

aHUS Patient Input Program – Wave 1

PRESENTATION OF RESULTS

KEY FINDINGS

Prepared for:



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bylok & associates

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Bylok and Associates has been commissioned by Alexion Canada to implement a multi-wave/multi-year 'Patient Input' Program to better understand the patient pathway related to aHUS, from pre-diagnosis through to treatment and beyond.

Research Objectives

Identify patient experiences and impressions on an **ongoing basis**.

Obtain **regular** feedback with regard to various nodes in the patient pathway.

To understand the Patient Journey from pre-diagnosis to treatment and beyond and identify the changes that occur on a **regular basis**.

Profile experiences related to past and current treatment, and determine familiarity and success with **current and future** treatment options.

Obtain **suggestions** for improving patient pathway experiences.



Methodology

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Patients and Caregivers were recruited from the aHUS Canada membership list to participate in the multi-wave 'aHUS Patient Input Program.'

Social media was also used to recruit participants.

Respondents agreed to take part in telephone WEBEX interviews.

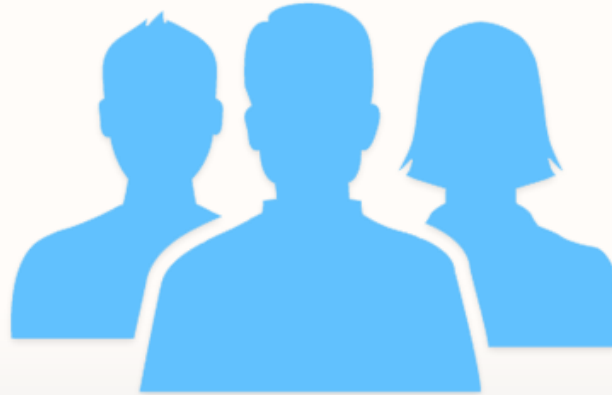
Interviews averaged 50-70 minutes in length and began on August 27, 2014.

A total of 22 respondents (12 patients; 10 caregivers) have participated as of October 17, 2014.

Interviews took place in English and French.

Respondents received an honourarium of \$100.00.





RESPONDENT PROFILE



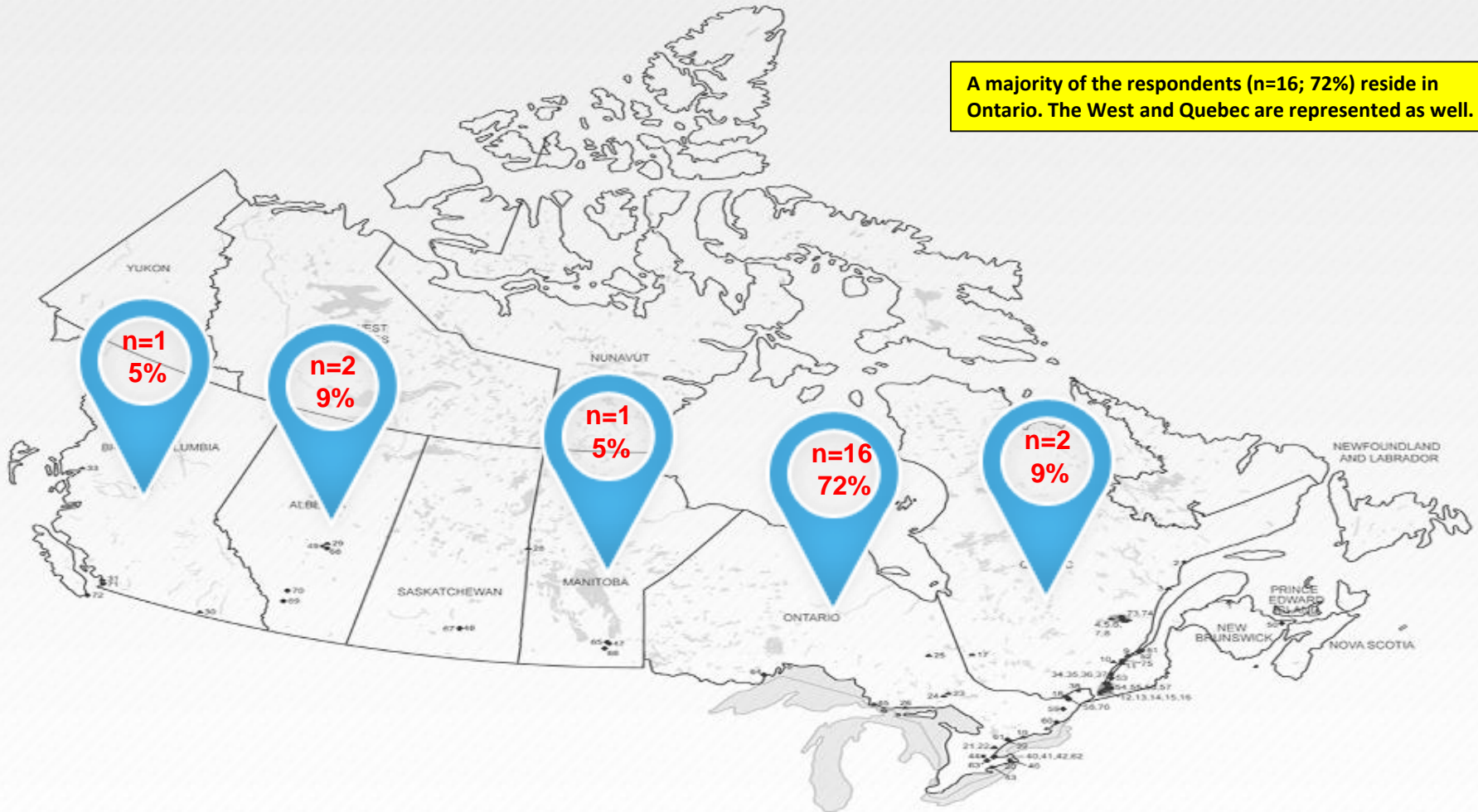
Note: Figures in all Tables/Charts are percentages unless otherwise noted

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Province – Respondents (N=22)



A majority of the respondents (n=16; 72%) reside in Ontario. The West and Quebec are represented as well.

