

Access to aHUS Treatment: 2016 aHUS Global Poll White Paper

Orphan Drug Access – Are Rare Disease Treatments like Eculizumab Equally Available Worldwide?

In an era of social media, sharing patient stories has become increasingly common and has connected rare disease patients formerly isolated by geographic distance from others affected by the same medical condition. The aHUS Alliance, a multi-national affiliation of patient organizations, collaboratively launched a 2016 aHUS global poll for patients and caregivers of pediatric patients, to gain information and insight into varied aspects of diagnosis, treatment, and the entire patient experience. Data was also collected to offer insight into how medical and research teams may better understand patient needs and interests, to improve this interface and optimize all facets of patient care, with the goal to improve patient outcomes. This article explores the 2016 aHUS Patient Global Voice for question 21: “Is the complement inhibitor eculizumab currently available in your country?”

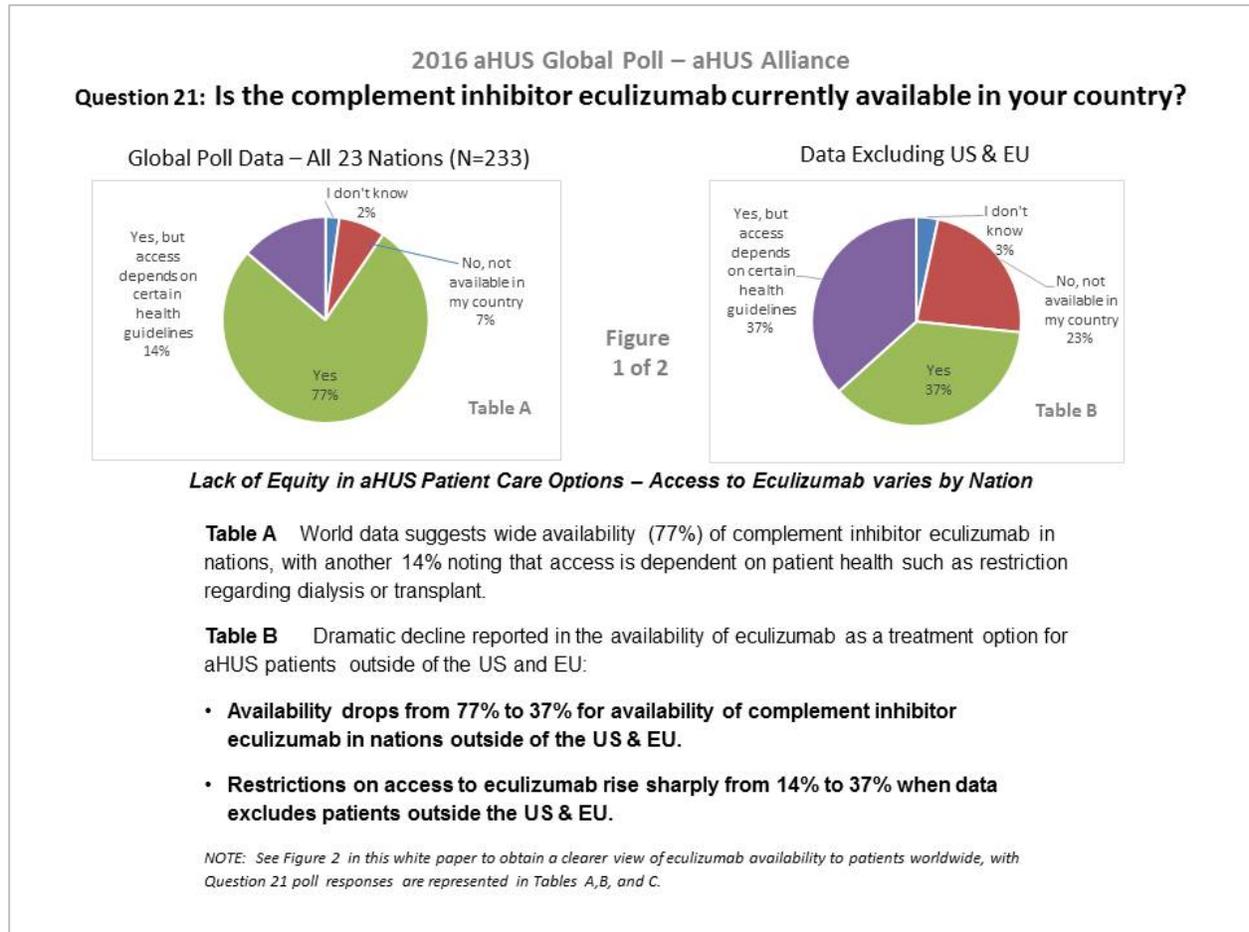
The aHUS Alliance reviewed the 2016 poll results, with 233 respondents from 23 countries, to gauge the degree to which the global aHUS patient community has access to eculizumab, currently the only drug used for treatment of aHUS patients. Data collected illustrates a sharp disparity in access to treatment dependent on the patient’s country. (See Figure 1, **Tables A and B**)

When aHUS global data includes the high number of poll responses from the USA (N=100), where eculizumab is available to aHUS patients regardless of health status, fully three-quarters of 2016 respondents (77%) were able to receive this drug should their physician prescribe therapeutic dosing of eculizumab (Table A). An additional 14% note that eculizumab is available conditionally in their nation but not for all aHUS patients, with the most common patient restriction being a denial for aHUS patients on dialysis but needing eculizumab for a kidney transplant. (Note: Alexion Pharmaceuticals, makers of eculizumab, currently lists 26 countries on their corporate site’s [Global Presence](#) page.)

