

# Aptitude Towards Patient-Centered Care Among Physicians Managing Rare Diseases in the United States: A Cross-Sectional Survey

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## Background

Patient centeredness describes providing healthcare that is respectful of the patient's preferences, needs, and values in clinical decision making<sup>1</sup>. In other words, where patient involvement is viewed as a focal point. This is particularly critical in rare disease where diagnostic delays, limited treatment options, and knowledge gaps are common<sup>1</sup>. Given these circumstances, patients and their caregivers may become experts by experience, providing valuable insights into their disease<sup>2</sup>. Therefore, involving patients and their caregivers may be essential to inform shared decision-making.

## Objective

To describe patient-centered practices and attitudes, including communication, empathy, and involvement of patients and caregivers in decision-making among physicians managing patients with rare diseases.

## Methods

- This was a cross-sectional study conducted among primary care physicians (PCPs) and specialists in the US, recruited via physicians' panels. Data were collected between October and November 2025.
- The study instrument was a structured online questionnaire comprising binary (yes/no) and Likert-like scale items to questions.
- Analyses were descriptive, with categorical variables summarized as frequencies and percentages (n, %). Differences between PCPs' and specialists' responses were assessed using Pearson's Chi-squared test or Fisher's exact test, as appropriate. Statistical significance was set at p < 0.05.



**References :**  
1. Abid MH, Kumah A, Newera A, Hafez P. Patient-Centered Healthcare: From Patient Experience to Human Experience. Glob J Qual Saf Healthc. 2024;7(4):144-148. doi:10.36401/JQSH-24-X2  
2. Perfetto EM, Oehrlein EM, Love TR, Schoch S, Kennedy A, Bright J. Patient-Centered Core Impact Sets: What They are and Why We Need Them. Patient. 2022;15(6):619-627. doi:10.1007/s40271-022-00583-x

## Results

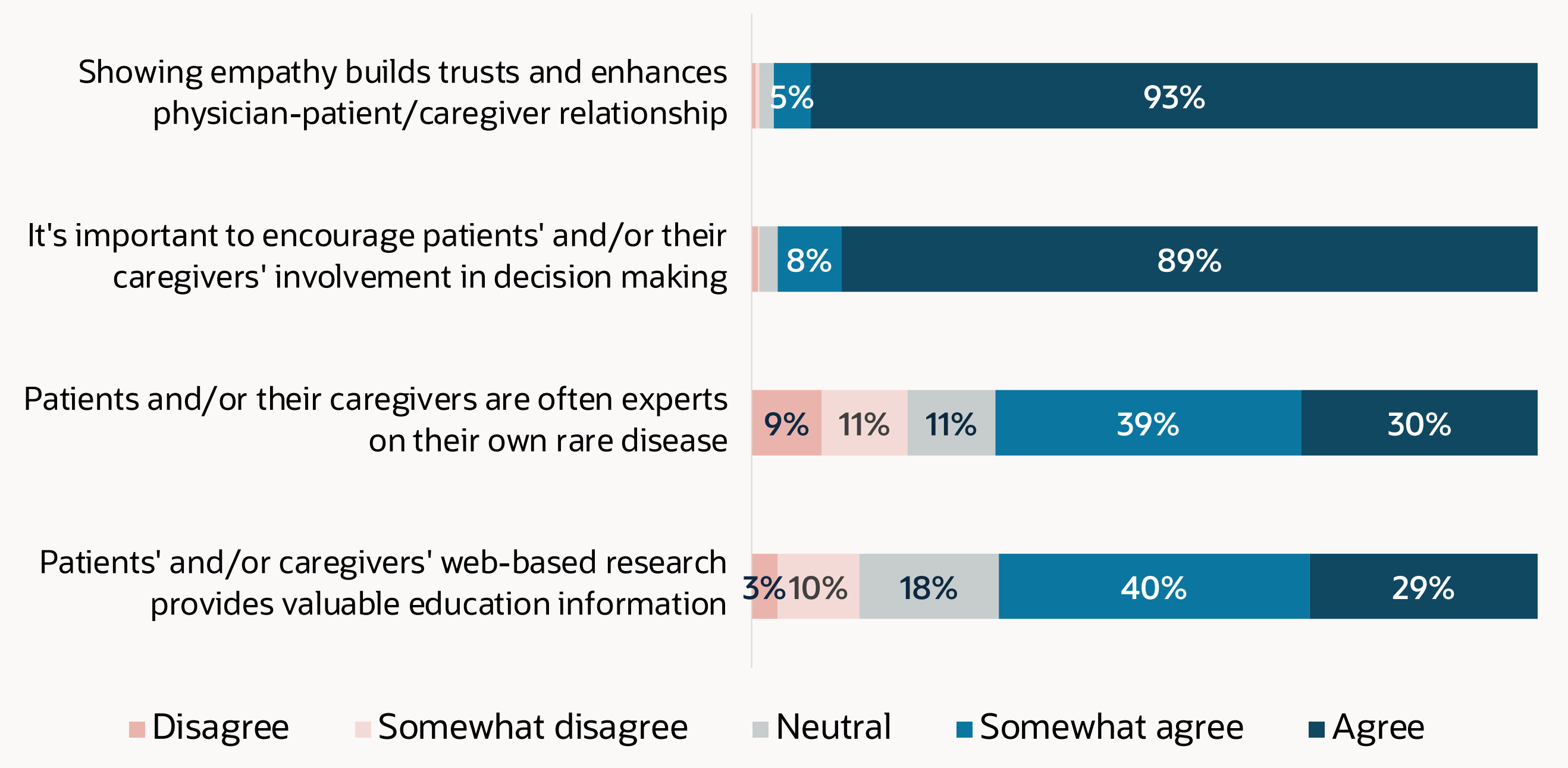
- Of the 617 participating physicians, one third were primary care physicians (family medicine, general practice, and internal medicine; n=206, 34%) and two thirds were specialists (n=411, 66%) (Table 1). Most of them had at least 10 years of clinical experience.