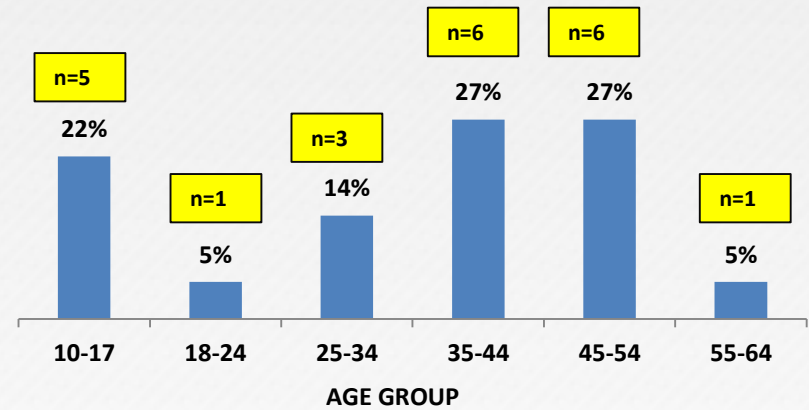


Age



Patient

Average: 35

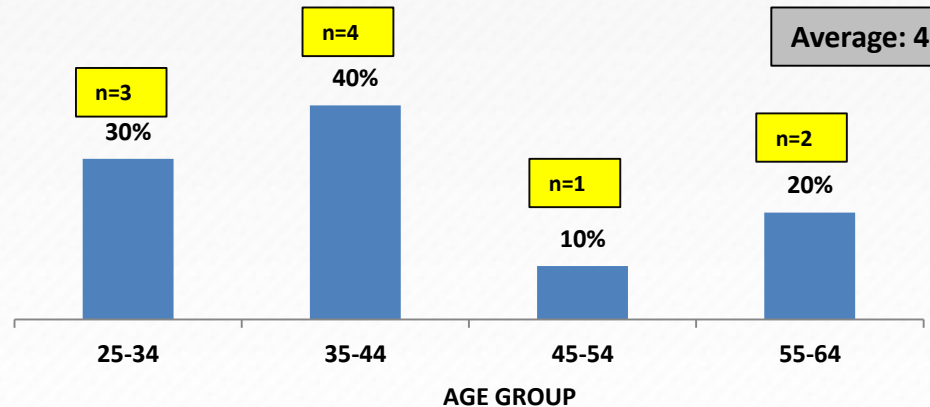


Patients average 35 years of age. Five patients are under 18 years of age while four patients are 18-34 years of age. Twelve patients are 35-54 years of age while one patient is over 54 years of age.



Caregiver

Average: 42



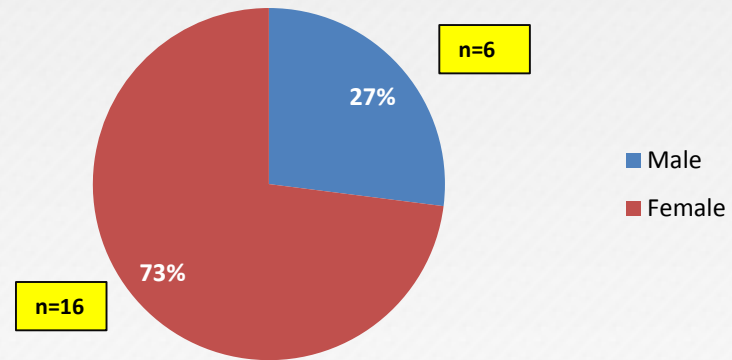
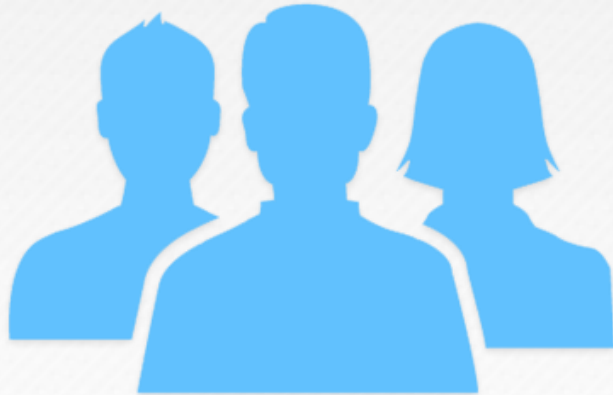
Caregivers average 42 years of age. A majority of the caregivers (n=7; 70%) are 25-44 years of age.

Gender



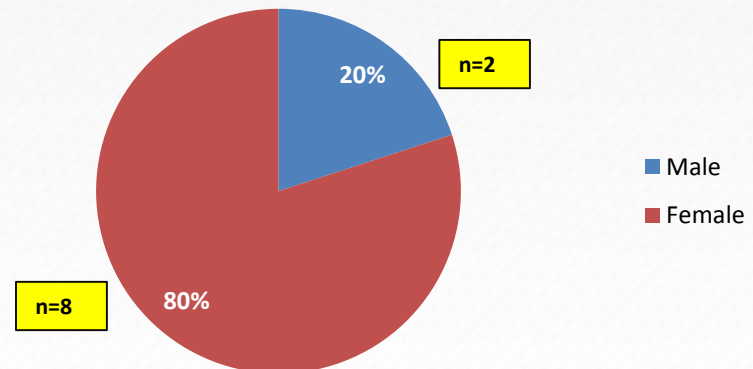
Patient

Most of the patients included in this research are female (n=16; 73%).

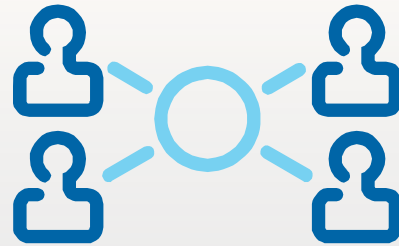


Caregiver

A strong majority of the caregivers participating in this research are female (n=8; 80%).



