aHUS Key Facts & Information

Atypical Hemolytic Uremic Syndrome

2017 September

Re-Thinking aHUS in 2017

Diagnosis: Modified Hamm Test for atypical Hemolytic Uremic Syndrome (Gavriilaki, E et al 2015)

Multi-Disciplinary Care Teams: Thrombotic Microangiopathy: A Multidisciplinary Team Approach (Gordon et al, 2017)

Atypical HUS is a type of TMA: Clinical evaluation of thrombotic microangiopathy: identification of patients with suspected atypical hemolytic uremic syndrome (Yu-Min Shen, 2016)

aHUS Alliance

CONNECT ► INFORM ► COLLABORATE

An innovative and collaborative partnership between patients with rare disease and industry-supported registries: the Global aHUS Registry (Woodward, L et al 2016)

aHUS Clinicians & Investigators – A Global Networking Hub

aHUS Advocacy & Patient Organizations – Connecting global aHUS advocates and efforts

Recent Research – aHUS & Thrombotic Microangiopathy

Atypical HUS (A Year in Research)

A look at aHUS research in over the last 12 months (2017), NCBI: US Library of Medicine

Thrombotic Microangiopathy, TMA (A Year in Research)

A look at TMA research in over the last 12 months (2017), NCBI: US Library of Medicine

---

About aHUS

- Atypical Hemolytic Uremic Syndrome (aHUS) is a very rare, chronic and life-threatening genetic condition
- aHUS can occur at any age, with roughly 60 per cent of children affected and 40 per cent adults [2]
- aHUS is caused by chronic, uncontrolled activation of the complement system, a part of the body's natural immune system [1]

- As a result, the immune system attacks the body's unhealthy and healthy cells, which can cause abnormal blood clotting and blood vessel damage [2, 3]
- The presence of blood clots causes damage to organs, leading to heart attack, stroke, kidney failure and death [2]
- Within a year of diagnosis, over half of patients will need dialysis, will have irreversible kidney damage, or will not survive [2]
- The majority of patients progress to end-stage kidney failure within three years of diagnosis [2,5]
- Death rates amongst aHUS patients are as high as 25 per cent, and progression to end-stage kidney disease occurs in more than 50 per cent of patients [2, 5]
- Kidneys are often transplanted in aHUS patients with permanent kidney failure, however, the disease recurs in 60 per cent of patients, and more than 90 per cent of patients experience failure of transplanted kidney [2]

Diagnosis

- Atypical HUS encompasses a group of diseases that share in the clinical features of a microangiopathic hemolytic anemia associated with thrombocytopenia and renal failure. In practice there is little agreement on what defines or limits classifying someone as an aHUS patient, given the nonspecific nature of the term aHUS. aHUS clumps together a group of diseases with very different underlying pathologies. [25]
- The causes of aHUS are not fully understood, but in 70 per cent of cases it is associated with an underlying genetic or acquired abnormality of the complement system [10]
- Doctors and their healthcare team must look at many factors when making a diagnosis – including clinical symptoms, lab findings, and results from more specialized tests such as gene analysis [11]
- During initial onset of aHUS, or during recurring episodes, tell-tale signs can be detected from lab findings relating to [9]
    - platelet levels
    - hemoglobin and haptoglobin levels
    - creatinine levels
    - BUN (blood urea nitrogen) levels

Symptoms

- aHUS disease can be characterized by three key features:[12]
    - thrombocytopenia (low platelet count in the blood)
    - anemia (low red blood cell/platelet count in the blood)
    - kidney symptoms (starting as acute kidney failure but can progress to end-stage kidney disease)
- There are a number of symptoms secondary to kidney failure, which include [10]
    - nausea and vomiting
    - confusion
    - shortness of breath (dyspnea)
    - fatigue
- aHUS can impact multiple organs and body systems. Central nerve system involvement is the most frequent extra-renal organ manifestation of aHUS (10–48%)[29] , but issues due to TMA may occur in the heart, lungs, GI tract, skin, eyes as well.
- In aHUS, patients present with symptoms of diarrhea, fatigue, irritability, and lethargy to a point where hospitalization is needed [12]
- The majority of patients have genetic abnormalities that impair cell surface control of complement [18]

Treatment

Plasma Therapy & Dialysis

- The prognosis for patients with aHUS is very poor,[13] with existing supportive therapies unproven and unreliable
- The management of aHUS has relied on plasma infusion and plasma exchange therapies with variable results[14]
- To date, there have been no well-controlled trials that show plasma exchange or plasma infusion to be safe or effective in aHUS [15]
- In studies where the majority of patients with aHUS were treated with plasma therapy, patient outcomes were reported as being poor[16]
- Despite plasma exchange or plasma infusion, 65 per cent of all aHUS patients die, require dialysis, or have permanent renal damage within the first year after diagnosis [6]
- Dialysis cannot completely compensate for the loss of kidney function, and can lead to deadly infections and shortened life expectancy [17]
- Complications related to plasma exchange have been reported to occur in up to 55 per cent of plasma exchange sessions in children and in 15 per cent of sessions in adults [16]

