caregiving in America* may be underestimating the difficulties that a broader group of rare caregivers face, as having social connections and higher education levels often makes it easier to navigate care systems and buffer caregiver stress and strain.[22]

C. Questionnaire and Caregiver Qualification

Rare Disease Caregiving in America was designed to explore topics unique to rare caregivers and their recipients. It was drafted by Greenwald & Associates, with input from the National Alliance for Caregiving and Global Genes. The full questionnaire can be found in online at www.caregiving.org/rare in Appendix A.

This study conducted 1,406 quantitative online surveys with caregivers ages 18 or older, who were currently providing care or had done so in the year prior to the time of survey, for an adult or child with a rare disease or condition.

Caregivers were those who self-identified as providing unpaid care, as described in the two following questions:

> Adult Caregiving: At any time during the last 12 months, have you provided unpaid care to a relative or friend age 18 years or older to help them take care of themselves? This may include helping with personal needs or household chores. It might be managing a person's finances, arranging for outside services, doing medical or nursing things, or visiting regularly to see how they are doing. This adult does not need to live with you. If you are paid some amount as part of a Medicaid, state government, or grant program to care for a close family member in home, please select "yes".
>
> Child Caregiving: At any time during the last 12 months, have you provided unpaid care to any child under the age of 18 because of a medical, behavioral, or other condition or disability? This kind of unpaid care is more than the normal care required for a child of that age. This could include care for an ongoing medical condition, a serious short-term condition, or emotional or behavioral problems, or developmental problems. If you are paid some amount as part of a Medicaid, state government, or grant program to care for your own child in home, please select "yes".

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[20] "Caregiving in the U.S. 2015," National Alliance for Caregiving and AARP Public Policy Institute, 2015. http://www.caregiving.org/caregiving2015/

[21] "Caregiving in the U.S. 2009"," National Alliance for Caregiving and AARP, 2009. http://www.caregiving.org/research/general-caregiving/

[22] "Caregiving in the U.S. 2015"

Caregivers then had to indicate that at least one person they care for had a rare disease, disorder, or condition and then indicate their care recipient's specific condition from a database of 6,552 diseases as provided by Genetic and Rare Diseases Information Center (GARD).[23] Caregivers who cared for more than one person with a rare disease were asked to answer about their experience with one person only. Caregivers were only eligible for this study if they reported caring for someone with a rare disease or condition, either broadly or by naming a specific rare disease, illness or condition. In total, rare caregivers in this study reported they provided care for 405 different conditions. [24] In keeping with other research on caregiving, to qualify for this survey, adult rare caregivers had to assist their care recipient with at least one Activity of Daily Living (ADL), Instrumental Activity of Daily Living (IADL), or medical/nursing task.[25] For those caring for a child, the only qualifying factor was self-identification as a caregiver.[26]

D. Sample

Rare Disease Caregiving in America utilized targeted convenience and snowball samples, recruiting potential caregivers through three different main points of contact. The first avenue of contact was outreach through targeted distribution of the survey link via advocacy groups (including the global patient advocacy group, Global Genes), disease organizations, and at a national conference for rare illnesses. This included email distribution to patient advocacy groups and distribution through news media, including the Rare Disease Report blog and RARECast podcast. The second avenue was through targeted online and social media placed advertisements, specifically targeting individuals caring for someone with a rare disease, through the National Alliance for Caregiving's Facebook page, and the social media pages for the Alliance and Global Genes. The third avenue was an email invitation sent to 424 rare caregivers who had completed the study as of October 10, 2017, asking them to share a message and the survey link with other rare caregivers they knew.

Regardless of avenue, all potential survey respondents were instructed or directed to click on a website link which sent potential respondents to the National Alliance for Caregiving's landing page, who were then redirected to the Greenwald & Associates' survey platform.

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[23] List generated by GARD on August 1, 2017.

[24] For a full look at the rare diseases, conditions, or illnesses that rare caregivers report caring for, see Appendix B: List of Rare Conditions.

[25] Activities of Daily Living (ADLs) include helping the care recipient with: bathing or showering, getting to and from the toilet, getting in and out of beds or chairs, dressing, dealing with incontinence or diapers, or feeding them. Instrumental Activities of Daily Living (IADLs) include helping with: arranging or providing transportation, shopping, housework, preparing meals, managing finances, giving medicines or injections, or arranging services.

[26] This qualification criteria are comparable to past research done on caregivers of children under age 18 in "Caregiving in the U.S. 2009," as assisting with ADLs or IADLs is necessary for all children.

E. Fielding

The questionnaire was programmed and hosted by Greenwald & Associates using mobile optimized online survey software, allowing respondents to more easily take the survey on a mobile device, such as a tablet or smartphone. Six in ten took the survey on a smartphone (60%), 34% used a desktop or laptop, and 6% used a tablet. The average length of time to complete the survey was 18.4 minutes. The online surveys were conducted between September 13 and November 1, 2017.

F. Data Cleaning

After fielding, data were reviewed for quality purposes. Respondents were removed from the analysis if they did not fit the study population definition. Reasons for removal from analysis included:

- duplicate response from the same person (the first response to the survey was kept, any latter responses were deleted);
- the reason for care was *not* a rare disease or condition;
- care was provided more than 12 months prior to survey (but not within the 12 months leading up to, and including, the time of survey);
- the respondent answered about multiple people and it was not possible to distinguish which care recipient the responses were about; or,
- care was paid as part of employment or job description.

Because no unique identifiers were used in the survey link, the survey platform utilized browser cookies to prevent duplication of respondents. Respondents were also asked to provide their email address to check for duplicate respondents. A total of 1,423 people responded to the survey and 76% of respondents provided their email address for duplication checks. Additionally, data were reviewed for quality issues such as straight-lining, speeding, or inconsistent results. A total of 1% of respondents (17 completes) were removed from the final results for duplication or quality issues.

The final result was a total of 1,406 online surveys conducted with caregivers of an adult or child with rare disease or condition.

G. Weighting and Margin of Error

Data presented in this report are not weighted. Methods for weighting the 1,406 rare caregivers were explored with the intent to make respondents demographically representative. However, weighting benchmarks or demographic targets for caregivers of an adult or child with rare disease are unknown based on any current nationally-representative study.

The sample used for this project was a non-probability convenience sample, which means traditional estimates like margin of error do not apply. However, if a project were completed with a comparable number of rare caregivers using a probability-based sample, we would expect a margin of error of plus or minus approximately 2.7 percentage points at the 95% confidence level. This means that 95 times out of 100, a difference of greater than roughly 2.7 percentage points would not have occurred by chance. Subgroup analyses would have a larger margin of error.

IV. Detailed Findings

A. Basics of the Caregiving Situation

Who is the Care Recipient?

It is estimated that 25 to 30 million Americans have a rare disease or condition.[27] According to *Rare Disease Caregiving in America*, caregiving for a child is the norm among caregivers of someone with a rare disease or condition, with 62% caring for a child under the age of 18, while 38% care for someone age 18 or older. Because past research has shown that the caregiving situation of those caring for a child can vary from those caring for an adult,[28] this report compares these two groups – rare caregivers of a child and rare caregivers of an adult – throughout.

Gender and Age of Care Recipient

Among rare caregivers, half are caring for a male (50%) and half are caring for a female (50%). Those caring for children were more likely to report that the child is a male (54%), as opposed to rare caregivers caring for an adult (43%). Past research has shown that as a care recipient's age increases, so does the likelihood that the care recipient is a female.[29]

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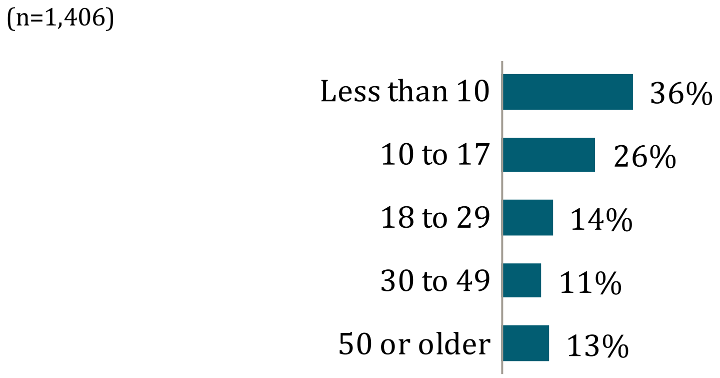
[27] NIH Genetic and Rare Diseases Information Center (GARD), National Center for Advancing Translational Sciences (NCATS).

[28] "Caregiving in the U.S. 2009"

[29] "Caregiving in the U.S. 2009" found that among caregivers of children, 59% report caring for a male, while just 35% of caregivers of adults cared for a male. This pattern may be due to demographic patterns due to longer life expectancy of women in the United States. For more information on life expectancy by gender and age, see "Health, United States, 2016," U.S. Department of Health and Human Services, Centers for Disease Control and Prevention, National Center for Health Statistics, 2016. https://www.cdc.gov/nchs/data/hus/hus16.pdf#015.

Rare caregivers are caring for a care recipient who is 20.9 years old, on average. One in three are under age 10 (36%) and 26% are between the ages of 10 and 17. On average, rare caregivers of a child report that the child is 8.5 years old, while rare caregivers of an adult say their care recipient is 41.4 years old, on average. General caregivers of a child care for someone 8.7 years old, about the same as rare caregivers of a child.[30] By comparison, general caregivers of an adult care for someone nearly 30 years older -- 69.4 years old, on average.[31]

Figure 1: Care Recipient Age



Child CR (n=871)	Adult CR (n=535)
58%^	--
42%^	--
--	38%^
--	28%^
--	33%^

CR = Care Recipient
^ significantly higher than comparison group

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[30] "Caregiving in the U.S. 2009"
[31] "Caregiving in the U.S. 2015"

Spotlight: Care Recipients Age 50 or Older

Spotlight: More than one in ten rare caregivers are providing care to an adult age 50 or older (13%). Figure 2 shines a spotlight on these caregivers by highlighting what is unique about them.[32]

Figure 2: Spotlight on Rare Caregivers of Recipient Age 50+

Who?

- On average, 56 year old caring for 64 year old
- 63% care for spouse; 27% care for a parent
- Been providing care for 6.8 years

Care situation

- 63% are sole caregiver; 74% have no paid help
- 69% do medical/nursing tasks
- Perform 2.3 ADLs and 5.5 IADLs, on average

Impact?

- Lowest use of respite (13%)
- 41% report high financial strain (lowest)
- More often have long-range care (53%) and end-of-life (47%) plans

Rare caregivers of an adult age 50 or older are the oldest group of rare caregivers surveyed – 55.8 years old on average. Most care for a spouse/partner (63%) or parent (27%) who is covered by Medicare (59%): 34% are male and 66% are female. They are relatively new to their role, having provided care for 6.8 years. Rare caregivers of an adult age 50+ take on a variety of tasks: 2.3 ADLs, 5.5 IADLs, and 69% do medical/nursing tasks, comparable to rare caregivers of younger adults. However, they more often tackle care alone: 63% are the sole caregiver and 74% have no paid help. They rarely get a break, with just 13% using respite. Long-range planning is more common: 53% have plans in place for their recipient's future care and 47% say their recipient has end-of-life plans. Comparable to rare caregivers of younger adults, 58% report high emotional stress, 56% are in a high-burden care situation, and 35% report high physical strain. They face fewer financial impacts: 41% report high financial strain and 77% have experienced at least one negative financial event due to caregiving. However, 61% still worry about paying for care.

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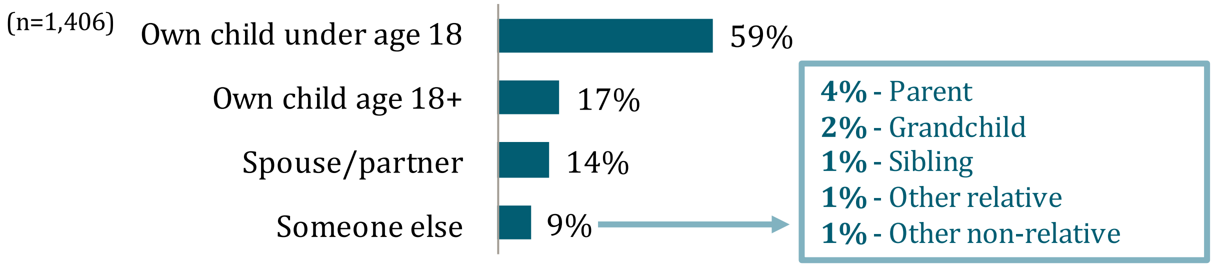
[32] Spotlights on rare caregivers provide a snapshot glance at significant findings that make this subgroup unique relative to comparison groups—in this case, the comparison group is rare caregivers caring for a younger aged adult (aged 18-49).

Relationship

Rare caregiving seems to be a role taken on by only the most immediate family members. Past research has shown that the younger the care recipient is[33] or the greater the care needs[34] are, the more often care falls to a close relative rather than extended family or friends. The study methodology, however, of recruiting rare caregivers via patient advocacy groups, disease organizations, and caregiver groups may be skewing these results. It may be that extended family or friends of someone with a rare disease or condition are out there and providing care but are less likely to be involved in these kinds of groups and therefore, may be less likely to be represented in this survey data.

Three out of five rare caregivers are a parent who provides care to their own child under the age of 18 (59%), while another 17% are a parent caring for an adult child (age 18 or older). One in seven rare caregivers care for a spouse or partner (14%). Roughly one in ten assist some other relative or a non-relative with their rare disease or condition (9%).

Figure 3: Relationship of Care Recipient to Caregiver



Younger generations of caregivers tend to care for younger recipients. Millennial caregivers (age 18-36) and Gen X caregivers (age 37-52) tend to be caring for their own child (83% and 82%, respectively), while Boomers and older generations of caregivers (age 53 or older) more often care for a spouse or partner (27% vs. 7% of Millennial and 12% of Gen X caregivers).

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[33] "Caregiving in the U.S. 2009" showed that among caregivers of children, most were parents or grandparents of the child needing care (55% parents, 18% grandparents).

[34] "Caregiving in the U.S. 2015" showed that among adult care recipients, the greater the needs of the recipient, the more often caregiving falls on the shoulders of a relative. Caregivers who provide the greatest hours of care and do the most activities for their care recipient (also known as "high-burden" caregivers) are more often taking care of a relative (90%). The "burden" of care is a simplified version of the Level of Care index, which is created by combining caregiver hours of care and assistance with ADLs and IADLs.

Spotlight: Parents Caring for Their Own Child

Spotlight: Six in ten rare caregivers surveyed are a parent caring for their own child under age 18 (59%). Figure 4 shines a spotlight on these caregivers by highlighting what is unique about them.[35]

Figure 4: Spotlight on Parents Caring for their Own Child Under Age 18

Who?
- On average, 40 year old caring for an 8.5 year old
- 54% care for a son; 46% care for a daughter
- Been providing care for 7.3 years

Care situation
- Half provide constant care (47%)
- Medical/nursing tasks are a hallmark (92%)

Impact?
- High burden of care (74%)
- Struggles to maintain friendships (57%); feels alone (58%)
- 66% have difficulty maintaining their own health
- 67% stopped saving for long term; 65% used up personal savings

Parents who are providing care to their own child with a rare disease or condition often provide around-the-clock care and have been doing so for close to their child's entire life. Rare caregiver parents often face impacts on their well-being, reporting lower levels of emotional or mental health and struggles with maintaining friendships and feeling alone. Two out of three find it difficult to maintain their own health (66%), though just 30% report having been asked about their own self-care by a care provider. Many report financial impacts, such as not saving for the long term or using up personal savings at a phase in their lives of typical prime, working, and earning years in that parents caring for their own child are 40 years old, on average. This financial ripple effect is likely to be felt for many years to come.

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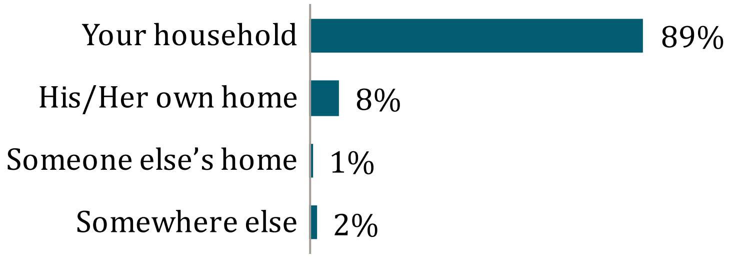
[35] Spotlights on rare caregivers provide a snapshot glance at significant findings that make this subgroup unique relative to comparison groups—in this case, the comparison group is rare caregivers caring for someone of another relationship (spouse, sibling, parent, friend) or parents caring for an adult child.

Residence of Care Recipient

About nine out of ten rare caregivers (89%) report that their care recipient lives in the same household with them, more commonly reported when the care recipient is a child under age 18. Three out of four (76%) rare caregivers of an adult live with their care recipient and only 7% report that their care recipient lives alone.

Figure 5: Residence of Care Recipient

(n=1,406)



	Child CR (n=871)	Adult CR (n=535)
Your household	96%^	76%
His/Her own home	3%	16%^
Someone else's home	<0.5%	3%^
Somewhere else	<0.5%	5%^

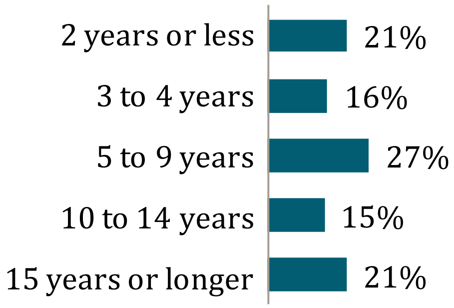
CR = Care Recipient
^ significantly higher than comparison group

Duration of Care

Rare caregiving is a long-term role, lasting 8.9 years on average, more than double the caregiving commitment found among caregivers more generally.[36] The majority of rare caregivers (63%) has provided care for longer than five years and 36% have provided care for ten years or longer. Nearly two out of every five rare caregivers of adults (37%) have provided care for 15 years or longer.

Figure 6: Duration of Care

(n=1,391)



	Child CR (n=866)	Adult CR (n=525)
2 years or less	20%	24%
3 to 4 years	18%^	12%
5 to 9 years	30%^	21%
10 to 14 years	20%^	7%
15 years or longer	11%	37%^

CR = Care Recipient
^ significantly higher than comparison group

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[36] "Caregiving in the U.S. 2015" found caregivers of adults care for 4.0 years on average, while "Caregiving in the U.S. 2009" found that caregivers of children under age 18 care for 4.2 years on average.