Key Findings

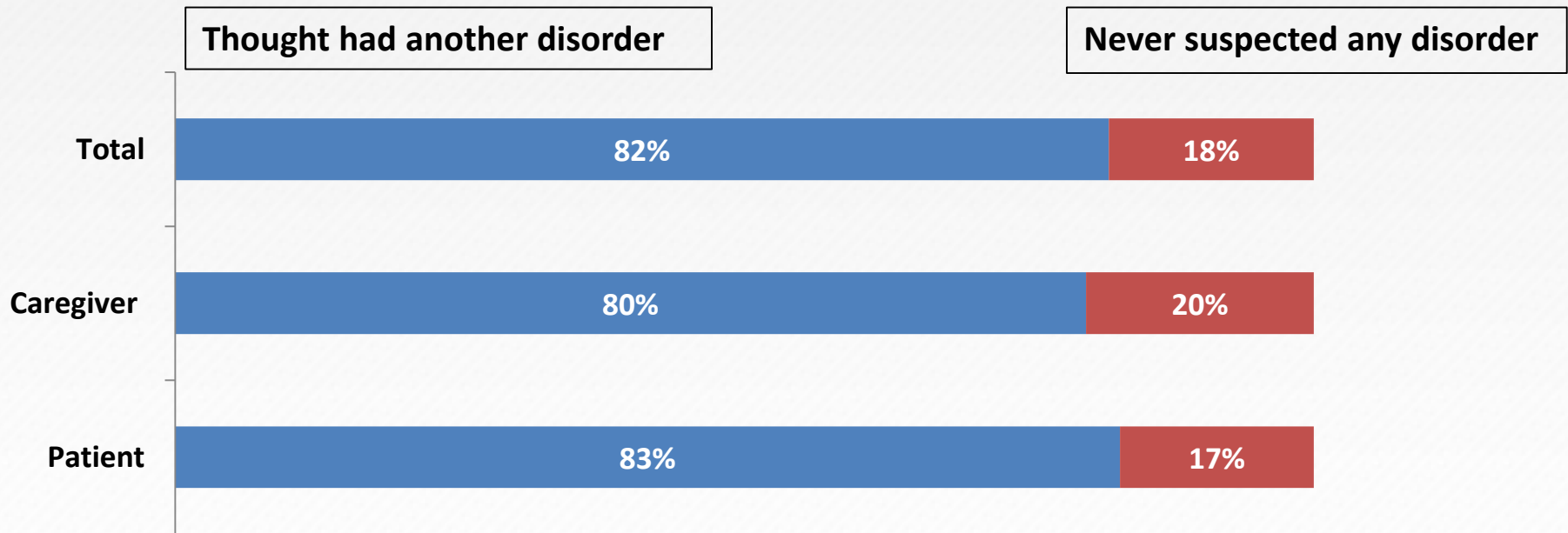


PATIENT JOURNEY

Which of the following statements best describe you prior to being diagnosed with aHUS? AIDED
What symptoms did you experience that caused you to initially go to the doctor? UNAIDED



- Most patients/caregivers suspected another disorder prior to the aHUS diagnosis
- Most common symptoms exhibited initially vary with 'lethargic/fatigue/tired' most prevalent

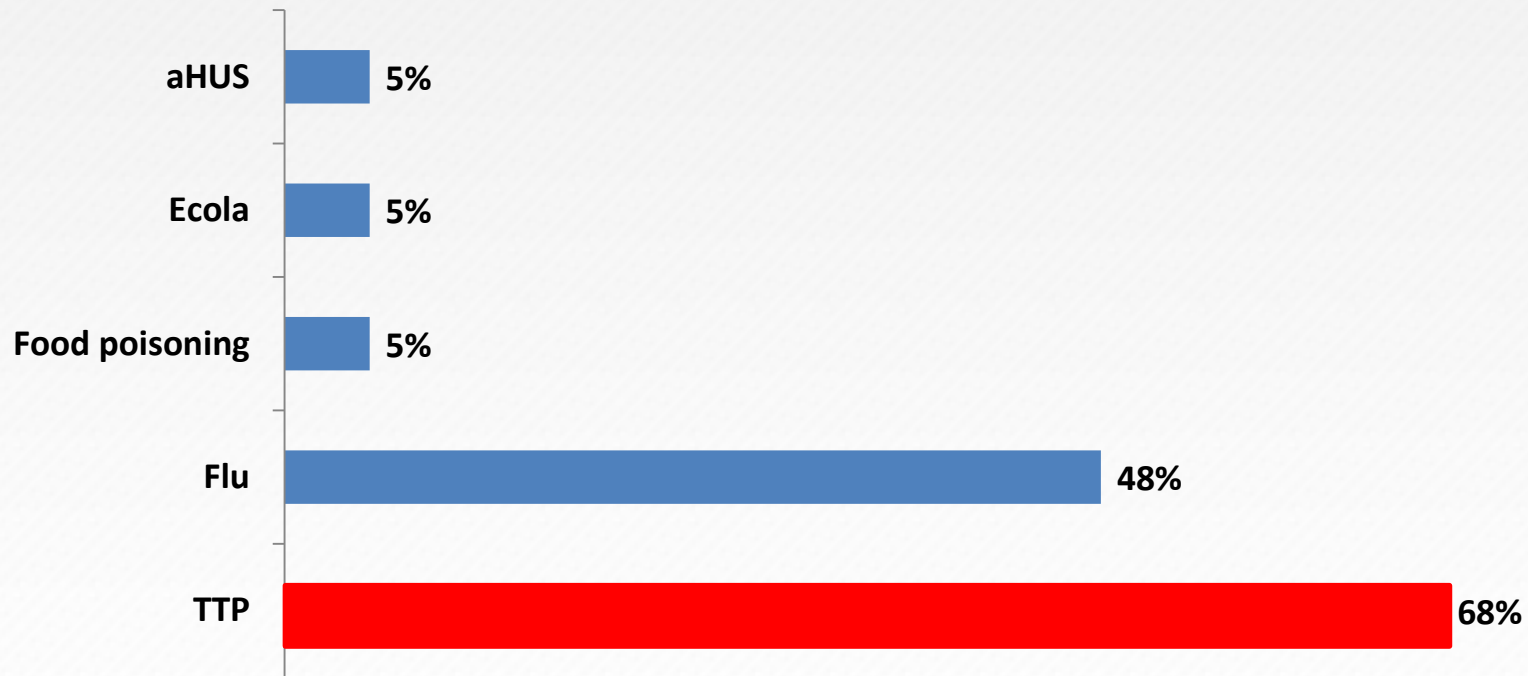


PATIENT JOURNEY



What condition/disorder did you/your doctor suspect you had prior to being diagnosed with aHUS by your doctor? UNAIDED

- In a majority of cases patients were diagnosed with TTP before the correct aHUS diagnosis was made

