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Cystinosis Patient Survey Data Analysis Final Report

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1420 K Street, NW, Suite 1050
Washington, DC 20005

202.591.4000 Main
202.591.4020 Fax

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Reaching healthcare influencers, producing measurable results.

Scope of Research



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- 1. Partnership initiated with CRN to reach patients and families with cystinosis.**

- 2. JPA fielded survey via online, mail and phone calls – July 17 through present**

- 3. Total responses to date: 192**
 - US = 162
 - Ex-US = 21
 - Unknown = 9

* The following analysis is based on US surveys

Key Takeaways

- While the vast majority (94%) of cystinosis patients have experienced crystal formation, only 61% currently use the eye drops.
- For those that aren't using the drops, it is primarily because they haven't been recommended or the patient doesn't experience symptoms; Currently, price is not a major factor in use of the drops.
- The primary source of the eye drops is NIH Eye Clinic.
- Most patients start using the drops over 1 year after cystinosis diagnosis. Compliance in terms of daily use and storage varies.
- The majority (89%) of patients have health insurance, but household income varies widely among patients and families.

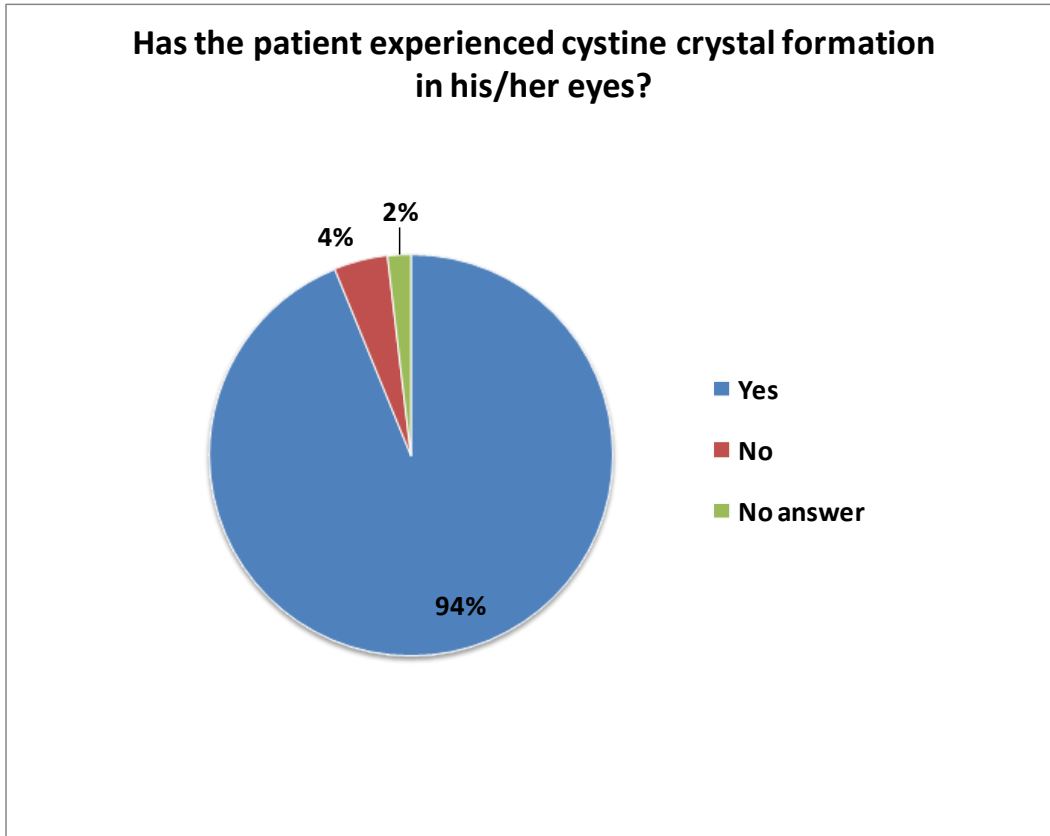
Cystinosis Diagnosis



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3.4 years	Average age at cystinosis diagnosis
Prenatal	Minimum age at cystinosis diagnosis
25 years	Maximum age at cystinosis diagnosis

Cystine Crystal Formation



Age when crystals were first identifiable:

Average age = 4.5 years

Min age = .25 years

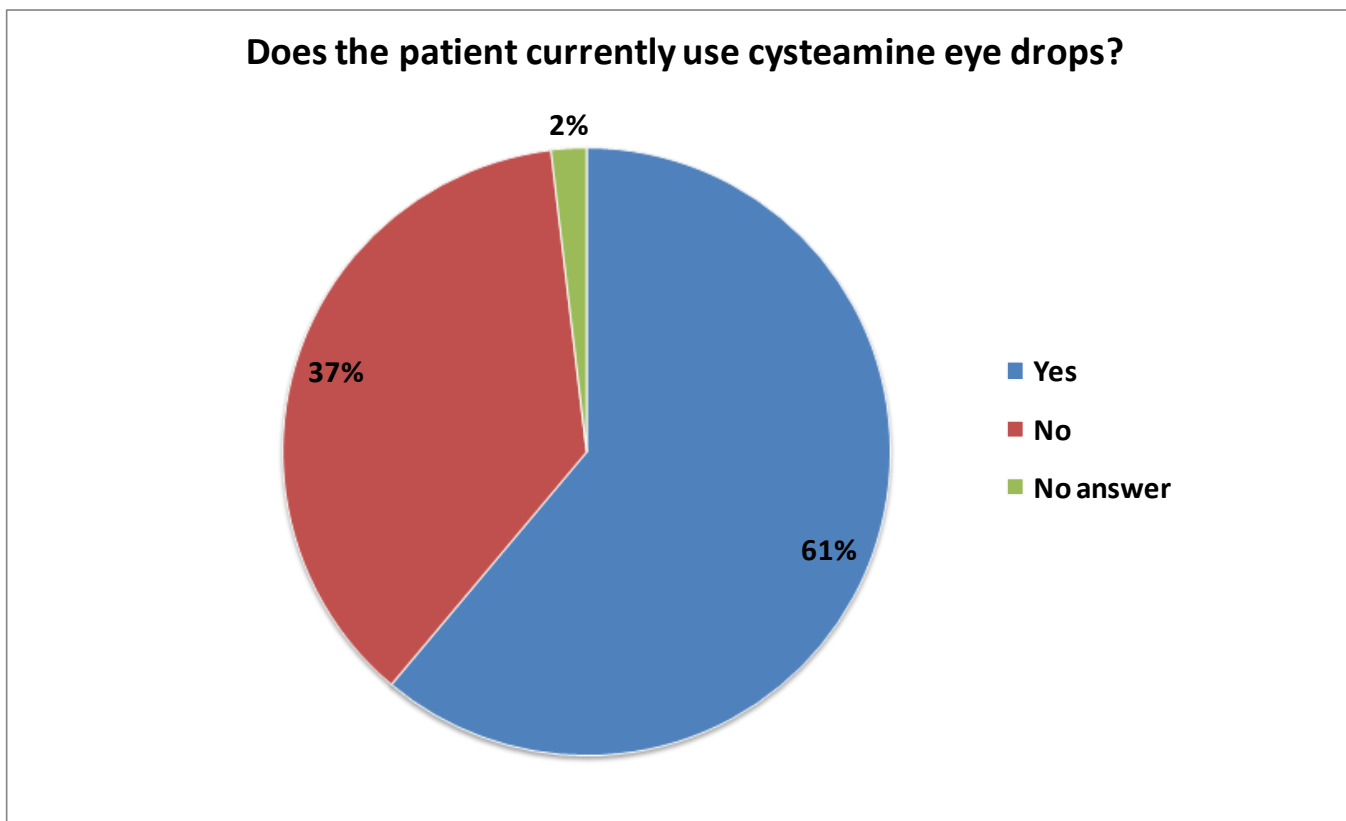
Max age = 28 years

Only 16 respondents indicated that they know their crystal score:

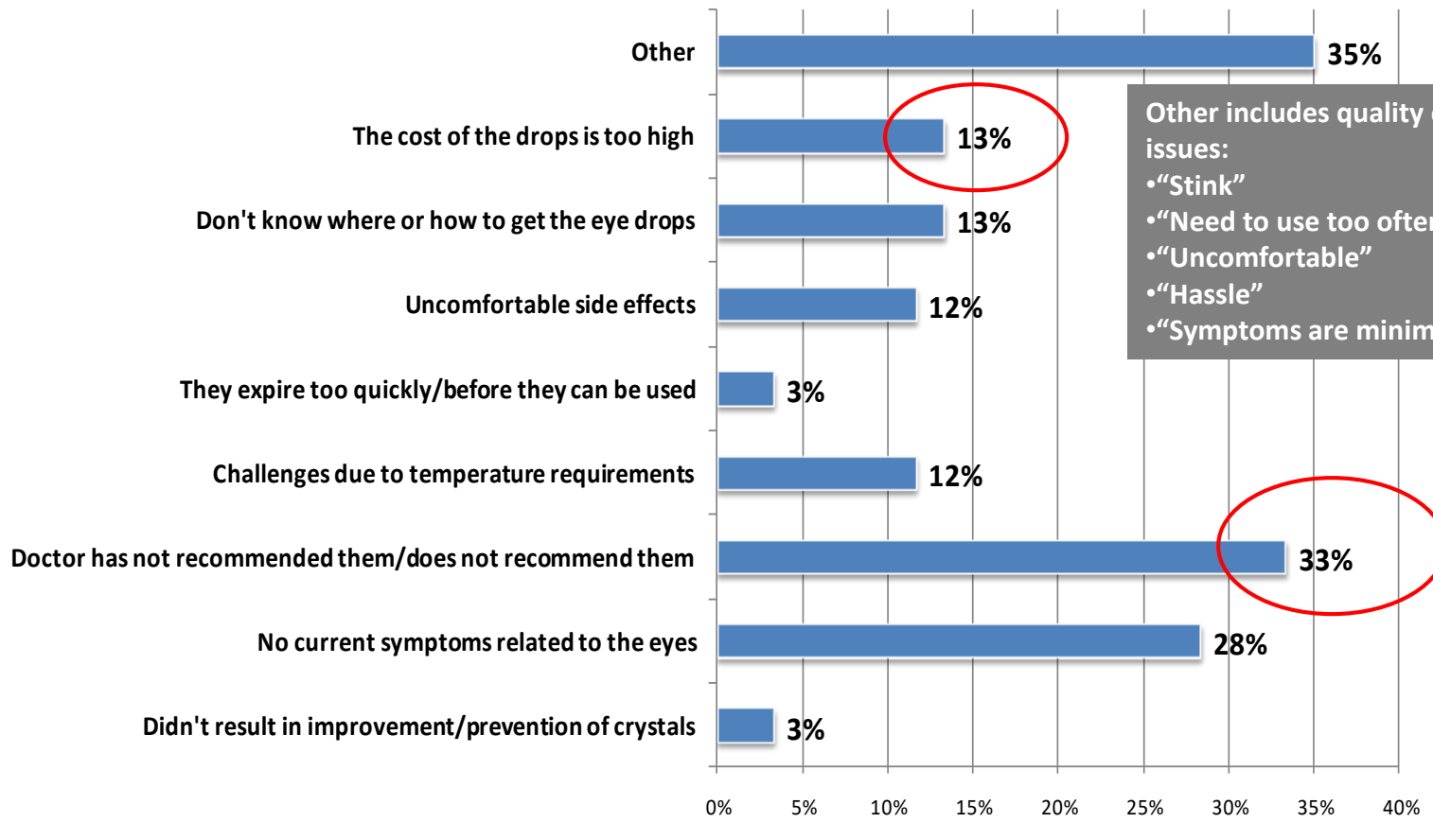
Min score = 1

Max score = 8

Eye Drop Usage

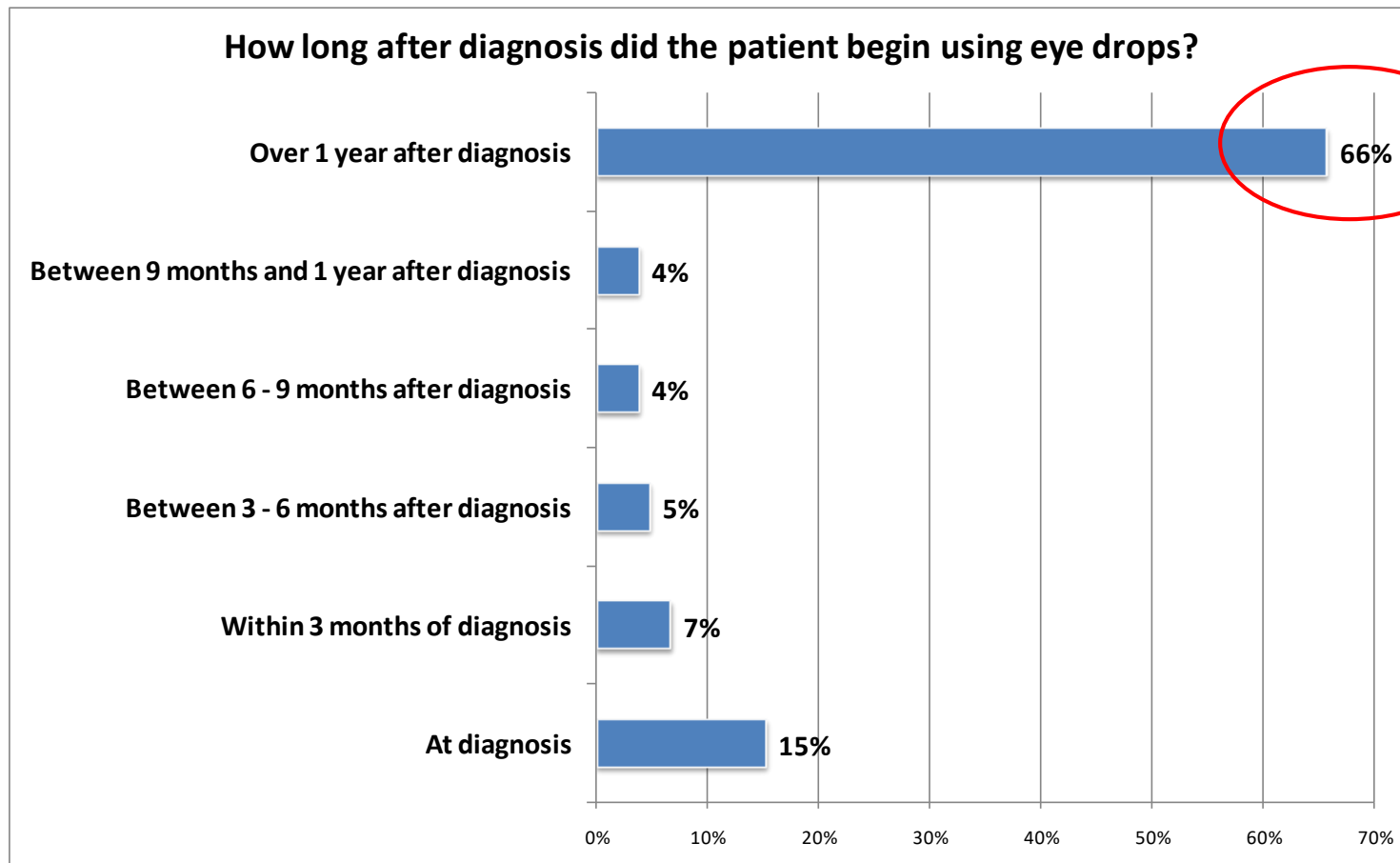


Why isn't the patient using cysteamine eye drops?

