

aHUS Patient Input Program – Wave 2

KEY FINDINGS - FINAL

Prepared for:



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June 6, 2016

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.

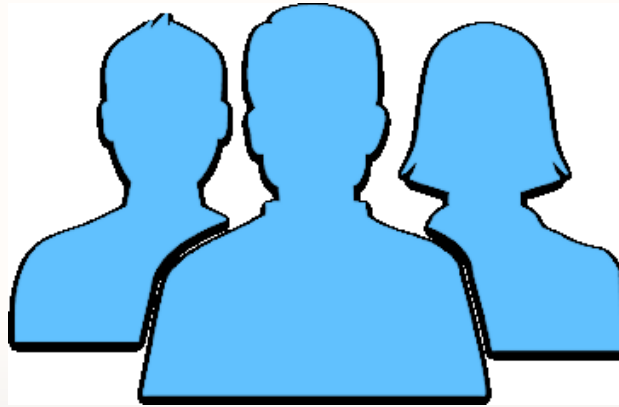
Respondents agreed to take part in telephone WEBEX interviews.

Interviews averaged 50-90 minutes in length and began on June 17, 2015 and ended on October 18, 2015

A total of 32 Canadian respondents (15 patients; 17 caregivers) have participated in the second wave of interviews as of October 26, 2015

Twelve of these respondents (8 caregivers; 4 patients) are new to the Program

Interviews took place in English and French. Respondents received an honourarium of \$100.00 CDN.



CANADIAN RESPONDENT PROFILE



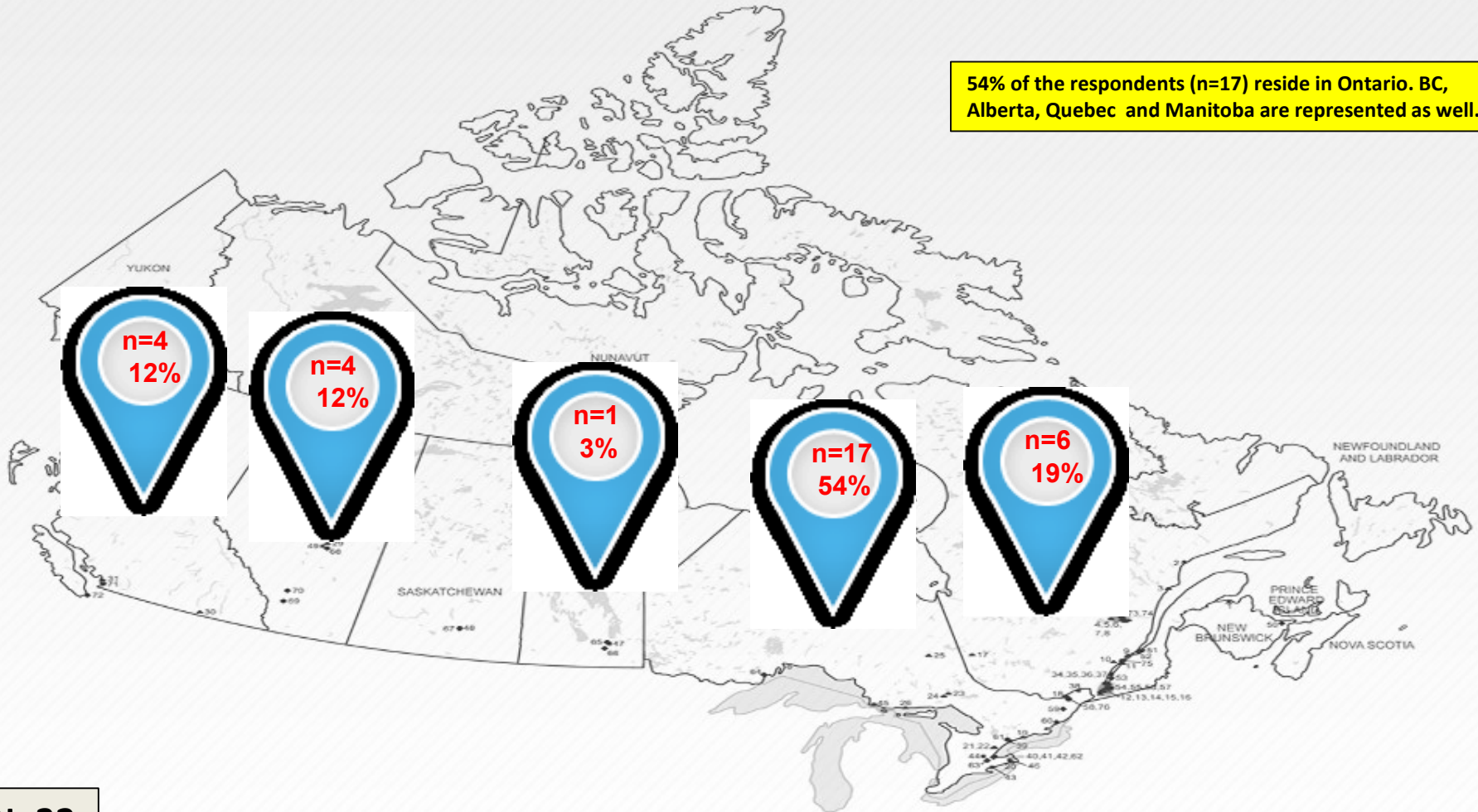
Note: Figures in all Tables/Charts are percentages unless otherwise noted