Treatment - Therapeutics

- Eculizumab has shown greater efficacy than plasma therapy in the prevention and treatment of aHUS [16, 19]
- In June 2013, an international study in the *New England Journal of Medicine* showed aHUS patients treated with eculizumab were able to discontinue plasma infusion/exchange and dialysis therapies, and saw improved kidney function, reduced blood vessel damage and decreased risk of blood clots [22]
- KDIGO Controversies Conference on aHUS and C3G: See Treatment Strategies, Section V. Goodship et al, Dec 2016. Kid. Intl. http://ow.ly/DCjf30euh7n
- aHUS Alliance – Therapeutic Drugs, R & D Landscape. Jan 2017. http://ow.ly/vlDJ30euhZQ
- Clinical Trials – Currently recruiting as of Sept 2017 on www.ClinicalTrials.gov (atypical HUS): ALXN1210 and OMS721, with more aHUS clinical trials expected soon. Click HERE for clinical trial updates.

Access to Treatment

- As of Sept 2017 aHUS patients in many nations still do not have access to eculizumab, and coverage within some of those countries is further restricted: dependent on the aHUS patient's location within their nation or their individual health status. [26,28]
- In September 2013, National Health Service (NHS) England recommended that eculizumab be funded for aHUS patients, following a positive reimbursement recommendation from the Clinical Priorities Advisory Group (CPAG). The final draft guidance recommending eculizumab for funding for treating aHUS was issued by the National Institute for Health and Care Excellence (NICE) in November of 2014.
- Inequality in Treatment Options among Nations - Access to eculizumab for treatment of aHUS patients worldwide plummets from 77% to only 37% for poll respondents in nations outside of the US & EU. [28] (White Paper at http://ow.ly/Dbzb303ZqhU, with 2016 Poll Results: http://ow.ly/1DA7303FoJx)

Note: The aHUS Alliance wishes to extend thanks to aHUS Canada for their efforts in providing core facts contained in this document.

SOURCES: See our CITATIONS section later in this document

A Short Version of this Document is Available HERE

Diseases/Disorders: Potential for Cross-Over to aHUS Research

AAV	(ANCA)-associated vasculitis Note: ANCA – anti-neutrophil cytoplasmic Abs
AMD	Age-related Macular Degeneration
AMR	Antibody mediated rejection
CAD	Cold Agglutinin Disease
CAD	Coronary Artery Disease
CMV	Cytomegalovirus
C3G	C3 glomerulopathy, Subtypes: Dense deposit disease (DDD) and C3 glomerulonephritis (C3GN)
CMND	Complement-Mediated Neurodegeneration
COPD	Chronic Obstructive Pulmonary Disease
CVD	Cardiovascular DiseaseÂ
DDD	Dense Deposit Disease (see also C3G)
DFG	Delayed Graft Function
DM	Dermatomyositis
GBS	Guillain “Barr Syndrome
GvHD	Acute Graft v Host Disease
HAE	Hereditary Angioedema
HSCT	Hematopoietic Stem Cell Transplant
IBMIR	Instant Blood-mediated Inflammation Reaction
IgAN	Immunoglobulin A Nephropathy (form of glomerulonephritis) Note: IgA – Immunoglobulin A
IRI	Ischemia-reperfusion Injury
MG	Myasthenia Gravis
MMN	Multifocal Motor Neuropathy
MPGN	Membranoproliferative glomerulonephritis
NMOSD	Relapsing Neuromyelitis Optica Spectrum Disorder
PNH	Paroxysmal Nocturnal Hemoglobinuria

RA	Rheumatoid arthritis
SLE	Systemic Lupus Erythematosus
STEC	HUS – Shiga toxin-releasing Escherichia coli-Hemolytic Uremic Syndrome
TMA	Thrombotic microangiopathy (often plural)
TTP	Thrombotic Thrombocytopenic Purpura

Note: Research done for other complement-mediated diseases, or those with similar underlying mechanisms, may provide knowledge to advance aHUS research and therapeutic drug discovery. Listed are some diseases for which future investigations may provide cross-over information for aHUS researchers.

FMI please visit http://www.ahusallianceaction.org/ahus-therapeutic-drugs-research-development/

Advancing aHUS Treatment - Pipeline of R & D for new Therapeutics

(Table created Sept 2017) [26,27]

Therapeutic Drug Discovery
aHUS and other Complement Mediated Diseases

COMPANY	DRUG/Molecule	TARGET/Mechanism	Other INFO
Alexion	ALXN1210	longer-acting C5 inhibitor	aHUS and PNH
	ALXN1007	C5a	Focus: GvHD
	ALXN1102 (TT30)	C3	Focus: PNH
	Soliris®/ eculizumab	C5	aHUS, PNH, More
Achillion	ACH-4471	Factor D	Focus: PNH Complement Mediated Diseases
ADIENNE Pharma & Biotech	MUBODINA®	C5	Focus: Typical HUS
Akari Therapeutics	Coversin®	C5	PNH Trial, Initial Data (PNH)
Alnylam	ALN-CC5	RNAi, C5	Focus: PNH, and Others
Amgen	ABP 959	Biosimilar to eculizumab, C5	ANZCTR Trial
Amyndas Pharmaceuticals	AMY-101	C3, Cp40	Focus: C3G, PNH, Others
	AMY-201	C3	Other: mini-FH