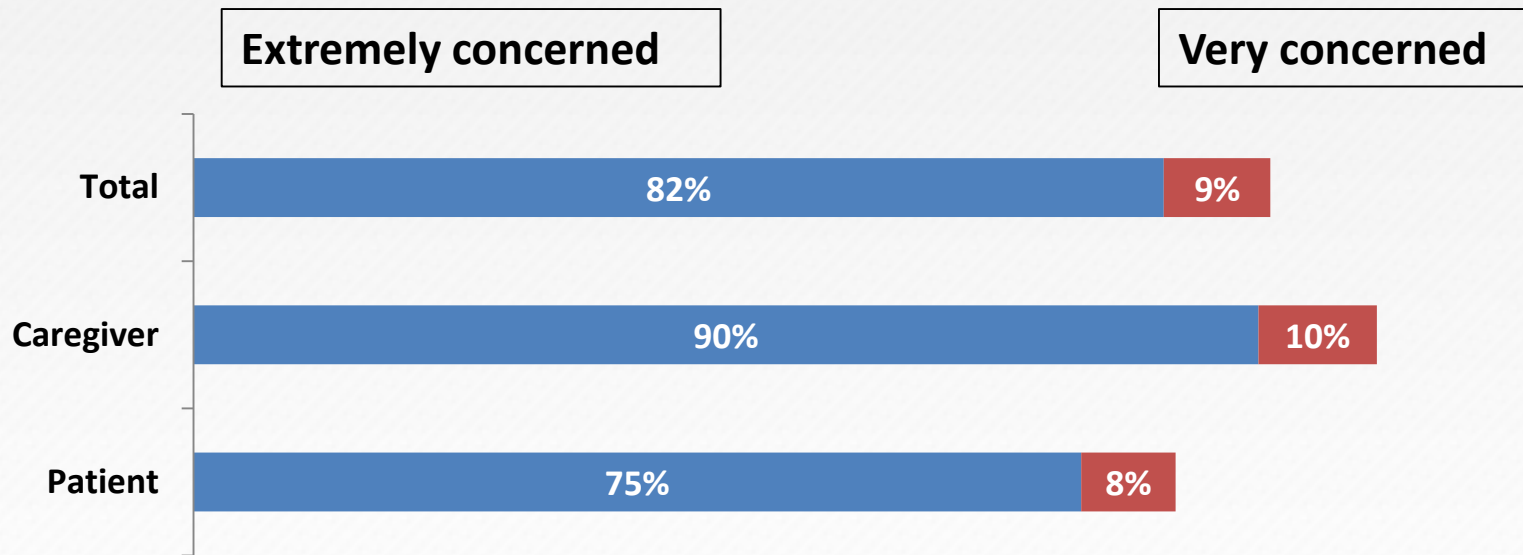


PATIENT JOURNEY



How would you describe your level of concern when you realized you may have aHUS? AIDED

- Patients/Caregivers react very strongly to an aHUS diagnosis
- Their level of concern is 'extremely high'



N=22

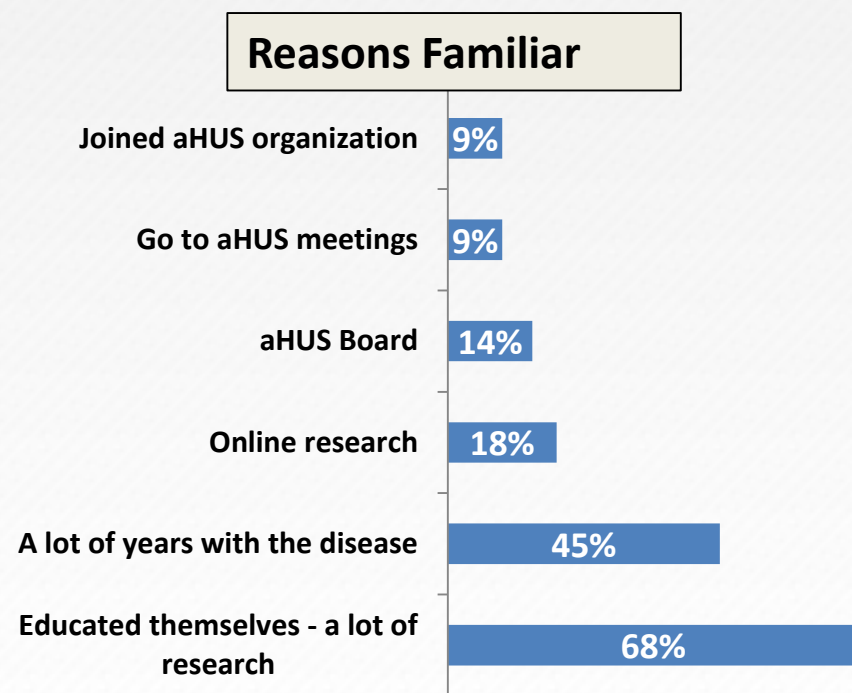
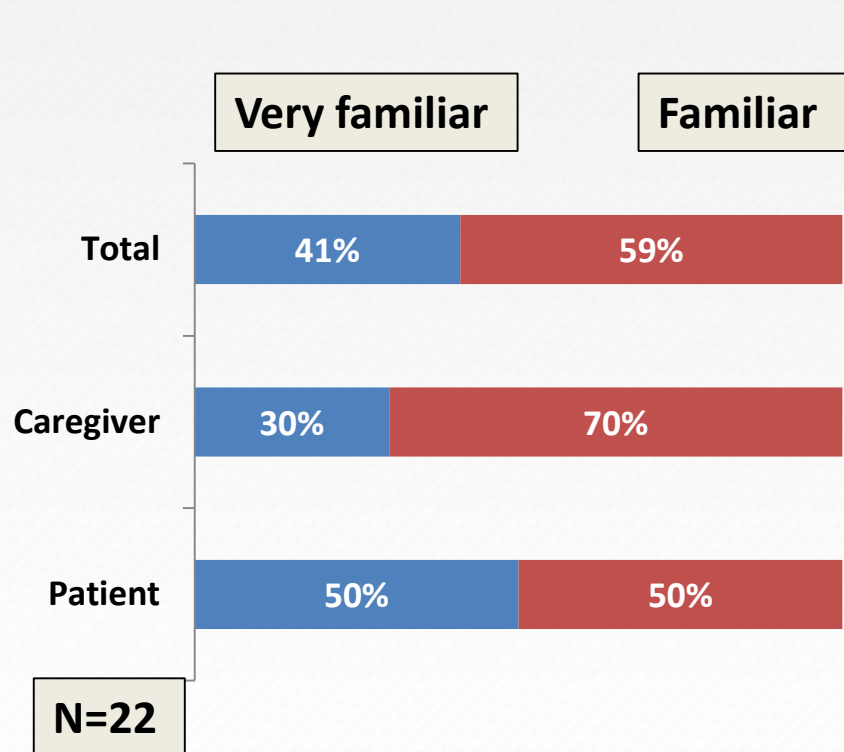
PATIENT JOURNEY



How familiar would you say you are with aHUS and its treatment?

