An Observational, Non-Interventional, Multicenter, Multinational Registry of Patients With Atypical Hemolytic Uremic Syndrome: Initial Patient Characteristics

Christoph Licht, Giana Luigi Ardissino, Gema Ariceta, Jon Beauchamp, David Cohen, Larry A. Greenbaum, Sally Johnson, Masayo Ogawa, Franz Schafer, Johan Vande Walle, Véronique Fremeaux-Bacchi

The Hospital for Sick Children, Toronto, Ontario, Canada; Yonsei University HSC Ctr Grando; Osaka Kogakuin University; Kobe, Japan; Temple University of Philadelphia, Spain; University of Pharmacology, Lawrance, Switzerland; Chiba University, New York, NY, USA; Loyola University, Atlanta, GA, USA; Tübingen University Hospital, Tübingen University, Tübingen, Germany; Holt Park Central, Leeds, Spain; Assistance Publique–Hôpitaux de Paris, Paris, France

INTRODUCTION

Atypical Hemolytic Uremic Syndrome Background

Atypical hemolytic uremic syndrome (aHUS) is a genetic, progressive, life-threatening disease mostly resulting from mutations in the complement (C) system. The aHUS registry is supported by Alexion Pharmaceuticals, Inc., with governance by an independent scientific advisory board (SAB), a scientific board of directors, and an executive committee.

31 (29.0)

Time to first and subsequent occurrence of specified events

Data are collected at study enrollment and every 6 months thereafter and include the following:

- Written informed consent from a patient or parent/legal guardian (if applicable as determined by the central registry that received consent)
- Registry enrollment date, date of birth, and sex
- Associated treatments and concomitant medications
- Laboratory values
- Proportion of patients who experience specified events

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Table 2. aHUS Diagnostic Criteria in Registry Entry (as of September 25, 2013)

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The Global aHUS Patient Registry (GAPR) is an ongoing, non-interventional, observational, worldwide registry of patients with atypical hemolytic uremic syndrome (aHUS). The registry is supported by Registry Support from Peloton Advantage, LLC, which provided editorial support with funding from Alexion Pharmaceuticals, Inc.

The registry is dedicated to increasing the understanding and awareness of aHUS and its manifestations. The registry aims to achieve the following milestones: (1) To follow each patient and assess long-term outcomes for a minimum of 5 years; (2) To capture genetic and clinical data from patient medical records; (3) To assess the effectiveness and safety of eculizumab and other aHUS treatments; (4) To report refinements (all as appropriate).

Inclusion for the Current Analysis

• Male or female patients of any age who have been diagnosed clinically with aHUS
• With or without an identified complement regulatory factor genetic abnormality or anti-complement factor antibody (if tested)
• With or without an identified complement genetic mutation
• Stated family history of aHUS
• Any prior plasma exchange/infusion

Methods

The following data were mandatory for all enrolled patients to be included in this analysis: (1) Patient demographics; (2) Baseline clinical characteristics; (3) Number of baseline laboratory tests; (4) Date of initial symptoms; (5) Age at registry enrollment; (6) Sex; (7) Previous plasma exchange/infusion; (8) Any identified complement genetic mutation; (9) Stated family history of aHUS; (10) Medical and disease history; (11) Assessment of the long-term manifestations of TMA complications of aHUS; other clinical outcomes, including safety of eculizumab and other aHUS treatments.

The global aHUS patient registry is dedicated to increasing the understanding and awareness of aHUS disease history and progression.

Registry Support

Milestones Achieved for the Global aHUS Patient Registry

- To follow each patient and assess long-term outcomes for a minimum of 5 years, information from patient medical records is entered via a secure web portal and maintained anonymously.
- The following data were mandatory for all enrolled patients to be included in this analysis:
- As of September 25, 2013, a total of 211 patients have enrolled in the global aHUS patient registry.
- Germany, 14.7% (n=31)
- Israel, 4.7% (n=10)
- Sweden, 1.4% (n=3)

Table 1. Patient Demographics in Global aHUS Patient Registry (as of September 25, 2013)

