4204

Baseline Demographics and Characteristics of 532 Patients With Atypical Hemolytic Uremic Syndrome in the Global aHUS Registry

Christoph Licht,[1] Gianluigi Ardissino,[2] Gema Ariceta,[3] David Cohen,[4] Christoph Gasteyger,[5] Larry A. Greenbaum,[6] Masayo Ogawa,[7] Franz Schaefer,[8] Johan Vande Walle,[9] Véronique Frémeaux-Bacchi[10]

[1]The Hospital for Sick Children, Toronto, Ontario, Canada; [2]Ospedale Maggiore Policlinico, Milan, Italy; [3]Hospital Universitario Cruces, Barakaldo, Spain; [4]Columbia University Medical Center, New York, NY, USA; [5]Alexion Pharmaceuticals, Lausanne, Switzerland; [6]Emory University, Atlanta, GA, USA; [7]Alexion Pharmaceuticals, Inc., Cheshire, CT, USA; [8]Heidelberg University Medical Center, Heidelberg, Germany; [9]UZ Gent Dienst Nefrologie, Ghent, Belgium; [10]Assistance Publique-Hôpitaux de Paris, Hôpital Européen Georges Pompidou, Paris, France

INTRODUCTION

- Atypical hemolytic uremic syndrome (aHUS) is a rare, life-threatening disease of chronic, uncontrolled complement activation leading to systemic thrombotic microangiopathy (TMA) and end-organ damage[1,2]
- Plasma exchange/plasma infusion (PE/PI) historically has been used to manage aHUS but provides little clinical benefit compared with supportive therapy[1,2]
- Eculizumab, a terminal complement inhibitor, is a humanized monoclonal antibody that binds with high affinity to the human C5 complement protein, blocking the generation of the pro-inflammatory C5b-9 terminal complement complex[3,4]
- Eculizumab is the first approved treatment for aHUS in pediatric and adult patients[3-5]
- The global aHUS Registry (US National Institutes of Health www.ClinicalTrials.gov Identifier NCT01522183) was initiated in April 2012 to record information on the progression of disease in all aHUS patients and to prospectively collect postmarketing effectiveness and safety data on patients treated with eculizumab
- 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
  - The industry sponsor fosters relationships with academic partners 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 and treatment safety and efficacy data in a population with a very rare disease

OBJECTIVE

- To report baseline demographics and patient characteristics of, and describe important milestones achieved by, patients enrolled in the global aHUS Registry from its inception through September 30, 2014

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Patient Eligibility Criteria

- Inclusion criteria
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    - With or without identified complement abnormalities or anti-complement factor antibody (if tested)
    - ADAMTS13 activity >5%, if performed
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Data Collection

- Data collected at study enrollment and every 6 months thereafter include:
  - Demographics
  - Medical and disease history
  - Symptomatology
  - Targeted laboratory results (including genetic results)
  - TMA complications
  - Associated treatments and concomitant medications
  - Clinical and patient-reported outcomes
  - Safety of eculizumab and other aHUS treatments
- 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

Primary Outcome Measures

- The proportion of patients who experience specified events
- 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
- Assessment of long-term manifestations of TMA complications of aHUS and other clinical outcomes

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- The Registry is supported by Alexion Pharmaceuticals, Inc., with governance by an independent scientific advisory board (SAB) (**Table 1**) and national coordinators representing each participating country

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- Provide scientific advice on global aHUS Registry-related matters
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aHUS, atypical hemolytic uremic syndrome; SAB, scientific advisory board.

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- The following data were required for enrolled patients to be included in this analysis:
  - Registry enrollment date, date of birth, and gender
  - For treated patients, date of first eculizumab treatment

RESULTS

Enrollment

- As of September 30, 2014, a total of 532 patients have enrolled in the global aHUS Registry
- The countries from which patients were enrolled are shown in **Table 2**
- Characteristics of enrolling clinicians and sites are displayed in **Figure 1**

Table 2. Countries Enrolling Patients in the Global aHUS Registry (as of September 30, 2014)

Country, n (%)	N=532
United States	112 (21.1)
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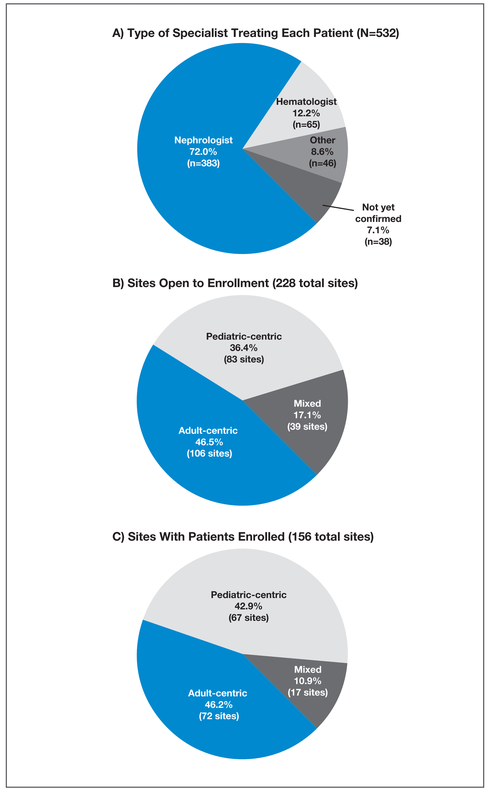

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- **Tables 3–6** provide information on demographics, aHUS diagnosis, baseline clinical, and eculizumab treatment characteristics
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Demographic Characteristic	Ever Treated With Eculizumab (n=304)	Never Treated With Eculizumab (n=228)	Total (N=532)
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Mean baseline eGFR, mL/min/1.73 m^2 (SD)	25.9 (34.9) n=86	72.0 (48.7) n=99	50.6 (48.6) n=185

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Eculizumab Treatment Characteristic	Ever Treated With Eculizumab (n=304)
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≥5 to <12 years	45 (14.8)
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≥18 years	181 (59.5)
Mean total time on eculizumab by age group, years (SD)	
<18 years (n=65)	0.7 (0.75)
≥18 years (n=139)	0.3 (0.51)
All (n=204)	0.4 (0.62)

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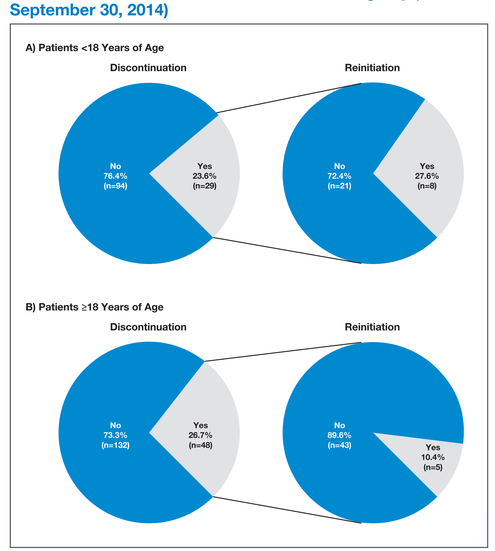
Figure 2. Proportion of Patients Who Discontinued and Reinitiated Eculizumab in the Global aHUS Registry (as of September 30, 2014)



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Milestones Achieved for the Global aHUS Registry

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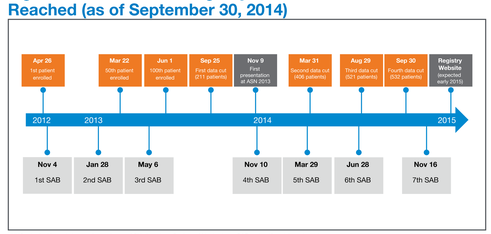

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CONCLUSIONS

- The global aHUS Registry is dedicated to increasing the understanding and awareness of aHUS disease history and progression
- Results of analyses from collected data and outcomes provide an opportunity to optimize care and improve quality of life for patients with aHUS
- Although enrollment has increased considerably since initiation of the global aHUS Registry, caution should be exercised when formulating scientific conclusions based on the data presented herein
- New clinical sites are encouraged to participate
- As of September 30, 2014, a total of 532 pediatric and adult patients have enrolled in the global aHUS Registry

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DISCLOSURES

C. Licht: Alexion Pharmaceuticals–Consultancy, honoraria, membership on the Board of Directors/Advisory Committees, research funding, and speakers bureau; Achillon Pharmaceuticals Consultancy, honoraria, membership on the Board of Directors/Advisory Committees, research funding, and speakers bureau. **G. Ardissino:** Alexion Pharmaceuticals–Consultancy, membership on the Board of Directors/Advisory Committees, and speakers bureau. **G. Ariceta:** Alexion Pharmaceuticals–Consultancy and membership on the Board of Directors/Advisory Committees. **D. Cohen:** Alexion Pharmaceuticals–Consultancy. **C. Gasteyger:** Alexion Pharma International Sàrl–Employment and equity ownership. **L.A. Greenbaum:** Alexion Pharmaceuticals–Consultancy, honoraria, membership on the Board of Directors/Advisory Committees, and research funding. **M. Ogawa:** Alexion Pharmaceuticals–Employment. **F. Schaefer:** Alexion Pharmaceuticals–Consultancy, membership on the Board of Directors/Advisory Committees, and research funding. **J. Vande Walle:** Alexion Pharmaceuticals–Consultancy, honoraria, membership on the Board of Directors/Advisory Committees, research funding, and speakers bureau. **V. Frémeaux-Bacchi:** Alexion Pharmaceuticals–Consultancy, membership on the Board of Directors/Advisory Committees, and research funding; CLS Behring–Honoraria.

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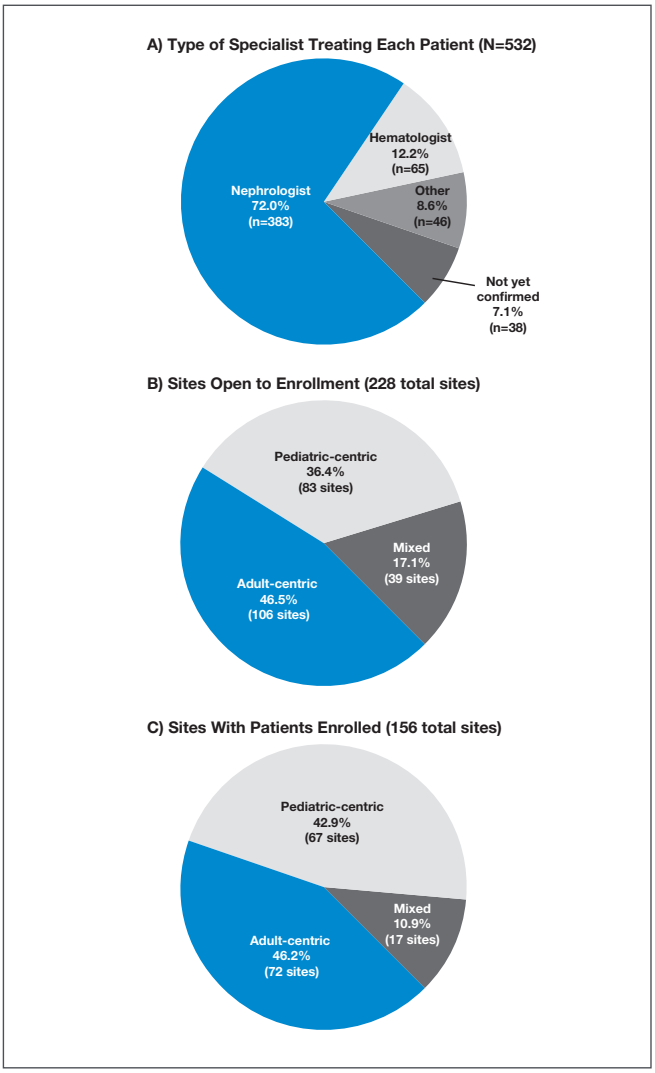

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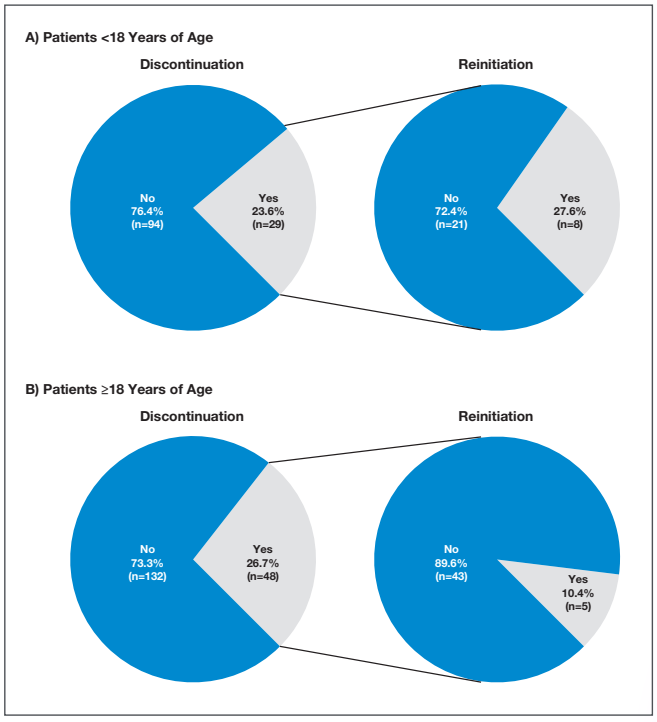

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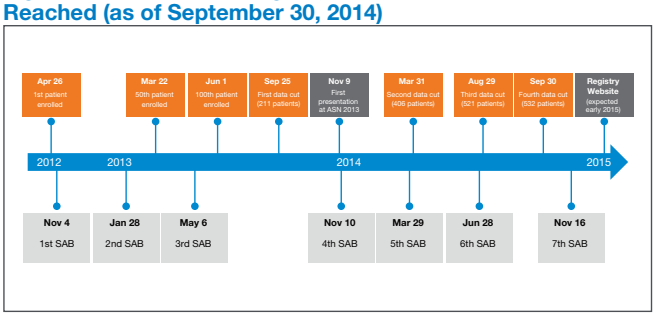

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DISCLOSURES

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