Why are you familiar with aHUS and its treatment? UNAIDED

- Families become (very) familiar with aHUS - in many cases very quickly
- aHUS research amongst these families is high
- Passivity is not an option

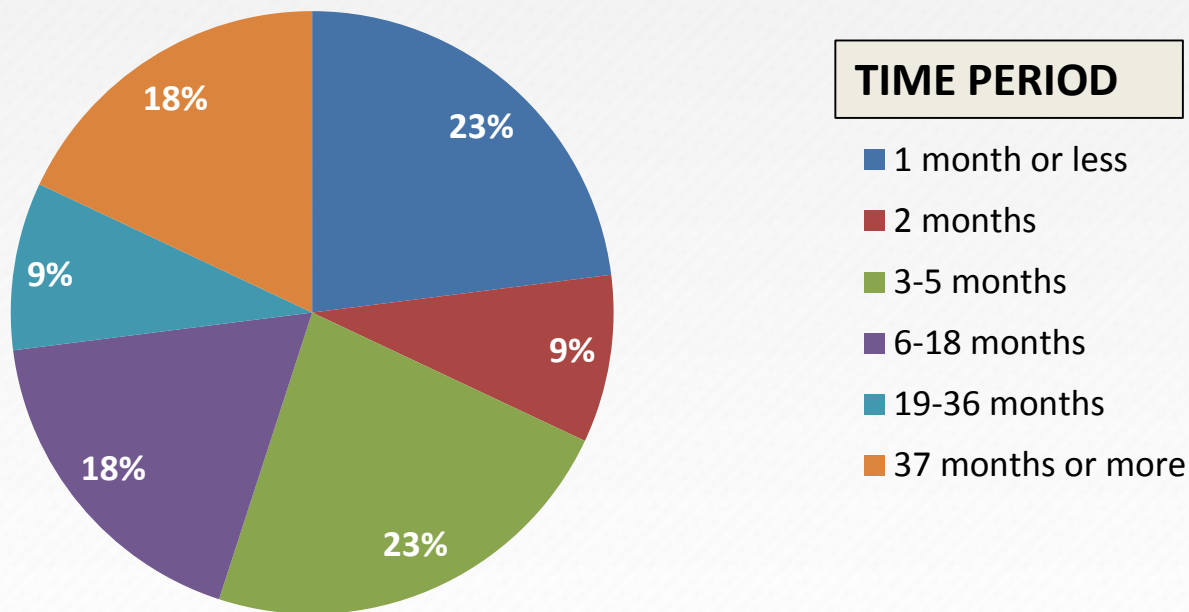


PATIENT JOURNEY



What was the length of time between when you/your doctor first suspected you had a health problem to when you were diagnosed with aHUS? UNAIDED

- The average time between when the doctor first suspected a health problem to when the patient was diagnosed with aHUS is **35 months**



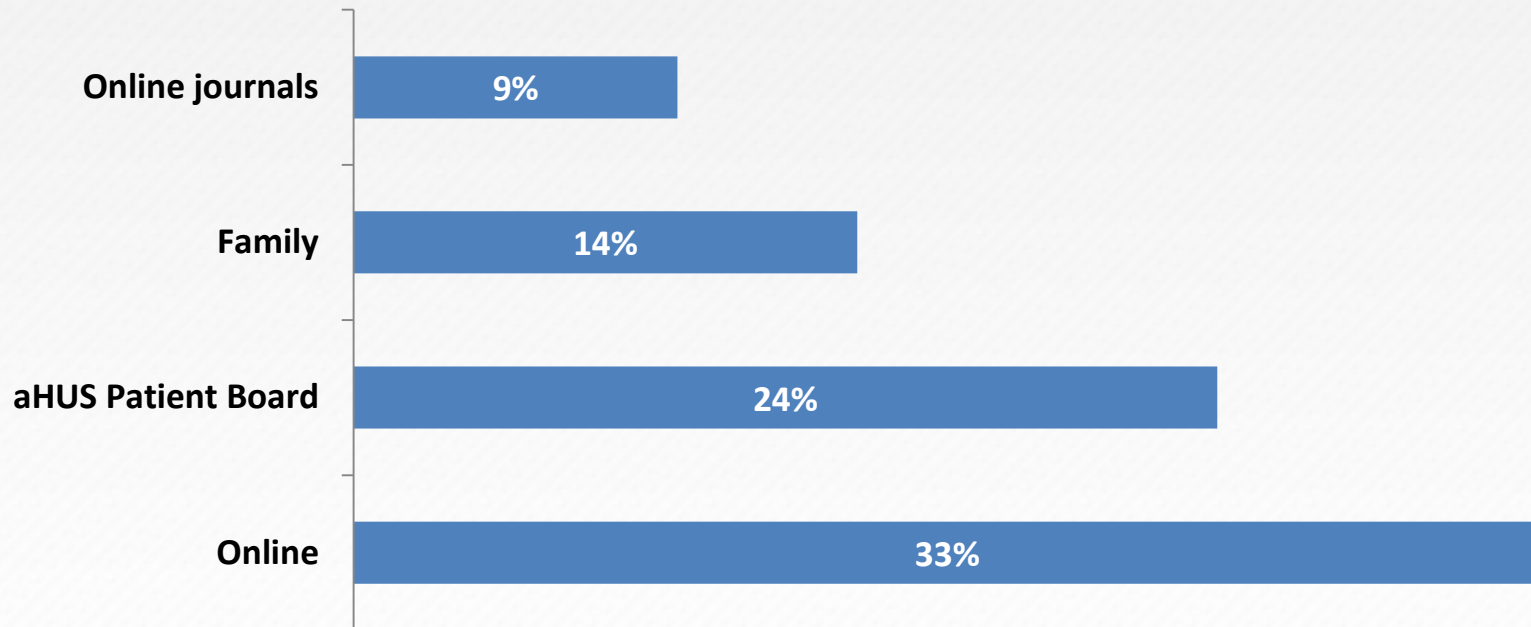
N=22

PATIENT JOURNEY



Which of these resources do you find most useful? UNAIDED

- **Online searches and the aHUS Patient Board are considered to be the two most useful resources available to patients and their families - outside of the medical community**