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Province



54% of the respondents (n=17) reside in Ontario. BC, Alberta, Quebec and Manitoba are represented as well.



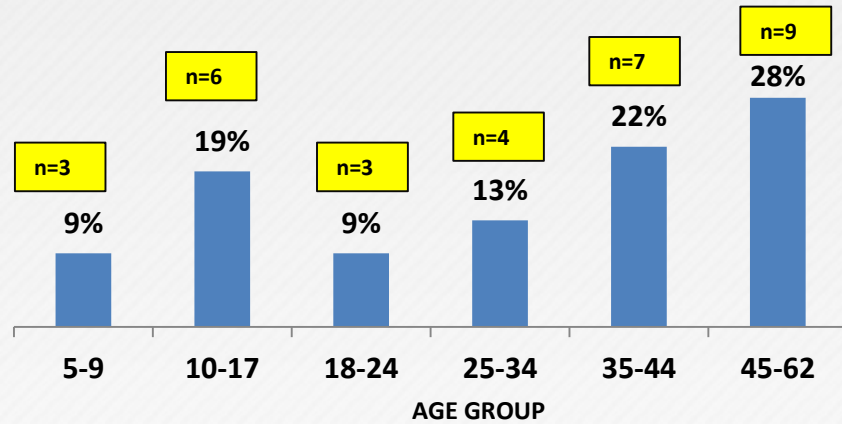
N=32

Age

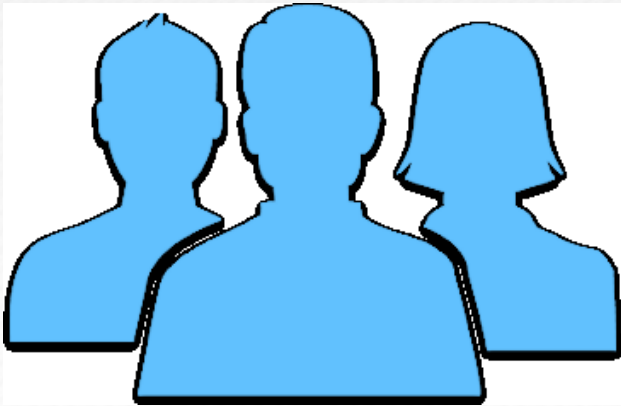


Patient

Average: 32



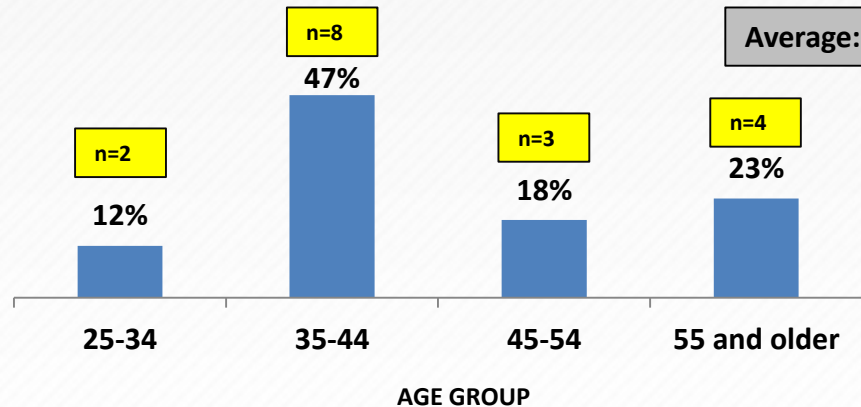
Patients average 32 years of age. Nine patients are under 18 years of age while seven patients are 18-34 years of age. Sixteen patients are 35-62 years of age.



Caregivers average 44 years of age. A majority of the caregivers (n=10; 59%) are 25-44 years of age.