The relatively rosy global picture of most aHUS patients having access to eculizumab as noted in Table A dims with the harsh reality, a marked decline within 2016 poll statistics that illustrates eculizumab is not available in most countries to some or all aHUS patients (Table B). Access to eculizumab for treatment of all aHUS patients worldwide plummets from 77% to only 37% of poll respondents in nations outside of the USA and Europe.

Disparity in treatment options is further revealed in the wide difference between eculizumab availability in countries limiting use within certain aHUS medical restrictions; there is a sharp increase in restricted use from only 14% globally (Table A) to a jump of 37%, or over 1 in 3 patients in countries having difficulty in obtaining eculizumab in nations outside the USA and EU (Table B). Treatment options are few for aHUS patients outside the USA and Europe, with 3 out of 5 respondents (60%) having no or limited access to eculizumab, as 23% note that eculizumab is not available at all and additionally 37% report their country places restrictions on its use for aHUS patients (Table B). Simply put, physicians in the majority of nations worldwide do not have this orphan drug available within hospitals, clinics, or

other healthcare settings despite medical reports and research that demonstrates the efficacy of eculizumab in treatment of both adult and pediatric aHUS patients.

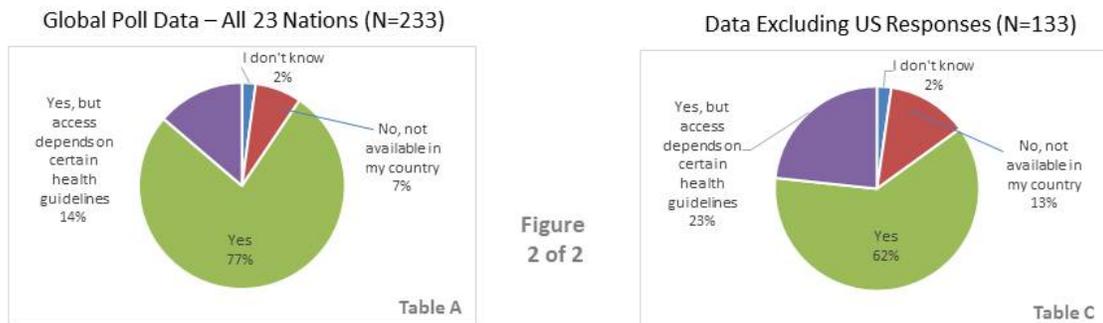


To provide additional insight, **Figure 2 Table C** provides a different view by omitting the high USA response rate to highlight data received from the other 22 nations participating in the 2016 aHUS global poll. When USA poll responses are omitted, eculizumab availability drops from 77% to only 62% of aHUS patients noted to have full access to this drug in their country. Treatment access around the world is conditionally restricted in many nations represented in the aHUS survey, evidenced by the steep increase from 14% to 23% of 2016 poll responses categorizing their nation as one that limits eculizumab availability to only select aHUS patient medical situations.

Simply put, 2016 aHUS poll results indicate that physicians in the majority of nations worldwide do not have this orphan drug available for aHUS patients within hospitals, clinics, or other healthcare settings despite medical reports and research that demonstrates the efficacy of eculizumab in treatment of both adult and pediatric aHUS patients.

2016 aHUS Global Poll – aHUS Alliance

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



Lack of Equity in aHUS Patient Care Options – Access to Eculizumab varies by Nation

Table A Data from 233 respondents in 23 nations reports wide availability (77%) of complement inhibitor eculizumab to treat aHUS patients and maintain renal transplants, with another 14% noting limited access.

Table C Eculizumab is widely available in the US, and the 2016 aHUS Global Poll response rate from the US was high (N=100). Excluding the US, access to eculizumab declines from 77% to 67%, which sharply drops to only 37% of aHUS patients able to be treated with eculizumab when both US & EU nations are excluded (See Figure 1, Table B.) **Overall:**

- Less than 4 in 10 aHUS patients (37%) have eculizumab broadly available as a treatment option if they live in a country outside of the US or EU. 60% of aHUS patients outside the US & EU have no or limited access to eculizumab. (Not Available- 23%, Access Restricted- 37%; Figure 1, Table B)
- Restrictions on access to eculizumab rise from 14% to 23% when US data is excluded, but limited access to eculizumab jumps to 37% for patients living in nations outside the US & EU. (Figure 1, Table B)

NOTE: See Figure 1 in this white paper to obtain a clearer view of eculizumab availability to patients worldwide, with Question 21 poll responses are represented in Tables A, B, and C.