N=22

PATIENT JOURNEY

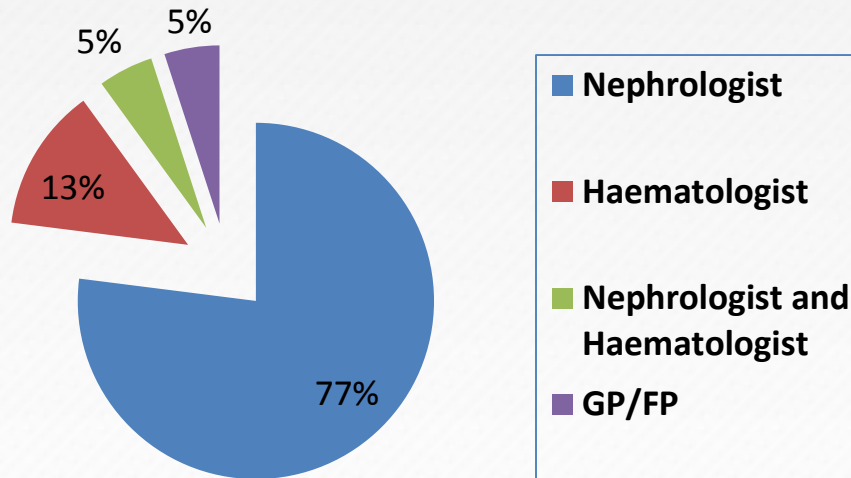
In total, how many doctors (including the doctor who made the diagnosis) did you see before you were diagnosed with aHUS? UNAIDED

What type of doctor made the aHUS diagnosis? UNAIDED



- The average number of doctors seen is **fourteen**
- **Nephrologists** made most of the aHUS diagnoses

Type of Doctor Who Made aHUS Diagnosis



N=22

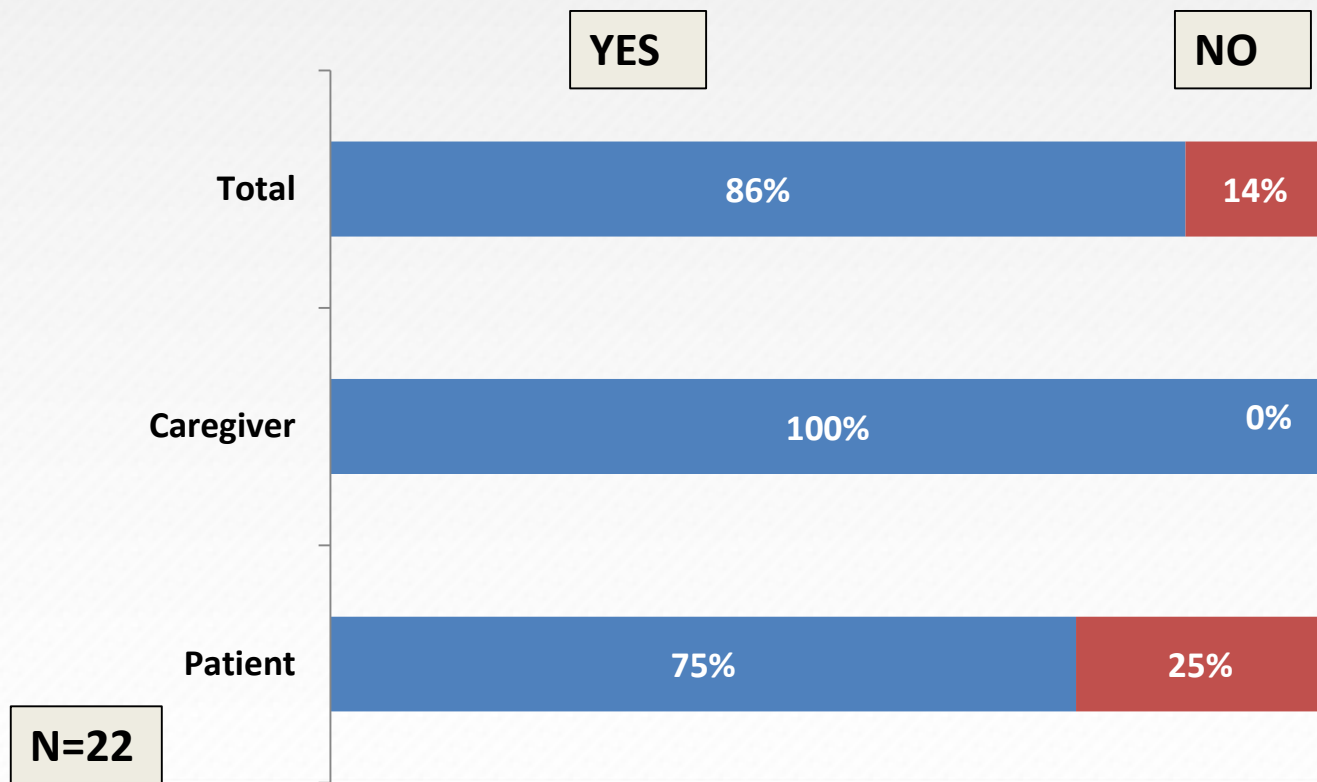
Patient	Number of Doctors Seen
1	5
2	10
3	8-12
4	4-6
5	7-8
6	15-20
7	8
8	2
9	10-15
10	10-15
11	20
12	8-10
13	15-25
14	6-8
15	3-4
16	52
17	4
18	6-8
19	6-7
20	50-60
21	20
22	5

PATIENT JOURNEY



Are you aware that plasma infusion/exchange does not treat the underlying cause of aHUS?

- Most patients/caregivers are aware that plasma infusion/plasma exchange do not treat the underlying cause of aHUS



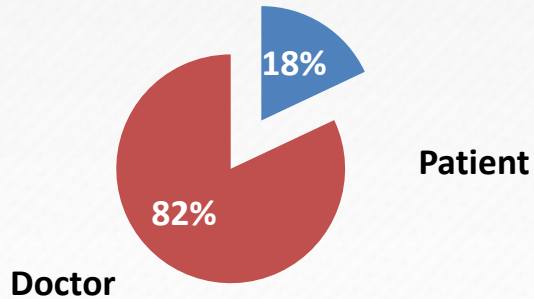
PATIENT JOURNEY



Who initiated conversation about a prescription medication?
Which of the following topics were discussed? AIDED