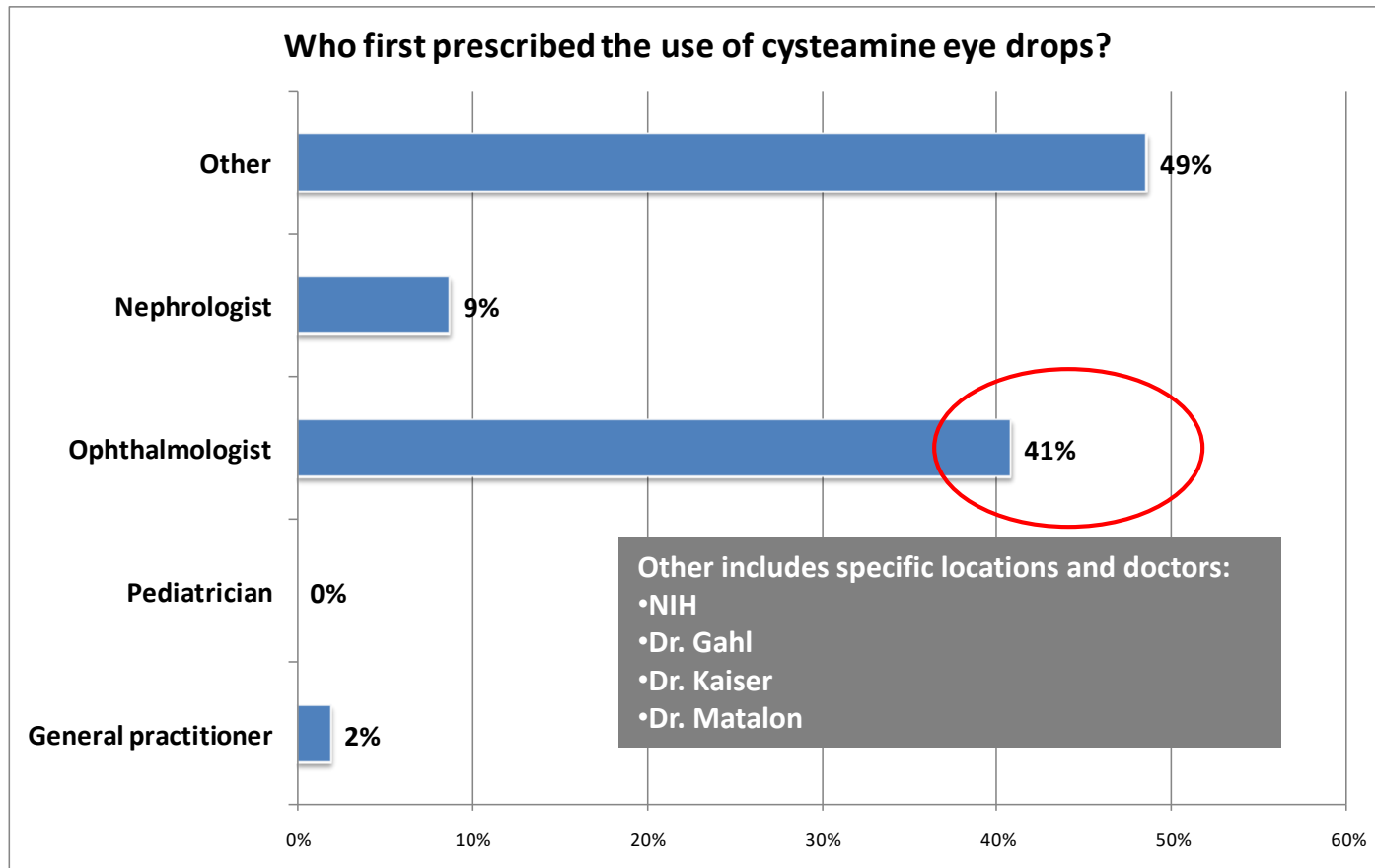


Respondents selected multiple responses.