Caregiver

Average: 44

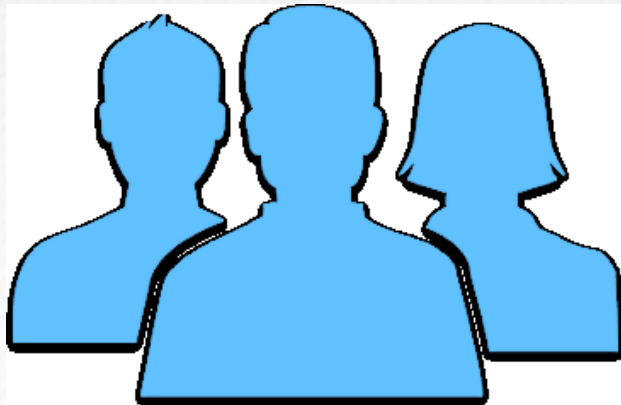


N=32

Gender



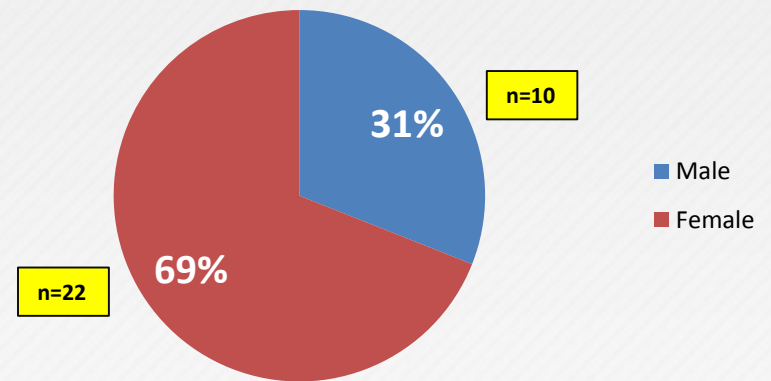
A majority of the patients included in this research are female (n=22; 69%).



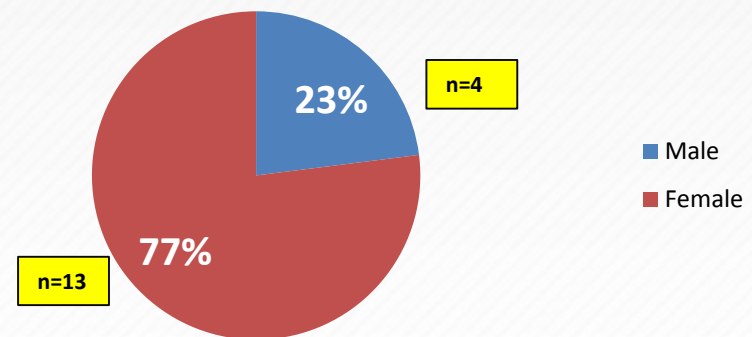
A strong majority of the caregivers participating in this research are female (n=13; 77%).

N=32

Patient



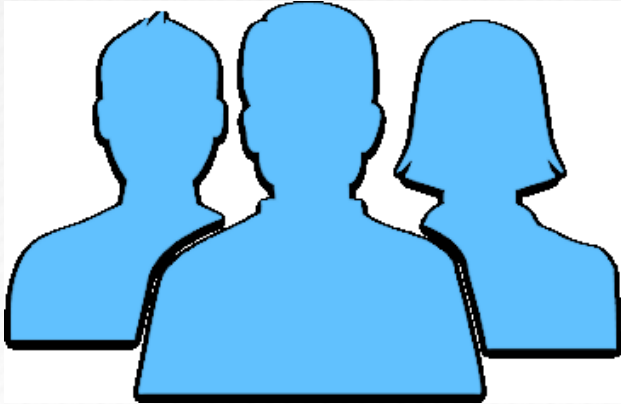
Caregiver



Hospital/Insurance



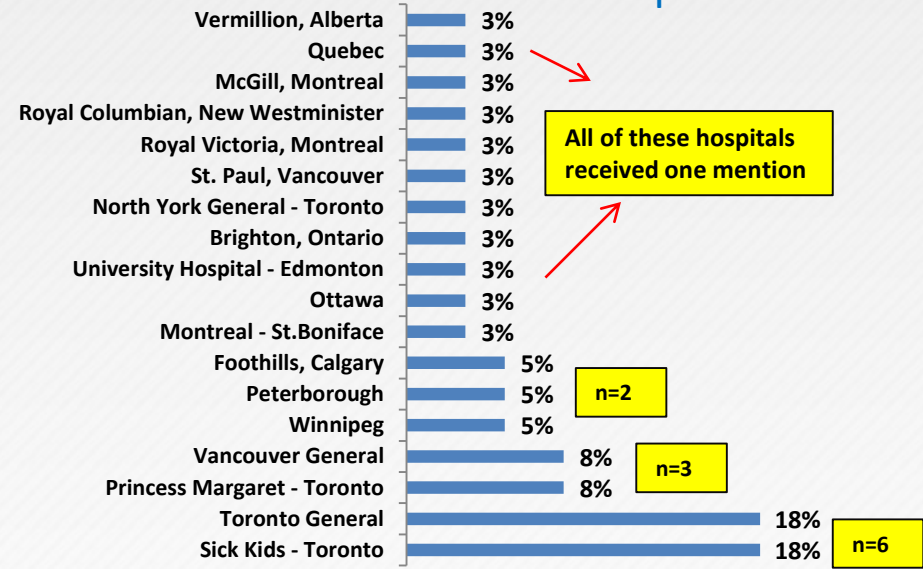
Sick Kids, Toronto General and Princess Margaret are the primary care hospitals in Ontario. Vancouver General is the likely care facility in BC.



