## Supplementary material

## Text S1. The development of surveys for adult patients with aHUS and caregivers of pediatric patients with aHUS

### ***Targeted literature review***

A targeted literature review was conducted to identify previous research on the key signs, symptoms and impacts of atypical hemolytic uremic syndrome (aHUS), key factors related to treatment with eculizumab or ravulizumab, and existing measures that could be used to inform the content of the surveys. No aHUS-specific measures and no treatment preference studies in patients with aHUS were identified. A measure of patient experience for patients with aHUS was identified – the Patient Experiences and Satisfaction with Medications questionnaire [1] – but this had not been validated at the time of the present study. The literature review (n = 17 articles) identified 16 key signs and symptoms of aHUS, in addition to a range of emotional, socioeconomic, and physical functioning factors related to aHUS and its treatment. Qualitative outcomes from the literature review were summarized and used to create a preliminary conceptual model of signs, symptoms and impacts of aHUS, as well as treatment-related factors of eculizumab and ravulizumab.

### ***Clinician interviews***

Interviews aimed to build upon the preliminary conceptual model with expert insight about aHUS and its treatment with eculizumab and ravulizumab. Clinicians with expertise in aHUS were directly recruited by Alexion, AstraZeneca Rare Disease. Interviews were web-assisted and were held by trained qualitative interviewers using a semi-structured interview guide and lasted approximately 60 minutes. Participants (*N* = 7) were US-based hematologists (n = 3) or nephrologists (n = 4) with 5–27 years of experience with adults with aHUS (n = 4 clinicians), pediatric patients (n = 1 clinician) or both adult and pediatric patients (n = 2 clinicians). The conceptual model was updated to include any additional signs and symptoms, impacts of aHUS and its treatment, other treatment considerations and benefits and burdens of treatment identified by clinicians.

### ***Adult patient and caregiver interviews***

Semi-structured concept elicitation telephone interviews were held with adult patients with aHUS (n = 5) and caregivers of pediatric patients with aHUS (n = 5) in the USA recruited through the Alexion OneSource™ patient program, the aHUS Foundation or via a recruitment agency. Interviews lasted 60–90 minutes and involved discussion of the main symptoms and impacts of aHUS, important factors when considering their preferred treatment for aHUS, benefits and burdens of eculizumab and ravulizumab and the relevance of possible survey questions to their experiences with aHUS. The output of these interviews was used to create separate conceptual models, based on the model from the clinician interviews, for adult patients and caregivers of children with aHUS separately.

### ***Survey pilot tests and follow-up interviews***

Separate web-based surveys were developed for adult patients and caregivers based on an existing measure developed to assess treatment preference between eculizumab and ravulizumab for paroxysmal nocturnal hemoglobinuria [2] and were informed by the respective conceptual models from the previous development stages. A subset of patients (n = 3) and caregivers (n = 3) from the concept elicitation interviews participated in pilot testing and follow-up interviews to assess the survey content and technical performance of the online surveys. Interviews were conducted by telephone, followed a semi-structured interview discussion guide and lasted approximately 45 minutes. Participant responses informed minor amendments to wording and some of the response options to some items in the survey.

### ***Final surveys***

After incorporating minor amendments to the surveys from the pilot testing phase, final surveys for patients and caregivers were hosted online and distributed via the Qualtrics XM platform. After the surveys were finalized, a survey item and response option were excluded from both surveys owing to ambiguity in the interpretation of the item. In the treatment preference part of both surveys, participants were asked to indicate whether they preferred eculizumab or ravulizumab on a 5-point Likert scale (strongly prefer ravulizumab, somewhat prefer ravulizumab, no preference, somewhat prefer eculizumab, strongly prefer eculizumab) regarding the ‘length of time that the treatment has been approved by the Food and Drug Administration for treating aHUS’. This item was also included as a response option to the instruction for participants to select up to five factors that are most important to them when deciding their overall preference between eculizumab and ravulizumab.