<table>
<thead>
<tr>
<th>-ever treated with eculizumab (n=104)</th>
<th>ever treated with PE/PI (n=18)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Year of registry enrollment, n (%)</td>
<td>Sex, n (%)</td>
</tr>
<tr>
<td>&lt;2 years</td>
<td>&lt;2 years</td>
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<tr>
<td>0 (0.0)</td>
<td>0 (0.0)</td>
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<tr>
<td>1 (0.5)</td>
<td>1 (0.5)</td>
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<tr>
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<td>104 (97.2)</td>
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<td>9 (8.4)</td>
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<td>21 (10.0)</td>
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<tr>
<td>18 (16.8)</td>
<td>18 (16.8)</td>
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<td>16 (15.0)</td>
<td>16 (15.0)</td>
</tr>
<tr>
<td>55 (51.4)</td>
<td>55 (51.4)</td>
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Table 3. Baseline Clinical Characteristics of Patients at Registry Entry (as of September 25, 2013)

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<thead>
<tr>
<th>ever treated with eculizumab (n=104)</th>
<th>ever treated with PE/PI (n=18)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Year of registry enrollment, n (%)</td>
<td>Age at registry enrollment, n (%)</td>
</tr>
<tr>
<td>&lt;2 years</td>
<td>&lt;2 years</td>
</tr>
<tr>
<td>0 (0.0)</td>
<td>0 (0.0)</td>
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<tr>
<td>1 (0.0)</td>
<td>1 (0.0)</td>
</tr>
<tr>
<td>2 (2.4)</td>
<td>2 (2.4)</td>
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<tr>
<td>6 (5.7)</td>
<td>6 (5.7)</td>
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<tr>
<td>19 (18.1)</td>
<td>19 (18.1)</td>
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<tr>
<td>86 (83.5)</td>
<td>86 (83.5)</td>
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<td>8 (7.7)</td>
<td>8 (7.7)</td>
</tr>
<tr>
<td>16 (15.4)</td>
<td>16 (15.4)</td>
</tr>
<tr>
<td>41 (39.4)</td>
<td>41 (39.4)</td>
</tr>
<tr>
<td>92 (43.6)</td>
<td>92 (43.6)</td>
</tr>
</tbody>
</table>

Table 2. Baseline Laboratory Tests in Global aHUS Patient Registry (as of September 25, 2013)

<table>
<thead>
<tr>
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<th>ever treated with PE/PI (n=18)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Year of registry enrollment, n (%)</td>
<td>Mean baseline eGFR (SD), mL/min/1.73 m²</td>
</tr>
<tr>
<td>&lt;2 years</td>
<td>&lt;2 years</td>
</tr>
<tr>
<td>0 (0.0)</td>
<td>0 (0.0)</td>
</tr>
<tr>
<td>1 (0.0)</td>
<td>1 (0.0)</td>
</tr>
<tr>
<td>5 (4.8)</td>
<td>5 (4.8)</td>
</tr>
<tr>
<td>54 (25.6)</td>
<td>54 (25.6)</td>
</tr>
<tr>
<td>65 (30.8)</td>
<td>65 (30.8)</td>
</tr>
<tr>
<td>92 (43.6)</td>
<td>92 (43.6)</td>
</tr>
</tbody>
</table>

Mean baseline eGFR (SD), mL/min/1.73 m²: 17.1 (16.36)

Presented at the American Society of Nephrology Kidney Week 2013 Annual Meeting, November 5–10, 2013, Atlanta, Georgia.
INTRODUCTION
Atypical Hemolytic Uremic Syndrome: Background

- Atypical hemolytic uremic syndrome (aHUS) is a genetic, progressive, life-threatening disease mostly resulting from chronic, uncontrolled complement activation. It is characterized by systemic thrombotic microangiopathy (TMA) leading to renal and other end-organ damage.
- Plasma exchange and infusion (PE/PI) has been the treatment of choice for aHUS; however, evidence suggests that PE/PI offers no significant benefit over simple supportive therapy.2,3
- Eculizumab (Soliris®, Alexion Pharmaceuticals, Inc., Cheshire, CT, USA), a terminal complement inhibitor, is a humanized monoclonal antibody that binds with high affinity to the human C5 complement protein, blocking the generation of pro-inflammatory C5a and C5b-9.4
- Eculizumab is the first approved treatment for aHUS in pediatric and adult patients.4-7
- The global aHUS patient registry (US National Institutes of Health ClinicalTrials.gov Identifier: NCT01522183) was initiated in April 2012 to prospectively capture postmarketing effectiveness and safety data on patients treated with eculizumab; the registry will record information on the progression of disease in all aHUS patients (whether treated with eculizumab or with other disease management strategies).
- The registry fulfills postmarketing regulatory requirements by providing follow-up on the aHUS indication for eculizumab.
- Successful registry implementation is contingent on contributions from both academia and the industry sponsor.
  - Academia provides access to global, longitudinal data and increased scientific knowledge to better manage patients, and
  - The industry sponsor fosters relationships with academic partners, building credibility and scientific integrity, while also providing transparency and clear guidelines for publication.
- A single, global aHUS patient registry can maximize both physician and patient participation to best capture information on disease, safety, and efficacy data in a population with a very rare disease.

OBJECTIVE
To report patient characteristics and describe important milestones achieved by patients enrolled in the aHUS registry from its inception (April 2012) through September 2013.

METHODS
Patient Eligibility Criteria
- Inclusion criteria
  - Male or female patients of any age who have been diagnosed clinically with aHUS
    - With or without an identified complement regulatory factor genetic abnormality or anti-complement factor antibody (if tested)
    - ADAMTS13 (a disintegrin and metalloproteinase with a thrombospondin type 1 motif; C-terminal carboxy-terminal) >5%, if performed
  - Written informed consent from a patient or parent/legal guardian (if applicable as determined by the central institutional review boards/independent ethics committees)
- Exclusion criteria for patient registry
  - HUS due only to Shiga toxin-producing Escherichia coli

Primary Outcome Measures
- Proportion of patients who experience specified events
- Collection and evaluation of safety and efficacy data specific to the use of eculizumab in patients with aHUS
- Time to first and subsequent occurrence of specified events
- Assessment of the long-term manifestations of TMA complications of aHUS; other clinical outcomes, including morbidity and mortality in patients with aHUS receiving eculizumab treatment or treated with other disease-management approaches

Data Collection
- Data are collected at study enrollment and every 6 months thereafter and include the following:
  - Demographics
  - Medical and disease history
  - Symptomatology
  - Targeted laboratory results (including genetic results)
Presented at the American Society of Nephrology Kidney Week 2013 Annual Meeting, November 5–10, 2013, Atlanta, Georgia.

Patient Eligibility Criteria
• Data are collected at study enrollment and every 6 months thereafter and include the following:
  • Proportion of patients who experience specified events
  • Successful registry implementation is contingent on contributions from both academia and the industry sponsor.
  • The registry fulfills postmarketing regulatory requirements by providing follow-up on the aHUS indication for
    the global aHUS patient registry (US National Institutes of Health ClinicalTrials.gov Identifier: NCT01522183)
• Atypical hemolytic uremic syndrome (aHUS) is a genetic, progressive, life-threatening disease mostly resulting
  from TMA leading to renal and other end-organ damage.
• Eculizumab is the first approved treatment for aHUS in pediatric and adult patients.4-7
• Eculizumab (Soliris®; Alexion Pharmaceuticals, Inc., Cheshire, CT, USA), a terminal complement inhibitor, is a
  humanized monoclonal antibody that binds with high affinity to the human C5 complement protein, blocking the
  use of the aHUS registry name.
• Some key responsibilities of the SAB are to:
  • For treated patients: date of first eculizumab treatment
  • Review and provide guidance on future amendments to the protocol, data variables to be collected, and case
    report refinements (all as appropriate).
  • Advise on analyses and scientific questions of interest.
  • Review and provide feedback on publication goals and logistics.
  • Contribute to the development of the publication plan.
  • Establish and follow protocols for the review and approval of external requests for analyses and publications
    from individual investigators or national coordinators.
  • Advise, counsel, and guide individuals on publications that utilize aHUS registry data and resources and/or
    use the aHUS registry name.
  • Review publication drafts before submission to journals or public release.

Inclusion for the Current Analysis
• The following data were mandatory for all enrolled patients to be included in this analysis:
  • Previously treated with eculizumab at any time or never treated with eculizumab
  • Registry enrollment date, date of birth, and sex
  • For treated patients: date of first eculizumab treatment

RESULTS

Patient Characteristics in Global aHUS Patient Registry
• Tables 1–4 provide information on demographics, aHUS diagnosis, baseline clinical characteristics, and
  eculizumab treatment characteristics.

Countries Enrolling Patients into aHUS Patient Registry (as of September 25, 2013)
• Australia, 9.0% (n=19)
• Austria, 3.8% (n=8)
• Denmark, 0.5% (n=1)
• France, 2.4% (n=5)
• Germany, 14.7% (n=31)
• Israel, 4.7% (n=10)
• Italy, 4.3% (n=9)
• Russia, 1.4% (n=3)
• Spain, 9.5% (n=20)
• Sweden, 1.4% (n=3)
• United Kingdom, 19.4% (n=41)
• United States, 28.9% (n=61)

Breakdown of Enrolling Sites: Specialist Type
• Nephrologists (86%)
• Hematologists (14%)

Breakdown of Enrolling Sites: Adult versus Pediatric Centric
• Pediatric-centric sites (60%)
• Adult-centric sites (40%)
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### Primary Outcome Measures

**Patient Eligibility Criteria**

- Data are collected at study enrollment and every 6 months thereafter and include the following:
  - Time to first and subsequent occurrence of specified events
  - Collection and evaluation of safety and efficacy data specific to the use of eculizumab in patients with aHUS

**Exclusion criteria for patient registry**

**Inclusion criteria**

- Atypical hemolytic uremic syndrome (aHUS) is a genetic, progressive, life-threatening disease mostly resulting from its inception (April 2012) through September 2013.

- Eculizumab was initiated in April 2012 to prospectively capture postmarketing effectiveness and safety data on patients (TMA) leading to renal and other end-organ damage.

- Even though PE/PI offers no significant benefit over simple supportive therapy, it is characterized by systemic thrombotic microangiopathy that is a result of chronic, uncontrolled complement activation.

### Results

#### Table 1. Patient Demographics in Global aHUS Patient Registry (as of September 25, 2013)

<table>
<thead>
<tr>
<th></th>
<th>Ever Treated With Eculizumab (n=104)</th>
<th>Never Treated With Eculizumab (n=107)</th>
<th>Total (N=211)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Mean age at registry enrollment (SD), years</strong></td>
<td>24.9 (21.00)</td>
<td>25.0 (17.22)</td>
<td>25.0 (19.17)</td>
</tr>
<tr>
<td><strong>Age at registry enrollment, n (%)</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>&lt;2 years</td>
<td>17 (16.3)</td>
<td>2 (1.9)</td>
<td>19 (9.0)</td>
</tr>
<tr>
<td>≥2 to &lt;5 years</td>
<td>8 (7.7)</td>
<td>7 (6.5)</td>
<td>15 (7.1)</td>
</tr>
<tr>
<td>≥5 to &lt;12 years</td>
<td>15 (14.4)</td>
<td>18 (16.8)</td>
<td>33 (15.6)</td>
</tr>
<tr>
<td>≥12 to &lt;18 years</td>
<td>5 (4.8)</td>
<td>16 (15.0)</td>
<td>21 (10.0)</td>
</tr>
<tr>
<td>≥18 years</td>
<td>55 (52.9)</td>
<td>55 (51.4)</td>
<td>110 (52.1)</td>
</tr>
<tr>
<td>N/A</td>
<td>4 (3.8)</td>
<td>9 (8.4)</td>
<td>13 (6.2)</td>
</tr>
<tr>
<td><strong>Sex, n (%)</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Female</td>
<td>58 (55.8)</td>
<td>45 (42.1)</td>
<td>103 (48.8)</td>
</tr>
<tr>
<td>Male</td>
<td>46 (44.2)</td>
<td>54 (50.5)</td>
<td>100 (47.4)</td>
</tr>
<tr>
<td>N/A</td>
<td>0 (0.0)</td>
<td>8 (7.5)</td>
<td>8 (3.8)</td>
</tr>
<tr>
<td><strong>Race, n (%)</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Asian</td>
<td>2 (1.9)</td>
<td>3 (2.8)</td>
<td>5 (2.4)</td>
</tr>
<tr>
<td>Black</td>
<td>5 (4.8)</td>
<td>1 (0.9)</td>
<td>6 (2.8)</td>
</tr>
<tr>
<td>Caucasian/African Caribbean</td>
<td>0 (0.0)</td>
<td>1 (0.9)</td>
<td>1 (0.5)</td>
</tr>
<tr>
<td>Caucasian</td>
<td>96 (92.3)</td>
<td>90 (84.1)</td>
<td>186 (88.2)</td>
</tr>
<tr>
<td>Latino</td>
<td>1 (1.0)</td>
<td>0 (0.0)</td>
<td>1 (0.5)</td>
</tr>
<tr>
<td>Mixed race (Black/Caucasian)</td>
<td>0 (0.0)</td>
<td>1 (0.9)</td>
<td>1 (0.5)</td>
</tr>
<tr>
<td>Mixed, Mother adopt</td>
<td>0 (0.0)</td>
<td>1 (0.9)</td>
<td>1 (0.5)</td>
</tr>
<tr>
<td>Unknown</td>
<td>0 (0.0)</td>
<td>1 (0.9)</td>
<td>1 (0.5)</td>
</tr>
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<td>9 (8.4)</td>
<td>9 (4.3)</td>
</tr>
<tr>
<td><strong>Year of registry enrollment, n (%)</strong></td>
<td></td>
<td></td>
<td></td>
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<tr>
<td>2012</td>
<td>18 (17.3)</td>
<td>3 (2.8)</td>
<td>21 (10.0)</td>
</tr>
<tr>
<td>2013</td>
<td>86 (82.7)</td>
<td>104 (97.2)</td>
<td>190 (90.0)</td>
</tr>
</tbody>
</table>

*aHUS, atypical hemolytic uremic syndrome; N/A, not available; SD, standard deviation.

<table>
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<th>Total (N=211)</th>
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<tbody>
<tr>
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<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Asian</td>
<td>2 (1.9)</td>
<td>2 (1.9)</td>
<td>4 (1.9)</td>
</tr>
<tr>
<td>Black</td>
<td>7 (6.5)</td>
<td>10 (9.5)</td>
<td>17 (8.0)</td>
</tr>
<tr>
<td>Caucasian/African Caribbean</td>
<td>7 (6.5)</td>
<td>7 (6.5)</td>
<td>14 (6.6)</td>
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<tr>
<td>Caucasian</td>
<td>85 (80.2)</td>
<td>85 (80.2)</td>
<td>170 (79.7)</td>
</tr>
<tr>
<td>Latino</td>
<td>1 (1.0)</td>
<td>1 (1.0)</td>
<td>2 (0.9)</td>
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</tr>
<tr>
<td>Unknown</td>
<td>5 (4.8)</td>
<td>5 (4.8)</td>
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<tr>
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<td>9 (8.4)</td>
<td>18 (8.5)</td>
</tr>
</tbody>
</table>

#### Table 2. aHUS Diagnosis Characteristics at Registry Entry (as of September 25, 2013)

<table>
<thead>
<tr>
<th></th>
<th>Ever Treated With Eculizumab (n=107)</th>
<th>Never Treated With Eculizumab (n=104)</th>
<th>Total (N=211)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Mean age at initial symptoms (SD), years</strong></td>
<td>23.8 (21.36)</td>
<td>17.1 (16.36)</td>
<td>20.8 (19.51)</td>
</tr>
<tr>
<td>n=93</td>
<td></td>
<td>n=76</td>
<td>n=169</td>
</tr>
<tr>
<td><strong>Mean age at diagnosis (SD), years</strong></td>
<td>23.9 (21.44)</td>
<td>17.1 (16.79)</td>
<td>20.9 (19.75)</td>
</tr>
<tr>
<td>n=94</td>
<td></td>
<td>n=75</td>
<td>n=169</td>
</tr>
<tr>
<td><strong>Stated family history of aHUS, n (%)</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Yes</td>
<td>16 (15.4)</td>
<td>20 (18.7)</td>
<td>36 (17.1)</td>
</tr>
<tr>
<td>No</td>
<td>88 (84.6)</td>
<td>87 (81.3)</td>
<td>175 (82.9)</td>
</tr>
<tr>
<td><strong>Any identified complement genetic mutation or auto-antibody, n (%)</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>N/A</td>
<td>16 (15.4)</td>
<td>38 (35.5)</td>
<td>54 (25.6)</td>
</tr>
<tr>
<td>No</td>
<td>41 (39.4)</td>
<td>24 (22.4)</td>
<td>65 (30.8)</td>
</tr>
<tr>
<td>Yes</td>
<td>47 (45.2)</td>
<td>45 (42.1)</td>
<td>92 (43.6)</td>
</tr>
</tbody>
</table>

*aHUS, atypical hemolytic uremic syndrome; N/A, not available; SD, standard deviation.

#### Table 3. Baseline Clinical Characteristics of Patients at Registry Entry (as of September 25, 2013)

<table>
<thead>
<tr>
<th></th>
<th>Ever Treated With Eculizumab (n=104)</th>
<th>Never Treated With Eculizumab (n=107)</th>
<th>Total (N=211)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Any prior kidney transplant, n (%)</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Yes</td>
<td>14 (13.5)</td>
<td>20 (18.7)</td>
<td>34 (16.1)</td>
</tr>
<tr>
<td><strong>Any prior dialysis, n (%)</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Yes</td>
<td>58 (55.8)</td>
<td>33 (30.8)</td>
<td>91 (43.1)</td>
</tr>
<tr>
<td><strong>Any prior plasma exchange/infusion, n (%)</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Yes</td>
<td>59 (56.7)</td>
<td>31 (29.0)</td>
<td>90 (42.7)</td>
</tr>
<tr>
<td>Mean baseline eGFR (SD), mL/min/1.73 m²</td>
<td>42.8 (48.11)</td>
<td>76.0 (63.81)</td>
<td>57.0 (57.34)</td>
</tr>
<tr>
<td>n=39</td>
<td></td>
<td>n=29</td>
<td>n=88</td>
</tr>
</tbody>
</table>

*eGFR, estimated glomerular filtration rate; SD, standard deviation.*
Presented at the American Society of Nephrology Kidney Week 2013 Annual Meeting, November 5–10, 2013, Atlanta, Georgia.

**Patient Eligibility Criteria**

**Atypical Hemolytic Uremic Syndrome: Background**

**METHODS**

• A single, global aHUS patient registry can maximize both physician and patient participation to best capture information on disease, safety, and efficacy data in a population with a very rare disease.

• Assessment of the long-term manifestations of TMA complications of aHUS; other clinical outcomes, including plasma exchange and infusion (PE/PI) has been the treatment of choice for aHUS1; however, evidence suggests that targeted laboratory results (including genetic results) and symptomatology can be used to monitor aHUS disease activity.

• Exclusion criteria for patient registry

• Successful registry implementation is contingent on contributions from both academia and the industry sponsor.

• The registry fulfills postmarketing regulatory requirements by providing follow-up on the aHUS indication for patients with aHUS from chronic, uncontrolled complement activation. It is characterized by systemic thrombotic microangiopathy (TMA) leading to renal and other end-organ damage.

• Plasma exchange and infusion (PE/PI) has been the treatment of choice for aHUS1; however, evidence suggests that targeted laboratory results (including genetic results) and symptomatology can be used to monitor aHUS disease activity.

**Inclusion criteria**

• Atypical hemolytic uremic syndrome (aHUS) is a genetic, progressive, life-threatening disease mostly resulting from complement activation.

• Eculizumab (Soliris®; Alexion Pharmaceuticals, Inc., Cheshire, CT, USA), a terminal complement inhibitor, is a targeted laboratory results (including genetic results) and symptomatology can be used to monitor aHUS disease activity.

**Exclusion criteria for patient registry**

• Male or female patients of any age who have been diagnosed clinically with aHUS will be included.

• Previously treated with eculizumab at any time or never treated with eculizumab.

• Review publication drafts before submission to journals or public release.

• Advise, counsel, and guide individuals on publications that utilize aHUS registry data and resources and/or medical records is entered via a secure web portal and maintained anonymously.

• Nephrologists (86%)

• United States, 28.9% (n=61)

• Spain, 9.5% (n=20)

• Italy, 4.3% (n=9)

• Australia, 9.0% (n=19)

**RESULTS**

- Targeted laboratory results (including genetic results)

- HUS due only to Shiga toxin-producing

- Symptomatology

- Academia provides access to global, longitudinal data and increased scientific knowledge to better manage aHUS patients, and

- New clinical sites are encouraged to participate.

- As of September 25, 2013, a total of 211 patients have enrolled in the global aHUS patient registry.

**Milestones Achieved for the Global aHUS Patient Registry**

- Figure 1 shows the milestones that have been reached to date since enrollment of the first patient on April 26, 2012.

**Figure 1. aHUS Patient Registry: A Time Line of Milestones Reached to Date**

[![Figure 1](image-url)](image-url)

- aHUS, atypical hemolytic uremic syndrome; SAB, scientific advisory board.

**CONCLUSIONS**

- The global aHUS patient registry is dedicated to increasing the understanding and awareness of aHUS disease history and progression.

- The results of analyses from collected data and outcomes provide an opportunity to optimize care and improve quality of life for aHUS patients.

- Based on the limited enrollment at this time, reflecting the early stage of the registry, it would be premature to draw scientific conclusions from the data presented herein.

- New clinical sites are encouraged to participate.

- As of September 25, 2013, a total of 211 patients have enrolled in the global aHUS patient registry.

**REFERENCES**


**ACKNOWLEDGMENTS**

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