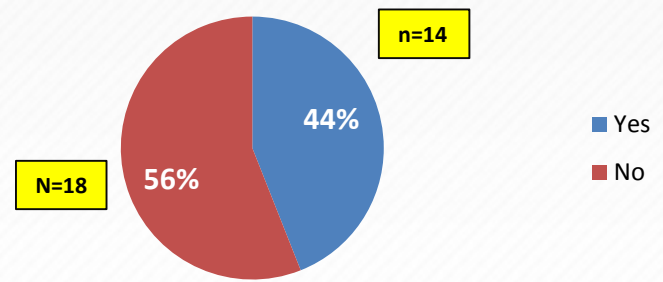
Just under half of the patients (n=15) have a private insurance plan.
 Carriers include: Manulife (N=3); GWL (N=3); Co-op (N=2) and ODSP (N=1)

N=32

Hospital

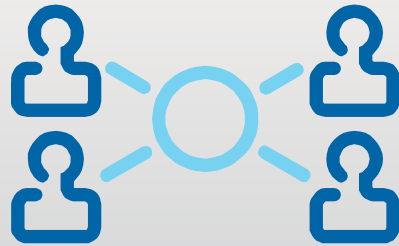


Insurance



Key Findings

Patient Feedback



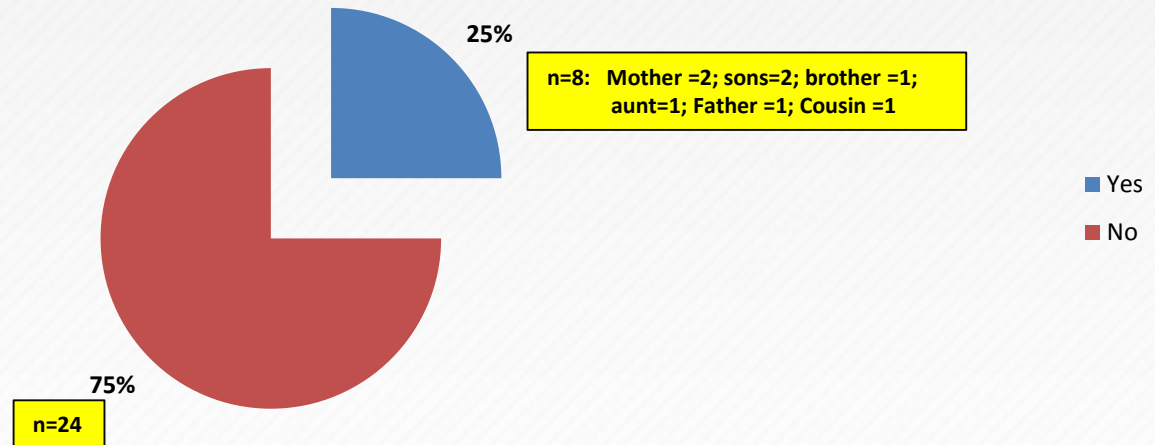
PATIENT JOURNEY

Anyone else in the family with aHUS?



- Most aHUS patients/caregivers (24 of 32) stated that no one else in their family has been diagnosed with aHUS

Incidence of Other Family Members Having aHUS



N=32

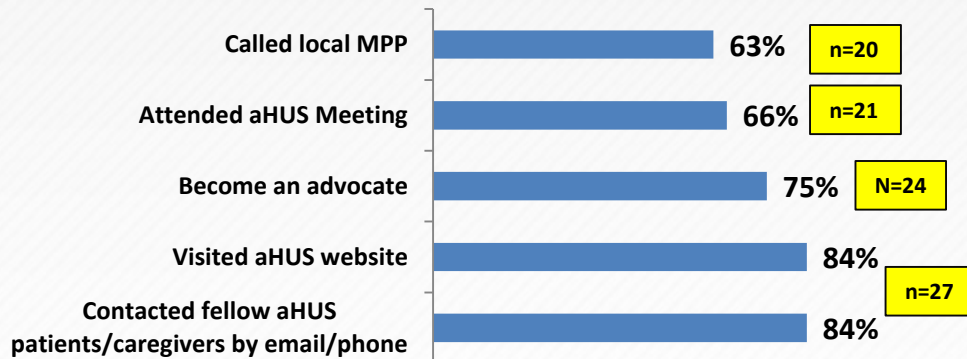
PATIENT JOURNEY

Which of the following activities have you done in the last three years? AIDED
Perceived success of these activities so far?