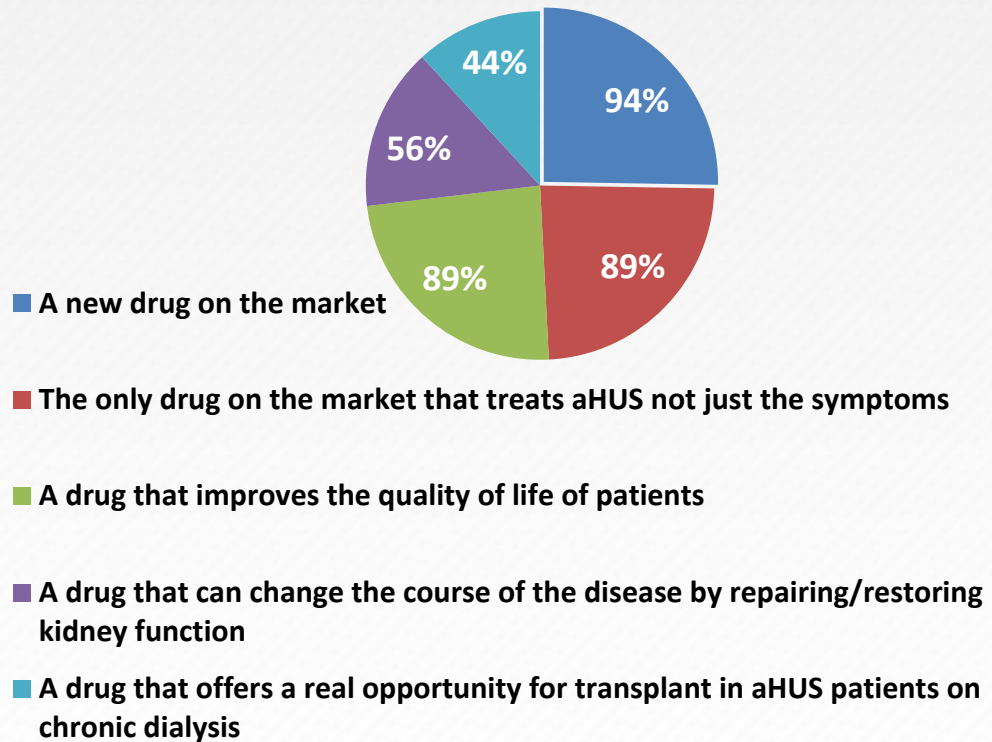
- Doctors generally initiate the conversation about prescription medication

Initiator of Prescription Medication Conversation



N=22

Topics Discussed in Conversation



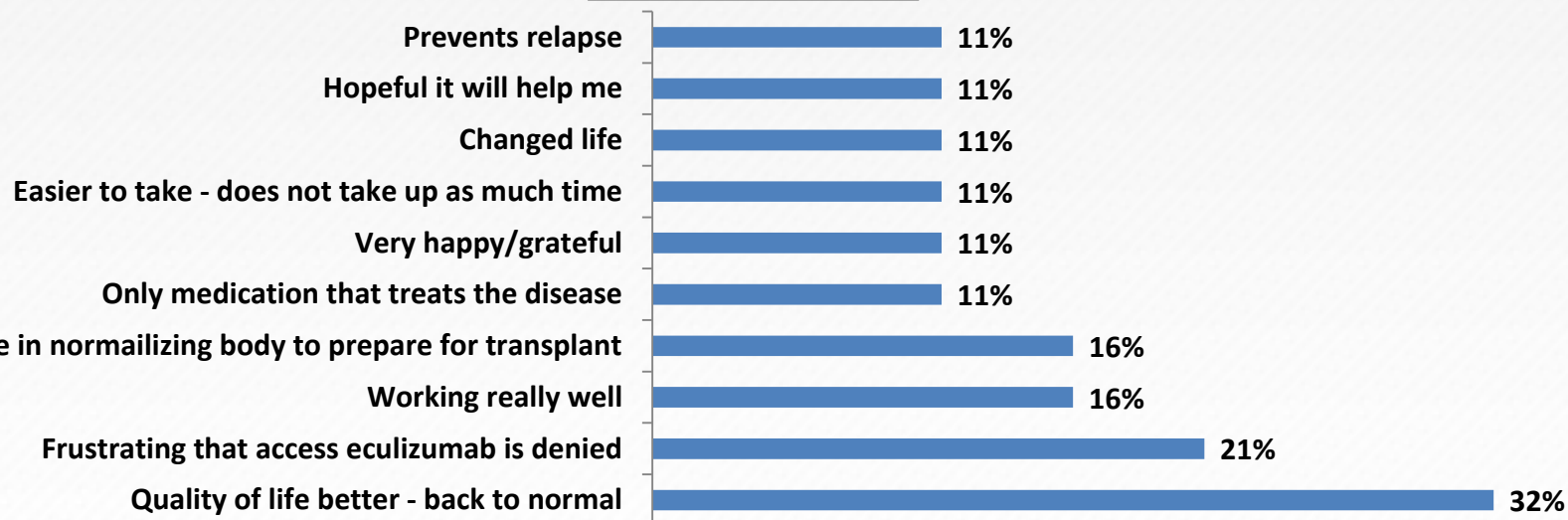
PATIENT JOURNEY



What was your reaction to this prescription medication? AIDED
Why did you react this way? UNAIDED

- **Reaction to eculizumab is ‘very positive’**
- **Patients/caregivers like the fact eculizumab :**
 - ‘offers a better quality of life –chance to get back to normal’,
 - ‘offers only hope in normalizing the body to prepare for a transplant’
 - ‘appears to be working very well (so far)’
- **Patients denied access are frustrated**