Treatment Continuum



Eye Drop Prescribers

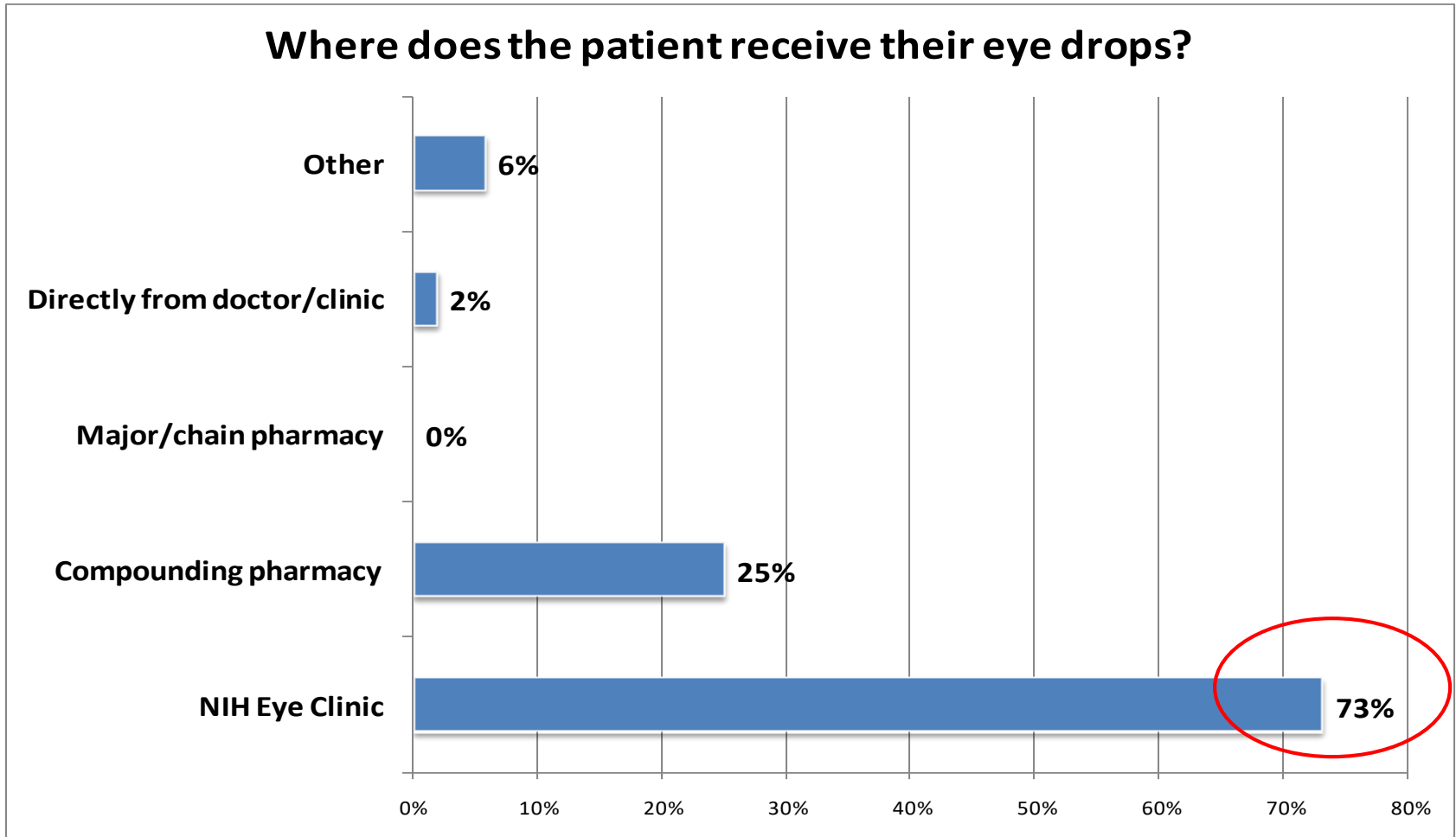


Method of Distribution



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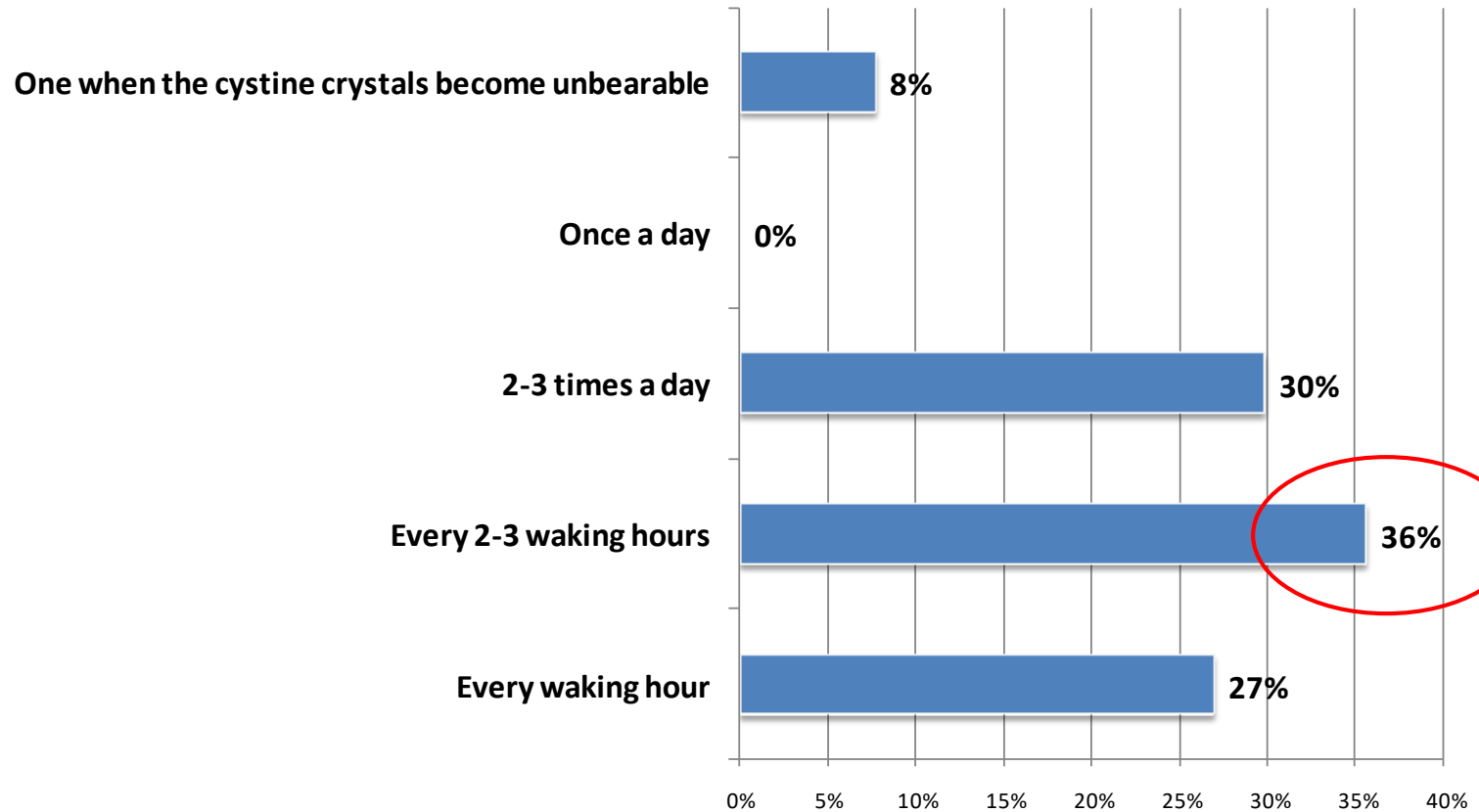
Where does the patient receive their eye drops?

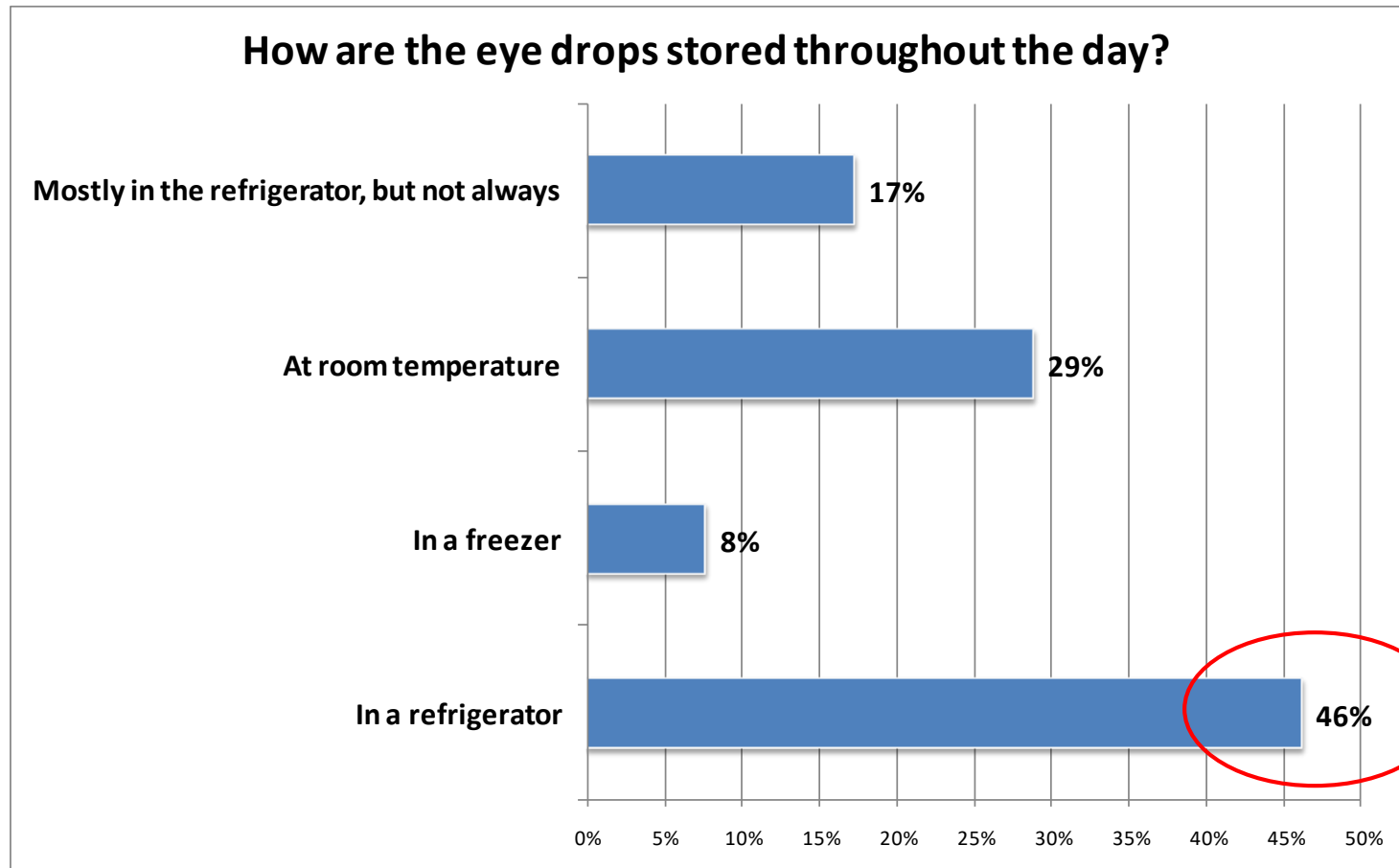


Other includes:
• Mayo Clinic
• S&R Drug Company in Kirbyville, TX

Respondents selected multiple responses.

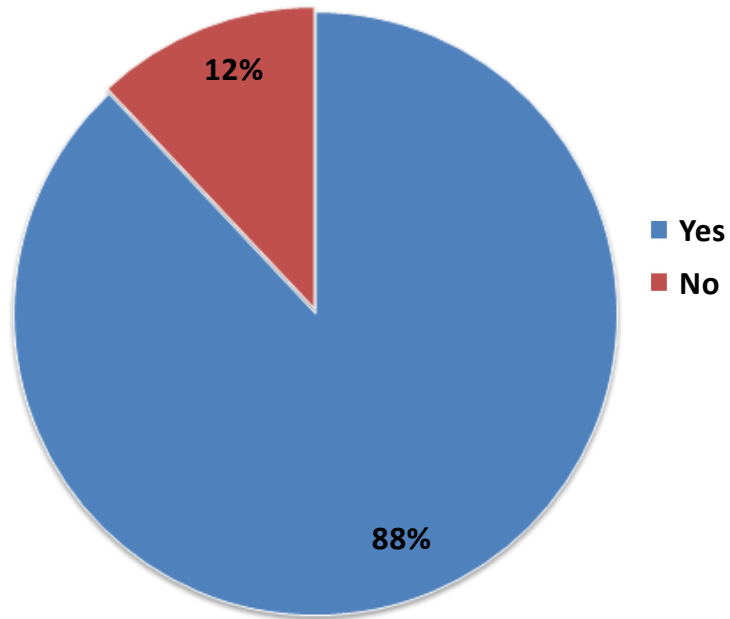
How many times a day does the patient use the eye drops?





Eye Drop Effectiveness

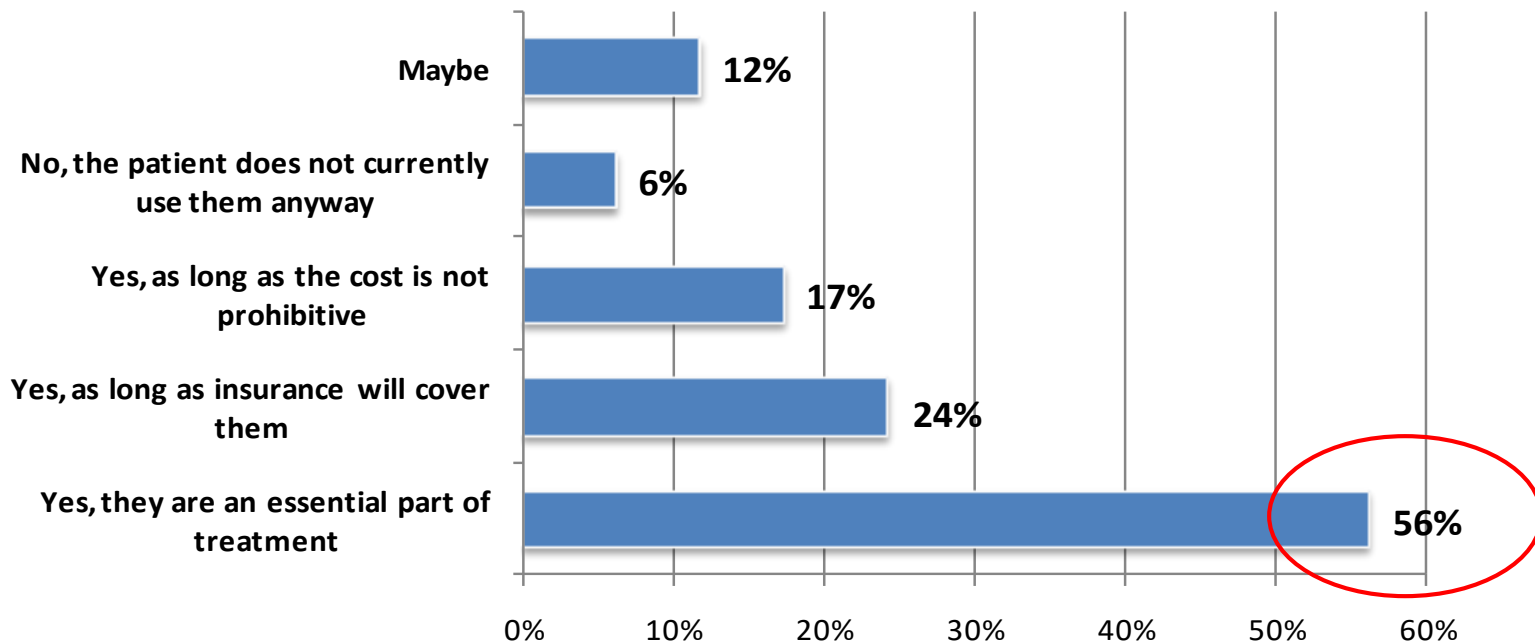
Has the patient experienced a decrease in cystine crystal formation or prevention since using the drops?



Commercially Available Eye Drops

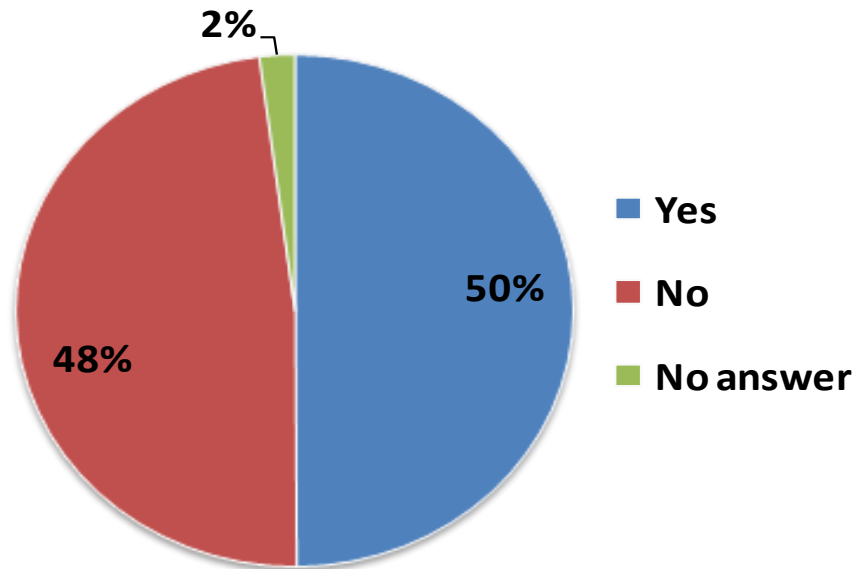


Is the patient likely to use cysteamine eye drops if they become commercially available and accessible at the pharmacy?



Kidney Transplants

Has the patient received a kidney transplant?



Age at transplant

Average age = 14

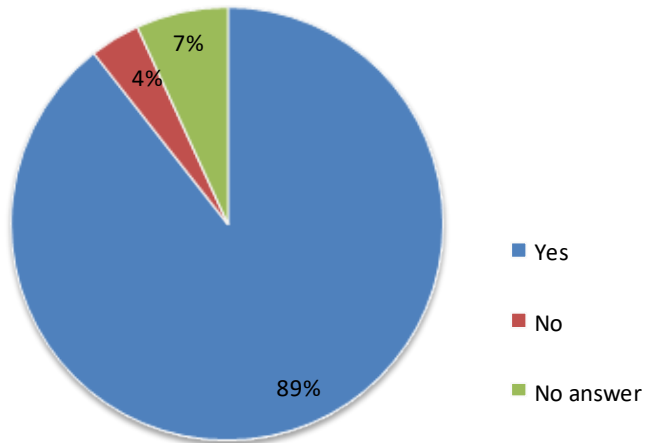
Min age = 5

Max age = 56

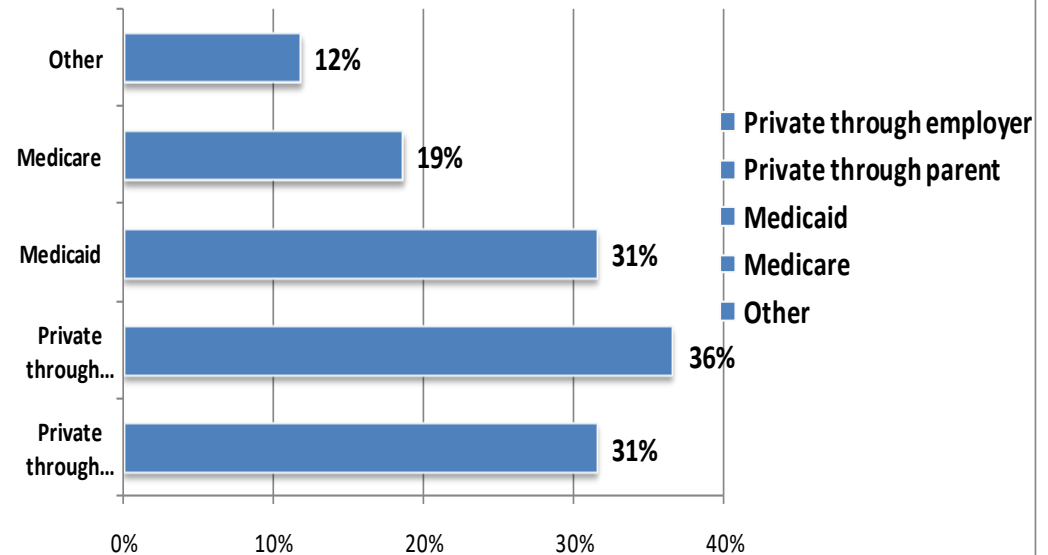
Health Insurance Status



Does the patient currently have health insurance?



What type of insurance does the patient have?

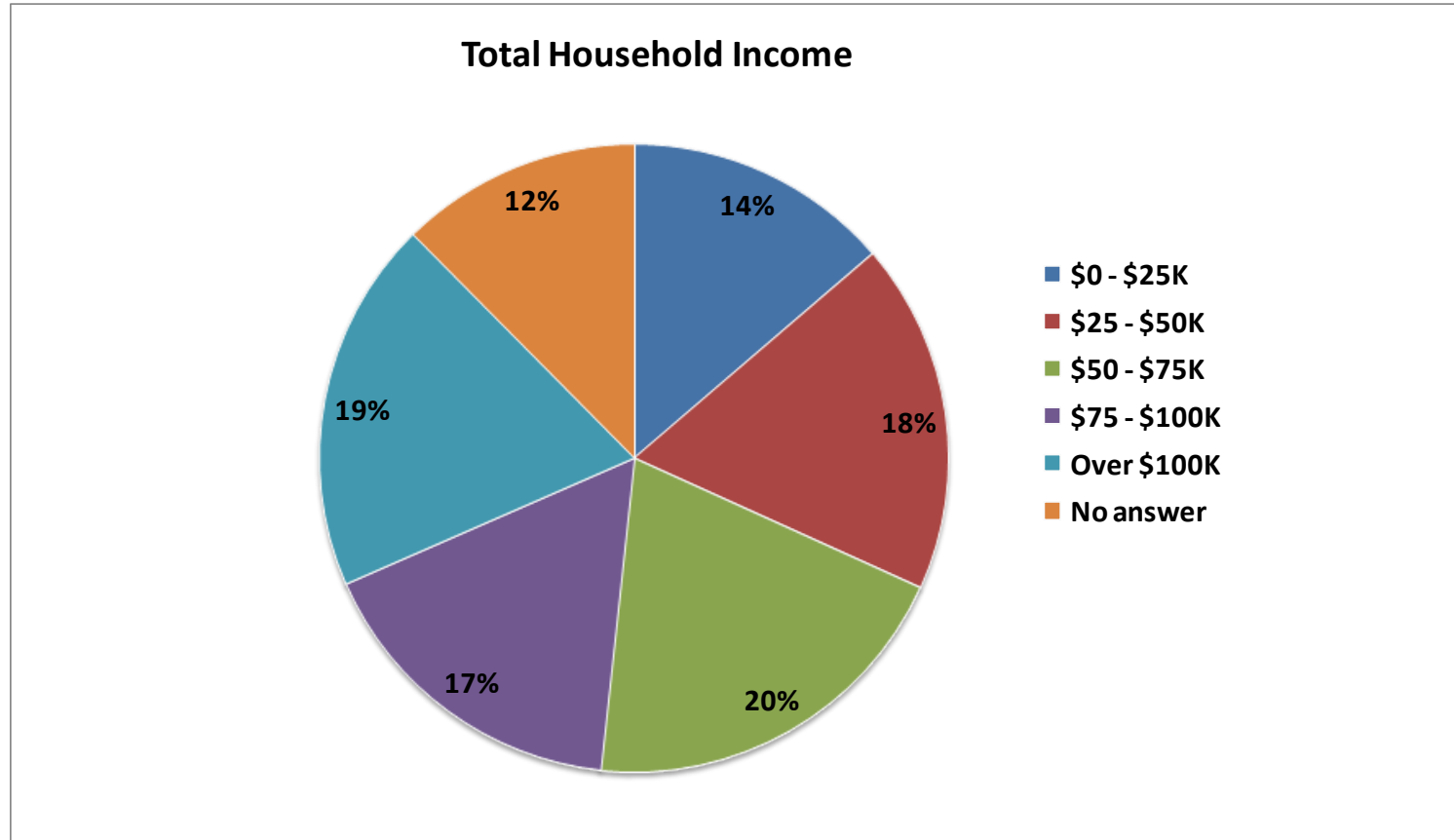


Other primarily includes state-run health programs, e.g., Mass Safety Net, Illinois Public Aid

Demographic Information



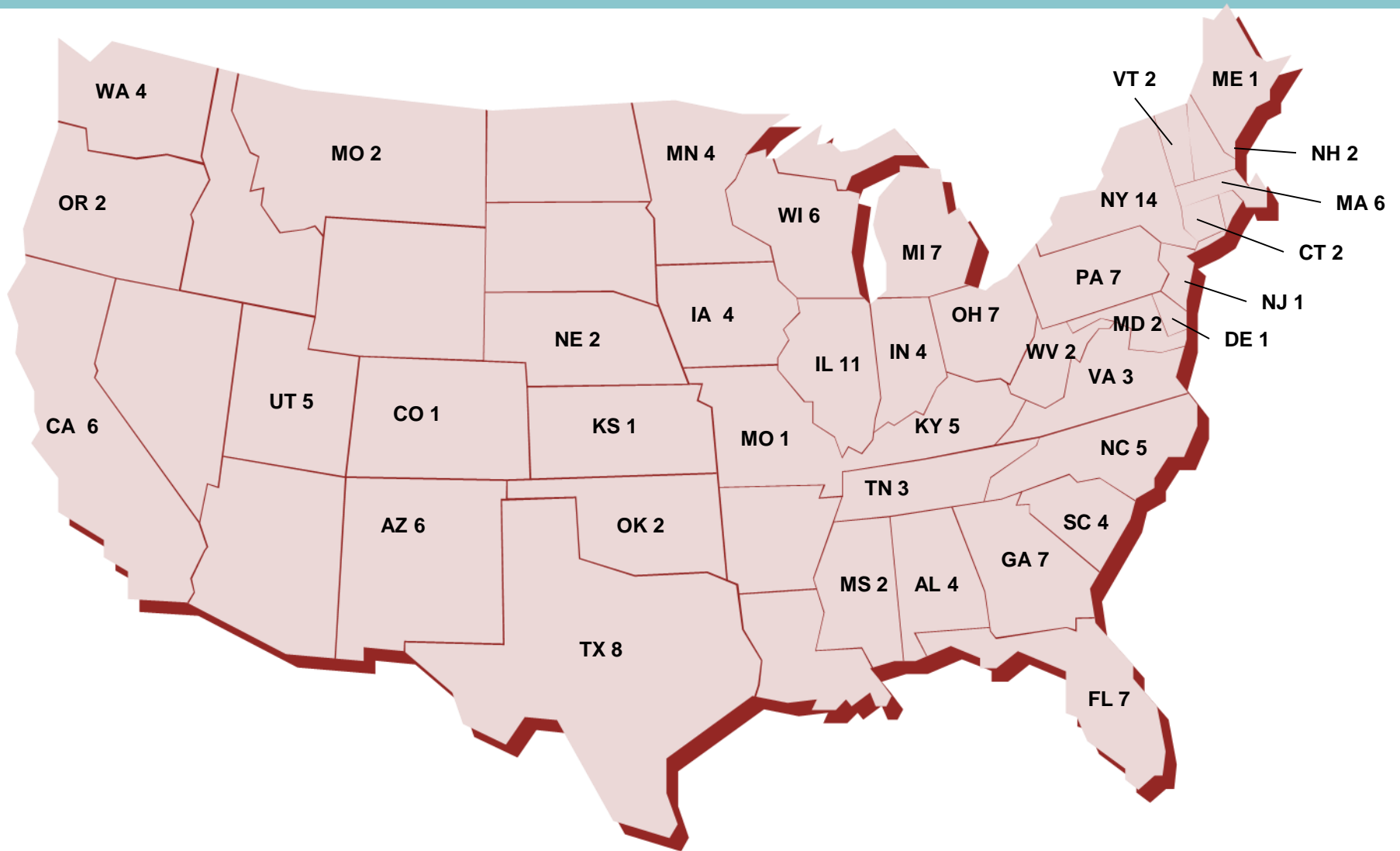
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Patient Location (n=162)



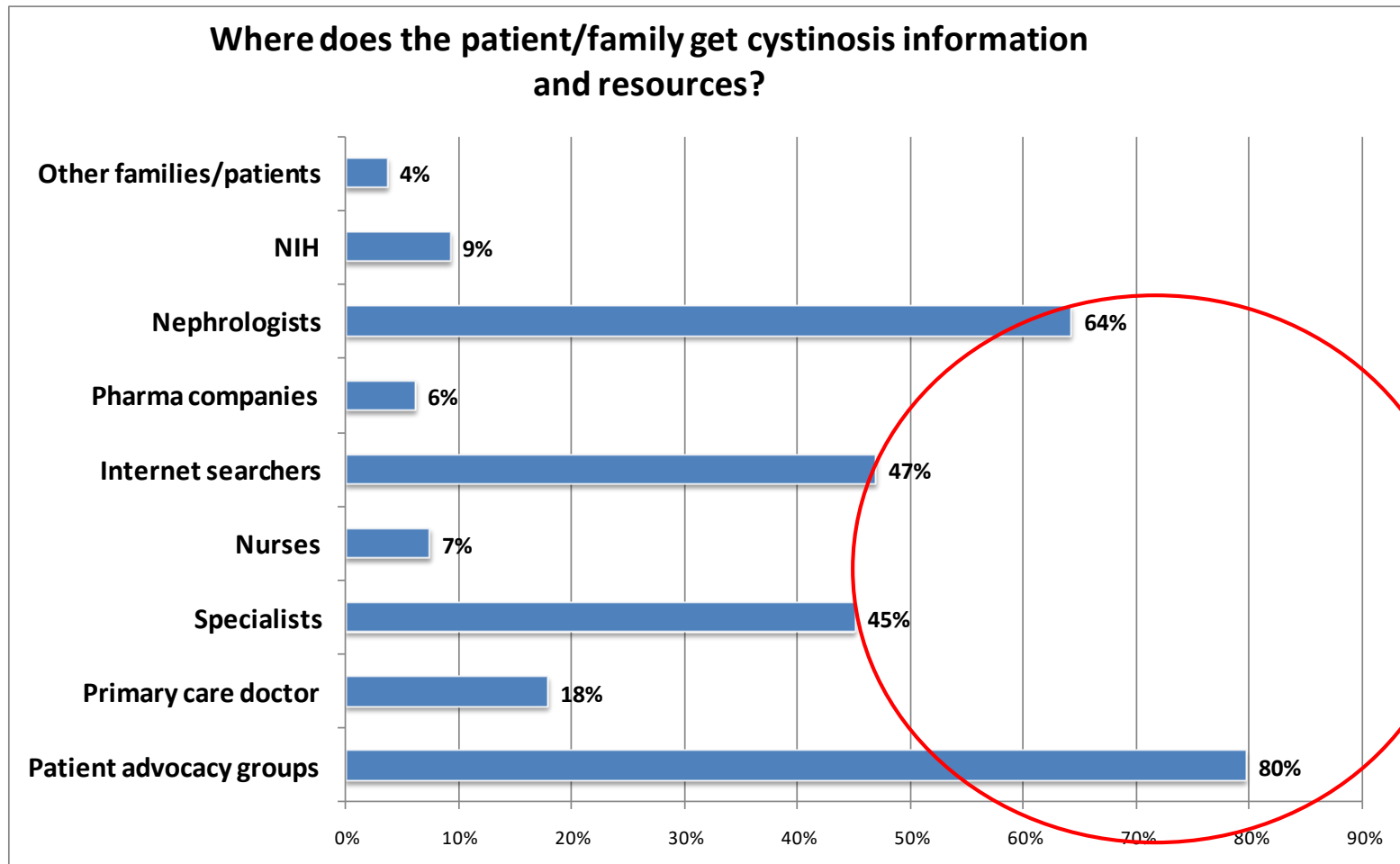
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Cystinosis Information & Resources

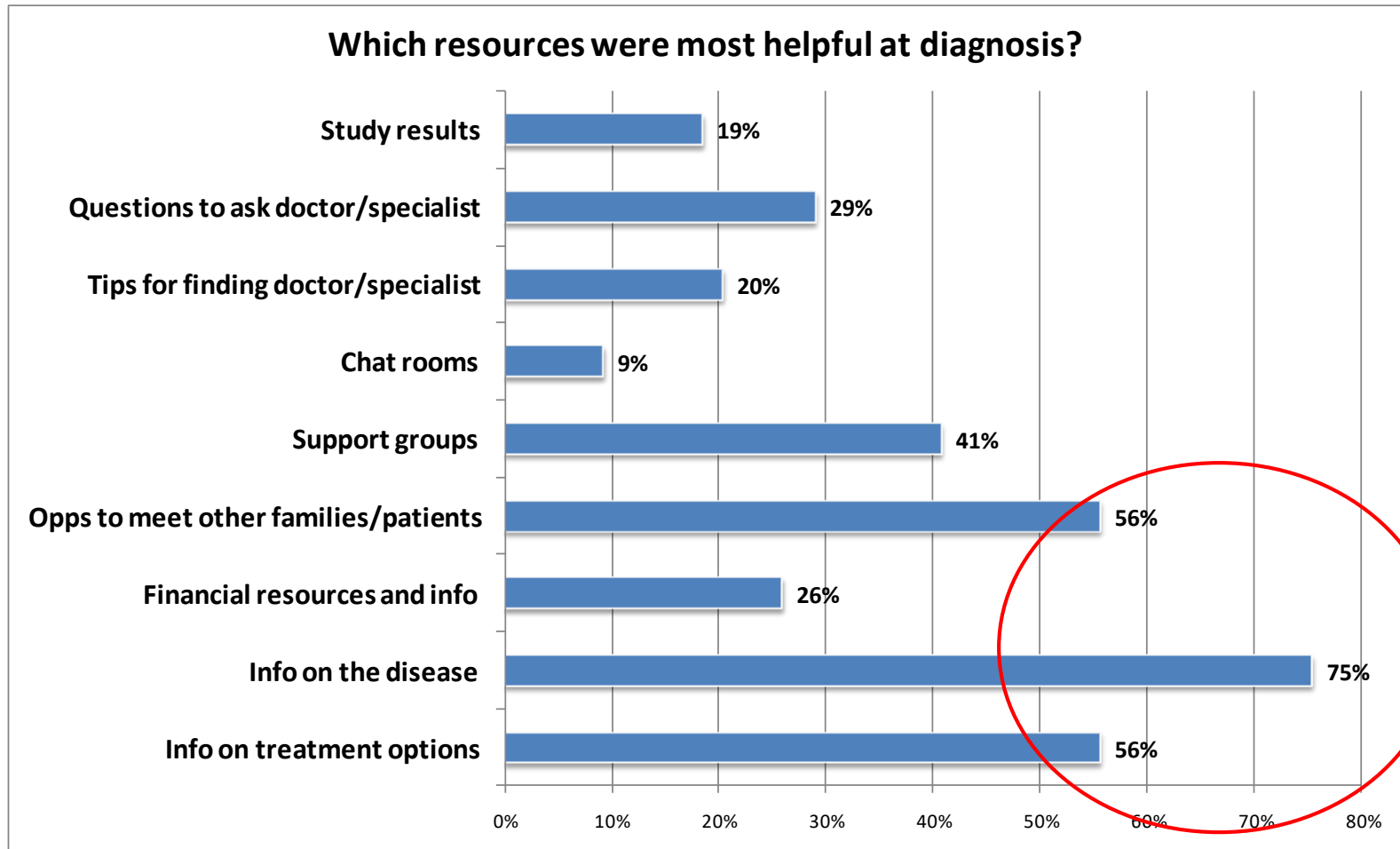


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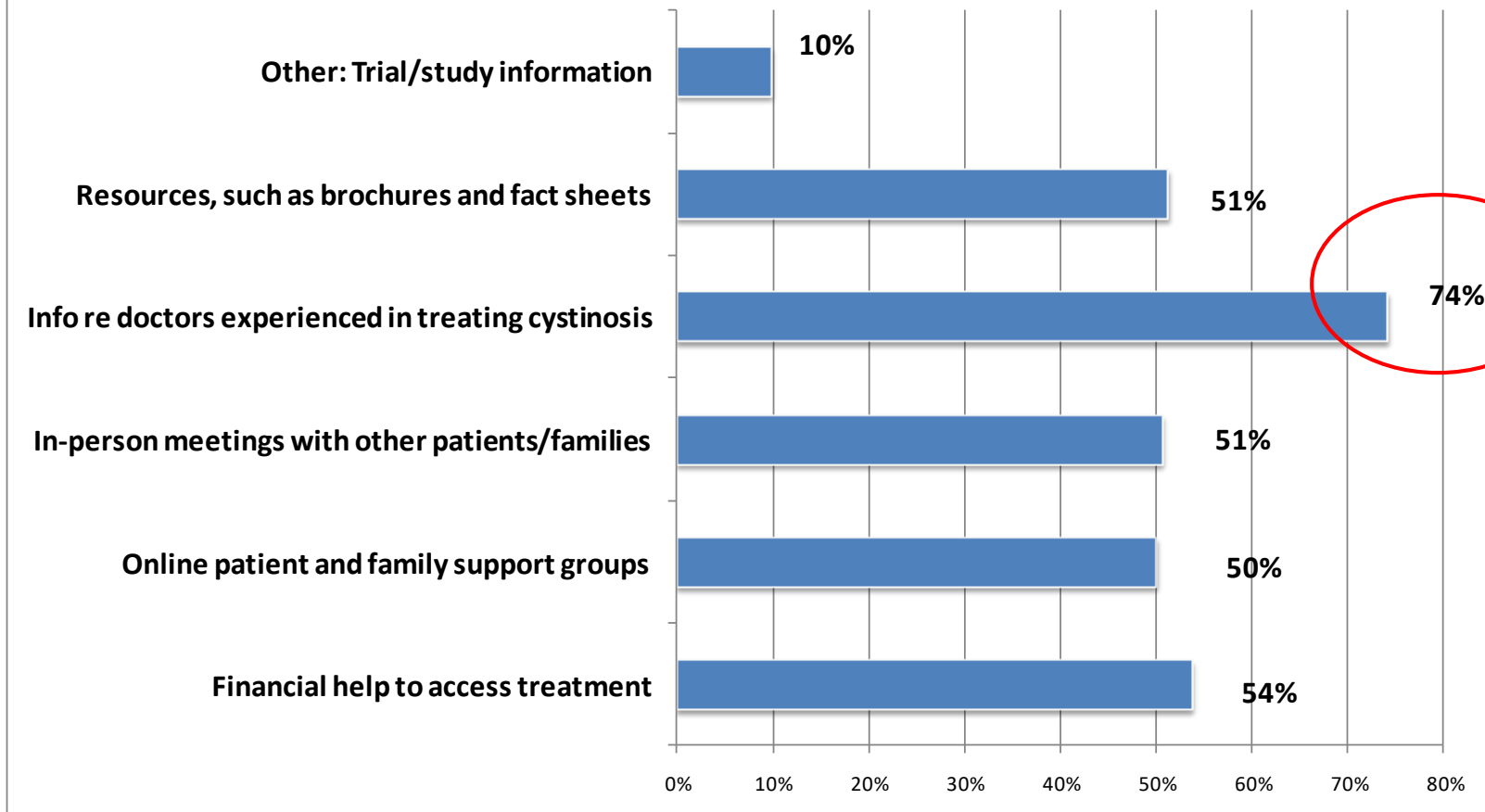
Respondents selected multiple responses.

Which resources were most helpful at diagnosis?



Respondents selected multiple responses.

Which resources would be helpful if they were made available?



Respondents selected multiple responses.

Jones Public Affairs Contact



Please direct any questions to:

Kelly Butterworth

202.591.4052

kelly@jonespa.com