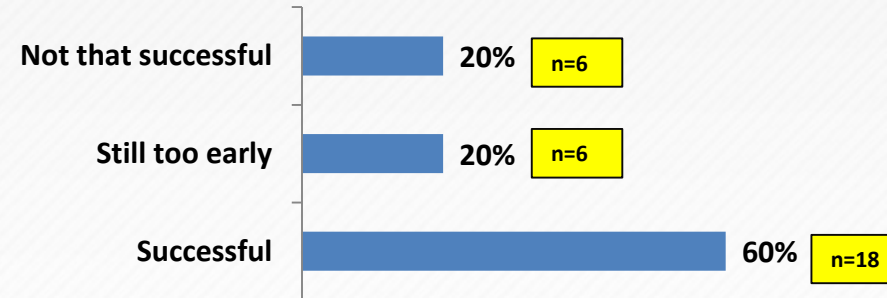


- Most patients/caregivers are **very active** and involved in their aHUS situation and community
- A majority of these patients consider their efforts (in most cases) to have some positive impact (particularly in Ontario) but feel there is 'still a long way to go' before all aHUS patients can gain access quickly

Activities



Perceived Success of Activities (N=30)



NOTE: 2 respondents were very early in the process and had not yet become involved

N=32

PATIENT JOURNEY

Please think of all the doctors you have seen since your aHUS journey began to when you were diagnosed with aHUS, how many were nephrologists, haematologists, transplant surgeons, GP/FPs, emergency physicians and other types of specialists?



- Patients have seen an average of **14 doctors** since their journey began
- An average of **6 nephrologists**, 3 haematologists, 1 GP/Paediatrician, 1 transplant surgeon, 1 emergency physician and 2 other specialists are seen during patient journeys

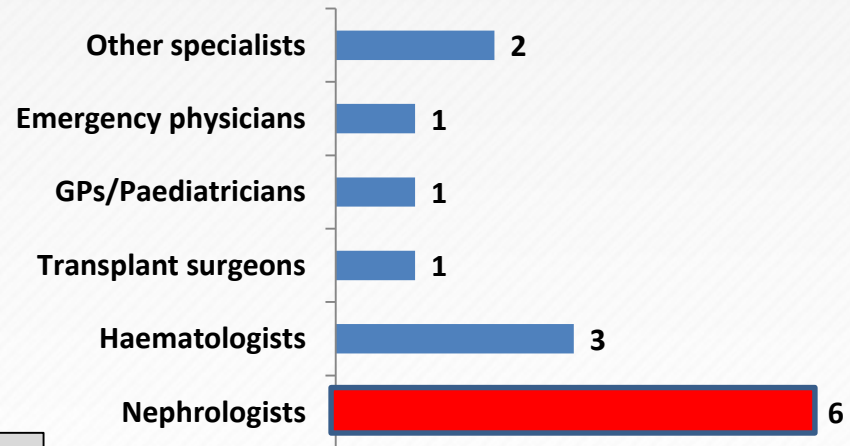
Number of Doctors Seen to aHUS Diagnosis

3	12
50	8
15	18
20	20
8	16
15	8
20	20
22	20
3	27
45	5
9	9
11	3
9	3
9	8
4	16
4	4

N=32

**AVERAGE
TOTAL: 14**

Number of Different Types of Doctors Seen to aHUS Diagnosis



PATIENT JOURNEY

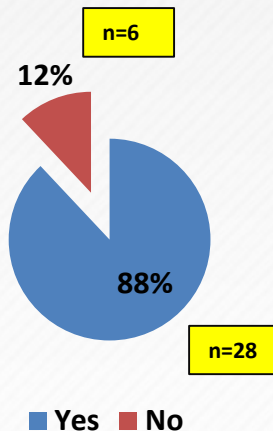


Have you had any issues/problems with plasmapheresis?

What issues, problems have you had? UNAIDED

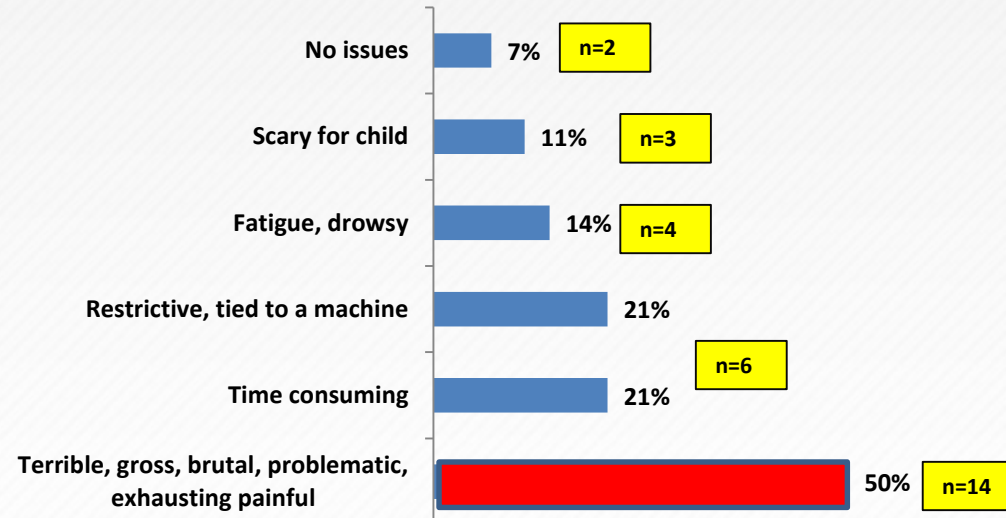
- Almost all the patients have had plasmapheresis (28 of 32)
- All but two of these patients consider the plasmapheresis treatment to be problematic
- Key dislikes include: **'terrible', 'brutal', 'exhausting', 'time consuming', 'scary' and 'gross'**

Whether had Plasmapheresis Treatment



N=32

Issues with Plasmapheresis (N=28)



PATIENT JOURNEY

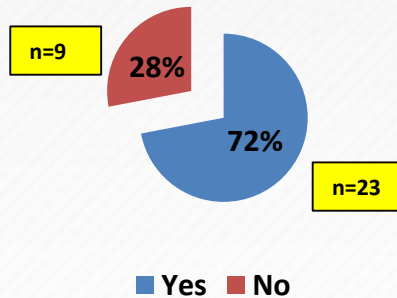


Have you had any issues/problems with dialysis?

What issues, problems have you had? UNAIDED

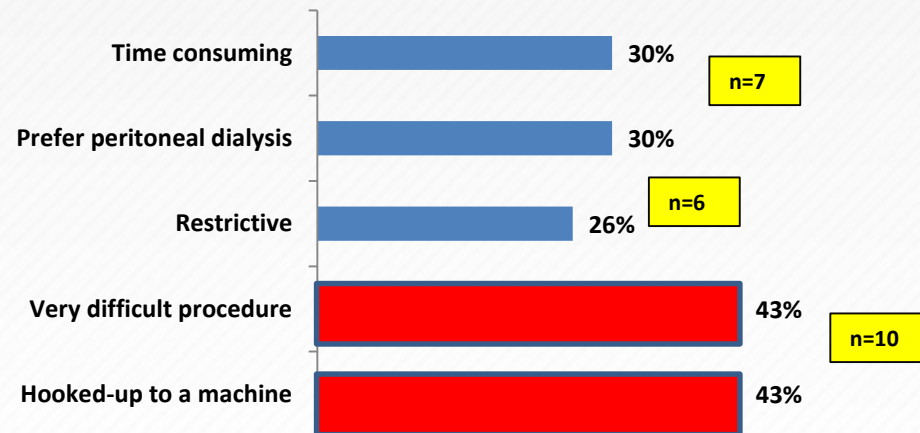
- Most patients have had dialysis (23 of 32)
- These procedures are (however) considered to be **'very difficult'** , **'restrictive'** – **'hooked up to a machine for long periods of time'**

Whether had Dialysis Treatment



N=32

Issues with Dialysis N=23



PATIENT JOURNEY

How has Soliris (eculizumab) changed your life? UNAIDED
How life changed? UNAIDED
Reactions to Soliris (eculizumab)?