**Table 1.** Distribution of medical specialties among participating physicians

Medical specialty	N=617 n (%)
Family Medicine / General Practice	124 (20.1%)
Internal Medicine	82 (13.3%)
Pediatrics	44 (7.1%)
Cardiology	44 (7.1%)
Gastroenterology	39 (6.3%)
Ophthalmology	36 (5.8%)
Obstetrics & Gynecology	31 (5.0%)
Nephrology	30 (4.9%)
Neurology	30 (4.9%)
Rheumatology	30 (4.9%)
Pulmonology	29 (4.7%)
Dermatology	27 (4.4%)
Infectious Disease	21 (3.4%)
Urology	19 (3.1%)
Endocrinology/Diabetes	18 (2.9%)
Hemato-oncology	13 (2.1%)

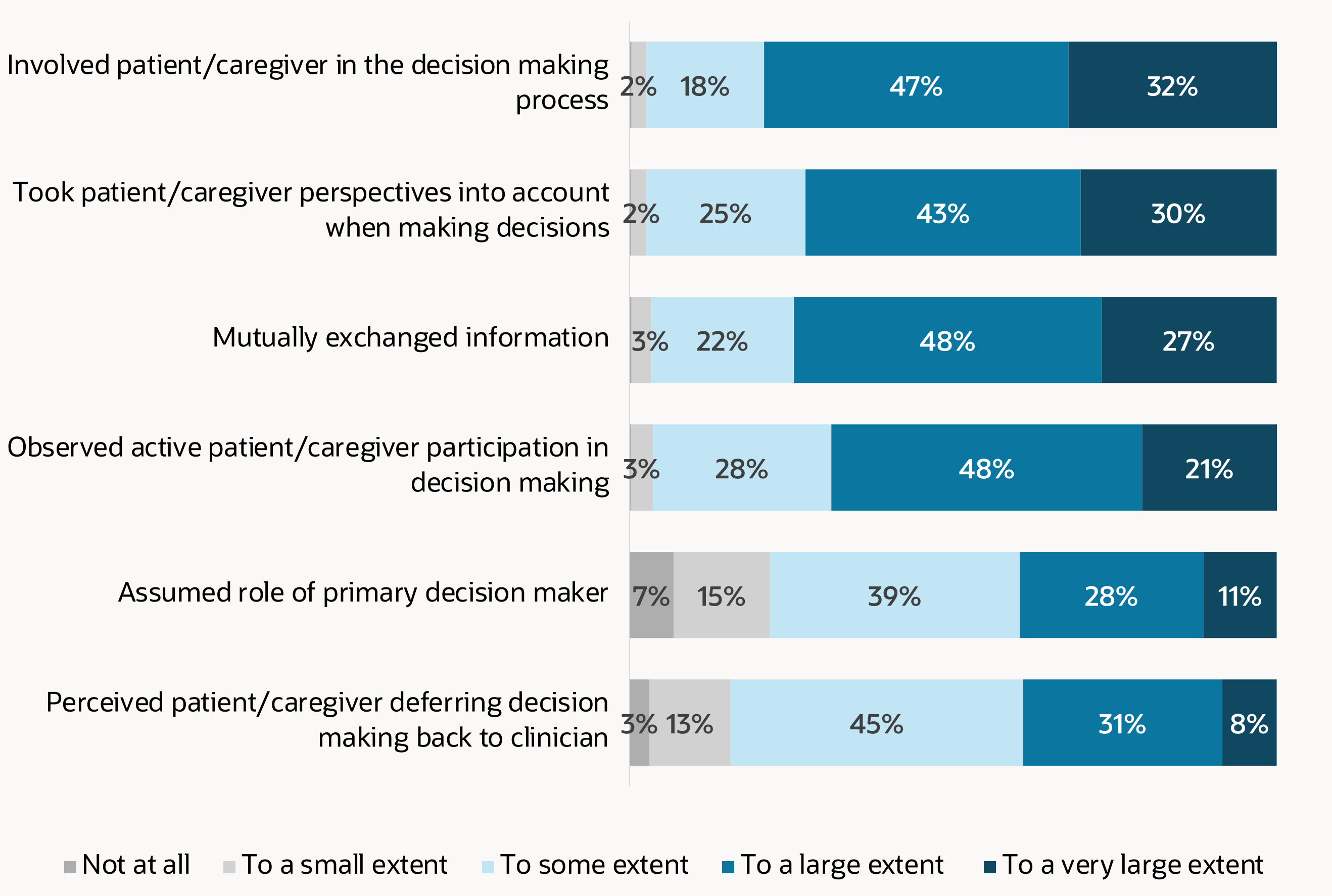
- Most physicians (n=547, 89%) agreed that it is important to encourage patients' and caregivers' involvement in decision-making. Around a third of physicians (n=186, 30%) agreed that patients and/or caregivers are experts in their rare disease (Figure 1).
- More than 90% of physicians agreed that empathy can build trust and enhances their relationship with their patients, a similar attitude was observed between PCPs and specialists.