B. Care Recipient Condition

Rare caregivers tend to provide care to someone whose rare disease or condition is genetic in nature, though the list of conditions for which care is provided is long and varied (for full list of conditions in this study, see *Appendix B* available on www.caregiving.org/rare). Many rare caregivers report there is no treatment for their care recipient's condition, and rather, that treatments or medications are used for therapy for symptoms, rather than actual treatment of the disease or condition.

Genetic

Seven in ten rare caregivers say their care recipient's condition is genetic. More than three out of four rare caregivers of a child say the child's condition has a genetic cause or component (78%), significantly more than those caring for an adult (60%). Caring for multiple people who have the same rare condition or disease is not uncommon. One in five (19%) report that multiple people in their family have the same genetically-linked rare disease or condition.

Although most rare caregivers report that their care recipient with a genetic condition has undergone testing (84%) and counseling (73%), many have not (13% and 23%, respectively).

Figure 7: Genetic Testing and Counseling

Among those whose recipient's condition is genetic (n=999)

	Child CR (n=679)	Adult CR (n=320)
Got genetic testing	88%^	76%
Consulted with genetic counselor	79%^	60%

CR = Care Recipient
^ significantly higher than comparison group

Most Common Rare Conditions

The list of rare conditions and diseases that rare caregivers report for their care recipient is long – with over 400 indicated (see full list of conditions mentioned by rare caregivers in *Appendix B*, available online at www.caregiving.org/rare). The most commonly mentioned rare conditions were cystic fibrosis (9%), pulmonary arterial hypertension (4%), atypical hemolytic uremic syndrome (2%), Ehlers-Danlos syndrome (2%), Fabry disease (2%), congenital central hypoventilation syndrome (2%), and pediatric autoimmune neuropsychiatric disorder (2%).

Classifying the full list of rare conditions or diseases into categories,[37] rare caregivers most often reported their care recipient having a condition related to congenital and genetic diseases (52%) or nervous system diseases (30%).

This pattern varied by child caregivers and adult caregivers, with those caring for a child more often reporting the presence of rare diseases related to congenital/genetic, digestive, lung, male reproduction, and musculoskeletal. Those caring for an adult more often report conditions related to the nervous system, blood, eye, skin, metabolic, heart, kidney/urinary, endocrine, and rare cancers.

Figure 8: Top Rare Disease Categories

Categories with 5% or greater, Multiple categories per condition allowed	All Rare Caregivers (n=1,406)	Child Care Recipient (n=871)	Adult Care Recipient (n=535)
Congenital and genetic disease	**52%**	56%^	46%
Nervous system disease	**30%**	26%	38%^
Digestive disease	**15%**	18%^	10%
Blood disease	**14%**	11%	17%^
Newborn screening	**13%**	15%^	9%
Lung disease	**12%**	15%^	8%
Male reproductive disease	**12%**	14%^	7%
Eye disease	**12%**	9%	15%^
Skin disease	**11%**	9%	14%^
Metabolic disorders	**10%**	8%	13%^
Heart disease	**10%**	5%	18%^
Kidney and urinary disease	**9%**	7%	13%^
Musculoskeletal disease	**8%**	10%^	5%
Rare cancers	**6%**	5%	8%^
Endocrine disease	**6%**	4%	8%^
Unclassified by NIH	**10%**	13%^	5%

^ significantly higher than comparison group

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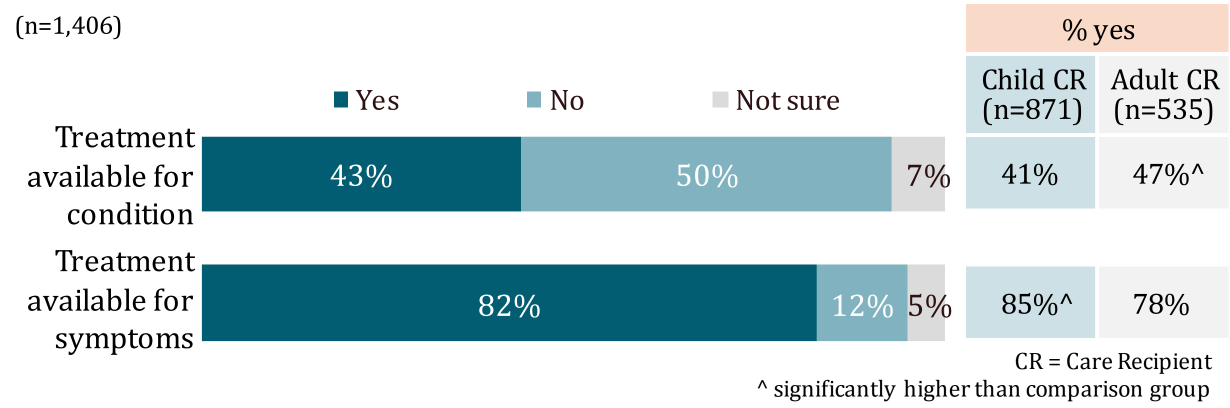
[37] The Genetic and Rare Diseases Information Center; National Center for Advancing Translational Services; National Institutes of Health: Disease listing by category, accessed and classified December 2017. https://rarediseases.info.nih.gov/diseases/categories

Treatment and Therapy

Although many rare caregivers report feeling that their care recipient was adequately diagnosed (88%), there is not always a next step toward treatment. Even though 82% report there is some sort of treatment or therapy available for managing their care recipient's symptoms, only 43% report that there is a treatment available for the underlying rare disease or condition. Those caring for an adult more often report there is a treatment available for the actual disease or condition (47%), compared to just 41% of rare caregivers of a child.

Figure 9: Treatments Available



CR = Care Recipient
^ significantly higher than comparison group

Prescription Medication

More than eight in ten rare caregivers indicate their recipient manages symptoms with a prescription medication (81%), more commonly done so when the care recipient is an adult (84% vs. 79% child care recipient). Only 59% of rare caregivers say their care recipient is taking a prescription medication to help *treat* their disease or condition. Again, adult care recipients more often take prescription medication (65%) than recipients under the age of 18 (55%).

Spotlight: Caregivers of Recipients Without Treatment

Spotlight: More than half of rare caregivers surveyed report they are unaware of any treatment for their care recipient's actual condition (57%). Figure 10 shines a spotlight on these caregivers by highlighting what is unique about them.[38]

Figure 10: Spotlight on Caregivers Who Report No Treatment for the Recipient's Rare Condition

Who?
- 44 year old female (88%) caring for own child (79%) who is 19.4 years old, on average
- 73% have children or grandchildren living in their household

Care situation
- Provide 52.0 hours of care per week
- 55% do medical/nursing tasks without training
- 64% have other unpaid help; 40% have paid help

Impact?
- High emotional stress (71%); physical strain (41%)
- 56% need medical professional help but have trouble finding one
- High burden of care (74%)

Rare caregivers who report their recipient's rare disease has no treatment report more difficulty finding formal avenues of care. Three out of four report their care recipient has a genetic issue or related condition (76%) and frequently, they have gotten genetic testing (87%) and seen a genetic counselor (78%). However, just 72% say there are therapies or treatments for symptoms and 38% report having difficulty accessing those. One in three feel their care recipient's local hospital can handle their condition (33%), and a majority (56%) report their care recipient needs, but has trouble finding a medical professional to care for their condition. Although half say their recipient needs a clinical trial (51%), just 20% report their care recipient has participated in one. The uncertainty of having no treatment seems to take an emotional toll, with 71% of rare caregivers reporting high emotional stress. They may be filling care gaps by taking on more themselves: helping for 52.0 hours per week, doing 2.5 ADLs and 3.5 IADLs, on average, causing physical strain (41%). They do rely on other caregivers (64%) or services to provide care: 40% have paid help and the rare caregivers have more often modified their recipient's home (46%) or used respite services (27%). Despite this additional help, they more often feel alone (58%) or feel they have been unable to maintain friendships (56%).

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[38] Spotlights on rare caregivers provide a snapshot glance at significant findings that make this subgroup unique relative to comparison groups—in this case, the comparison group is rare caregivers who report their care recipient's actual condition has a treatment.

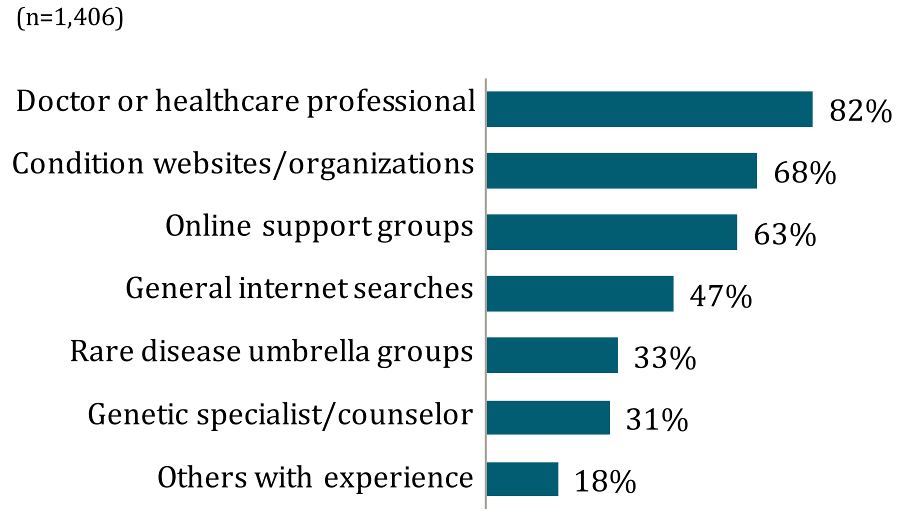
C. Navigating the Health Care System

Doctors and medical specialists are trusted and heavily relied upon by rare caregivers to help them address a rare disease or condition but are often difficult to access. Some rare caregivers report having difficulty accessing treatments, therapy, and services: few have had conversations with care professionals about self-care.

Information Sources

Doctors and health-care professionals are instrumental in providing help to rare caregivers' care recipients. More than eight in ten (82%) rare caregivers rely on doctors and health-care professionals for information, while 68% turn to condition-specific websites or organizations, 33% look to general rare disease groups,[39] and 31% turn to a genetic counselor or specialist. Those caring for a child are more likely than those caring for an adult to report having used most sources of help or information.

Figure 11: Information Sources



Child CR (n=871)	Adult CR (n=535)
84%^	78%
73%^	60%
74%^	45%
46%	49%
37%^	27%
40%^	18%
17%	21%

CR = Care Recipient
^ significantly higher than comparison group

Rare Caregivers Information Sources

- Women caregivers more often report using condition-specific websites or organizations (70% vs. 52% of men), online support groups (66% vs. 42%), general rare disease or condition umbrella groups (34% vs. 24%), and genetic counselor or specialist (33% vs. 22%).

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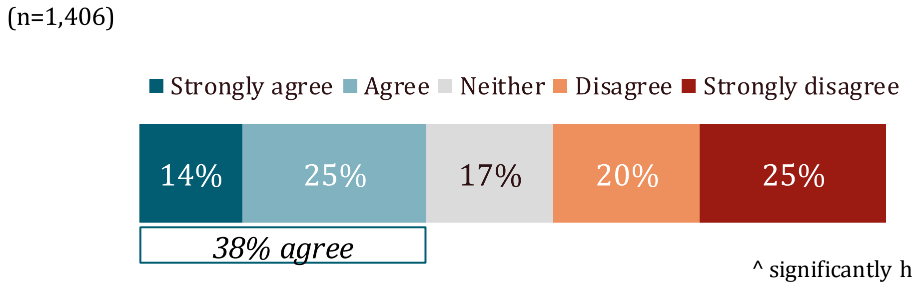
[39] Due to the sampling and recruitment method for this study of outreaching to caregiver and disease support groups, this result is likely higher than one would expect in a general population of rare disease caregivers.

- Non-Hispanic Caucasians more often report using condition-specific websites or organizations (69% vs. 61% of other racial/ethnic groups) and online support groups (65% vs. 52%).[40]
- Millennial (those age 18-36) and Gen X (ages 37-52) rare caregivers turn more often to online support groups (68%) than Boomer or older rare caregivers (ages 53 or older; 48%). Gen X and older caregivers are more likely to turn to in-person support groups (20%) than Millennial rare caregivers (13%).
- Those whose care recipient has a genetic condition more often turn to a genetic specialist or counselor for help or information (39% vs. 13% caring for someone with an unknown or non-genetic condition).

Hospital, Treatments, and Therapies

Despite the high reliance upon doctors and medical professionals, only 38% feel their care recipient's local hospital can handle their underlying rare disease or condition, with rare caregivers of a child more often feeling that the local hospital was prepared to handle the condition (41% vs. 34% among those caring for an adult).

Figure 12: Ability of Hospital to Handle Condition



% agree	
Child CR (n=871)	Adult CR (n=535)
41%^	34%

CR = Care Recipient
^ significantly higher than comparison group

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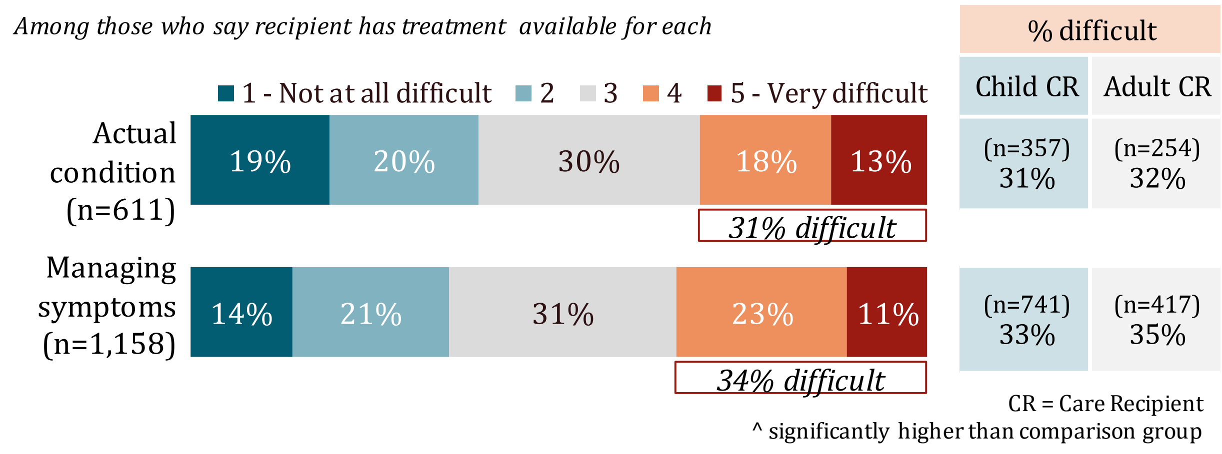
[40] As has been found in other caregiver surveys with recruitment through disease-specific organizations and groups (see Gibson Hunt, G., R. Greene, C. Grace Whiting, L. Weber-Raley; "On Pins and Needles: Caregivers of Adults with Mental Illness," National Alliance for Caregiving, 2015), women and whites overwhelmingly responded and were also more likely to report engagement in, and use of, these types of groups for information or help. Therefore, this may indicate that disease and caregiver support organizations should strive to diversify their outreach to these under-represented populations.

Among those rare caregivers who report that there is a treatment or therapy for managing their care recipient's symptoms, one out of three report difficulty accessing that treatment or therapy (34%, see Figure 13). Among those who say there is a treatment for the condition, nearly as many have difficulty accessing it (31%).

Figure 13: Accessibility of Therapies and Treatments



CR = Care Recipient
^ significantly higher than comparison group

Rare Caregivers Difficulty with Local Services

- Rare caregivers who have more "clarity" about their care recipient's condition – in that they know the condition is genetic or that a treatment is available – more often feel their care recipient's local hospital can handle the condition (42% of rare caregivers whose care recipient has a genetic condition vs. 29% otherwise; and 45% with a known treatment vs. 33% otherwise).

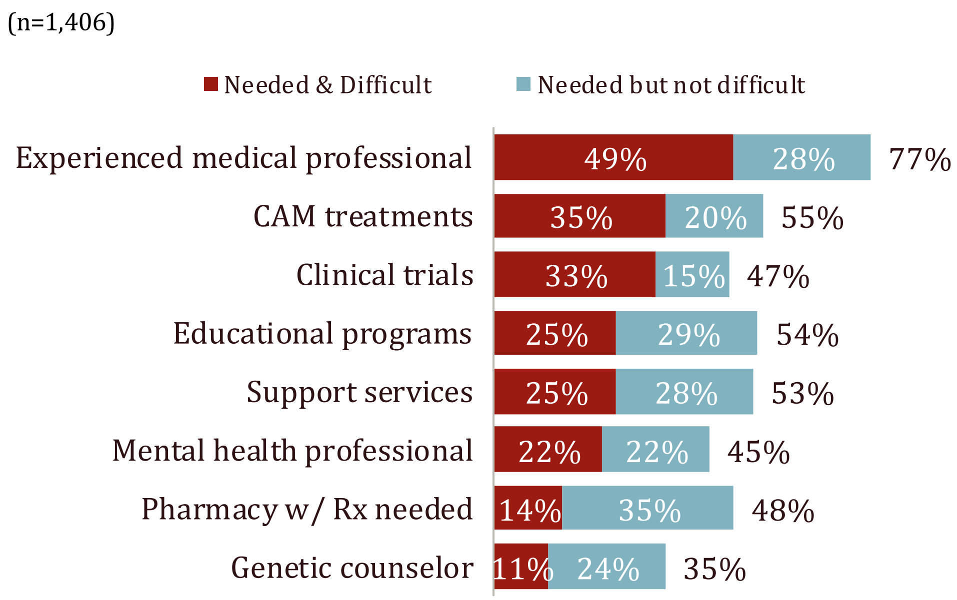
- Rare caregivers whose care recipient lives in a rural area express more doubts about the local hospital: only 27% agree that their care recipient's local hospital can handle their condition, compared to 44% of those caring for someone in an urban or suburban area. In addition, rare caregivers of a rural-dwelling care recipient more often report troubles accessing therapies or treatments for symptoms (39% vs. 32%).

Services Utilized and Needed by Care Recipient

When asked about a series of eight services their care recipient needs, three out of four rare caregivers say their care recipient needs a medical professional with experience treating the underlying rare condition (77%), while more than half feel their care recipient needs complementary or alternative (CAM) treatments (55%), educational support services or programs (54%), and/or a case manager (53%). Rare caregivers of a child often report needing each of these services, more so than rare caregivers of an adult, with the exception of a mental health professional.