## References

1. Kimman ML, Rotteveel AH, Wijsenbeek M *et al*. Development and pretesting of a questionnaire to assess patient experiences and satisfaction with medications (PESaM questionnaire). *Patient.* 10, 629–642 (2017).

2. Kaiser K, Yount SE, Martens CE *et al*. Assessing preferences for rare disease treatment: qualitative development of the Paroxysmal Nocturnal Hemoglobinuria Patient Preference Questionnaire (PNH-PPQ©). *Patient. Prefer. Adherence.* 14, 705–715 (2020).

## Table S1. Summary of adult and caregiver survey contents and study variables

|  |  |
| --- | --- |
| Survey section | Survey items/study variables |
| Part 1, demographics and health information | **Adult patients and caregivers (6 items)**  Gender, age, ethnicity, race, education, work status  **Pediatric patients (5 items)a**  Gender, age, ethnicity, race, education |
| Part 2, aHUS disease and management | **Adult patients and caregivers (12 items)**  Date of diagnosis; aHUS triggering event; dates of first and last/most recent infusions, location treatments took place and number of infusions; formulation of ravulizumab received; use of a venous access port |
| Part 3, preference between treatments | **Adult patients (16 items) and caregivers (18 items)b**  Preference between treatments for a range of factors on a 5-point scale:   * strongly prefer ravulizumab * somewhat prefer ravulizumab * no preference * somewhat prefer eculizumab * strongly prefer eculizumab   Overall preference between treatments on the same 5-point scale  Participants selected up to five treatment-related factors that they considered to be the most important when indicating preference between treatments |
| Part 4, perceived impact of eculizumab treatment on daily life  Part 5, perceived impact of eculizumab on productivity  Part 6, perceived impact of ravulizumab treatment on daily life  Part 7, perceived impact of ravulizumab on productivity | **Adult patients (10 items per treatment) and caregivers (13 items per treatment)b**  Participants indicated whether each in a range of statements about treatment-specific impacts applied on a 5-point scale:   * not at all * a little bit * somewhat * quite a bit * very much |
| Part 8, time lost owing to treatment | **Adult patients, caregivers and pediatric patients**   * time needed, per treatment session, to travel for/receive treatment * time needed, per month, to manage aHUS (excludes time to receive treatment) |

aPediatric patient demographics and characteristics were provided by caregivers.

bCaregivers answered additional questions regarding their own experiences.

aHUS, atypical hemolytic uremic syndrome.

## Text S2. Ravulizumab and eculizumab treatment impact and preference survey content.

### Adult patient survey

#### Part 1: demographics and health information questions

1. What is your gender?
   * Female
   * Male
   * Other: (open text)
2. What is your current age?
3. Do you consider yourself Hispanic/Latino or not Hispanic/Latino?
   * Hispanic/Latino
   * Not Hispanic/Latino
4. How would you describe your race? Select all that apply.
   * White/Caucasian
   * Black/African American
   * American Indian/Alaska Native
   * Asian/Asian American
   * Native Hawaiian/Pacific Islander
   * Other: (open text)
5. What is your highest level of education completed?
   * Did not complete high school
   * High school diploma or GED (General Education Development)
   * Some college, certification program or currently enrolled
   * College, technical college or university degree
   * Graduate degree (MS, PhD, MD, etc.)
6. How would you describe your current work status? Select all that apply.
   * Employed full-time (≥ 40 hours per week)
   * Employed part-time (< 40 hours per week)
   * Homemaker
   * Student
   * Retired
   * Disabled
   * Unemployed