**Figure 1.** Physician agreement levels on rare disease patient management



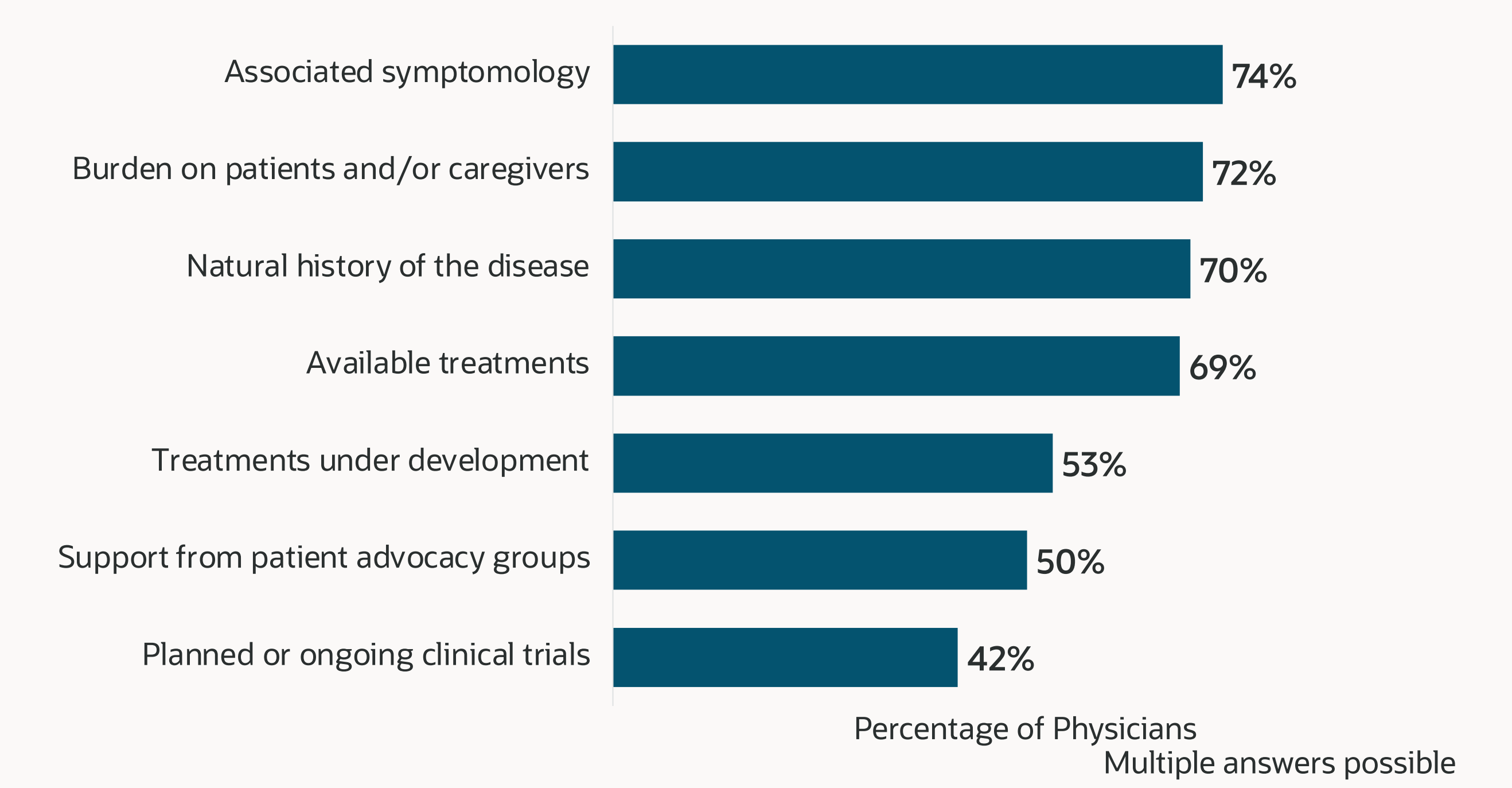
- Physicians reported a high degree of patient/caregiver involvement in treatment initiation or treatment switching discussions with patients and caregivers (Figure 2).
  - The majority reported involving their patient in decision-making to a large extent (n=489, 79%), while mutually exchanging information on their rare disease (n=460, 75%) and taking their patients' and/or caregivers' perspectives into account in the process (n=449, 73%).
  - Only 39% of physicians reported, to a large extent, assuming the role of primary decision maker or perceiving patients deferring decisions back to them about treatment.

**Figure 2.** Extent of physician behavior during treatment initiation or treatment switch discussion with patients and/or caregivers

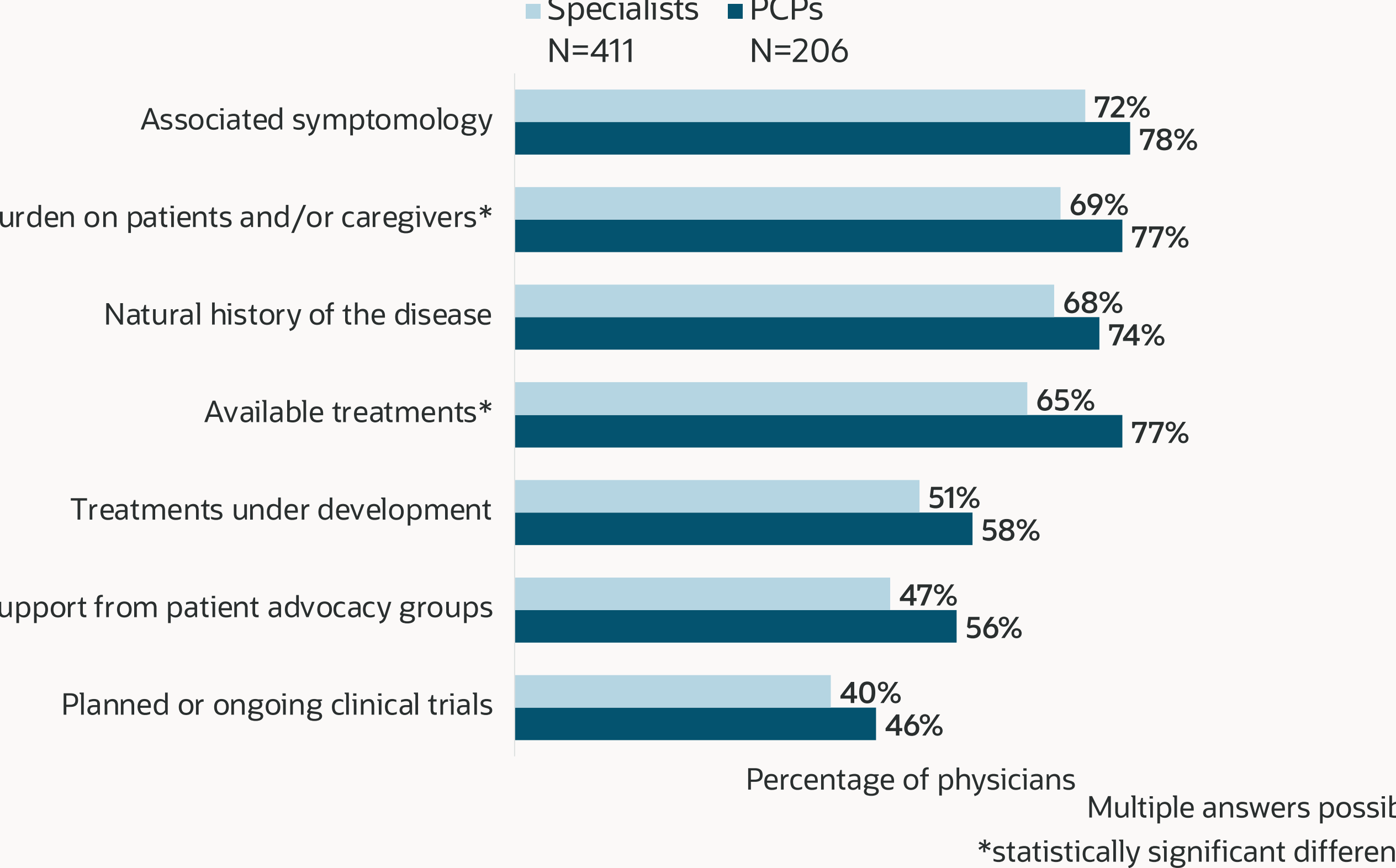


- When asked to report on what they have learned from their patients about the rare disease, physicians mostly reported learning about the rare disease symptomatology (74%), burden of the disease on quality of life (72%), and the natural history of the disease (70%) (Figure 3).
- PCPs were more likely than specialists to learn about available treatments and disease burden (Figure 4).

**Figure 3.** Physician-reported insights gained from patients and/or caregivers on their rare disease



**Figure 4.** Physician-reported insights gained from patients and/or caregivers on their rare disease, reports from primary care physicians vs specialists



- Discussions most often occur throughout the patient's management journey rather than at diagnosis or at the start of treatment. The most discussed topics were the importance of treatment adherence, associated psychological challenges, and the burden of the disease on quality of life (Table 2).

**Table 2.** Physician-reported topics discussed with patients and/or caregivers across the rare disease patient journey

Topic discussed	Disease stage			
	At diagnosis n (%)	At treatment start n (%)	Throughout the patient management n (%)	Upon patient request n (%)
Burden on quality of life	276 (44.7%)	204 (33.1%)	436 (70.7%)	186 (30.1%)
Importance of treatment adherence and consequences of discontinuation	231 (37.4%)	315 (51.1%)	446 (72.3%)	154 (25.0%)
Psychological challenges	261 (42.3%)	216 (35.0%)	438 (71.0%)	192 (31.1%)
Evidence-based resources for information	246 (39.9%)	184 (29.8%)	330 (53.5%)	222 (36.0%)
Patient advocacy groups	221 (35.8%)	161 (26.1%)	294 (47.6%)	257 (41.7%)
Sources for financial support	186 (30.1%)	176 (28.5%)	290 (47.0%)	274 (44.4%)

## Conclusion

Physicians recognize the value of patient involvement and input in decision making at large, although they still have some reservations when it comes to considering patients as experts of their own rare disease. Decision making around treatment is largely perceived as a collaborative process rather than exclusively physician driven, with a little less than half of physicians assuming the role of sole decision-maker. While most physicians have presented an aptitude towards patient centeredness in their attitude, there remains a small gap in practice to foster true collaborative patient-centered care.