Rare caregivers indicate there are gaps in care or support services for their recipient. Among all rare caregivers, half indicate that an experienced medical professional is needed and difficult to find (49%). More than one in three say it is difficult to find complementary or alternative (CAM) treatments in their care recipient’s area (35%).

Figure 14: Service Needs and Availability



% need	
Child CR (n=871)	Adult CR (n=535)
81%^	70%
57%^	51%
51%^	41%
64%^	38%
58%^	45%
46%	44%
54%^	39%
43%^	23%

CR = Care Recipient
^ significantly higher than comparison group

Among those who do need it, access to a variety of treatment and support services is problematic, with two out of three who would need a clinical trial reporting they are difficult to find (69%). More than six in ten have trouble accessing complementary or alternative treatments (64%) or an experienced medical professional (63%).

Figure 15: Most Difficult to Find Services

	% Difficult to Find *Among those who need it*		
	All Rare Caregivers	Child Care Recipients	Adult Care Recipients
Clinical trials	69%	70%	68%
Complementary or alternative (CAM) treatments	64%	66%	60%
Medical professional with experience treating condition	63%	61%	66%
Mental health professional	50%	49%	51%
Case manager or social worker	47%	45%	51%
Educational support services	46%	44%	51%

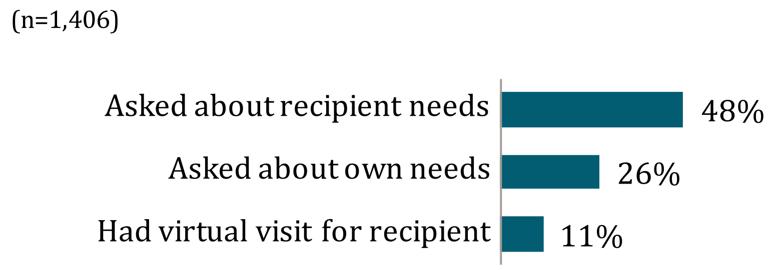
^ significantly higher than comparison group

Caregiver Interaction with Care Professionals

Rare caregivers may need more support from the doctors and care professionals with whom they interact. Fewer than half (48%) have had a doctor, nurse, or social worker ask what they need to care for their care recipient. Even fewer (26%) have had a medical professional or social worker ask the rare caregiver what they need to take care of themselves. Just 11% report having had a virtual or online visit with a doctor who could provide care for their care recipient.

Rare caregivers of a child more often report having experienced each of these three interactions. This is an area where rare caregivers seem to be getting more support than caregivers generally: just 32% of caregivers had a care professional ask about care recipient needs and 16% had been asked about their own self-care.[41]

Figure 16: Caregiver Interaction with Care Professionals



	Child CR (n=871)	Adult CR (n=535)
Asked about recipient needs	52%^	41%
Asked about own needs	29%^	22%
Had virtual visit for recipient	13%^	8%

CR = Care Recipient
^ significantly higher than comparison group

Rare Caregivers Differential Interactions

- Rare caregivers who are parents caring for their own child under age 18 are most likely to report having a care professional ask about their needs, both for caring for their child (53% vs. 40% all other) and for themselves (30% vs. 21% all other).
    - Perhaps reflecting a tendency of pediatric care providers to focus on the family unit, rare caregivers of a child under age 10 more often report being asked about care needs (57% care recipient needs; 31% self-care needs).
- Rare caregivers with household incomes of at least $50,000 more often have had an online doctor visit or consult (13%) than those with lower incomes (6%).

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[41] "Caregiving in the U.S. 2015"

Rare caregivers voice concerns about accessing services for their care recipient and the challenges faced navigating the medical care system:

“Of the 75+ doctors she's seen, there has only been one with the desire to truly help her, understand her needs and the interplay between her conditions, and who is willing to be her advocate when needed. We need more doctors who can do that.”

“I have lost a lot of confidence in the medical community… I don't feel like my daughter has ever been adequately diagnosed or treated…I wish she could be adequately treated for pain and get an accurate, complete diagnosis.”

“The treatments for [his issues] are either unavailable in our area or unworkable because of the complications of his condition. The goal is to maintain quality of life at as high of a level as possible for as long as possible. But, given his rapid decline over the last two years, I don't have hope for improvement or better treatment options in his lifetime.”

“We just don't fit into any box, making it more difficult to find resources.”

“[In our state] the whole disability housing model has changed. They are no longer looking to provide group homes for individuals, and it is our job to locate housing with an apartment, buy a home with other parents, and then use the paid staff to come in…Right now, we are backup for all the staff. A day will come when we cannot do this!”

D. The Life-Changing Nature of Rare Caregiving

Rare caregiving becomes a role that fundamentally alters the way the caregiver lives their daily life. They find themselves spending many hours doing a variety of tasks for the person with the rare disease or condition, combining to place a high “burden of care” [42] on rare caregivers, lived day-to-day over the course of many years.

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[42] “Burden of care” refers to the Burden of Care Index, which gages the complexity and challenges of the caregiving situation. For more detail see section below on Burden of Care.

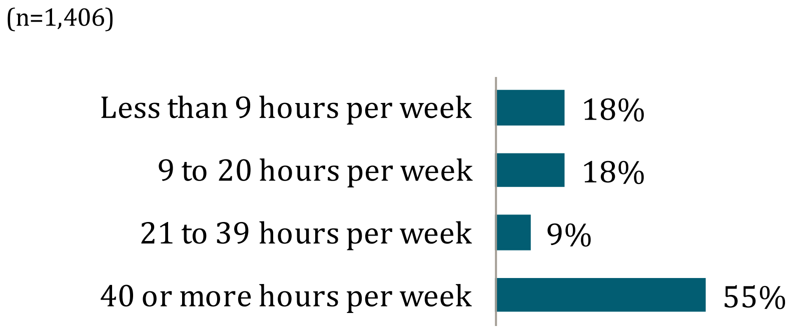
Choice

Becoming a caregiver for someone with a rare disease or condition can be a life-changing event. Among those who were asked,[43] 69% of rare caregivers say they had no choice other than to take on their role. Seven out of ten rare caregivers of an adult say they had no choice (72%), while 49% of caregivers of adults nationally felt they had no choice in taking on their role.[44]

Hours of Care

Rare caregiving is time-intensive: most rare caregivers are the primary care provider and devote many hours each week to providing care. More than half of rare caregivers spend 40 hours a week or more caregiving (55%), with 38% reporting they provide constant care “around the clock.” On average, rare caregivers of adults spend 36.7 hours a week providing care – 12 hours more a week than caregivers of adults generally.[45] Even more striking is the amount of care provided weekly by rare caregivers of a child: 53.0 hours compared to 29.7 hours weekly for general child caregivers.[46]

Figure 17: Hours of Care



	Child CR (n=871)	Adult CR (n=535)
Less than 9 hours per week	12%	26%^
9 to 20 hours per week	15%	24%^
21 to 39 hours per week	8%	10%
40 or more hours per week	64%^	40%

CR = Care Recipient
^ significantly higher than comparison group

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[43] Rare caregivers who were caring for their own child under the age of 18 were not asked about their choice in taking on care for their own child.
[44] “Caregiving in the U.S. 2015”.
[45] Caregivers of adults nationally, caring for an adult age 18+ for any condition, spend 24.4 hours per week providing care, on average. “Caregiving in the U.S. 2015”.
[46] Caregivers of children nationally, who are caring for a child age 17 or younger for any condition, spend 29.7 hours per week providing care, on average. “Caregiving in the U.S. 2009”.

Rare Caregivers Putting in High Amounts of Care Hours

- Rare caregivers with the youngest care recipients – those under age 10 – have the most time-intensive care role: 57.4 hours per week, on average. Those with older child care recipients (age 10 to 17) provide 47.0 hours of care weekly.
    - Rare caregivers of adults show no differentiation of hours of care as their care recipient ages, as those caring for someone age 18-29 (38.9 hours) show comparable time commitment as those caring for older adults (35.7 hours when caring for someone age 30-49 and 35.1 hours caring for someone age 50 or older).
- Rare caregivers who report there is no treatment available for their care recipient's rare disease or condition seem especially likely to put in even more hours. On average, those rare caregivers who report there is no treatment available spend 12 more hours each week providing care (52 hours), as compared to their counterparts who say their care recipient's condition does have a treatment available (40 hours per week).
- Rare caregivers who report their care recipient relies upon government insurance (includes Medicare, Medicaid, and/or Children's Health Insurance Program) spend about 55.7 hours a week providing care on average, which is significantly higher than rare caregivers who report their care recipient has private insurance coverage (includes employer or individually-purchased health plans; 44.7 hours per week).
- Rare caregivers who perform medical/nursing tasks (a body of caregiving tasks that are highly skilled like giving injections, wound care, tube feedings, and so on[47]) spend double the amount of time providing care – 51.2 hours on average – as those caregivers who do not perform these tasks (24.0 hours).

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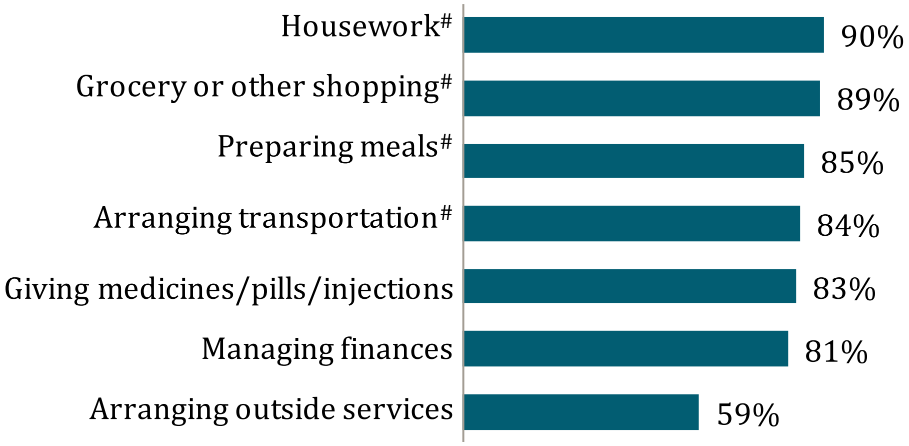
[47] For more detail, see Section IV. E. The Underlying Job of Rare Disease Expert; Medical/Nursing Tasks.

Instrumental Activities of Daily Living (IADL)

Rare caregivers assist their care recipients with a variety of activities, including Instrumental Activities of Daily Living (IADLs). Nearly all rare caregivers help their care recipient with at least one IADL. Among rare caregivers of an adult, help is most often provided with housework (90%), grocery or other shopping (89%), meal preparation (85%), and transportation (84%).[48] Rare caregivers of a child more often help their care recipient with medication administration (91% vs. 70% of rare caregivers of an adult) and service coordination (64% vs. 52%), though a majority of rare caregivers of an adult still do both activities.

Figure 18: Instrumental Activities of Daily Living

(n=1,406)



	Child CR (n=871)	Adult CR (n=535)
Housework#	n/a	90%
Grocery or other shopping#	n/a	89%
Preparing meals#	n/a	85%
Arranging transportation#	n/a	84%
Giving medicines/pills/injections	91%^	70%
Managing finances	80%	83%
Arranging outside services	64%^	52%

Asked of caregivers of adults only

CR = Care Recipient
^ significantly higher than comparison group

On average, rare caregivers of an adult perform 5.5 out of seven possible IADLs, while rare caregivers of a child perform 2.3 out of three possible IADLs.

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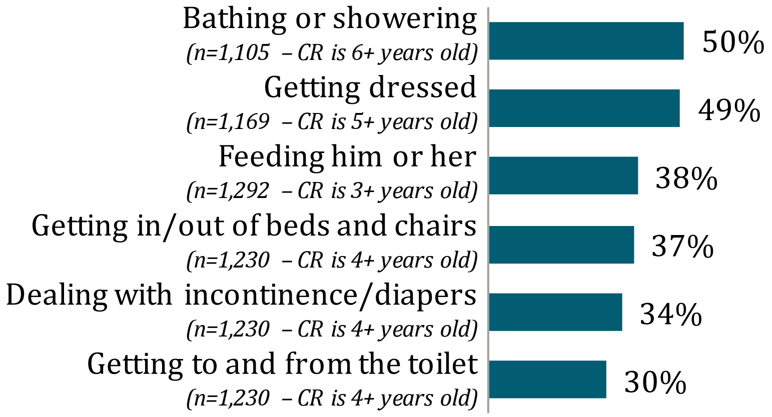
[48] Given the age of their care recipients, rare caregivers of a child were not asked about helping with housework, grocery or other shopping, preparing meals, or transportation – as all of these activities would be presumed to be something done for a child regardless of the presence of a rare condition or disease.

Activities of Daily Living (ADL)

Three in five rare caregivers (62%) help with at least one Activity of Daily Living (ADL). Bathing (50%) and dressing (49%) are the most common ADLs with which care recipients need assistance. Rare caregivers of a child are more likely to help with each of the six ADLs with one notable exception: rare caregivers of an adult more often have to help their care recipient in and out of beds and chairs. On average, rare caregivers help with 2.0 out of six ADLs.

Figure 19: Activities of Daily Living



Child CR (n=871)	Adult CR (n=535)
55%^	44%
53%^	45%
44%^	31%
33%	42%^
40%^	27%
30%	29%

CR = Care Recipient
^ significantly higher than comparison group

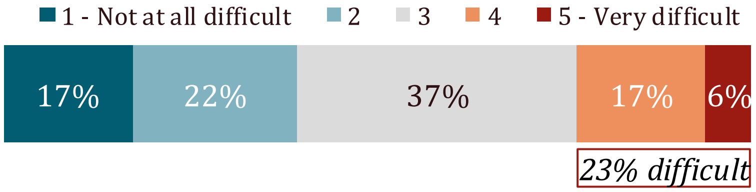
Rare Caregivers Doing More ADLs

- Rare caregivers who report there is no treatment available for their care recipient's rare disease or condition perform 2.5 ADLs, on average. They are more likely than those who say there is a treatment available to do each of the six ADLs.
- Rare caregivers who say their care recipient receives paid help perform 3.1 ADLs on average, significantly more than those who have no paid help (1.5 ADLs), perhaps pointing to the higher needs of their care recipient.
- Those with household incomes under $100,000 do more ADLs (2.1 on average), as compared to their higher income counterparts (1.8 on average).

Among rare caregivers doing at least one ADL, 23% find it difficult to perform these kinds of tasks.

Figure 20: Difficulty Performing ADLs

Among those assisting with ADLs (n=868)



% difficult	
Child CR (n=530)	Adult CR (n=338)
20%	28%^

CR = Care Recipient
^ significantly higher than comparison group

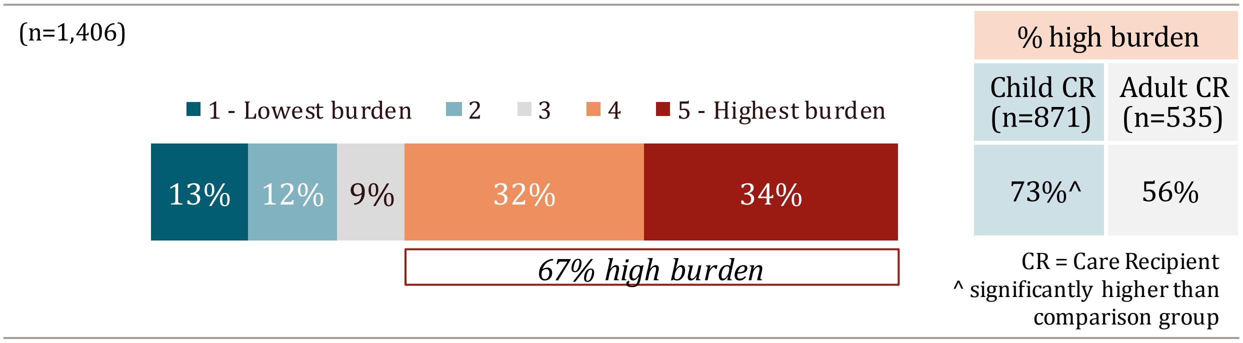
Those especially likely to feel helping with ADLs is difficult include those caring for their own adult child (30% difficult), long-term caregivers (29% when caring for 10 or more years), and older caregivers (27% age 50 or older). This suggests that the difficulty of performing ADLs, many of which are physical in nature, increases over time as both the rare caregiver and the care recipient age.

Burden of Care Index

The Burden of Care Index was created to gauge the complexity and challenges of the *care situation* for the caregiver. This Index provides one way to articulate the impact of a disease or disability on the people who care for a patient during the caregiver journey.

Considering all the responsibilities that rare caregivers often shoulder, it is no surprise that the Burden of Care Index[49] places rare caregivers as being in an exceptionally high burden care situation. Two out of every three rare caregivers have a high caregiver burden (67%) – far higher than general caregivers.[50]

Figure 21: Burden of Care Index



Rare Caregivers Unique Burden of Care

- Rare caregivers have a high burden of care that tends to last a long period of time. Rare caregivers' burden is comparable to that of cancer caregivers where 62% of cancer caregivers have a high burden. However, that burden is relatively short and episodic: just 1.9 years on average,[51] while rare caregiving lasts much longer – 8.9 years on average.

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[49] "Burden of Care" is an index that is based on the number of hours of care provided by the caregiver, the number of ADLs performed, and the number of IADLs performed. Note that for caregivers of children, Caregiving Support Activities (CSAs) are substituted for IADLs performed. Points are assigned for each of them: ultimately, these points are consolidated into three burden categories: low (values 1–2), medium (3), and high (values 4–5). See "Caregiving in the U.S. 2015" Appendix B, Detailed Methodology for the details of creating the index (www.caregiving.org/caregiving2015).

[50] 40% of general caregivers of an adult from "Caregiving in the U.S. 2015" and 39% of general caregivers of a child from "Caregiving in the U.S. 2009" have a high caregiver burden.

[51] G.G. Hunt, C.G. Whiting, M.L. Longacre, L. Weber-Raley, and L. Popham, "Cancer Caregiving in the U.S.—An Intense, Episodic, and Challenging Care Experience," National Alliance for Caregiving, National Cancer Institute, and Cancer Support Community, 2016.

- As the Burden of Care Index increases, rare caregivers have increasing levels of stress and strain, reduced emotional and physical health, and increased feelings of isolation and personal neglect.

Figure 22: Impacts on Caregiver by Burden of Care Index

	Low burden (n=345) A	Mid burden (n=124) B	High burden (n=937) C
Stress and Strain *(rating 4 or 5 on a 5-point scale)*			
High level of emotional stress	46%	65%^{A}	75%^{AB}
High level of physical strain	12%	23%^{A}	48%^{AB}
Health			
Fair/poor emotional or mental health	27%	41%^{A}	45%^{A}
Fair/poor physical health	21%	33%^{A}	33%^{A}
Isolation *(strongly/somewhat agree)*			
Feeling alone	36%	42%	62%^{AB}
Inability to maintain friendships	30%	39%	60%^{AB}
Personal Care *(strongly/somewhat agree)*			
Difficult to care for own health	39%	54%^{A}	65%^{AB}
Able to find time for self	62%^{BC}	48%^{C}	33%

^{ABC} indicates result is significantly higher than lettered comparison group

All of the tasks that rare caregivers take on and the hours of care can add up to create a life-changing role for the rare caregiver:

> "Caring for my son has been the hardest constant trial I've ever experienced."

> "I am a well-educated person who has seen the horrors of the health-care system. There are no services for pediatric hospice or pediatric live-in nursing. This has taken a significant toll on us. To care for my son means I cannot work. Not working means that I will eventually lose my house, and my other four children will suffer."

> “Caring for someone with a rare disease…is all consuming. My family's life is totally structured around the schedule that we have to keep. As a caregiver, I am in a constant state of guilt over one child having to live with this disease and the rest of the family being constrained to living within its boundaries.”

> “Caring for a medically fragile [care recipient] is financially burdening. No one seems to understand that when your child falls ill with a disease like [condition], there is no slow progression and time to plan out a financial strategy. Things happen very quickly, and immediate decisions have to be made…When my son fell ill, I had no choice but to quit working. Because of my son's situation, I had to stop paying on loans due to loss of income, and…now my credit is ruined.”

E. The Underlying Job of Rare Disease Expert

Rare caregivers often find their daily lives changing in other ways, such that they become the “expert” on their care recipient’s condition. They “become” nurses, performing medical/nursing tasks; they are the voice of care, doing Caregiving Support Activities like coordinating care and advocating; they “become” teachers, educating health-care professionals about their care recipient’s condition; and, they “become” researchers, finding and accessing clinical trials for their care recipient.

Medical/Nursing Tasks

In addition to helping their care recipient with IADLs and ADLs, the vast majority of rare caregivers take on “medical/nursing tasks”: a body of caregiving tasks that are highly skilled in nature – things that may have typically been handled by someone like a health-care aide, nurse, or other health-care professional.[52]

More than eight in ten rare caregivers help their care recipient with medical/nursing tasks (84%). Rare caregivers more often help with these tasks than general caregivers of adults (57%[53]) – with 92% of rare caregivers of a child performing medical/nursing tasks and 71% of rare caregivers of an adult helping with these things.

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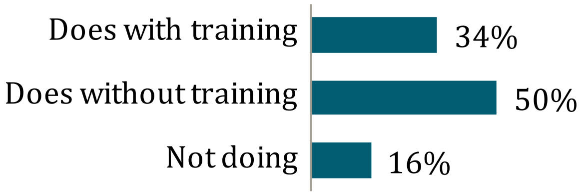
[52] This includes giving medicines like pills, eye drops, or injections; preparing food for special diets; tube feedings; wound care; monitoring blood pressure or blood sugar; helping with incontinence; or, operating equipment like hospital beds, wheelchairs, oxygen tanks, nebulizers, or suctioning tubes. See: Reinhard, S.C., Levine, C., & Samis, S. Home Alone: Family Caregivers Providing Complex Chronic Care. AARP Public Policy Institute & United Hospital Fund, 2012.

[53] “Caregiving in the U.S. 2015”

Often, these complex tasks are performed without having any prior training: half of rare caregivers do medical/nursing tasks without any preparation.

Figure 23: Medical/Nursing Task Preparation

(n=1,406)



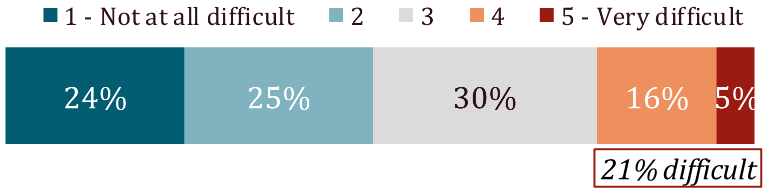
	Child CR (n=871)	Adult CR (n=535)
Does with training	41%^	21%
Does without training	50%	49%
Not doing	8%	29%^

CR = Care Recipient
^ significantly higher than comparison group

One in five rare caregivers find it difficult to help with medical/nursing tasks (21%), higher than caregivers generally.[54]

Figure 24: Difficulty Performing Medical/Nursing Tasks

Among those doing medical/nursing tasks (n=1,177)



% difficult	
Child CR (n=799)	Adult CR (n=378)
21%	20%

CR = Care Recipient
^ significantly higher than comparison group

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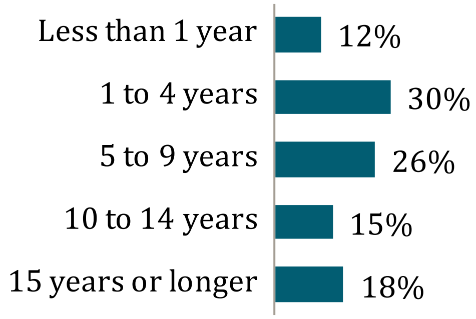
[54] 14% of caregivers of adults doing medical/nursing tasks report it is difficult; "Caregiving in the U.S. 2015"

On average, rare caregivers have been doing medical/nursing tasks for 8.2 years, with rare caregivers of a child doing these tasks for 6.6 years, with rare caregivers of an adult performing these skilled tasks for 11.8 years on average.

Figure 25: Length of Time Performing Medical/Nursing Tasks

Among those doing medical/nursing tasks (n=1,161)



	Child CR (n=795)	Adult CR (n=366)
Less than 1 year	11%	14%
1 to 4 years	32%	25%
5 to 9 years	30%^	16%
10 to 14 years	19%^	6%
15 years or longer	8%	39%^

CR = Care Recipient
^ significantly higher than comparison group

Rare Caregivers Medical/Nursing Challenges

- The longer rare caregivers have been in their role, the more likely they are to be performing medical/nursing tasks without prior preparation: 57% of rare caregivers who have provided care for 10 years or more are performing medical/nursing tasks without preparation, compared to 46% of shorter-term caregivers.

- Rare caregivers who report there is no treatment available for their care recipient's condition are especially likely to take on medical/nursing tasks without training (55%), as compared to 43% of rare caregivers who report there is treatment available. These rare caregivers are also especially likely to report difficulty with performing these tasks (24% vs. 17% of those with a treatment available).

- Rare caregivers of the youngest children are among the most likely to report doing these tasks with preparation (48% of those caring for a child under age 10), which could suggest that the newest caregivers are more often receiving training. However, there is no difference in receiving preparation for medical/nursing tasks by the length of time one has been doing medical/nursing tasks. Rather, it may be that rare caregivers of this particularly young and dependent group receive preparation *because of* the assumption that these care recipients cannot do medical/nursing tasks on their own.

- The length of time that rare caregivers have been providing care and how long they have been performing medical/nursing tasks is related to the age of their care recipient – perhaps reflecting onset and rare disease trajectory. In every case, rare caregivers have been doing medical/nursing tasks for approximately the same amount of time they have been providing care: medical/nursing tasks are a defining feature of rare caregiving.

Figure 26: Length of Caregiving and Medical/Nursing Task Performance by Age of Care Recipient

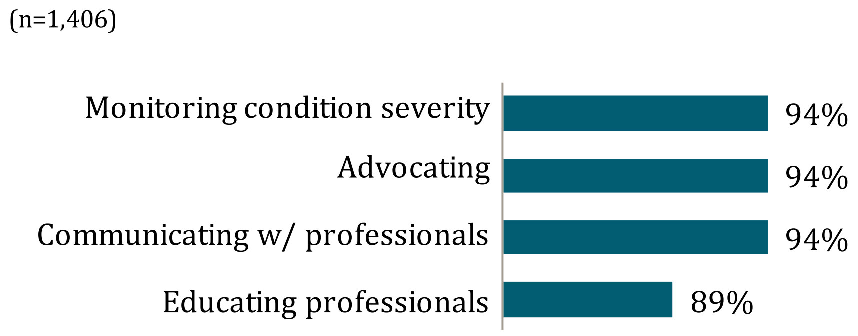
	Age of Care Recipient				
	Younger than 10 (n=502) A	10 - 17 (n=369) B	18 - 29 (n=204) C	30 - 49 (n=152) D	50 or older (n=179) E
Average years of providing care	4.4	10.9^{AE}	16.1^{ABDE}	11.9^{AE}	6.8^{A}
Average years of doing medical/nursing tasks	4.1	10.1^{AE}	16.5^{ABDE}	11.7^{AE}	6.0^{A}

^{ABCDE} indicates result is significantly higher than lettered comparison group

Caregiving Support Activities

In addition to helping with IADLs, ADLs, and medical/nursing tasks, rare caregivers often become a voice for their care recipient. Nearly all rare caregivers monitor the severity of their care recipient's condition (94%), advocate for them (94%), communicate with care providers (94%) and, find themselves educating health-care professionals (89%) about their care recipient's rare condition.

Figure 27: Caregiving Support Activities



Child CR (n=871)	Adult CR (n=535)
97%^	91%
98%^	89%
97%^	90%
92%^	83%

CR = Care Recipient
^ significantly higher than comparison group

Rare Caregivers Differences in Caregiving Support Activities

- Those rare caregivers who report their care recipient's condition has no treatment available are more often taking on the roles of condition monitoring (96% vs. 92% of those whose care recipient has a treatment), communication about care (96% vs. 92%), and educator (91% vs. 86%).
- Notably, rare caregivers whose care recipient has a genetically-linked condition more often report taking on the role of educator (91%), as compared to those caring for someone without a genetic link or cause (83%).
- Rare caregivers are more often taking on these four Caregiving Support Activities when their care recipient is younger.

Figure 28: Caregiving Support Activities by Age of Care Recipient

	Age of Care Recipient				
	Younger than 10 (n=502) A	10 - 17 (n=369) B	18 - 29 (n=204) C	30 - 49 (n=152) D	50 or older (n=179) E
Monitoring severity of condition to adjust care accordingly	96%^{DE}	98%^{DE}	95%^{D}	87%	90%
Advocating with health-care providers, services, schools, or agencies	97%^{DE}	98%^{CDE}	94%^{DE}	86%	84%
Communicating with health-care professionals about care	96%^{DE}	98%^{CDE}	94%^{D}	84%	91%
Educating health-care professionals about the rare condition	90%^{DE}	95%^{ADE}	91%^{DE}	80%	77%

^{ABCDE} indicates result is significantly higher than lettered comparison group

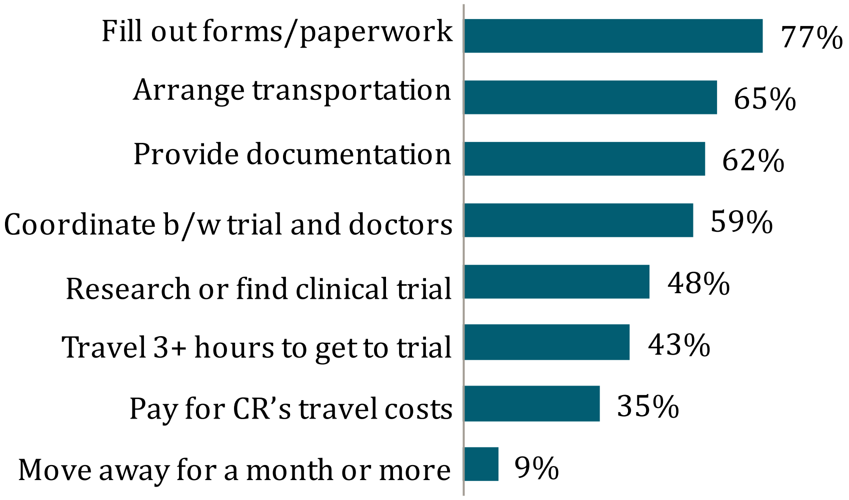
Clinical Trials

One in four rare caregivers say their care recipient has participated in a clinical trial (26%). Those caring for an adult are more likely to report their care recipient has ever been enrolled in a clinical trial (30% vs. 24% caring for a child).

Among those who do participate in a clinical trial, the rare caregiver takes on significant responsibility. Very few caregivers report *not* being involved in helping their care recipient with a clinical trial (8%). A majority help with trial paperwork (77%), provide or arrange transportation (65%), monitor condition to provide trial documentation (62%), or coordinate communication between the trial and the regular care team (59%).

Figure 29: Clinical Trial Support

Among those whose recipient has participated in a clinical trial (n=365)



	Child CR (n=205)	Adult CR (n=160)
Fill out forms/paperwork	85%^	67%
Arrange transportation	68%	61%
Provide documentation	68%^	56%
Coordinate b/w trial and doctors	66%^	51%
Research or find clinical trial	50%	44%
Travel 3+ hours to get to trial	46%	39%
Pay for CR's travel costs	34%	37%
Move away for a month or more	10%	8%

CR = Care Recipient

^ significantly higher than comparison group

Rare Caregivers: Longer Caregiver and Clinical Trial Support

- Rare caregivers who have been providing care for a greater number of years more often report their care recipient has participated in a clinical trial. Ultimately, this means that the longest-term rare caregivers – those who have cared for 10 years or longer – have the biggest role in clinical trials for their care recipient.

Figure 30: Clinical Trials by Length of Time Caregiving

	Length of Time Caregiving		
	Less than 5 years (n=517) A	5 – 9 years (n=372) B	10 or more years (n=502) C
Care recipient participated in any clinical trial(s)	**17%**	**27%^{A}**	**34%^{AB}**
Caregiver helped with… *(out of all caregivers in group)*			
Filling out forms or handling other paperwork	11%	21%^{A}	29%^{AB}
Providing or arranging transportation	9%	17%^{A}	24%^{AB}
Monitoring condition to provide documentation to clinical trial	9%	15%^{A}	24%^{AB}
Coordinating care or communication between trial and regular care team	8%	15%^{A}	24%^{AB}
Researching or finding the clinical trial	7%	12%^{A}	18%^{AB}
Traveling more than a 3-hour drive to get to trial	7%	11%^{A}	15%^{A}

^{ABC} indicates result is significantly higher than lettered comparison group

In the caregivers' own words, taking on the role of expert can be overwhelming:

> "For me, the most frustrating thing is having to educate doctors about her condition instead of them telling me what the problem might be. I am the one that researched [condition] and found a geneticist who confirmed my suspected diagnoses. I'm also the one who figured out that she has [other conditions] and found methods of managing her symptoms…We've never had a doctor who did any research to try to help her."

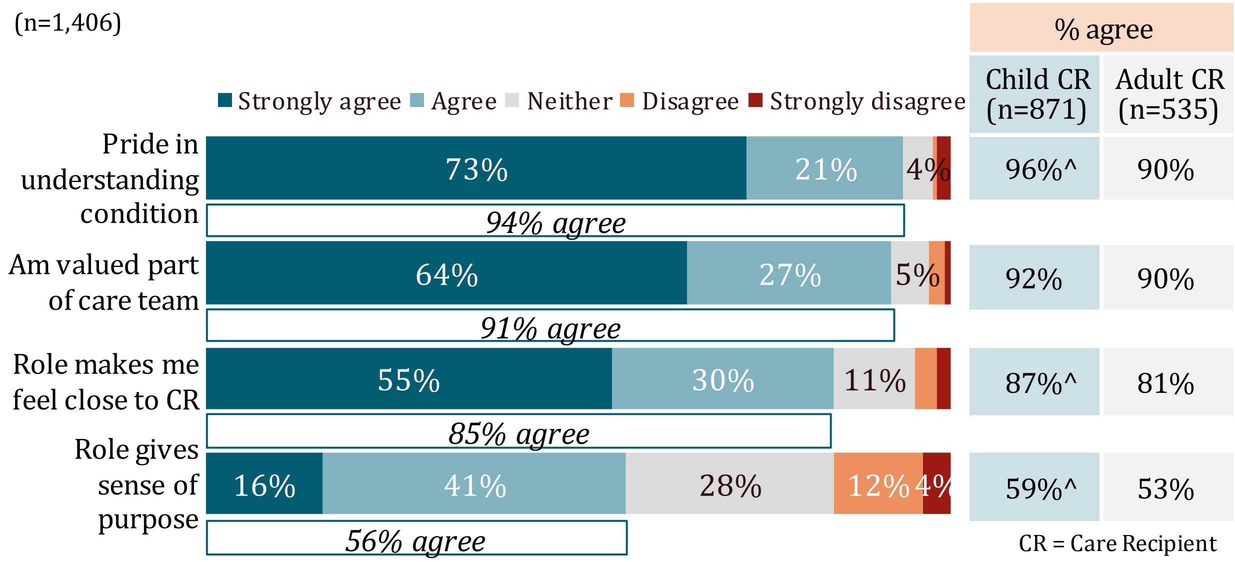
"There is little research being done in the U.S. for my daughter's condition, and few physicians know anything about it. I diagnosed her condition myself after she had been seen by multiple specialists over 15 years…I have to educate everyone we deal with, and I have to take the lead on all her care: doing the research, reading the studies, calling – even internationally – to speak with physicians and researchers who do work on her condition…Nonetheless, I am exhausted, sad, and discouraged."

"Advocacy: I have learned to fight back and share with others the lesson I have learned through our journey."

Positive Aspects of Caregiving

Rare caregivers also experience positive emotions related to their role in helping their care recipient. Nearly all rare caregivers take pride in improving their understanding about their care recipient's condition (94%) and nine out of ten feel like a valued part of the care team. Rare caregivers also report feeling close to their care recipient because of their role (85%) and feeling a sense of purpose (56%).

Figure 31: Positive Aspects of Caregiving



CR = Care Recipient

^ significantly higher than comparison group

Rare Caregivers of the Youngest Recipients

- Rare caregivers of the youngest recipients – those under age 10 – are especially likely to feel that providing care helps them feel close to their care recipient (90% vs. 82% of those caring for someone age 10 or older), as well as to feel rare caregiving provides a sense of purpose (64% vs. 52% of those caring for an older recipient).

Rare caregivers reflect the pride and importance of their multifaceted role as expert on their care recipient’s condition:

> “[I am motivated by] seeing her progress in areas professionals told me I could never hope to see progress in because of all the work I do with her.”

> “I have made his life as happy as I possibly can, and he is very well taken care of.”

> “I think my daughter has a real opportunity to have a nearly-normal adulthood if we continue to encourage skills and personal development. The idea that she could have some independence and find her own joys as an adult motivates me.”

> “My son gives me hope. I know that I am here raising him and caring for him myself. I have more knowledge now about many things I didn't have before. I have hope that he will grow up knowing all that I have sacrificed for him to have a longer life, to know that I am a great mother, and that I love him with all my heart.”

> “My son's positive attitude keeps me going…We feel blessed to have him yet strained due to uncontrolled circumstances.”

F. The Ripple Effect on Those Around the Rare Caregiver

Rare caregivers find themselves taking on much of their care recipient's care, with the majority acting as the primary caregiver, without paid help or relief from support services like respite and transportation services. That day-to-day load often means that immediate and extended family members in the "constellation of care" are also called upon to provide unpaid care, including other children living in the home.

Paid Help

Despite rare caregivers being in a higher-burden care situation than caregivers generally, they are no more likely to rely on paid help than caregivers generally. Just one in three rare caregivers (33%) receives help from paid aides. The use of paid help is more common among rare caregivers of a child (35%) than among rare caregivers of an adult (30%). These figures match general caregivers' experiences: 35% of general caregivers of a child[55] and 32% of general caregivers of an adult[56] used paid help.

Rare Caregivers using Paid Help

- The use of paid help among rare caregivers is more common among those with a household income of $50,000 or more (36%), compared to lower income households (26%). Similarly, those caring for someone with insurance of some kind (either private and/or government provided) more often report having paid help (38%) than those without any insurance (18%).
- Those caring for the youngest care recipients under age 10 are more likely to report having paid help (37%) than those caring for someone 30 or older (26%).
- Rare caregivers who care for a care recipient who lives in a suburban/urban area more often report having paid help (35%) than those caring for someone in a rural area (28%).
- Rare caregivers who report there is no treatment available for their care recipient's condition more often lean on paid help (40%) than those with a treatment available (24%).

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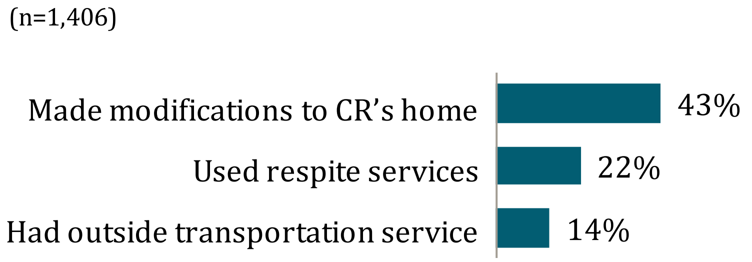
[55] "Caregiving in the U.S. 2009"
[56] "Caregiving in the U.S. 2015"

Support Service Use

Support service use is not common among rare caregivers. Most often, rare caregivers report having made modifications to their care recipient's home to make things easier for him/her (43%) – a task more often undertaken by rare caregivers of an adult than rare caregivers of a child. Only 22% report having used a respite service to get a break and 14% have used an outside transportation service. Use of transportation is more common among rare caregivers of an adult (19%) than a child (11%).

Figure 32: Support Service Use by Rare Caregivers



Child CR (n=871)	Adult CR (n=535)
39%	48%^
22%	22%
11%	19%^

CR = Care Recipient
^ significantly higher than comparison group

Presence of Other Unpaid Caregivers

Six out of ten rare caregivers report that at least one other person provides unpaid help to their care recipient (59%). Rare caregivers of a child more often say there is other help (66%) than rare caregivers of an adult (47%).

In addition, it seems that the "rare" part of rare caregiving *for a child* tends to pull in more support: rare caregivers of a child more often report help than general caregivers of a child (54%).[57] However, the "rare" part of rare caregiving for an adult seems to have the opposite impact: rare caregivers of an adult are less likely to say they have other unpaid helpers than general caregivers of an adult (53%).[58]

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[57] "Caregiving in the U.S. 2009"
[58] "Caregiving in the U.S. 2015"

Rare caregivers of an adult seem to be especially likely to provide care on their own: 44% report having neither paid help, nor other unpaid caregivers. Just one out of four rare caregivers of a child is similarly providing care on their own.

Figure 33: Paid and Unpaid Help

	All Rare Caregivers (n=1,406)	Child Care Recipients (n=871)	Adult Care Recipients (n=535)
No paid help but has unpaid	**35%**	40%^	27%
No paid or unpaid help	**32%**	25%	44%^
Has both paid and unpaid help	**24%**	26%^	21%
Has paid help but no unpaid	**9%**	9%	9%

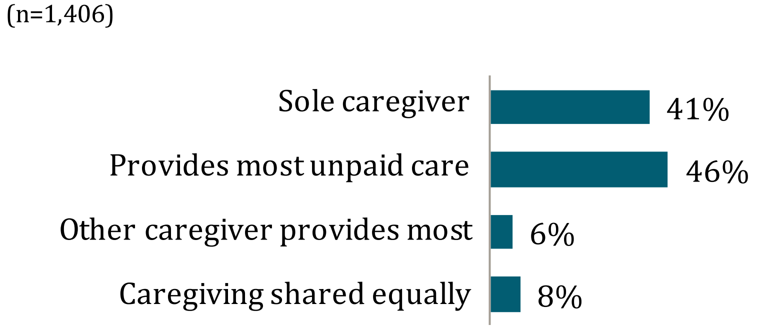
^ indicates significantly higher than comparison group

The lack of help does not seem to translate into increased feelings of isolation. Among rare caregivers without *any* help (paid or unpaid), 57% of those caring for a child report feeling alone, compared to just 44% of rare caregivers of adults. Further research is needed, but it may be that caregivers of adults are able to stave off feelings of isolation due to the interactions they have with their care recipient (an adult), while caregivers of children feel more isolated or alone.

Primary Caregiver

Despite having other help, the majority of rare caregivers are the primary caregiver (87%). The pattern of primary rare caregiving for a child and an adult are mirror imaged: Rare caregivers of a child tend to be the main caregiver among several (53%), while rare caregivers of an adult tend to be the sole caregiver (53%).

Figure 34: Primary Caregiver Status



	Child CR (n=871)	Adult CR (n=535)
Sole caregiver	34%	53%^
Provides most unpaid care	53%^	34%
Other caregiver provides most	5%	6%
Caregiving shared equally	8%	7%

CR = Care Recipient

^ significantly higher than comparison group

Rare Caregivers as Sole Caregiver

- The sole rare caregiver is especially common when caring for one's spouse or partner: 72% are the only unpaid caregiver.
- Those who report being in a racial/ethnic minority group[59] are more often sole caregivers (49%) than non-Hispanic Caucasians (40%).

Other Unpaid Caregivers

Among rare caregivers who report having other unpaid help, the most common helpers are immediate family of the care recipient: a parent (78%), a grandparent (40%), or sibling (32%). As might be expected, who these other unpaid helpers are varies by the age of the care recipient. The younger the care recipient is, the more often help comes from family who are a generation or two older; while the older the care recipient is, the more often help comes from those of a similar age or younger.

Figure 35: Who Unpaid Caregivers Are by Age of Care Recipient

	Age of Care Recipient				
Other unpaid caregivers are the care recipient's…	Younger than 10 (n=351) A	10 - 17 (n=226) B	18 - 29 (n=119) C	30 - 49 (n=69) D	50 or older (n=66) E
Older generation					
Parent	$87\%^{CDE}$	$89\%^{CDE}$	$66\%^{E}$	$72\%^{E}$	17%
Grandparent	$59\%^{BCDE}$	$46\%^{CDE}$	$18\%^{DE}$	4%	--
Aunt or Uncle	$20\%^{BCDE}$	$12\%^{E}$	$12\%^{E}$	$9\%^{E}$	--
Same or Younger generation					
Sibling	24%	$42\%^{ADE}$	$44\%^{ADE}$	25%	29%
Cousin	$5\%^{E}$	$4\%^{E}$	$4\%^{E}$	1%	--
Spouse or Partner	--	--	27%	22%	23%
Child	--	--	5%	$14\%^{C}$	$47\%^{CD}$
Niece, Nephew, Grandchild	--	<0.5%	--	3%	$15\%^{CD}$

^{ABCDE} indicates result is significantly higher than lettered comparison group

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[59] Includes Hispanic, African-American, Asian, Pacific Islander, Alaskan Native, Native American, and/or multi-racial/ethnic groups.

Youth Caregivers

One out of four rare caregivers report that at least one of these other care providers is a child under the age of 18 (24%). Rare caregivers caring for a child more often report the presence of a youth caregiver (27%) than rare caregivers of an adult (17%).

The presence of other unpaid help is more common the younger the care recipient's age. About six in ten rare caregivers of an older recipient (age 30 or older) are the sole caregiver, compared to just 30% of those caring for the youngest care recipients. Although it may be easier to garner additional rare caregivers for the youngest recipients, this more often means that youth are pulled into providing rare care.[60]

Figure 36: Caregiving Support Activities by Age of Care Recipient

	Age of Care Recipient				
	Younger than 10 (n=502) A	10 - 17 (n=369) B	18 - 29 (n=204) C	30 - 49 (n=152) D	50 or older (n=179) E
Presence of other unpaid help	70%^{BCDE}	61%^{DE}	58%^{DE}	45%	37%
Sole caregiver	30%	39%^{A}	42%^{A}	55%^{ABC}	63%^{ABC}
Children under age 18 who help provide care	23%^{E}	34%^{ACDE}	19%	14%	14%

^{ABCDE} indicates result is significantly higher than lettered comparison group

Youth Rare Caregivers

- Those who report being a racial/ethnic minority group[61] more often report their care recipient has a youth caregiver (34%) compared to non-Hispanic Caucasians (23%).

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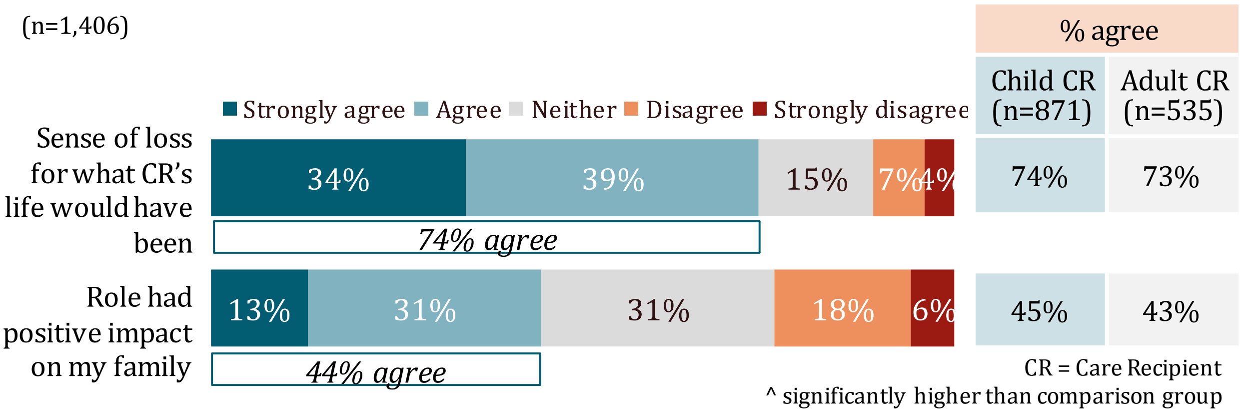
[60] Our data do not allow us to fully explore who these child caregivers are and their relationship to the care recipient, though the data do suggest that the vast majority of these children who are providing unpaid rare care are the sibling of the person with the rare disease or condition (85%). For additional research on who child caregivers are, see "Young Caregivers in the U.S.," National Alliance for Caregiving in collaboration with United Hospital Fund, 2005. http://www.caregiving.org/pdf/research/youngcaregivers.pdf

[61] Includes Hispanic, African-American, Asian, Pacific Islander, Alaskan Native, Native American, and/or multi-racial/ethnic groups.

Impact on the Family and Sense of Loss

Three out of four rare caregivers struggle with a sense of loss for what their care recipient's life would be like without their condition (74%). Only 44% feel their role as a rare caregiver has had a positive impact on their family.

Figure 37: Impact on Family Life



Rare Caregivers Impact on Family

- Relatively new rare caregivers – those who have been providing care for less than five years – more often struggle with a sense of loss about their care recipient's life (79%), as compared to those who have been in their role longer (71%).

Extended Absence from Home

One in five rare caregivers reports having to live away from home for more than three weeks to help their care recipient get needed care (19%), with these extended absences more common among rare caregivers of a child (21%) than rare caregivers of an adult (15%).

Rare Caregivers Moving Away from Home

- Women caregivers are more likely to report having to live away from home for a period of three weeks or more (19%) than men (13%).

G. The Impact on the Rare Caregiver

Rare caregivers take on a lot of responsibilities, performing many hours of care each week, often with others helping, but likely with few breaks. This constant daily role can impact the caregiver's employment, their attending school or continuing their education, as well as cause increased emotional stress, physical strain, and declining health.

Employment

Two-thirds (65%) of rare caregivers worked at some point during the past year while also providing care. Rare caregivers of an adult are more likely to report having worked (70%) than are rare caregivers of a child (63%).

Rare Caregivers Likely to Work

- Pointing to the difficulties of juggling work and caregiving, rare caregivers who provide 20 or fewer hours of care each week are more likely to be employed (75%) than those who provide more care (60%).
- Gen X rare caregivers are more likely to be working (age 37-52; 70%) than either Millennial rare caregivers (age 18-36; 64%) or Boomer or older rare caregivers (age 53 or older; 58%).
- Male rare caregivers more often report working while caregiving (75%) than their female counterparts (64%).

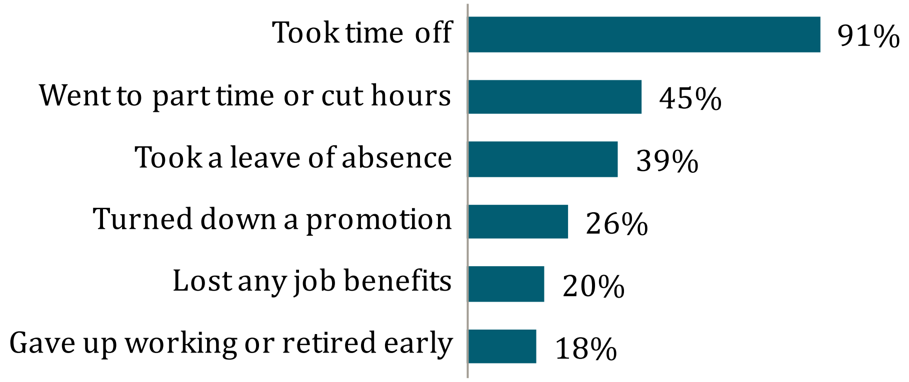
Impacts of Caregiving on Employment

Employed rare caregivers can find their caregiving role bleeding over to impact their work life, often in ways that are more impactful than general caregivers' experience.[62] Nearly all rare caregivers have gone in late or left work early to provide care (91%) – a significantly more common occurrence than general caregivers (74% among general caregivers of a child[63] and 49% among general caregivers of an adult[64]).

Significant numbers also have cut back on their hours (45%), taken a leave of absence (39%), or turned down a promotion (26%). Each of these work impacts are more often reported by rare caregivers of a child than by rare caregivers of an adult. Of note, half of employed rare caregivers of a child went from working full-time to part-time or otherwise cut back their work hours in order to provide care (52%).

Figure 38: Work Impacts as a Result of Rare Caregiving

Among those employed in the past year while providing care (n=919)



	Child CR (n=546)	Adult CR (n=373)
Took time off	93%	89%
Went to part time or cut hours	52%^	36%
Took a leave of absence	42%^	34%
Turned down a promotion	31%^	20%
Lost any job benefits	23%^	16%
Gave up working or retired early	21%^	14%

CR = Care Recipient
^ significantly higher than comparison group

The greater the number of hours a rare caregiver puts in, the greater the chance they have experienced each of these six work impacts – suggesting that the uniquely demanding experience of rare caregiving adversely impacts rare caregivers' work lives.

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[62] "Caregiving in the U.S. 2009" found that of employed general caregivers of a child: 30% cut back work hours; 32% took a leave of absence; 11% turned down a promotion; 15% lost job benefits; and 21% gave up work entirely (the only comparable result to rare caregivers' experience). "Caregiving in the U.S. 2015" found that of employed general caregivers of an adult: 14% cut back work hours; 15% took a leave of absence; 5% turned down a promotion; 3% lost job benefits; and 6% gave up work entirely.

[63] "Caregiving in the U.S. 2009".

[64] "Caregiving in the U.S. 2015".

The Relationship of Work and Income Among Rare Caregivers

- The rare caregivers' household income is related to their employment: those with higher incomes are more often employed. Just 55% of rare caregivers with a household income under $50,000 report working, compared to 67% of those with $50,000 to less than $100,000 and 77% of those with $100,000 or more.[65]
- Data suggest that those rare caregivers with household incomes above $100,000 are less likely to report leaving one's job entirely, either through a leave of absence (33%) or giving up work/early retirement (11%); as well as being less likely to cut back working hours (41%). The lowest income rare caregivers are more often doing each of these things.

Education

In addition to providing care, 10% of rare caregivers are also students. Comparable percentages of rare caregivers of a child are enrolled in school (11%), as is the case with rare caregivers of an adult (9%).

Of note, 8% of rare caregivers are both enrolled in school and working while providing care. These caregivers are 39 years old, on average – about 6 years younger than the total group of surveyed rare caregivers (44.9 years old). These rare caregivers otherwise show comparable levels on the Burden of Care Index, hours of care provided, and duration of care.[66]

Rare Caregivers More Often in School

- One out of every five Millennial rare caregivers (age 18-36) reports being in school while caregiving (19%), significantly more than Gen X (age 37-52; 9%) and Boomer or older (age 53 or older; 3%) rare caregivers.
- Rare caregivers with household incomes under $50,000 are more often in school (15%) than those with higher incomes (9%).
- Rare caregivers who are of racial/ethnic minority groups more often report being a student while caregiving (18%) as compared to non-Hispanic Caucasians (9%).

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[65] It is not entirely clear whether rare caregivers who are in lesser burden-care situations are better able to maintain employment which increases the caregiver's household income or rather, that rare caregivers with higher household incomes are more able to maintain employment, due to more flexible workplace accommodations or the ability to pay for paid help, which lessen the burden of the care situation. Additional research is needed.

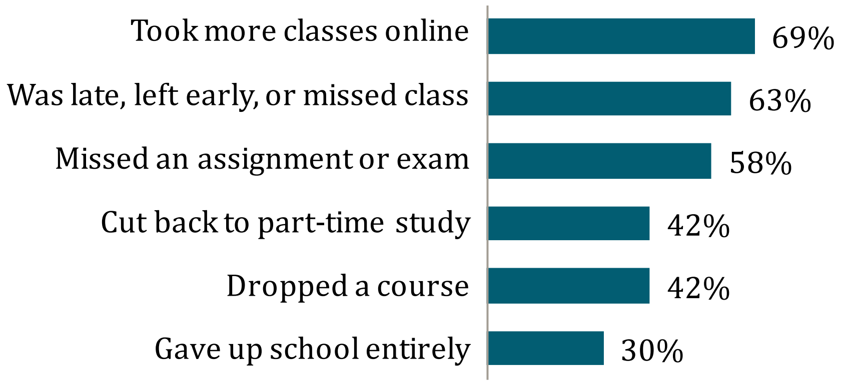
[66] Rare caregivers who are both in school and employed while providing care have been providing care for 9.3 years, 70% have a high burden on the Burden of Care Index, and 57% care for 40 or more hours each week.

Impact of Caregiving on Student Life

As with work, rare caregivers often struggle to fulfill their school responsibilities. Among student rare caregivers, roughly two out of three have taken more online classes (69%) or missed class to provide care (63%). More than half say they have missed an exam or assignment (58%).

Figure 39: School Impacts as a Result of Rare Caregiving

Among those who were students in the past year while providing care (n=143)



Child CR (n=94)	Adult CR (n=49)
72%	63%
67%	55%
63%	49%
48%^	31%
46%	35%
33%	24%

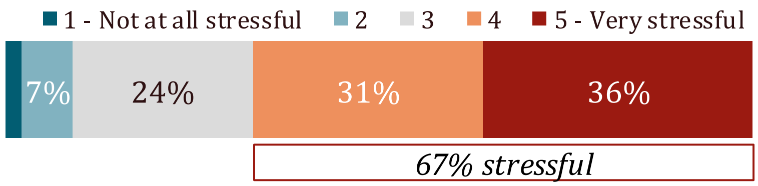
CR = Care Recipient
^ significantly higher than comparison group

Emotional Stress and Health

Rare caregivers say providing care to their care recipient is emotionally stressful (67%), with a majority of both adult and child caregivers feeling highly stressed. Rare caregivers' emotional stress is about twice as high as that of general caregivers: just 34% of general caregivers of a child[67] and 38% of general caregivers of an adult[68] report high emotional stress.

Figure 40: Emotional Stress

(n=1,406)



% stressful	
Child CR (n=871)	Adult CR (n=535)
69%^	64%

CR = Care Recipient
^ significantly higher than comparison group

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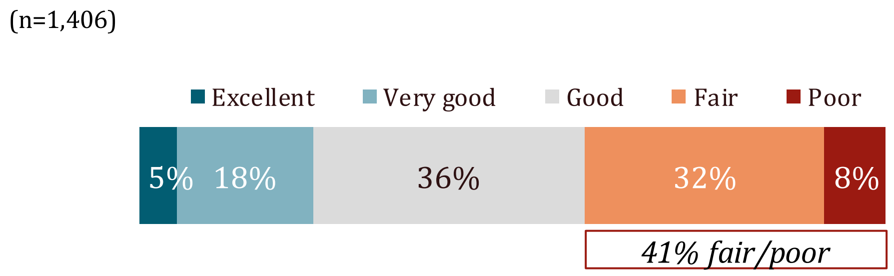
[67] "Caregiving in the U.S. 2009"
[68] "Caregiving in the U.S. 2015"

This emotional stress can impact the emotional and mental health of the rare caregiver: 41% rate their emotional health as fair or poor. Those caring for a child more often report fair or poor mental health (43% vs. 36% caring for adults).

Figure 41: Mental/Emotional Health of Caregiver



% fair/poor	
Child CR (n=871)	Adult CR (n=535)
43%^	36%

CR = Care Recipient
^ significantly higher than comparison group

Rare Caregivers with Emotional Health Concerns

- Seven out of ten rare caregivers who are caring for their own child under age 18 report high emotional stress (71%), compared to those caring for a spouse/partner (58%) or another adult or child recipient (57%). Nearly half (45%) report fair/poor mental health, more than those caring for their own adult child (31%) or some other recipient (35%).
- Women caregivers are more likely to say their emotional stress level is high (69% vs. 51% men), and to feel their mental health is fair or poor (43% vs. 25% of men).
- Rare caregivers who report their care recipient has *no* treatment options available are also especially likely to experience high stress (71% vs. 63% of rare caregivers who report their care recipient's condition has a treatment).
- Nearly half of rare caregivers who have only been doing medical/nursing tasks for less than five years say their emotional or mental health is fair or poor (47%): more than those not doing medical/nursing tasks (35%) or those who have been performing these longer (38%).

Rare caregivers voice the stress and strain they face:

> "The thought of having to care for my son for the rest of his life is overwhelming and sad…The level of stress is unimaginable, as well as my level of anger. I'm angry there is not more help, and I am angry that this has happened to him, and caring for him is hard. The sense of isolation, even among family and friends, is indescribable. I love him so much, but I am so tired."

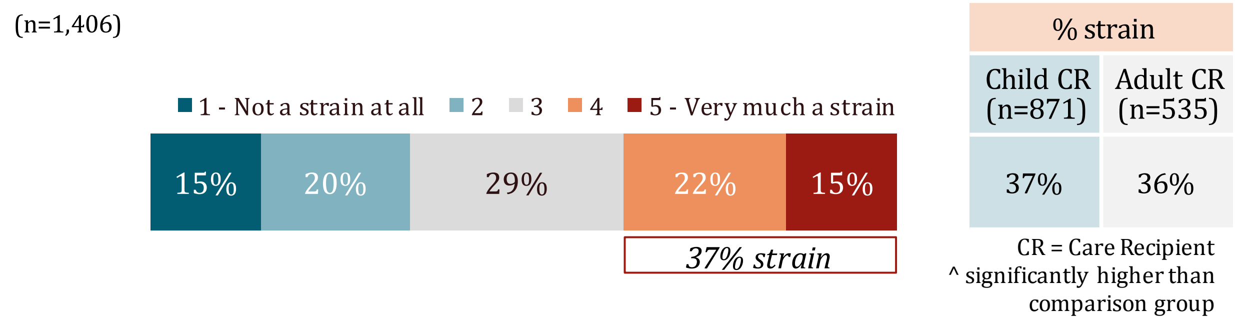
> "This is truly the hardest job I have ever had. It is so hard to watch someone you love go down so quickly, and all you can do is try to make them comfortable."

> "[I am] hoping to shed light on the strain of caregivers who provide care to those living with a rare condition and [our] feelings of isolation/helplessness."

Physical Strain

To a lesser degree, rare caregiving can be a physical strain to caregivers: nearly two in five report high levels of physical strain (37%). As with emotional stress, rare caregivers more often report feeling physical strain than general caregivers.[69]

Figure 42: Physical Strain



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[69] "Caregiving in the U.S. 2009" found that 17% of general caregivers of a child report high levels of physical strain, while "Caregiving in the U.S. 2015" found that 19% of general caregivers of an adult report high physical strain.

Rare Caregivers with Higher Levels of Physical Strain

- Rare caregivers who report high levels of physical strain are more often doing medical/nursing tasks and doing more ADLs than those who report low-to-mid physical strain. They also report higher difficulty in doing ADLs. This physical wear and tear does not seem to be age-related, as those feeling high levels of physical strain are the same age, on average, as those feeling lower strain.

Figure 43: Task Performance and Caregiver Age by Physical Strain

	Low-to-mid physical strain (n=890)	High physical strain (n=516)
Medical/nursing tasks	79%	92%^
Average number of ADLs performed	1.4	3.2^
Bathing/showering	36%	72%^
Dressing	37%	71%^
Feeding	26%	60%^
Getting in and out of beds and chairs	26%	55%^
Incontinence	21%	55%^
Getting to and from toilet	20%	46%^
Difficult to do ADLs	8%	39%^
Caregiver age (average, in years)	*44.8*	*45.1*

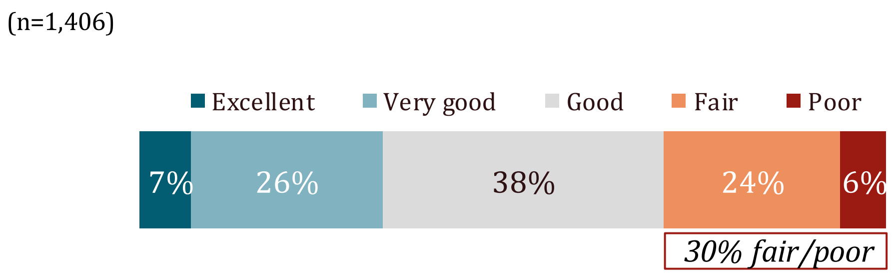
^ indicates significantly higher than comparison group

- Rare caregivers who report their care recipient's disease or condition has *no* treatment available are especially likely to report feeling physical strain (41% vs. 32% with treatment available).

Impact on Health

Rare caregiving takes a clear toll on the physical health of caregivers: one out of three (30%) rate their physical health as only fair or poor. Although rare caregivers of a child report their physical health (30% fair or poor) is about on par with general child caregivers (26% fair or poor[70]), rare caregivers of an adult seem to fare worse than caregivers generally (17%[71]). On the whole, however, rare caregivers' health is worse than that of adults in the United States overall, where just 10% report fair or poor health.[72]

Figure 44: Health Status of Caregiver



% fair/poor	
Child CR (n=871)	Adult CR (n=535)
30%	30%

CR = Care Recipient
^ significantly higher than comparison group

Rare Caregivers with Fair/Poor Health

- Long-term rare caregivers – those who have been providing care for 10 years or longer – are more likely to be in fair or poor health (36%) than shorter-term caregivers (26%).
- Rare caregivers with lower incomes are more often in fair or poor health: 20% of those with a household income of $100,000 or more report fair or poor health, which increases to 42% when household income drops under $50,000 per year.
- More than four in ten rare caregivers who are racial/ethnic minorities report fair or poor physical health (43%), significantly more than non-Hispanic whites (28%).

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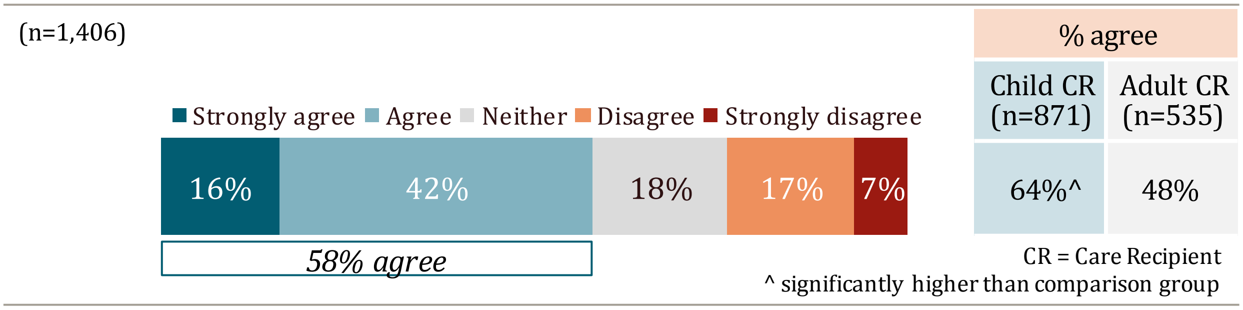
[70] "Caregiving in the U.S. 2009"
[71] "Caregiving in the U.S. 2015"
[72] CDC/NCHS, National Health Interview Survey, January–June 2017.

About six in ten rare caregivers find it difficult to take care of their own health (58%). Those caring for a child with a rare condition or disease find this more difficult (64%) than those caring for an adult (48%).

Figure 45: Caregiver Difficulty Taking Care of Own Health



CR = Care Recipient
^ significantly higher than comparison group

Rare Caregivers with Difficulty Taking Care of Health

- Rare caregivers who are performing medical/nursing tasks for their care recipient report it is difficult to take care of their own health (61% vs. 40% of rare caregivers not doing medical/nursing tasks).
- Rare caregivers who have children under the age of 18 living in their household also are especially likely to have difficulty with their own health (62% vs. 47% of rare caregivers without a child in the home).
- As with emotional stress, female rare caregivers more often claim they have difficulty taking care of their own health (61% vs. 35% of men).

H. Finances and Long-Range Planning

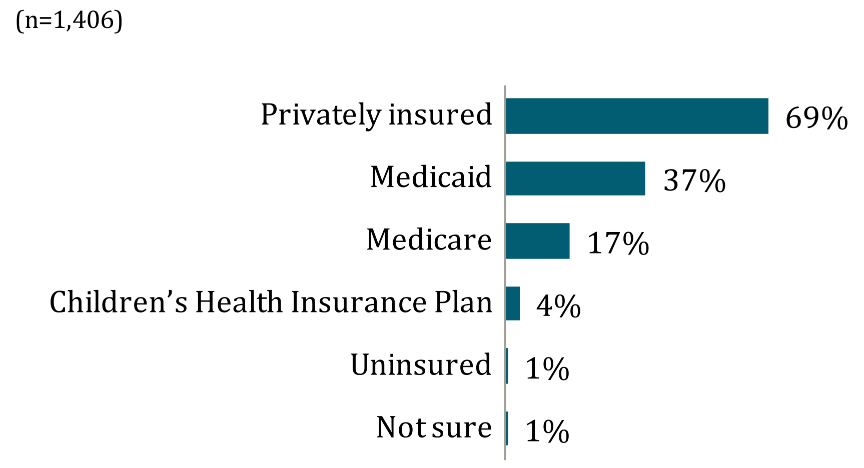
The ripple effect of rare caregiving can extend past day-to-day tasks of providing care, the need for others in the family to step up and pitch in, and the emotional and physical health of the caregiver. Rare caregiving can ultimately impact the finances of the rare caregiver and their family unit; and these financial impacts loom large. Rare caregivers face financial strain, many have experienced negative impacts on their finances, and paying for care is a constant worry. Few have long-range plans in place, either for the recipient's care, their own potential transition out of care, or end-of-life.

Financial Coordination and Recipient Insurance

Rare caregivers often take on the role of financial coordinator for their care recipient: 81% report that they manage finances, including paying bills or filling out insurance claims. This task is more commonly done by rare caregivers than by caregivers generally (54%).[73]

Rare caregivers report their care recipient uses various health insurance coverage types, including private insurance[74] (69%), Medicaid (37%), and Medicare (17%).

Figure 46: Care Recipient Health Insurance Coverage



	Child CR (n=871)	Adult CR (n=535)
Privately insured	75%^	60%
Medicaid	40%^	32%
Medicare	5%	38%^
Children's Health Insurance Plan	6%^	2%
Uninsured	<0.5%	2%
Not sure	1%	1%

CR = Care Recipient
^ significantly higher than comparison group

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[73] "Caregiving in the U.S. 2015", among general caregivers of adults. No comparison data exist for caregivers of a child in "Caregiving in the U.S. 2009".

[74] Private insurance is inclusive of coverage obtained through an employer, union, or other group plan, or plans purchased privately by an individual direct from an insurance carrier, broker, third party, or on an exchange.

- Rare caregivers are especially likely to be involved in their care recipient’s paperwork and finances if he or she is in early adulthood (age 18-29, 91% involved), more so than if the care recipient is younger or older. As might be expected, insurance patterns vary by the care recipient’s age, with a child more often covered through private insurance, Medicaid, or the Children’s Health Insurance Plan (CHIP), while adults are more often covered by Medicare.

Figure 47: Caregiver Help with Finances and Insurance Status by Age of Care Recipient

	Age of Care Recipient				
	Younger than 10 (n=502) A	10 - 17 (n=369) B	18 - 29 (n=204) C	30 - 49 (n=152) D	50 or older (n=179) E
Caregiver manages finances	78%	82%	91%^{ABDE}	78%	78%
Insurance status of recipient…					
Privately insured	74%^{CDE}	76%^{CDE}	66%^{E}	58%	56%
Medicaid	43%^{E}	37%^{E}	51%^{ABDE}	36%^{E}	8%
Medicare	5%	5%	18%^{AB}	41%^{ABC}	59%^{ABCD}

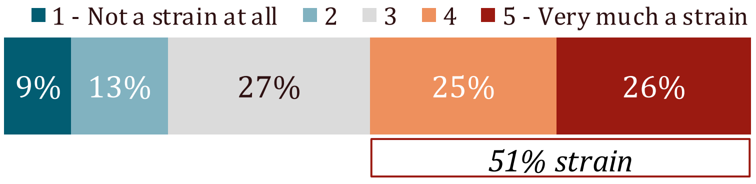
^{ABCDE} indicates result is significantly higher than lettered comparison group

Financial Strain and Worry

Half of rare caregivers report high levels of financial strain (51%), with those caring for a child more often reporting financial strain.

Figure 48: Financial Strain

(n=1,406)



% strain	
Child CR (n=871)	Adult CR (n=535)
54%^	47%

CR = Care Recipient
^ significantly higher than comparison group

Rare Caregivers with Financial Strain

- Rare caregivers with lower household incomes more often report facing financial strain as a result of their caregiving role: 56% of caregivers with income below $100,000 report financial strain, while just 42% of those with higher incomes report facing strain.

- Rare caregivers who are the primary (56%) or sole (49%) caregiver are also likely to report facing financial strain, more so than those who are not the primary rare caregiver (38%).

Three out of every four rare caregivers worry about the ability of their own family (or their care recipient's family, if not a close relative) to pay for care (74%). Again, those with incomes under $100,000 are especially likely to worry (76%) compared to higher income rare caregivers (68%).

Financial Help for Care Recipient

More than half of rare caregivers report they have requested information about how to get financial help for their care recipient (55%). Those caring for a child more often have requested information on financial help (58%) than those caring for an adult (51%). However, rare caregivers are significantly more likely than general caregivers to have sought out this kind of information.[75]

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[75] "Caregiving in the U.S. 2009" found that only 32% of child caregivers generally sought out information to get their care recipient financial help, while "Caregiving in the U.S. 2015" found just 28% of caregivers of adults had done so.

Caregiver Financial Strain, Worry, and Requests for Help

- Rare caregivers’ feelings of financial strain, worry, and outreach vary by the care recipient’s age.
  - Feelings of strain are highest as their care recipient reaches later childhood and early adulthood.
  - Concern about the ability to pay for care seems to peak when the care recipient is in early adulthood (age 18 to 29).
  - Requests for information about financial help are most common when the care recipient is very young (under age 10) or reaching young adulthood. Further research is needed to explore the interactions of length of time providing care, care recipient age, and financial strain on the caregiver.

Figure 49: Financial Strain and Worry by Age of Care Recipient

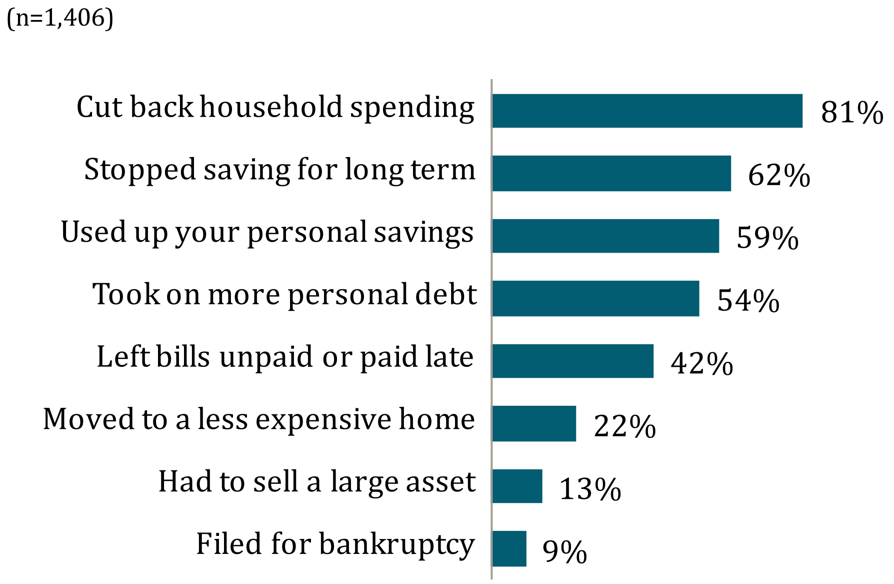
	Age of Care Recipient				
	Younger than 10 (n=502) A	10 - 17 (n=369) B	18 - 29 (n=204) C	30 - 49 (n=152) D	50 or older (n=179) E
Financial strain	$50\%^{E}$	$59\%^{ADE}$	$54\%^{DE}$	43%	41%
Worry about care recipient’s/care recipient’s family ability to pay for care	$72\%^{E}$	$76\%^{E}$	$87\%^{ABDE}$	71%	61%
Requested information about financial help for care recipient	$61\%^{BDE}$	$54\%^{E}$	$63\%^{BDE}$	49%	39%

^{ABCDE} indicates result is significantly higher than lettered comparison group

Financial Impacts on Caregiver

Rare caregiving impacts the financial situation of nearly all rare caregivers: more than four in five (86%) have experienced financial impacts because of their caregiving role. Common impacts include cutting back on household spending (81%), not saving for long-term goals (62%), using up personal savings (59%) and, taking on more personal debt (54%). Some have even taken drastic steps such as selling a large asset (13%) or filing for bankruptcy (9%). Rare caregivers of a child more often report having experienced five out of eight of these impacts, as compared to those caring for an adult.

Figure 50: Financial Impacts on Caregiver



	Child CR (n=871)	Adult CR (n=535)
Cut back household spending	84%^	75%
Stopped saving for long term	66%^	56%
Used up your personal savings	64%^	52%
Took on more personal debt	57%^	48%
Left bills unpaid or paid late	45%^	36%
Moved to a less expensive home	22%	21%
Had to sell a large asset	13%	14%
Filed for bankruptcy	9%	10%

CR = Care Recipient
^ significantly higher than comparison group

Rare Caregiver Financial Impacts

- Rare caregivers with household incomes under $100,000 more often experience each of these eight impacts, as compared to their higher income counterparts.
- Racial/ethnic minority caregivers more often report experiencing several financial impacts as a result of caregiving compared to non-Hispanic Caucasians such as: stopping saving for long-term goals (74% vs. 61% non-Hispanic whites), using up personal savings (73% vs. 58%), taking on more personal debt (63% vs. 53%), leaving household bills unpaid or paying late (58% vs. 40%), moving to a less expensive home (29% vs. 21%), or filing for bankruptcy (15% vs. 8%).

- Data indicate that the longer a rare caregiver provides care, the more likely they are to experience negative financial impacts,[76] especially major impacts of selling a large asset or filing for bankruptcy.

Figure 51: Financial Impacts by Length of Time Caregiving

	Length of Time Caregiving		
	Less than 5 years (n=517) A	5 – 9 years (n=372) B	10 or more years (n=502) C
Cut back household spending	81%	83%	79%
Stopped saving for long-term goals	59%	65%^{A}	64%
Used up personal savings	55%	63%^{A}	60%
Took on more personal debt	47%	56%^{A}	58%^{A}
Left household bills unpaid or paid late	38%	42%	46%^{A}
Moved to a less expensive home or apartment	16%	24%^{A}	26%^{A}
Had to sell a large asset	9%	12%	18%^{AB}
Filed for bankruptcy	5%	8%	14%^{AB}

^{ABC} indicates result is significantly higher than lettered comparison group

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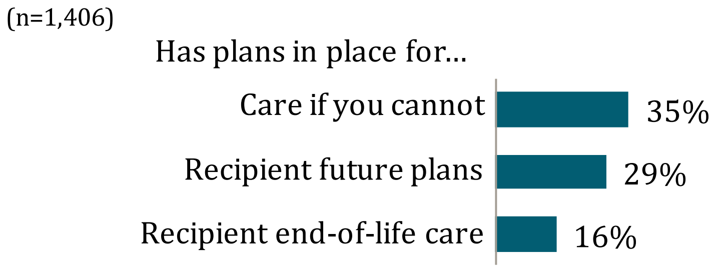
[76] There is no significant difference in income levels by length of time providing care.

Future Planning

It is possible that the combination of strain and financial impacts cause many rare caregivers to be unable to plan for the long term. Just one in three rare caregivers have a contingency plan in case they become unable to provide care (35%). Only 29% have plans in place for their care recipient's future care, including handling things like financial matters, health-care decisions, and living arrangements. Few report there are plans in place for their care recipient's end-of-life care. Rare caregivers of an adult are more likely to report that there are long-range, as well as end-of-life, plans in place.

Figure 52: Future Planning



Child CR (n=871)	Adult CR (n=535)
36%	34%
21%	43%^
7%	31%^

CR = Care Recipient
^ significantly higher than comparison group

Rare Caregiver Long Term Planning

- Alarmingly, just 29% of sole rare caregivers report there is a contingency plan in place in case of their inability to continue providing care.
- Long-term rare caregivers – those who have been providing care for 10 years or longer – more often report having plans in place in case of their own inability to provide care (43% vs. 30% of those caring for under 10 years) and for their care recipient's long-term arrangements (37% vs. 25%).

- Long-term planning is more common among those caring for older recipients.

Figure 53: Long-Term Plans by Age of Care Recipient

	Age of Care Recipient				
Plans are in place for…	Younger than 10 (n=502) A	10 - 17 (n=369) B	18 - 29 (n=204) C	30 - 49 (n=152) D	50 or older (n=179) E
Who will provide care if caregiver was unable to	34%	38%^{DE}	47%^{ABDE}	26%	26%
Recipient's future care (financial, health, and living)	20%	23%	40%^{AB}	35%^{AB}	53%^{ABCD}
Recipient's end-of-life care	7%	8%	21%^{AB}	25%^{AB}	47%^{ABCD}

^{ABCDE} indicates result is significantly higher than lettered comparison group

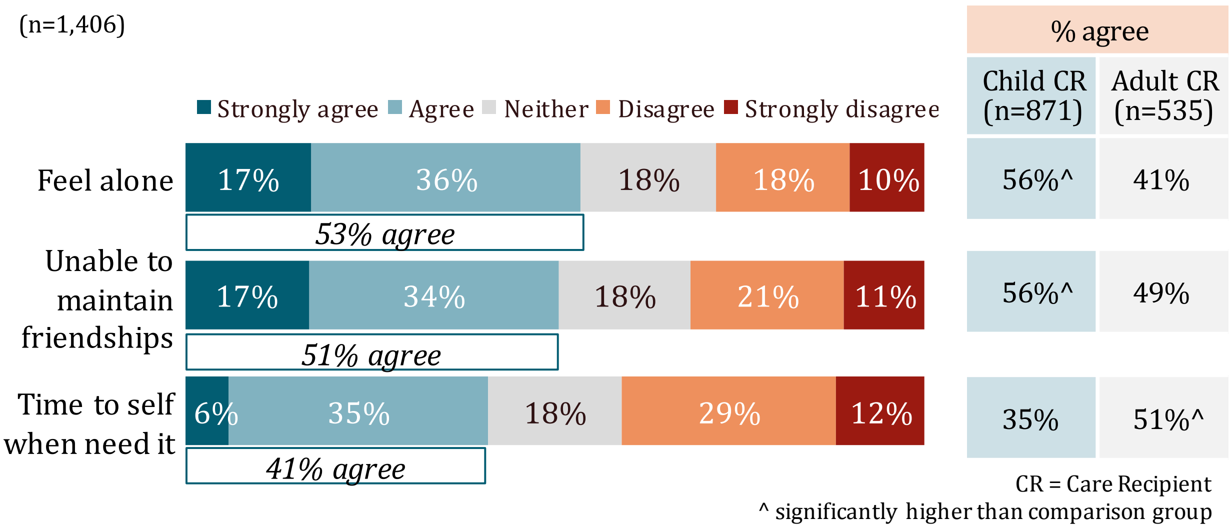
I. Importance of Community

Not only does rare caregiving impact the family unit and the caregiver's daily life, health, and finances, but their caregiving role can also impact their social and family lives. Many rare caregivers have difficulty maintaining friendships and face feelings of isolation. Yet, some find comfort in creating a sense of community with other rare caregivers, finding support online, and through reaching out to share information.

Social Support and Friendships

Half of rare caregivers have been unable to maintain friendships since taking on their caregiver role (51%), while 53% report feeling alone. Just four in ten indicate that they have been able to get some time to themselves when needed (41%). The loss of friendships and feeling isolated are more common among rare caregivers of a child, while getting time for oneself is more common among rare caregivers of an adult (see Figure 54, next page).

Figure 54: Social Support



CR = Care Recipient
^ significantly higher than comparison group

Rare Caregivers Lacking Social Support

- Rare caregivers who are Millennials or Gen X (age 18-52) are especially likely to report loss of friendship (54% vs. 40% of Boomers or older generations age 53 or older) or feeling alone (55% vs. 48%).
- Rare caregivers of the youngest care recipients – those under age 10 – are the least likely to say they are able to have time to themselves (32%), less than those caring for a child age 10-17 (40%) or an adult of any age (51%).
- Rare caregivers whose care recipient lives in a rural area are also less likely to say they can find time for themselves (37%) as compared to those whose care recipient lives in a suburban or urban area (43%).

Online Support

Perhaps because of the time-intensive demands of caregiving and to battle feelings of isolation, many rare caregivers report using an online support group (60%). Use of online support groups is more common among rare caregivers of a child (71%) than rare caregivers of an adult (41%).[77] However, it is important to note that rare caregivers were recruited to this study through online outreach via a variety of caregiver and disease groups, which may be overestimating the reliance of rare caregivers on online support.

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[77] Due to the way this study population was recruited, such as through targeted Facebook advertisements, results related to online use and online social support must be interpreted with caution, as the sample of respondents likely over-estimates reliance on online support.

Rare caregivers do talk about the importance of creating these social connections to not only share information, but to gain support and a sense of hope:

> “Talking to other caregivers and patients with the same disease gives me hope that she will learn how to manage her disease and treatment.”

> “The biggest thing that gives me hope is the rare condition community – meeting others who also deal with rare conditions themselves or of those they love. These are truly the strongest people that I know.”

> “[I feel hopeful] when I see my son connect with other kids that have [condition] and are leading full lives. When he meets young adults who go on to have successful careers, despite the disease. I wish we had more opportunities for him to meet and stay connected with people such as this.”

> “Seeing others in similar situations – making the best out of life, growing and gaining physical and mental strength – gives me hope for my son's [quality of life].”

> “The only good thing about a rare disease is the people you meet with the disease. It's a double-edged sword, though, because in some corner of your mind, you know they won't win either…will [a cure] come in time for my dear friends who have the disease now?”

Spotlight: Caregivers of those in a Rural Area

Spotlight: One out of three rare caregivers surveyed say their recipient lives in a rural area (34%). Figure 55 shines a spotlight on these caregivers by highlighting what is unique about them.[78]

Figure 55: Spotlight on Rare Caregivers of Someone in a Rural Area

Who?

- 44 year old caring for own child (73%) or spouse (17%) covered by Medicaid (45%)
- 68% have household income under $75,000

Care situation

- Provide 50.6 hours of care weekly
- 90% help with transportation; 87% do medical/nursing tasks
- Just 28% use paid help

Impact?

- High burden of care (70%)
- Hard to get needed services: doubt in local hospital
- Greater financial impacts: cutting back spending, no savings, bills unpaid

Rare caregivers of someone living in a rural area are lower income, with 68% having household incomes under $75,000. Half report high levels of financial strain and they more often report financial impacts due to caregiving, such as cutting back on household spending, stopping long-term saving, depleting personal savings, and leaving household bills unpaid. Many report transportation impacts: they more often help their care recipient with transportation (90%) and among those whose recipient has done a clinical trial, 53% of caregivers have traveled more than three hours to help them get there. Care and support service access is more difficult: just 28% use paid help and 39% report difficulty accessing symptom management therapies. With nearly all needed services, rare caregivers of someone in a rural setting report greater difficulty finding that service (e.g., medical professional, clinical trials, genetic counselor, or educational support). Only 27% feel their recipient's local hospital can handle their condition.

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[78] Spotlights on rare caregivers provide a snapshot glance at significant findings that make this subgroup unique relative to comparison groups—in this case, the comparison group is rare caregivers caring for someone who lives in a suburban/urban setting.

J. Rare Caregiver Case Studies

Below we present three case studies of rare caregivers to illustrate that while rare caregiving is on the whole, a life-changing situation that is lived day-by-day, there are variations in caregiving experiences, depending on the type of rare disease the care recipient has.

These three examples: Cystic Fibrosis, Fabry disease, and Pulmonary arterial hypertension (PAH) are meant to illustrate the variety of caregiving experiences, and not intended to minimize the experiences of rare caregivers caring for someone with one of the other 400 conditions represented in this study.

Case Study 1: Cystic Fibrosis

Who, caring for whom: Among those rare caregivers who replied to *Rare Caregiving in America* survey, the average age of a cystic fibrosis (CF) caregiver is 41 years old, 91% are women, and nearly all are white, non-Hispanic (95%). In many cases, the caregiver's responsibility did not come by choice, as nearly nine in ten care for their own child (87%). On average, the CF caregiver has provided care for nearly 9 years.

Employment, finances, and planning: Two-thirds of cystic fibrosis caregivers were employed during their past year of providing care (66%), and the median household income of such caregivers is $87,500. Caregiving almost always necessitates time off from work (93%), but more drastic impacts on employment are less common. Less than half report they have had to cut back on hours (40%), while only one-quarter took a leave of absence (28%) or turned down a promotion (24%). About one in eight reports retiring early or quitting the workforce (13%). Despite having relatively high household incomes, three-quarters are concerned about their ability to pay for the care (73%). Half have plans in place if they are no longer able to fulfill the role of caregiver (53%), and only a handful have developed an end-of-life plan (7%).

Care overview: Despite also being employed, the typical cystic fibrosis caregiver spends 40 hours a week providing care, performing an average of three IADL tasks. More than nine in ten conduct medical/nursing tasks (93%). Three-fifths of these caregivers receive unpaid assistance (61%), but only 16% receive paid assistance. Almost two-thirds of caregivers report that their local hospital is generally capable of handling cystic fibrosis (63%), and nine in ten consult with health-care professionals to enhance care (90%).

About the care recipient: Nearly all care recipients' cystic fibrosis results from a genetic issue (98%), but only one-fifth of those have a relative with the same condition (19%). Two-thirds report there being treatment available for cystic fibrosis (68%), but treatment or therapy to manage symptoms is reported to be accessible by nearly all caregivers (98%). Cystic fibrosis caregivers generally have little difficulty finding the support they need, although at least one in five report having trouble finding alternative treatments (36%), clinical trials (29%), pharmacies that carry the proper medications (28%), or mental health professionals (25%).

Impacts on the caregiver: Four in five cystic fibrosis caregivers report their own physical health to be at least good (82%), including half who say their health is very good or excellent (51%). Nearly all agree that they take pride in improving their understanding of cystic fibrosis (96%). However, providing care has taken an emotional toll, as only three in ten report very good or excellent emotional/mental health (31%). Although three in five report they struggle with a sense of loss over “what might have been” for their care recipients (60%), more than half agree that their caregiver status gives them a sense of purpose (62%), and has had a positive impact on their family (50%). Half also agree that they are able to find time for themselves when they can (53%). On average, the cystic fibrosis caregiver’s overall Burden of Care Index is 3 out of a possible 5.

Case Study 2: Fabry disease

Who, caring for whom: Among those rare caregivers who replied to *Rare Caregiving in America* survey, Fabry caregivers are 48 years old, on average, and seven in ten are women (69%). All Fabry caregivers surveyed care for a family member (100%). Two-fifths care for a spouse or partner (42%), while three in ten care for a child (31%) and one in five care for a parent (19%). The average Fabry care recipient is 44 years old.

Employment, finances, and planning: More than three-quarters of Fabry caregivers are employed (77%), but their status as caregivers impacts their professional life. Seven in ten report taking time off to give care (70%), and half say they have had to take a leave of absence (50%). These limitations likely contribute to a lower household income of just $40,000 on average, well below that of general rare caregivers ($62,500). Unsurprisingly, more than eight in ten worry about their ability to pay for needed care (85%). Half of Fabry caregivers have plans in place for future care if they are no longer able to provide care themselves (50%), but only one-quarter have plans for end-of-life care (27%).

Care overview: Fabry care is demanding and complex. On average, Fabry caregivers have provided care for an average of 12 years. Each week, Fabry caregivers spend an average of 28 hours providing care. They also assist in completion of an average of 5 IADL and 1 ADL tasks. Arranging for outside support is the only IADL task that less than half of Fabry caregivers assist in (46%). Only about one-third have received unpaid caregiving assistance in the past year (35%), and even fewer have received paid help (19%). Only two-fifths agree that their local hospital can handle Fabry (42%). To get more help and information about the condition, nine in ten caregivers consult with doctors or other health-care professionals (88%), and nearly all talk to genetic counselors about the condition (96%). However, two in five say medical professionals with treatment experience are difficult to find in their community (38%).

About the care recipient: Among all Fabry care recipients, the disease results from a genetic issue or component (100%), and in nine of ten cases, care recipients know of a family member also afflicted by Fabry (88%). Four of five say treatment is available for Fabry (81%), and a similar share report that therapy and treatments to address Fabry symptoms are available as well (77%). Less than a third report a need for clinical trials to receive treatment (31%), but closer to half see a need for mental health services (42%).

Impacts on the caregiver: All Fabry caregivers agree that they take immense pride in learning about and understanding the condition (100%). Only one in five consider their own physical health to be excellent or very good (19%), and a remarkable three out of five say their health is fair or poor (62%). However, three-quarters of Fabry caregivers are more likely to report that their mental/emotional health is at least good (73%). The overall Burden of Care Index for Fabry caregivers is about average, at 2.9 out of 5.

Case Study 3: Pulmonary arterial hypertension (PAH)

Who, caring for whom: Among those rare caregivers who replied to *Rare Caregiving in America* survey, Pulmonary arterial hypertension (PAH) caregivers are 54 years old on average, and two-thirds are women (67%). Nearly all care for relatives (94%), including half who care for a spouse or partner (51%). One-third care for a PAH-afflicted child (33%). Care recipients are an average of 46 years old, and seven in ten are women (71%).

Employment, finances, and planning: Just under two-thirds of PAH caregivers are employed (63%). Nine in ten report having altered their work schedules to provide care (91%), two in five took a leave of absence (38%), and a third cut back their hours (34%). Only about one in seven say they gave up working entirely or retired early (13%). On average, these caregivers report a household income of $62,500. Two-thirds are concerned about their ability to pay for care (65%). Only one-fifth have plans in place if they are unable to continue to provide care (20%) and three in ten have made end-of-life arrangements (31%).

Care overview: On average, PAH caregivers have been providing care for less time than rare caregivers in general – about 6.5 years, compared to nearly 9 years on average. PAH caregivers spend an average of 28 hours caring for their care recipient each week. These caregivers are involved with, on average, just under 5 IADLs, with the most common being household chores (95% partake in this task), grocery shopping (93%), and meal preparation (86%). The average PAH caregiver completes 1 ADL, with about one-third assisting in bathing (32%), getting in and out of chairs (31%) and getting dressed (30%). Two-thirds report having assisted their charge in medical/nursing tasks (67%). Unpaid assistance is received by two-fifths of PAH caregivers (41%). Paid assistance is much less common, as only one in ten receive such help (12%). Nine in ten caregivers consult with doctors or medical professionals to learn more about PAH (88%). Six in ten turn to condition-specific websites for advice (59%), and smaller but still significant shares use online or in-person support groups (45% and 37%, respectively).

About the care recipient: Only a handful of care recipients developed PAH as a genetic condition (14%), of which two-fifths report a relative that has the same condition (43%). Three-quarters report that treatment for PAH is available (73%), and nearly all state that therapy is accessible to treat the symptoms (94%). More than nine in ten care recipients use prescription medication to treat PAH (92%). However, less than half of caregivers agree that local hospitals can handle the condition (45%).

Impacts on the caregiver: Half of PAH caregivers consider caregiving to be emotionally stressful (49%), yet nine in ten agree that they take pride in learning and understanding PAH (90%). Three-quarters of caregivers consider their own physical and mental/emotional health to be at least good (73% for both aspects of their health). The average Burden of Care Index for PAH caregivers is 2.9 out of 5.

K. Policy Recommendations

Rare caregivers play a critical role in the health of those for whom they provide care. The purpose of this study is to showcase the enormity of the changes in caregivers' lives, as a result of taking on this important role. Rare disease management comes with little support and guidance. It comes with expanded responsibilities that include having to become activists and advocates for their care recipient. The challenges are great, and we hope that this important information and insight will help create a community of support for those in the rare disease fight.

With these unique challenges facing rare caregivers, we recommend the following:

- **Enhance the health of family caregivers through access to respite and counseling.**
  Only 22% of rare caregivers report having used a respite service to get a break. As the burden of care increases, rare caregivers experience increasing levels of stress and strain, reduced emotional and physical health, and increased feelings of isolation and personal neglect. This is compounded by the length of time the caregiver serves in this role—nearly 10 years on average. Two out of three rare caregivers find it difficult to maintain their own health, one out of three rate their physical health as only fair or poor. overall, rare caregivers' health is worse than United States adults overall, where just 10% report fair or poor health. Nearly seventy percent of rare caregivers say providing care is emotionally stressful, with a majority reporting that they are highly stressed. Policymakers should fund lifespan respite care services and incorporate plans for respite care and options to receive counseling into policy proposals intended to serve caregivers of those with rare diseases.

- **Achieve financial security and workplace protections for family caregivers.** The largest hurdle for many rare caregivers is balancing work and caregiving responsibilities--on average rare caregivers of adults spend between 40 and 50 hours a week providing care. Nearly all rare caregivers have gone in late or left work early to provide care (91%) – a significantly more common occurrence than general caregivers. Significant proportions also have cut back on their hours (45%), taken a leave of absence (39%), or turned down a promotion (26%). Of note, half of employed rare caregivers of a child went from working full-time to part-time or otherwise cut back their work hours to provide care (52%). Policymakers and employers should work together to foster a workplace environment that offers paid family and medical leave. Paid family and medical leave policies should: 1) Be available for family caregivers, not just new parents. 2) Offer compensation for time off at or around sixty percent of full pay. 3) Ensure caregivers are not discriminated against for taking time off for caregiving responsibilities.

- **Ensure family caregivers receive comprehensive training and information.** Rare caregivers often find that they become the expert on their care recipient's condition. They become nurses, performing medical/nursing tasks; they are the voice of care, doing Caregiving Support Activities like coordinating care and advocating; they become teachers, educating health care professionals about their care recipient's condition; they become researchers, finding and accessing clinical trials for their care recipient. Among all rare caregivers, half indicate that an experienced medical professional is needed and difficult to find. Often, complex tasks are performed without having any prior training: Half of rare caregivers do medical/nursing tasks without any preparation. More than eight in ten rare caregivers rely on medical professionals for information, while 68% turn to condition-specific websites or organizations. Health systems, medical professional societies, patient advocate organizations, and policymakers should find innovative ways to provide rare caregivers with accessible and understandable training and information on the condition of the person receiving care.

- **Improve care coordination between skilled home health providers, direct care workers, and family caregivers.**
  While many rare caregivers report feeling that their care recipient was adequately diagnosed, there is not necessarily always a next step toward treatment. While over eighty percent report there is some sort of treatment or therapy available for managing their care recipient's symptoms, only about forty percent report that there is a treatment available for the underlying rare disease or condition. Many rare caregivers report there is no treatment for their care recipient's condition, and rather, that treatments or medications are used for therapy for symptoms, rather than actual treatment. In addition, fewer than half have had a doctor, nurse or social worker ever ask what they need to care for their care recipient. Even fewer have had a medical professional or social worker ask themselves, the rare

caregiver, what he/she needs to take care of themselves. Policymakers and health systems should ensure care models are patient- and family-centered. For instances where treatment isn't available, health care professionals and students should be better educated on symptom management techniques such as high-quality palliative or hospice care as a measure to improve the quality of life, prolong survival, and increase family satisfaction, where applicable. In addition, policymakers should consider broadening criteria for patients to qualify for skilled home health nursing and private duty nursing so training could be ordered and overseen by a health care professional but executed in the home.

- **Expand services and supports available to family caregivers on the community level.**
  Not only does rare caregiving impact the family unit, the caregiver's daily life, their health, and finances, but their role can impact their social and family lives. Many rare caregivers have difficulty maintaining friendships and face feelings of isolation. Yet, some find comfort in creating a sense of community with other rare caregivers, finding support online and through reaching out to share information. Doctors and medical specialists are trusted and relied upon heavily by rare caregivers to help them through rare disease, but are often difficult to access. Rare caregivers whose care recipient lives in a rural area express more doubts about the local hospital: only 27% agree that their care recipient's local hospital can handle their condition. Policymakers, providers, health systems, and community living programs should expand to serve family caregivers in greater capacity. Community living programs should take into consideration the needs of those across the lifespan—including those who are aging, physically or mentally disabled, and those with rare health conditions—when developing home and community-based services and supports. Community support programs should include funding to patient advocacy groups to back and expand peer support services, and ensure peer support interventions are well trained and monitored so they can achieve improved health outcomes.

- **Include family caregiver as a vital member of the team in health care settings.**
  Nearly 90% of rare caregivers are the primary caregiver. In addition to helping their care recipient with IADLs and ADLs, the vast majority of rare caregivers take on "medical/nursing tasks": a body of caregiving tasks that are highly skilled in nature – things that may have typically been handled by someone like a health care aide, nurse, or another health care professional. More than eight in ten rare caregivers help their care recipient with medical/nursing tasks. Nearly all rare caregivers monitor the severity of their care recipient's condition (94%), advocate for them (94%), and communicate with care providers (94%); and 89% find themselves educating health care professionals about their care recipient's rare condition. Despite rare caregivers being in a higher burden care situation than caregivers generally, they are no more likely to rely on paid help than caregivers generally. Any entity responsible for interacting with a caregiver of those with rare

diseases should incorporate an evidence-based methodology into their practice to assess the needs of the *caregiver* and their ability to provide proper care. The paid care team should identify the primary caregiver and ensure they are documented and included in plans for treatment. Policymakers could also work with HRSA agencies to design grant opportunities or collaborative agreements for the rare disease community that translates the medical home model into this space; helping disease states become more educated on the medical home model, tools for constructing and maintaining, and how to partner with medical teams.

- **Recognize the family caregiver's role in clinical trial research and innovation.**
  Rare caregivers are often advocates within the health care system, educating professionals about their care recipient's rare disease or condition. Due to the lack of available therapies to support a person with a rare disease or condition, caregivers are also key catalysts in researching and accessing clinical trials. One in four report their care recipient has participated in a clinical trial and when that occurs, the rare caregiver often has to help with things like paperwork, transportation, trial response documentation, and care coordination. When managing existing medications or therapies, many report there is not always a next step toward treatment. Even though more than 80% of caregivers say that there is some sort of treatment or therapy available for managing their care recipient's symptoms, less than half report that there is a treatment available for the underlying rare disease or condition. Drug and device manufacturers should consider how the presence or absence of a family caregiver impacts the development process and consider caregivers as partners in clinical trial recruitment. As patient-reported outcome measurements (PROs) are developed, innovators should identify ways to capture the perspective of the caregiver, including both the role of the caregiver in medication or device management and the impact of disease on unpaid caregivers.

Rare caregivers face challenges that are multifaceted. Pressure points on this group are significantly compounded by the duration of time spent living as a caregiver and lack of available options to treat the care recipient's condition. This means that not only are rare caregivers marginalized in their ability to contract with qualified paid direct care workers, but that they are also struggling to find the time away from work and daily responsibilities to serve as a caregiver without facing financial consequences. It is for these reasons that this report recommends to policymakers that rare caregivers require empowerment to remain in their role as caregiver, not to replace them with potentially more costly services.

L. Respondent Profile

The majority of rare disease caregivers surveyed in this research are women (87%). On average, they are 44.9 years old, with those caring for a child about 12 years younger, on average, than those caring for an adult. Rare caregivers of a child are about the same age as general caregivers of a child,[79] while rare caregivers of an adult are about 3 years older than general caregivers of adults.[80]

Figure 56: Respondent Profile

	Total (n=1,406)	Child Care Recipient (n=871)	Adult Care Recipient (n=535)
Caregiver Gender			
Male	**12%**	6%	23%^
Female	**87%**	94%^	76%
Decline to respond	**<0.5%**	<0.5%	<0.5%
Caregiver Age			
18 to 29	**6%**	7%^	4%
30 to 39	**31%**	42%^	11%
40 to 49	**31%**	38%^	21%
50 to 59	**20%**	10%	36%^
60 or older	**12%**	3%	28%^
Mean age	***44.9***	*40.4*	*52.3^*
Caregiver Generation			
Millennial (age 18-36)	**26%**	35%^	10%
Gen X (age 37-52)	**49%**	58%^	36%
Boomer (age 53-74)	**24%**	8%	51%^
Silent / Greatest (age 75+)	**1%**	--	2%^

^ indicates significantly higher than comparison group

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[79] "Caregiving in the U.S. 2009" found that general caregivers of a child were 40.6 years old, on average.

[80] "Caregiving in the U.S. 2015" found that general caregivers of an adult were 49.2 years old, on average.

The majority of rare caregivers report having a child or grandchild under the age of 18 living in their household (71%). Nearly all rare caregivers are current caregivers (96%), with just 4% reporting they cared for someone with a rare disease or condition in the past year but are no longer doing so. Rare caregivers of an adult more often report that they have transitioned out of a current caregiving role. One in five rare caregivers report caring for more than one person with a rare disease or condition in the past year (22%). One in three rare caregivers report their care recipient lives in a rural area (34%), more than caregivers generally.[81]

Respondent Profile, Continued

	Total (n=1,406)	Child Care Recipient (n=871)	Adult Care Recipient (n=535)
Children Under Age 18 Living in Caregiver's Household			
Yes (includes child recipients)	**71%**	97%^	29%
No	**29%**	3%	71%^
Current Care			
Yes, currently providing care	**96%**	98%^	92%
Provided care in past 12 months	**4%**	2%	8%^
Caregiver Cared for Multiple People with Rare Disease			
Yes	**22%**	23%	21%
No	**76%**	76%	77%
Not sure	**1%**	1%	2%
Care Recipient Lives In…			
Rural area	**34%**	33%	35%
Suburban/Urban (not rural)	**66%**	67%	65%

^ indicates significantly higher than comparison group

The majority of rare caregivers have graduated college with a bachelor's degree or higher (59%). Six in ten rare caregivers report a household income of $50,000 or more, with those caring for a child likely to report a higher income.

The high levels of income[82] and education[83] among the surveyed rare caregivers suggests two things: 1) that the situation among rare caregivers may be underestimated

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[81] Among general child caregivers, 26% report their care recipient lives in a rural area ("Caregiving in the U.S. 2009") and among general adult caregivers, 28% their recipient resides in a rural area ("Caregiving in the U.S. 2015").

[82] Rare caregivers of an adult have comparable incomes to general caregivers of an adult. "Caregiving in the U.S. 2015" found that among general caregivers of an adult, 53% reported a household income of $50,000 or more. However, rare caregivers of a child more often have incomes of $50,000 or more (64%) vs. 37% general child caregivers, as found in "Caregiving in the U.S. 2009".

[83] Rare caregivers tend to be more educated than caregivers generally: 62% of rare caregivers of a child have a bachelor's degree or higher compared to 32% of general child caregivers ("Caregiving in the U.S.

in this report, as income and education tend to allow for improved caregiving situations;[84] and 2) networks of rare caregivers may need to improve outreach to lower-income rare caregivers, as the majority of the surveyed rare caregivers came from online social networks and disease groups of rare caregivers.

Rare caregivers responding to the survey self-identified as white (91%), with small proportions identifying as Hispanic, African-American, Asian/Pacific Islander, Native American/Alaskan Native, and/or Other.

Respondent Profile, Continued

	Total (n=1,406)	Child Care Recipient (n=871)	Adult Care Recipient (n=535)
Caregiver Education			
High school or less	**9%**	8%	10%
Some college	**18%**	16%	22%^
Technical school / Associate’s degree	**13%**	13%	13%
Bachelor’s degree	**32%**	35%^	26%
Graduate work or degree	**27%**	27%	28%
Caregiver Household Income			
Less than $30,000	**13%**	13%	14%
$30,000 to $49,999	**15%**	13%	18%^
$50,000 to $74,999	**18%**	18%	19%
$75,000 to $99,999	**15%**	16%^	12%
$100,000 or more	**27%**	30%^	22%
Decline to respond	**12%**	10%	15%^
Caregiver Race/Ethnicity			
White	**91%**	92%^	89%
Hispanic	**4%**	4%	4%
African-American	**2%**	2%	4%
Native American / Alaskan Native	**2%**	2%	3%
Asian/Pacific Islander	**2%**	3%	2%
Other	**1%**	<0.5%	1%
Decline to respond	**4%**	3%	5%

^ indicates significantly higher than comparison group

2009”). Similarly, 54% of rare caregivers of an adult have at least a bachelor’s degree, compared to 34% of general caregivers of an adult nationally (“Caregiving in the U.S. 2015”).

[84] As shown in “Caregiving in the U.S. 2015”

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About the National Alliance for Caregiving: Established in 1996, the National Alliance for Caregiving is a non-profit coalition of national organizations focusing on advancing family caregiving through research, innovation, and advocacy. The Alliance conducts research, does policy analysis, develops national best-practice programs, and works to increase public awareness of family caregiving issues. Recognizing that family caregivers provide important societal and financial contributions toward maintaining the well-being of those they care for, the Alliance supports a network of more than 80 state and local caregiving coalitions and serves as Secretariat for the International Alliance of Carer Organizations (IACO). Learn more at www.caregiving.org.

About Global Genes: Global Genes is a leading rare disease patient advocacy organization whose mission is to connect, empower, and inspire the rare disease community. With international scope, Global Genes develops educational resources, programs, and events that unite patients, advocates, and industry experts. Committed to fostering these meaningful connections, Global Genes is driven to catalyze therapeutics and cures for the estimated 7,000 rare diseases that impact approximately 1 in 10 Americans, and 350 million people worldwide. For more information, please visit www.globalgenes.org and follow @GlobalGenes on social media to join the RARE conversation.