Reasons Given



N=22

PATIENT JOURNEY

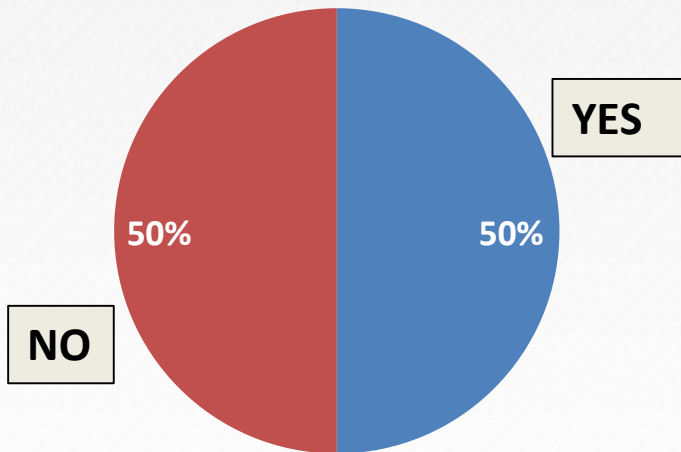


Have you received eculizumab? UNAIDED

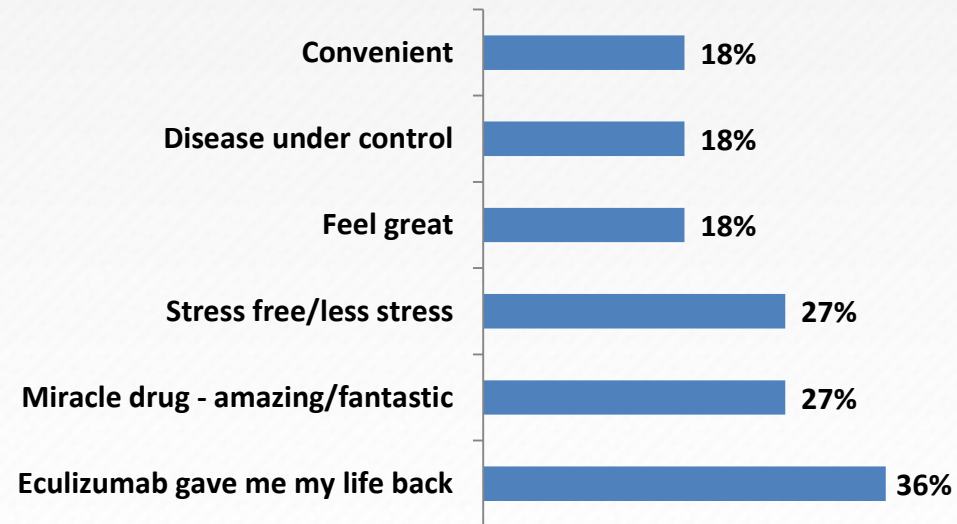
What do you like about your eculizumab treatment? UNAIDED

- 11 of the 22 patients participating in this research program have received eculizumab

Received eculizumab Treatment



KEY LIKES

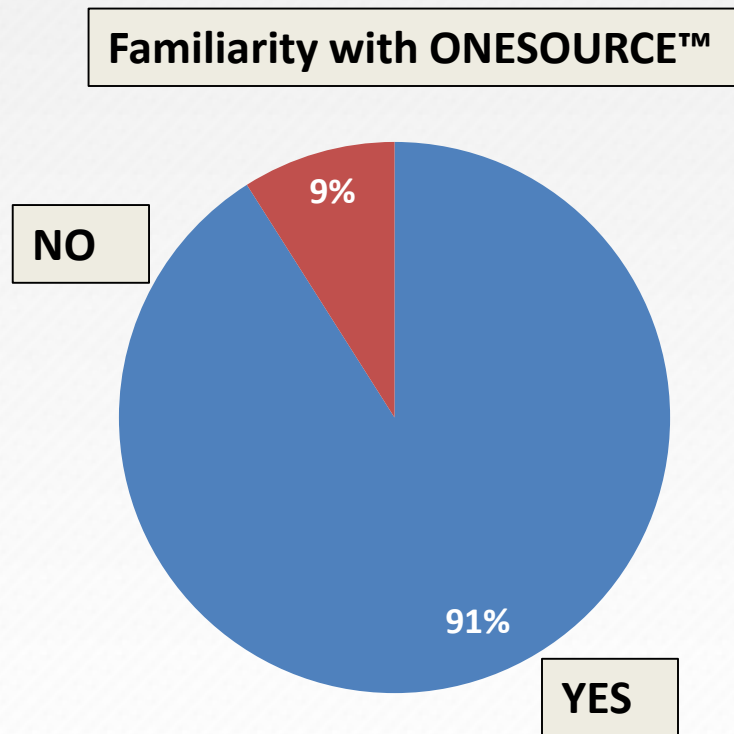


PATIENT JOURNEY



Are you familiar with a patient program called ONESOURCE?

- Most patients/caregivers are aware of ONESOURCE™ (20 of 22)



PATIENT JOURNEY

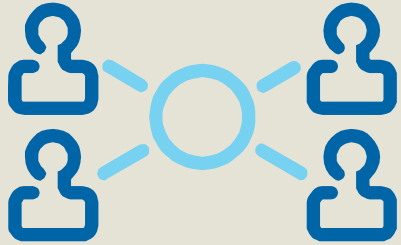


Do you have any suggestions on how the aHUS Community can help aHUS patients? UNAIDED

- The participants in the 'Patient Input' program are very vocal with regard to how the aHUS Community **can help patients**

SUGGESTIONS

Educate doctors/specialists more	45%
Universal access	36%
Stop misdiagnosing - diagnose correctly - faster	27%
Quicker access to funding	27%
More awareness of aHUS	18%
Treat faster	14%
Circulate new information regularly	14%
Ensure all patient cases are available online	9%
Less frequent treatment	9%
Lower price	9%



SUMMARY OF PATIENT JOURNEY

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aHUS Landscape and Journey

TREATMENT

The most common treatment pathway is: plasmapheresis – dialysis – kidney transplant – eculizumab. Currently eculizumab is most likely used as **the third/fourth therapy** in the treatment pathway.

Most patients/caregivers are aware that plasma infusion/plasma exchange **do not treat the underlying cause of aHUS**.

The introduction of prescription treatment – **eculizumab immediately changes the perspective and outlook of these patients** – taking the Patient Journey to a '(more) normal, active and emotionally sound – happy place.
Common feedback includes: '(almost) back to normal' ; 'more active' ; 'more energy' ; 'amazing difference' ; 'relieved – pressure gone – more relaxed' ; 'lot more positive' and 'blessed to have eculizumab'.

Changes in outlook are related to time spent – time put in - becoming more familiar with aHUS, understanding that their is treatment available that has already helped or can help **when access is gained** – many of these patients/caregivers are 'more hopeful' but others have had a very rocky journey without (yet) a 'happy ending' to their journey.

DIAGNOSIS

Patients feel that it takes 'forever' to be correctly diagnosed with aHUS. The average time between when the doctor first suspected a health problem to when the patient was diagnosed with aHUS is **1062 days (just under 3 years)**.

Most patients are **initially diagnosed with TTP**. The average number of **doctors seen is 14** before a correct diagnosis is made.

Patients and caregivers – in fact all family members and even friends – are (all) impacted with some degree of fear initially. The common words used to describe their feelings at the time of the aHUS diagnosis include: '**scared**' ; '**exhausted**' ; '**confused**' and '**awful/terrible**'.

Many of these patients also highlight the fact that 'the lack of information/knowledge they have seen/experienced' adds to their frustration and concern.

PRE-DIAGNOSIS

The Patient Journey is an **emotional rollercoaster** – starting with feelings of disbelief, anger, confusion and denial. The aHUS patients and their families start their Patient Journey not necessarily knowing what they actually have and why they have become ill so fast – after being healthy, active and fit.

The initial visit – in essence the starting point of the 'official' aHUS Patient Journey for a lot of patients - is fraught with **confusion, denial, surprise, and (of course) serious concern**. Many of these patients/caregivers/families are shifted very quickly from a fairly normal and healthy world into a 'scary' world where questions may not be answered or (even worse in many cases) take very long – too long – to be answered.