The aHUS Alliance understands the difficulties in bringing an orphan drug to market, the rigors of pharma R & D, stringent scientific requirements, and the myriad worldwide variations among governmental regulatory environments. Drug safety and supply, intellectual property protection, and national healthcare systems all impact patient healthcare options - as does treatment cost. Our alliance of nations appreciates that a bevy of socio-economic and geo-political issues come wrapped around hard economic realities, but rare disease patients now connect to an expanding pool of information and medical advancements through global advocacy efforts. Patients with aHUS face the challenge of knowing that this chronic and life-threatening disease may cause a catastrophic medical event without warning, yet we are lucky – reportedly 95% of rare disease patients do not have a drug, therapy or treatment for their condition.¹

Atypical HUS patients are rare even among the 6000 to 7000 different rare disease populations. Rare diseases affect millions of people worldwide, although in the USA to be defined as a rare disease means fewer than 200,000 Americans would be affected (2016 USA pop 2016 is about 324 million), and a disease or disorder in Europe is defined as rare when it affects fewer than 1 in 2000 people.² For those patients worldwide challenged by atypical HUS ours is an ultra-rare patient population, since aHUS is estimated to affect less than 2 people in a million.³

These 2016 aHUS global poll results clearly underscore the lack of equity in aHUS treatment options available in countries outside of the USA and EU, despite availability and effectiveness of eculizumab therapy for aHUS patients of all ages with varying degrees of kidney function. Without access to eculizumab, aHUS patients will languish on dialysis while co-morbidities mount – even though success in maintaining a renal graft with eculizumab therapy has been documented in medical literature.

“A good medication for rare disease patients is a medication that is both available in the country where they live and affordable. The drug is of little use if one of these two factors is missing.” (Quote from EURORDIS website.⁴) Development of orphan drugs and bringing them to market is difficult from a variety of perspectives. The 2016 aHUS global poll included multiple survey questions that address treatment availability and the impact of cost on aHUS patient care options, with full poll results available through the aHUS Alliance’s global networking hub at www.ahusallianceaction.org, including the [RareConnect webinar](#) analyzing 2016 survey results.

In conclusion, 2016 aHUS global poll data presents an overall picture of patient care and aHUS treatment options that vary widely from nation to nation, pointing to a worldwide lack of equitable and effective healthcare available for atypical HUS patients .

Citations

1. According to the EveryLife Foundation for Rare Diseases, although some groups have multiple treatment options. (former name Kakkis Foundation) <http://everylifefoundation.org/>
2. Rare Disease Day, Statistics – Annually recognized the last day of February. <http://www.rarediseaseday.org/>
3. Maga TK, Nishimura CJ, Weaver AE, Frees KL, Smith RJH. Mutations in alternative pathway complement proteins in American patients with atypical hemolytic uremic syndrome. Hum Mutat. 2010b;31:E1445–60. [[PubMed](#)]
4. EURORDIS: The Voice of Rare Disease Patients in Europe. Website article: ‘Access to Orphan Drugs’ <http://www.eurordis.org/content/access-orphan-drugs>

2016 aHUS Global Poll – Results & Resources

aHUS Alliance Website – Graphs of all 2016 aHUS Global Poll Results, Links to the Poll Questions, Blogs, RareConnect Webinar, and more at <http://ow.ly/f5sl3033y0x>

RareConnect – 21 June 2016: aHUS Webinar, hosted by RareConnect and featuring poll commentary and aHUS information presented by Dr. Christoph Licht of Toronto CA Sick Children’s Hospital. Video, slide downloads, and more at <http://ow.ly/Aj0d3033y4o>

Additional Information & Resources

[Eculizumab Prevents Thrombotic Microangiopathy: Long-Term Follow-up Study of Patients with Atypical Hemolytic Uremic Syndrome.](#) (Courtesy of ASH Conf. Dec 2015)

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[Discontinuation of eculizumab maintenance treatment for atypical hemolytic uremic syndrome: a report of 10 cases.](#) *Am J Kidney Dis.* 2014 Oct;64(4):633-7. doi: 10.1053/j.ajkd.2014.01.434. Epub 2014 Mar 19. ([Associated content, Graph](#): Ardissino et al: courtesy of aHUSUK)

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[Advances and challenges in the management of complement-mediated thrombotic microangiopathies.](#) *Therapeutic Advances in Hematology.* 2015 Aug; 6(4): 171–185. doi: [10.1177/2040620715577613](https://doi.org/10.1177/2040620715577613)

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aHUS Awareness Day is marked annually on 24 September.

Follow the global campaign to raise aHUS visibility on **Twitter @aHUS24Sept**