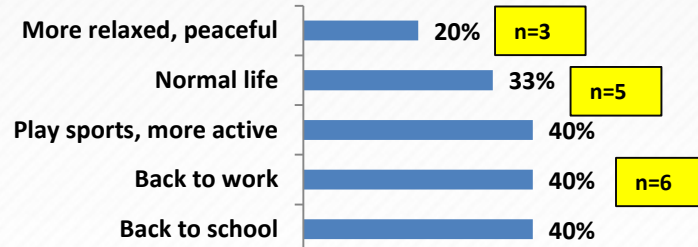


- All the patients on eculizumab (N=15) consider their treatment to be **'life changing/restoring'**
- Eculizumab allows these patients and their families to return to a state of 'normalcy' – 'going back to school', 'going back to work', 'playing sports' – 'being active again'
- Their lives have been changed for the better

Whether life has changed for the better since eculizumab introduced

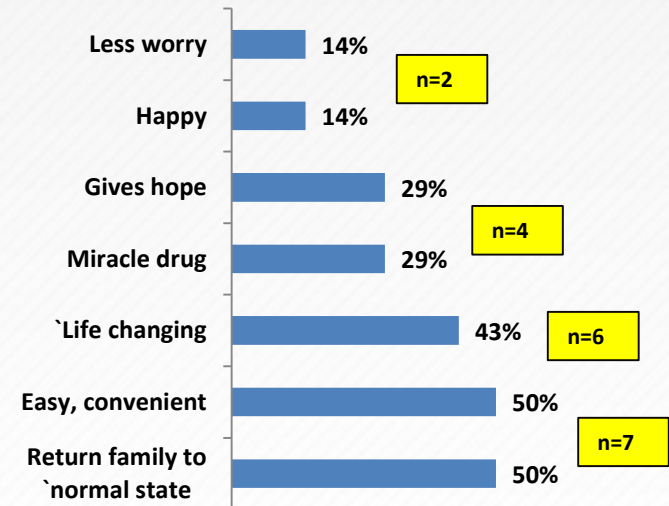
YES – 100%

How Life Changed



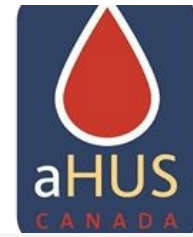
N=15

Reaction to eculizumab



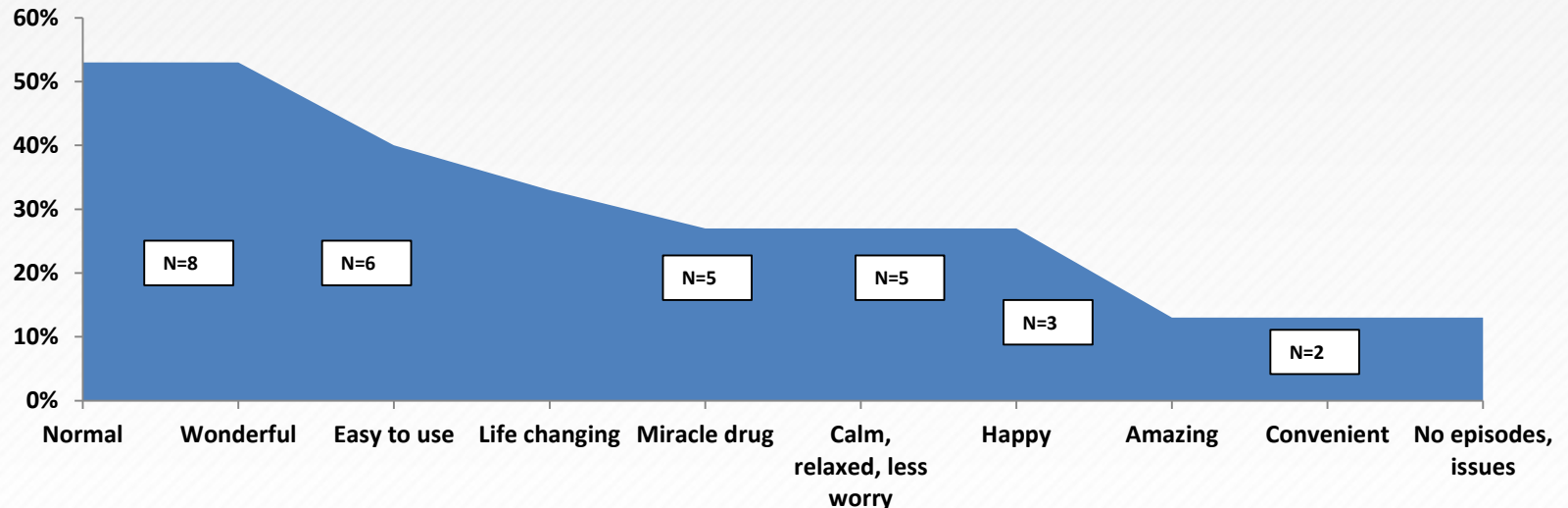
PATIENT JOURNEY

What words would you use to describe your Soliris (eculizumab) treatment?



- Soliris (eculizumab) is associated with **normalcy** – a return (thankfully) to that state – a means for patients/families to ‘move on –escape’ from a world they never wanted/imagined they would enter
- Key words used to describe the impact of eculizumab treatment include: **‘wonderful’, ‘easy to use’, ‘life changing’, ‘miracle drug’, ‘calm, relaxed, no-less worry’ and ‘convenient’**

Words used to describe the impact of Soliris (eculizumab) treatment



N=15

PATIENT JOURNEY

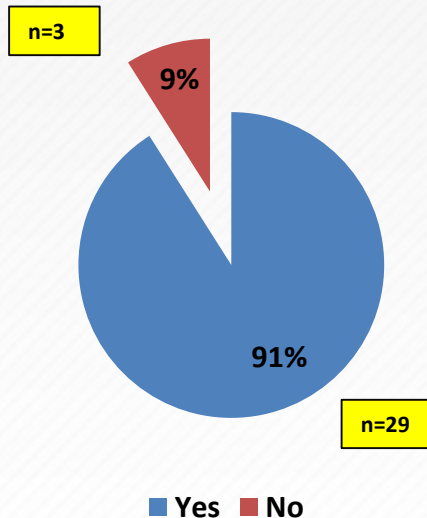


Are you familiar with a patient support program called ONESOURCE?

Has ONESOURCE helped with your reimbursement, that is, trying to help you gain access to eculizumab through private and public payers?

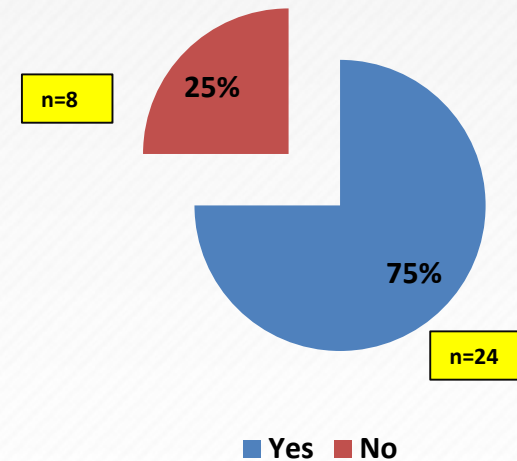
- Almost all the respondents (29 of 32) are **aware** of the patient support program – ONESOURCE
- Most patients/caregivers are **aware** that ONESOURCE helps with reimbursement – the paperwork required to apply for – gain access to eculizumab

Awareness of ONESOURCE



N=32

Awareness that ONESOURCE helps with reimbursement



PATIENT JOURNEY

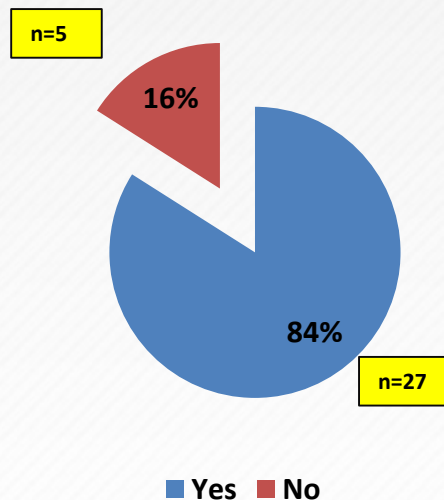


Do you keep up to date with new clinical data related to aHUS and its treatment?

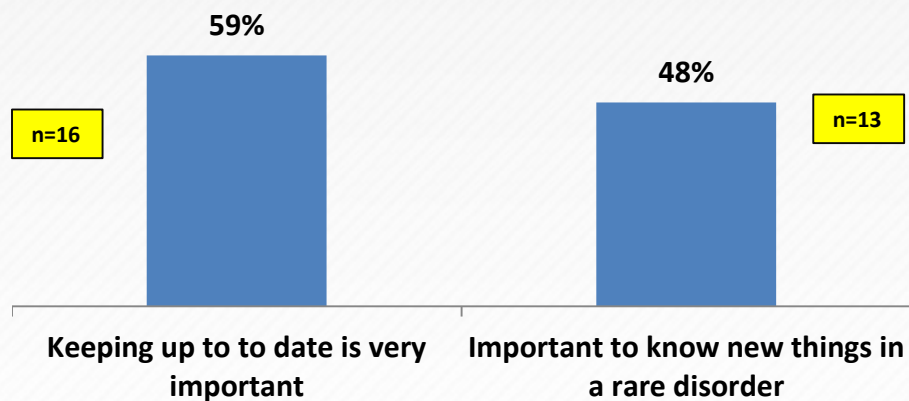
Why do you keep up to date with new clinical data related to aHUS and its treatment? UNAIDED

- Most patients/caregivers stay up to date with **new** clinical data related to aHUS
- These patients strongly believe that it is important 'to keep up to date' in a rare and 'new' disorder

Whether keep up to date with new clinical data



Reasons for keeping up to date with new clinical data (N=27)



N=32