Annexon	ANX005	C1q	Alternative pathway, Auto-immune, Clinical Trial
Apellis	Compstatin®/APL-2	C3	Clinical Trials: PNH, AMD, Other
ChemoCentryx	CCX168 Avacopan	C5aR	AAV , other
Genentech	Lampalizumab	Factor D (FCFD4514S, RG7417)	Clinical Trial: Eyes
	Rituxan/Rituximab	CD20	Focus: MPGN, IgAN, Other also RA
Greenovation	Moss-FH	Factor H, C3	Complement Disorders, Plant-based
InflaRx	IFX-1/ IFX-2	C5a	Clinical Trials AAV, other
Inflazyme	Mirococept®/APT070	C3 convertase inhibitor	Focus: IBMIR, IRI
LFB Group	hCFH	Factor H	**Focus: Factor H, DDD**
Novartis (Sandoz)	LFG316	C5	Clinical Trials: PNH, also Transplant
	KRP203	S1PR	Clinical Trials
	CFZ533	**CD40**	Clincal Trials
NovelMed	**NM9401**	Properdin	Pharma Focus: PNH, Hemodialysis, Others
Omeros	OMS721	MASP-2 ,Lectin pathway	Clinical Trials: aHUS TMA, and IgAN & Others
	OMS906	MASP-3 ,Alternative pathway	Early Focus: PNH, aHUS, Others
Opthotech	Zimura (ARC1905)	**C5**	Clinical Trial: AMD
Ra Pharma	RA101495	C5, oral small molecules	Focus: PNH, LN, MG, Others Clinical Trial PNH
Resverlogix	RVX-208/apabetalone (RVX-000222)	BET	ESRD w/ Hemodialysis CAD BETonMACE for CVD
Sobi	SOBI005	**C5**	Focus: PNH, aHUS
True North, Acquired by BIOVERATIV	BIVV009, formerly **TNT009**	C1s, Classical Pathway	Clinical Trial: Complement Mediated Disorders Autoimmune Hemolytic Anemia, CAD
Various Pharma	Cinryze, Berinert, Ruconest, Others	C1-INH	Clinical Trials

**Clinical Trials, Stages of Development: http://www.nlm.nih.gov/services/ctphases.html*
Check for updated information on Clinical Trials at www.ClinicalTrials.gov
aHUS Alliance (Jan 2017) - aHUS Therapeutic Drug Pipeline

2016 aHUS Global Poll: aHUS Patient Voice [28]

An international poll of aHUS patients and pediatric caregivers was launched on 29 February 2016 (world Rare Disease Day) and was completed 15 April 2016. The poll was offered in 6 languages and contained 45 questions to include patient profiles as well as diagnosis and treatment experiences. Additional information and insights were sought regarding aHUS challenges, patient engagement views, clinical trials, and orphan drug development issues.

233 respondents from 23 countries provided data for the 2016 aHUS Global Poll, with results reported within these assets, graphs and commentary:

- 2016 aHUS Global Poll OVERVIEW: http://ow.ly/gSj8303GcdH
- 2016 aHUS Global Poll, RESULTS & Graphs: http://ow.ly/1DA7303FoJx
- RareConnect 2016 aHUS Poll Webinar (commentary by Dr. C Licht): http://ow.ly/ACiN303GajE

2014 aHUS Poll: *In Collaboration with RareConnect, previous aHUS poll Results & Webinar with commentary by Dr. T Goodship:* http://ow.ly/hRau303OZG2

aHUS Insights – Select Info from the 2016 Global Poll [28] ***– (View Complete Data at Links above)***

Poll respondent Profile - 48% of responses were from caregivers of pediatric aHUS patients, with the remaining 52% of data representing adult patients. 66% of people completing the 2016 aHUS global poll were female, 34% were male.

- Response rate by Nation – Of the 23 countries participating in the 2016 poll, respondents living in these three countries had the highest participation rates with the other 20 nations created less than 10% of survey responses. (The poll was available in 6 languages:EN, ES, FR, RUS, IT and JPN)
  USA - 43% UK - 18% Canada - 11%
- Genetic Testing – 84% have or are awaiting Genetic Test Results
- aHUS Info Sources - When seeking Information, most:
- Check aHUS Patient Organizations– 37% Rely on their Doctor – 17% Utilize Search Engines – 26%

- Dialysis - 46% of poll respondents stated the most significant dialysis issue was it interferes with normal routines. Other dialysis issues:
  - Impact on Other ORGANS 29%
  - