

Survey for aHUS Adult Patients or Caregivers of Pediatric aHUS Patients

*(While * marks required fields, providing complete answers to all survey questions will provide data that offers a fuller understanding of needs, circumstances, and important issues that face aHUS patients and caregivers.)*

Section 1: About the Person Filling in the Survey

1 The person filing in this survey is: *

- A person living with atypical HUS
- A parent/guardian of a person living with atypical HUS
- A partner/caregiver of an person living with aHUS

2 Gender of Person filing in form *

- Male
- Female

3 Age of Person filling in the form (in years) *

- Under 20
- 21 - 30
- 31 - 40
- 41 - 50
- 51 - 60
- 61 - 70
- 71+

Section 2: About the Person living with aHUS

4 Age of the Patient in years *

- 0 - 2
- 3 - 5
- 6 - 10
- 11 - 20
- 21 - 30
- 31 - 40
- 41 - 50
- 51 - 60
- 61 - 70
- 71+

5 Gender of Patient *

- Male
- Female

6 Country of Residence of Patient *

7 Has genetic testing been done? *

- Yes, results are back
- Yes, but results still pending
- No
- No, but other family member(s) tested

- Not sure

8 If yes, what was the predisposing genetic cause of aHUS, as noted in the test results? *

- CFH (Factor H)
- CFI (Factor I)
- CFB (Factor B)
- MCP (CD 46)
- C3
- THBD (Thrombomodulin)
- DGKE
- Anti-factor H Autoantibodies
- CFHR1 and CFHR3 deletions
- Results still pending
- Inconclusive/Genetic Cause not Known
- Multiple mutations found (specify)
- Other

9 If genetic testing was done, how long did it take for you to receive results? *

- Less than 1 month
- 1 month or more but less than 3 months
- 3 months or more but less than 6 months
- 6 months or more but less than 12 months
- 12 months or more

Section 3: Patient Profile

10 Are you an adult aHUS Patient? *

- Yes
- No

11 How long ago did you (they) receive a diagnosis of aHUS? (in years)

12 During which time of the year did you (they) first become ill? *

- Jan - March
- Apr - June
- July - Sep
- Oct - Dec

13 How would you characterize the speed of your (their) diagnosis?

- Soon after presenting with symptoms
- After a delay of a few weeks
- After several months of illness
- After months, then going to see other specialists
- Diagnosis not determined until more than a year from onset of illness
- Diagnosis took more than 18+ months

14 Age at diagnosis? (in years) *

- Under 3
- 3 to 10
- 11 to 20

- 21 to 35
- 36 to 55
- 56 to 65
- Over 65

15 Did you (they) need dialysis during the initial illness? *

- Yes, but kidney function resumed and dialysis was stopped
- Yes, and dialysis is an ongoing requirement
- No dialysis needed

16 If you (they) are currently on dialysis, which type of dialysis is being done?

- Hemodialysis In-centre
- Home Hemodialysis
- Peritoneal Dialysis

17 Did you (they) receive plasma infusions and/or plasma exchanges during your initial illness? *

- Yes
- No

18 Which do you consider to be the most significant issues facing dialysis patients?

- Impacts quality at work/school (cognitive issues)
- Dialysis interferes with normal routine of work, school, and/or family

- Travel time to treatment
- Infections in line or fistula, etc.
- Dialysis interferes with ability to travel
- Home dialysis is difficult due to technical issues
- Fluid overload
- Peritonitis
- Impact on other organs (e.g. cardiac issues, pulmonary hypertension)
- Disruption to family schedule/lifestyle
- Anxiety or depression
- Other

19 Have you (they) had a transplant? *

- Yes, a kidney transplant
- Yes, a kidney & liver transplant
- Yes, but the transplant failed
- Yes, have had more than 1 transplant
- No, not needed
- No, but listed and awaiting a transplant
- No, treatment access or other circumstances block a transplant

20 If you (they) had a transplant, how long ago did the transplant occur? (in years)

- Less than 1
- 1 or more but less than 2
- 2 or more but less than 3
- 3 or more but less than 4

- 4 or more but less than 5
- 5 or more but less than 6
- 6 or more but less than 8
- 8 or more but less than 10
- 10 or more

Section 4: Treatment

21 Is the complement inhibitor eculizumab currently available in your country? *

- Yes
- Yes, but access depends on certain health guidelines
- No, not available in my country
- I don't know

22 If you answered yes to the question above, how are drug costs covered?

- By government plan
- By private insurance
- By other coverage
- It is not covered
- By multiple types of coverage
- Don't know / Not sure

23 Are you (they) currently using eculizumab?

- Yes

- No, have never used it
- No, use of this drug was discontinued

24 If you answered no to the question above, why are you (they) not using eculizumab?

- Not available in my country
- Physician has not recommended it
- Insurance or Government guidelines state aHUS patient status is not eligible
- Cannot afford it
- Other

Answer the next 4 questions only if you (they) are on the complement inhibitor eculizumab

25 How soon after your (their) illness was eculizumab used for patient treatment? (in months)

- Less than 1
- 1 or more but less than 3
- 3 or more but less than 6
- 6 or more but less than 12
- 12 or more

26 By what method is eculizumab administered?

- By port (or other access point)
- Intravenously (eg. an IV using a vein in arm)

27 Where is it given?

- At home, by someone specially trained
- At home, by a visiting nurse or other medical professional
- At a doctor's office or infusion center/clinic
- In a hospital

28 Difficulties to note?

- Venous access difficult
- Infection issues
- Lost time at work/school for treatment
- Travel to the treatment location is problematic
- Emotional distress with access
- Disruption to family schedule
- Location risks contact with sick patients

Section 5: Research – Patient Participation

29 Have you participated in aHUS research?

- Yes
- Yes, and would like to do so again
- No, I do not wish to participate
- No, but I would like to learn more about how to be included
- I don't know

30 Are you enrolled in an aHUS patient registry?

- Yes
- Yes, but I'd like to receive updates
- No, I do not wish to participate
- No, but I would like to learn more about how to be included
- I don't know

31 Do you think patients or patient organizations should play a role in setting the aHUS research agenda?

- Yes
- No
- I'm not sure

32 Is there a research topic which you think should be examined?

- Yes
- No

33 If you answered Yes above, please state in a few words, what topic you think should be examined.

34 In your opinion, what would encourage greater patient participation in research studies or clinical trials?

- Broader patient access to full articles and publications, not just abstracts
- Wider offering of opportunities to receive information and updates about ongoing studies or trials

- More information from my (their) medical team regarding current or ongoing studies or trials
- More information from patient organizations regarding current or ongoing studies or trials
- Inform patients how their participation can benefit their own care, help other patients and/or their own family members
- I don't know
- Other

Section 6: Insights from Patients & Caregivers

35 With future advancements in aHUS treatment options, would you prefer a treatment that you could give yourself such as a subcutaneous injection (shot)?

- Strongly prefer to give it myself
- Somewhat prefer to give it myself
- It doesn't matter if I or someone else administers it
- Somewhat prefer if someone else administers it
- Greatly prefer someone else to administer it

36 If you prefer a treatment that you could give yourself, what interval of time (frequency) is the most acceptable?

- Four times a day
- Twice a day
- Once a day
- Once every two days
- Once a week

37 How confident are you regarding information about aHUS research and developments updates reaching:

a. Your specialist or medical team?

- Confident
- Somewhat sure
- Uncertain
- Not confident

b. You, as patient or caregiver?

- Confident
- Somewhat sure
- Uncertain
- Not confident

c. aHUS patient organization(s)?

- Confident
- Somewhat sure
- Uncertain
- Not confident

38 How much time and effort do you expend trying to find more information about aHUS?

- Not much, I (we) rely on info from our doctor
- Enough to share articles with our medical team

- Some, I find most information on an aHUS patient organization site
- Some, I use a search engine to find info

39 Have you(they) experienced ongoing issues or discomfort with any of these issues, either serious or frequently enough to discuss these symptoms with a doctor?

- Headaches
- Vision issues
- Breathing issues
- Stomach or GI issues
- Skin issues
- Seizure activity
- Difficulty with mental confusion, focus, or memory
- Patient has not had ongoing issues of concern to alert medical staff

40 Have you (they) been told by a doctor that aHUS may have caused complications in other parts of the body (extra renal), aside from the blood or kidneys?

- Heart (cardiac)
- Lungs (pulmonary)
- Skin (dermatology)
- GI (stomach or colon)
- Neurological (headaches, vision, seizures, stroke, etc.)
- No ongoing issues, but the medical team mentioned some of these during hospitalization
- No, the medical team didn't discuss these issues

- No, but the medical team made me aware that these issues can affect aHUS patients

41 Have you (they) had discussions regarding the cost of aHUS treatment, to include complement inhibitors, with:

a. Your (their) specialist or medical team?

- No
- Yes, but it did not impact medical decisions
- Yes, it affected choices/quality of care

b. A representative from your (their) workplace?

- No
- Yes, but it did not impact medical decisions
- Yes, it affected choices/quality of care

c. An insurer or health policymaker?

- No
- Yes, but it did not impact medical decisions
- Yes, it affected choices/quality of care

d. Within your (their) family?

- No
- Yes, but it did not impact medical decisions
- Yes, it affected choices/quality of care

42 Is your (their) current treatment for aHUS the preferred treatment of choice?

- Yes
- No
- Other

43 Regarding access to patient care and treatment options

- Patients in my country seem to have full access to drugs (such as complement inhibitors) and varied treatment option choices
- Patients in other countries seem to have more access to drugs (such as complement inhibitors) and various treatment options
- My (their) health situation or financial circumstances limit choices for medical care or treatment
- Health Ministry or Government policies in my country limit choices for medical care or treatment
- Specialists and/or treatment locations are located far enough from me (them) that it impacts care or lifestyle of patient and/or their family
- Access to medical care and various treatment options are not a concern for me (them)
- Cost of medical care and treatment (to include complement inhibitors) are of concern to me and my family

44 Regarding cost of treatment with complement inhibitor eculizumab

- Prices are something seen on treatment or insurance bills, but it doesn't concern me as a patient or caregiver

- I don't know the price, but cost of this treatment doesn't concern me as a patient or caregiver
- Prices are something seen on treatment or insurance bills, but its cost seems too high
- Complement inhibitor cost is something my (their) medical team has mentioned in terms of prescribing its use, length of its use, or discontinuing its use longer term
- High cost of complement inhibitor drug treatment is something I (they) have seen online or in media reports

45 With future developments in new aHUS treatment options, what do you think would most likely affect your (their) use of any new safe and effective drug?

- Cost of the drug
- Type of delivery method or convenience to administer/take the drug
- Government Regulations or Health Care policies that do not limit the drug's use, or its application based on patient profile
- If clinical trial locations for the drug were offered in my (their) country
- Knowledge/opinion of my (their) specialists or medical team regarding this drug

46 As a participant in a registry, whether as an adult aHUS patient or a parent/guardian enrolling their child in an aHUS registry, which information collection method do you prefer?

- Completing a written questionnaire, with the physician or hospital submitting it to the aHUS registry

- Completing a written questionnaire, and personally submitting this to the aHUS registry
- Being provided an info sheet describing the purpose of an aHUS registry, attached to a written questionnaire to take home and fill in/return myself
- Completing an online questionnaire using a secure website or App
- I'm not sure/I have no preference

Thank you for participating in the 2016 aHUS global survey. Answers to these questions, along with the profile of respondents, will help to provide information about the needs, issues, and experiences of aHUS patients worldwide.

Once you go back to the Survey Home Page, don't forget to download the "I participated in the 2016 aHUS Survey" badge and proudly display it on your Facebook or Twitter page and spread awareness about aHUS.