#### Part 2: aHUS disease and management questions

1. When were you diagnosed with aHUS? Please enter at least the year.
   * Month
   * Year
2. What event triggered your aHUS?
   * Infection (e.g. bacterial infection or virus, including Covid)
   * Severe hypertension
   * A specific medication/drug
   * Cancer
   * Autoimmune disease (e.g. Lupus)
   * Bone-marrow transplant
   * Pregnancy or childbirth
   * None
   * Other: (open text)
   * Unsure
3. Approximately when did you first receive an IV infusion of Soliris (eculizumab) for your aHUS?
   * Month
   * Year
4. Approximately when did you last receive an IV infusion of Soliris (eculizumab) for your aHUS?
   * Month
   * Year
5. Where did you receive your last IV infusion of Soliris (eculizumab) for your aHUS?
   * Hospital (in-patient)
   * Hospital (out-patient)
   * Clinic (e.g. doctor’s office or infusion clinic)
   * Home
   * Other: (open text)
6. Approximately when did you first receive an IV infusion of Ultomiris (ravulizumab) for your aHUS?
   * Month
   * Year
7. Approximately when was your last (most recent) IV infusion of Ultomiris (ravulizumab) for your aHUS?
   * Month
   * Year
8. About how many IV infusions in total of Ultomiris (ravulizumab) have you received for your aHUS since starting on Ultomiris (ravulizumab) to now? Please include loading doses.
   * 3–5
   * 6–8
   * 9 or more
9. Where did you receive your last (most recent) IV infusion of Ultomiris (ravulizumab) for your aHUS？
   * Hospital (in-patient)
   * Hospital (out-patient)
   * Clinic (e.g. doctor’s office or infusion clinic)
   * Home
   * Other: (open text)
10. Are you currently receiving the new formulation of Ultomiris (ravulizumab) with reduced infusion time (100mg/ml) compared to the older formulation of Ultomiris (ravulizumab) (10mg/ml)?
    * Yes, the new formulation of Ultomiris (ravulizumab)
    * No, not the new formulation of Ultomiris (ravulizumab)
    * Unsure
11. Did you ever have an implanted venous access port (e.g. a “mediport” or “port”) placed to receive your Soliris (eculizumab) treatments?
    * Yes
    * No
    * Unsure
12. Do you currently have an implanted venous access port (e.g. a “mediport” or “port”) placed to receive your Ultomiris (ravulizumab) treatments?
    * Yes
    * No
    * Unsure

#### Part 3: preference questions

Indicate which of the two medications (Soliris or Ultomiris) you prefer when thinking about each of the factors asked about on the next screen. Consider the **current formulation of Ultomiris** you receive. **If you think something does not apply to you, please check “Not Applicable.”**

|  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- |
| **Which do you prefer based on …** | **Strongly prefer Ultomiris** | **Somewhat prefer Ultomiris** | **No preference** | **Somewhat prefer Soliris** | **Strongly prefer Soliris** | **Not applicable** |
| 19. Controlling your aHUS |  |  |  |  |  |  |
| 20. Frequency of infusion |  |  |  |  |  |  |
| 21. Length of time to receive infusion from start of the infusion time |  |  |  |  |  |  |
| 22. Your perception of side effects or safety of treatment |  |  |  |  |  |  |
| 23. Convenience of receiving infusion (e.g. at home, going to an infusion center, etc.) |  |  |  |  |  |  |
| 24. Convenience of infusion injection (e.g. having a mediport or peripheral injection) |  |  |  |  |  |  |
| 25. Being able to go to work/school |  |  |  |  |  |  |
| 26. Being able to plan social activities |  |  |  |  |  |  |
| 27. Being able to travel/vacation |  |  |  |  |  |  |
| 28. Your perception of the effectiveness of the infusion until the next treatment |  |  |  |  |  |  |
| 29. Concern related to receiving an infusion procedure |  |  |  |  |  |  |
| 30. Concern related to your aHUS getting worse |  |  |  |  |  |  |
| 31. Benefit to your quality of life |  |  |  |  |  |  |
| 32. Cost of treatment, including coverage by insurance |  |  |  |  |  |  |

|  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- |
| **33. Overall, which of the two medications do you prefer?** |  |  |  |  |  |  |

**34. Select up to five (5) factors below that are most important to you when deciding your overall preference between Soliris and Ultomiris.**

1. Controlling your aHUS
2. Frequency of infusion
3. Length of time to receive infusion from start of the infusion time
4. Your perception of side effects or safety of treatment
5. Convenience of receiving infusion (e.g. at home, going to an infusion center, etc.)
6. Convenience of infusion injection (e.g. having a mediport or peripheral injection)
7. Being able to go to work/school
8. Being able to plan social activities
9. Being able to travel/vacation
10. Your perception of the effectiveness of the infusion until the next treatment
11. Concern related to receiving an infusion procedure
12. Concern related to your aHUS getting worse
13. Benefit to your quality of life
14. Cost of treatment, including coverage by insurance
15. None of the above

#### Part 4: preference (eculizumab) Likert questions

For each statement, select how much you agree with it based on your own experience. **If you think something does not apply to you (e.g. you did not work or go to school while receiving Soliris), please check “Not Applicable.”**

|  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- |
|  | **Not at all** | **A little bit** | **Somewhat** | **Quite a bit** | **Very much** | **Not applicable** |
| 35. The frequency of my Soliris (eculizumab) infusions disrupted my life |  |  |  |  |  |  |
| 36. The frequency of my Soliris (eculizumab) infusions impacted my ability to go to work/school |  |  |  |  |  |  |
| 37. While receiving Soliris (eculizumab) treatments, I was able to enjoy life |  |  |  |  |  |  |
| 38. While receiving Soliris (eculizumab) treatments, I was able to talk to my doctor or nurse as often as I would have liked about my aHUS |  |  |  |  |  |  |
| 39. Side effects of Soliris (eculizumab) can disrupt a person’s life |  |  |  |  |  |  |
| 40. Soliris (eculizumab) is effective in treating symptoms of aHUS |  |  |  |  |  |  |

#### Part 5: disease and treatment (eculizumab) impacts

For each statement, select how much you agree with it based on your own experience. **If you think something does not apply to you (e.g. you did not work or go to school while receiving Soliris), please check “Not Applicable.”**

|  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- |
|  | **Not at all** | **A little bit** | **Somewhat** | **Quite a bit** | **Very much** | **Not applicable** |
| 41. My Soliris (eculizumab) treatments impacted how productive I was at work/school |  |  |  |  |  |  |
| 42. While receiving Soliris (eculizumab) treatments, I was able to keep my regular work/school schedule |  |  |  |  |  |  |
| 43. My Soliris (eculizumab) treatments made me change my school or career goals |  |  |  |  |  |  |
| 44. My Soliris (eculizumab) treatments impacted my overall financial well-being |  |  |  |  |  |  |

#### Part 6: preference (ravulizumab) Likert questions

For each statement, select how much you agree with it based on your own experience. Consider the **current formulation of Ultomiris** you receive. **If you think something does not apply to you (e.g. you do not work or go to school while receiving Ultomiris), please check “Not Applicable.”**

|  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- |
|  | **Not at all** | **A little bit** | **Somewhat** | **Quite a bit** | **Very much** | **Not applicable** |
| 45. The frequency of my Ultomiris (ravulizumab) infusions disrupts my life |  |  |  |  |  |  |
| 46. The frequency of my Ultomiris (ravulizumab) infusions impacts my ability to go to work/school |  |  |  |  |  |  |
| 47. While receiving Ultomiris (ravulizumab) treatments, I am able to enjoy life |  |  |  |  |  |  |
| 48. While receiving Ultomiris (ravulizumab) treatments, I am able to talk to my doctor or nurse as often as I would like about my aHUS |  |  |  |  |  |  |
| 49. Side effects of Ultomiris (ravulizumab) can disrupt a person’s life |  |  |  |  |  |  |
| 50. Ultomiris (ravulizumab) is effective in treating symptoms of aHUS |  |  |  |  |  |  |

#### Part 7: disease and treatment (ravulizumab) impacts

For each statement, select how much you agree with it based on your own experience. Consider the **current formulation of Ultomiris** you receive. **If you think something does not apply to you (e.g. you do not work or go to school while receiving Ultomiris), please check “Not Applicable.”**

|  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- |
|  | **Not at all** | **A little bit** | **Somewhat** | **Quite a bit** | **Very much** | **Not applicable** |
| 51. My Ultomiris (ravulizumab) treatments impact how productive I am at work/school |  |  |  |  |  |  |
| 52. While receiving Ultomiris (ravulizumab) treatments, I am able to keep my regular work/school schedule |  |  |  |  |  |  |
| 53. My Ultomiris (ravulizumab) treatments have made me change my school or career goals |  |  |  |  |  |  |
| 54. My Ultomiris (ravulizumab) treatments impact my overall financial well-being |  |  |  |  |  |  |

#### Part 8: time lost due to treatment (eculizumab and ravulizumab)

55. About how many **hours per treatment session** did you need to take to receive each of the following treatments? Please consider all the time involved in receiving the treatment, including all travel time, wait time, receiving the infusion itself, monitoring, etc.

|  |  |
| --- | --- |
|  | Hours taken per treatment |
| Soliris (eculizumab) |  |
| Ultomiris (ravulizumab) |  |

56. About how many **hours per month** did you need to take to manage your aHUS while on each of the following treatments? Please consider any follow-up visits, travel time for follow-up visits, and managing your aHUS in general. Please do not include time to receive the treatment.

|  |  |
| --- | --- |
|  | Hours taken per month to manage your aHUS |
| Soliris (eculizumab) |  |
| Ultomiris (ravulizumab) |  |

### Caregiver survey

#### Part 1: demographics and health information questions

1. What is your gender?
   * Female
   * Male
   * Other: (open text)
2. What is your age?
3. Do you consider yourself Hispanic/Latino or not Hispanic/Latino?
   * Hispanic/Latino
   * Not Hispanic/Latino
4. How would you describe your race? Select all that apply.
   * White/Caucasian
   * Black/African American
   * American Indian/Alaska Native
   * Asian/Asian American
   * Native Hawaiian/Pacific Islander
   * Other: (open text)
5. What is your highest level of education completed?
   * Did not complete high school
   * High school diploma or GED (General Education Development)
   * Some college, certification program, or currently enrolled
   * College, technical college, or university degree
   * Graduate degree (MS, PhD, MD, etc.)
6. How would you describe your current work status? Select all that apply.
   * Employed full-time (≥ 40 hours per week)
   * Employed part-time (< 40 hours per week)
   * Homemaker
   * Student
   * Retired
   * Disabled
   * Unemployed
7. What is your child’s gender?
   * Female
   * Male
   * Other: (open text)
8. What is your child’s current age?
9. Do you consider your child to be Hispanic/Latino or not Hispanic/Latino?
   * Hispanic/Latino
   * Not Hispanic/Latino
10. How would you describe your child’s race? Select all that apply.
    * White/Caucasian
    * Black/African American
    * American Indian/Alaska Native
    * Asian/Asian American
    * Native Hawaiian/Pacific Islander
    * Other: (open text)
11. What is your child’s highest level of education completed?
    * Not yet school age
    * Kindergarten
    * 1st Grade
    * 2nd Grade
    * 3rd Grade
    * 4th Grade
    * 5th Grade
    * 6th Grade
    * 7th Grade
    * 8th Grade
    * Freshman year of high school (9th Grade)
    * Sophomore year of high school (10th Grade)
    * Junior year of high school (11th Grade)
    * High school diploma or GED
    * Some college, certification program, or currently enrolled

#### Part 2: aHUS disease and management questions

1. When was your child diagnosed with aHUS? Please enter at least the year.
   * Month
   * Year
2. What event triggered your child’s aHUS?
   * Infection (e.g. bacterial infection or virus, including Covid)
   * Severe hypertension
   * A specific medication/drug
   * Cancer
   * Autoimmune disease (e.g. Lupus)
   * Bone-marrow transplant
   * None
   * Other: (open text)
   * Unsure
3. Approximately when did your child first receive an IV infusion of Soliris (eculizumab) for aHUS?

• Month

• Year

1. Approximately when did your child last receive an IV infusion of Soliris (eculizumab) for aHUS?

* Month

• Year

1. Where did your child receive the last IV infusion of Soliris (eculizumab) for aHUS?
   * Hospital (in-patient)
   * Hospital (out-patient)
   * Clinic (e.g. doctor’s office or infusion clinic)
   * Home
   * Other: (open text)
2. Approximately when did your child first receive an IV infusion of Ultomiris (ravulizumab) for aHUS?
   * Month
   * Year
3. Approximately when was your child’s last (most recent) IV infusion of Ultomiris (ravulizumab) for aHUS?

* Month
* Year

1. About how many IV infusions in total of Ultomiris (ravulizumab) has your child received for aHUS since starting on Ultomiris (ravulizumab) to now? Please include loading doses.
   * 3–5
   * 6–8
   * 9 or more
2. Where did your child receive the last (most recent) IV infusion of Ultomiris (ravulizumab) for aHUS?
   * Hospital (in-patient)
   * Hospital (out-patient)
   * Clinic (e.g. doctor’s office or infusion clinic)
   * Home
   * Other: (open text)
3. Is your child currently receiving the new formulation of Ultomiris (ravulizumab) with reduced infusion time (100mg/ml) compared to the older formulation of Ultomiris (ravulizumab) (10mg/ml)?
   * Yes, the new formulation of Ultomiris (ravulizumab)
   * No, not the new formulation of Ultomiris (ravulizumab)
   * Unsure
4. Did your child ever have an implanted venous access port (e.g. a “mediport” or “port”) placed to receive Soliris (eculizumab) treatments?
   * Yes
   * No
   * Unsure
5. Does your child currently have an implanted venous access port (e.g. a “mediport” or “port”) placed to receive Ultomiris (ravulizumab) treatments?
   * Yes
   * No
   * Unsure

#### Part 3: preference questions

Indicate which of the two medications (Soliris or Ultomiris) you prefer when thinking about each of the factors asked about on the next screen. Consider the **current formulation of Ultomiris**. **If you think something does not apply to you or your child with aHUS, please check “Not Applicable.”**

|  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- |
| **Which do you prefer based on …** | **Strongly prefer Ultomiris** | **Somewhat prefer Ultomiris** | **No preference** | **Somewhat prefer Soliris** | **Strongly prefer Soliris** | **Not applicable** |
| 24. Controlling your child’s aHUS |  |  |  |  |  |  |
| 25. Frequency of infusion |  |  |  |  |  |  |
| 26. Length of time to receive infusion from start of the infusion time |  |  |  |  |  |  |
| 27. Your perception of side effects or safety of treatment |  |  |  |  |  |  |
| 28. Convenience of receiving infusion (e.g. at home, going to an infusion center, etc.) |  |  |  |  |  |  |
| 29. Convenience of infusion injection (e.g. having a mediport or peripheral injection) |  |  |  |  |  |  |
| 30. Your child being able to go to school |  |  |  |  |  |  |
| 31. Your ability to go to work/school |  |  |  |  |  |  |
| 32. Your ability to plan social activities |  |  |  |  |  |  |
| 33. Your ability to travel/vacation |  |  |  |  |  |  |
| 34. Your perception of the effectiveness of the infusion until the next treatment |  |  |  |  |  |  |
| 35. Concern related to your child receiving an infusion procedure |  |  |  |  |  |  |
| 36. Concern related to your child’s aHUS getting worse |  |  |  |  |  |  |
| 37. Benefit to your child’s quality of life |  |  |  |  |  |  |
| 38. Benefit to your quality of life |  |  |  |  |  |  |
| 39. Cost of treatment, including coverage by insurance |  |  |  |  |  |  |

|  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- |
| **40. Overall, which of the two medications do you prefer?** |  |  |  |  |  |  |

**41. Select up to five (5) factors below that are most important to you when deciding your overall preference between Soliris and Ultomiris.**

1. Controlling your child’s aHUS
2. Frequency of infusion
3. Length of time to receive infusion from start of the infusion time
4. Your perception of side effects or safety of treatment
5. Convenience of receiving infusion (e.g. at home, going to an infusion center, etc.)
6. Convenience of infusion injection (e.g. having a mediport or peripheral injection)
7. Your child being able to go to school
8. Your ability to go to work/school
9. Your ability to plan social activities
10. Your ability to travel/vacation
11. Your perception of the effectiveness of the infusion until the next treatment
12. Concern related to your child receiving an infusion procedure
13. Concern related to your child’s aHUS getting worse
14. Benefit to your child’s quality of life
15. Benefit to your quality of life
16. Cost of treatment, including coverage by insurance
17. None of the above

#### Part 4: preference (eculizumab) Likert questions

For each statement, select how much you agree with it based on your and your child’s experiences. **If you think something does not apply to you (e.g. you did not work or your child did not go to school while receiving Soliris), please check “Not Applicable.”**

|  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- |
|  | **Not at all** | **A little bit** | **Somewhat** | **Quite a bit** | **Very much** | **Not applicable** |
| 42. The frequency of my child’s Soliris (eculizumab) infusions disrupted our lives |  |  |  |  |  |  |
| 43. The frequency of my child’s Soliris (eculizumab) infusions impacted my ability to go to work/school |  |  |  |  |  |  |
| 44. The frequency of my child’s Soliris (eculizumab) infusions impacted my child’s ability to go to school |  |  |  |  |  |  |
| 45. While receiving Soliris (eculizumab), our family was able to enjoy life |  |  |  |  |  |  |
| 46. While receiving Soliris (eculizumab) treatments, I was able to talk to my child’s doctor or nurse as often as I would have liked about my child’s aHUS |  |  |  |  |  |  |
| 47. Side effects of Soliris (eculizumab) can disrupt a family’s life |  |  |  |  |  |  |
| 48. Soliris (eculizumab) is effective in treating a child’s symptoms of aHUS |  |  |  |  |  |  |

#### Part 5: disease and treatment (eculizumab) impacts

For each statement, select how much you agree with it based on your and your child’s experiences. **If you think something does not apply to you (e.g. you did not work or your child did not go to school while receiving Soliris), check “Not Applicable.”**

|  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- |
|  | **Not at all** | **A little bit** | **Somewhat** | **Quite a bit** | **Very much** | **Not applicable** |
| 49. My child’s Soliris (eculizumab) treatments impacted how productive I was at work/school |  |  |  |  |  |  |
| 50. My child’s Soliris (eculizumab) treatments impacted how productive my child was at school |  |  |  |  |  |  |
| 51. While my child was receiving Soliris (eculizumab) treatments, I was able to keep my regular work/school schedule |  |  |  |  |  |  |
| 52. While my child was receiving Soliris (eculizumab) treatments, my child was able to keep their regular school schedule |  |  |  |  |  |  |
| 53. My child’s Soliris (eculizumab) treatments made me change my school or career goals |  |  |  |  |  |  |
| 54. My child’s Soliris (eculizumab) treatments impacted our overall financial well-being |  |  |  |  |  |  |

#### Part 6: preference (ravulizumab) Likert questions

For each statement, select how much you agree with it based on your and your child’s experiences. Consider **the current formulation of Ultomiris** your child receives. **If you think something does not apply to you (e.g. you do not work or your child does not go to school while receiving Ultomiris) check “Not Applicable.”**

|  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- |
|  | **Not at all** | **A little bit** | **Somewhat** | **Quite a bit** | **Very much** | **Not applicable** |
| 55. The frequency of my child’s Ultomiris (ravulizumab) infusions disrupts our lives |  |  |  |  |  |  |
| 56. The frequency of my child’s Ultomiris (ravulizumab) infusions impacts my ability to go to work/school |  |  |  |  |  |  |
| 57. The frequency of my child’s Ultomiris (ravulizumab) infusions impacts my child’s ability to go to school |  |  |  |  |  |  |
| 58. While receiving Ultomiris (ravulizumab), our family is able to enjoy life |  |  |  |  |  |  |
| 59. While receiving Ultomiris (ravulizumab) treatments, I am able to talk to my child’s doctor or nurse as often as I would like about my child’s aHUS |  |  |  |  |  |  |
| 60. Side effects of Ultomiris (ravulizumab) can disrupt a family’s life |  |  |  |  |  |  |
| 61. Ultomiris (ravulizumab) is effective in treating a child’s symptoms of aHUS |  |  |  |  |  |  |

#### Part 7: disease and treatment (ravulizumab) impacts

For each statement, select how much you agree with it based on your child’s experience. Consider the **current formulation of Ultomiris** your child receives. **If you think something does not apply to you (e.g. you do not work or your child does not go to school while receiving Ultomiris) check “Not Applicable.”**

|  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- |
|  | **Not at all** | **A little bit** | **Somewhat** | **Quite a bit** | **Very much** | **Not applicable** |
| 62. My child’s Ultomiris (ravulizumab) treatments impact how productive I am at work/school |  |  |  |  |  |  |
| 63. My child’s Ultomiris (ravulizumab) treatments impact how productive my child is at school |  |  |  |  |  |  |
| 64. While my child is receiving Ultomiris (ravulizumab) treatments, I am able to keep my regular work/school schedule |  |  |  |  |  |  |
| 65. While my child is receiving Ultomiris (ravulizumab) treatments, my child is able to keep their regular school schedule |  |  |  |  |  |  |
| 66. My child’s Ultomiris (ravulizumab) treatments have made me change my school or career goals |  |  |  |  |  |  |
| 67. My child’s Ultomiris (ravulizumab) treatments impact our overall financial well-being |  |  |  |  |  |  |

#### Part 8: monthly time lost due to treatment (eculizumab and ravulizumab)

68. About how many **hours per treatment session** did you need to take for your child to receive each of the following treatments? Please consider all the time involved in your child receiving the treatment, including all travel time, wait time, your child receiving the infusion itself, monitoring, etc.

|  |  |
| --- | --- |
|  | Hours taken per treatment you took |
| Soliris (eculizumab) |  |
| Ultomiris (ravulizumab) |  |

69. About how many **hours per month** did you need to take to manage your child’s aHUS while your child was on each of the following treatments? Please consider any follow-up visits, travel time for follow-up visits, and managing your child’s aHUS in general. Please do not include time to receive the treatment.

|  |  |
| --- | --- |
|  | Hours taken per month you took to manage your child’s aHUS |
| Soliris (eculizumab) |  |
| Ultomiris (ravulizumab) |  |

70. About how many **hours per treatment session** did your child need to take to receive each of the following treatments? Please consider all the time involved in receiving the treatment, including all travel time, wait time, receiving the infusion itself, monitoring, etc.

|  |  |
| --- | --- |
|  | Hours taken per treatment your child took |
| Soliris (eculizumab) |  |
| Ultomiris (ravulizumab) |  |

71. About how many **hours per month** did your child need to take to manage their aHUS while on each of the following treatments? Please consider any follow-up visits, travel time for follow up visits, and managing your child’s aHUS in general. Please do not include time to receive the treatment.

|  |  |
| --- | --- |
|  | Hours taken per month your child took to manage their aHUS |
| Soliris (eculizumab) |  |
| Ultomiris (ravulizumab) |  |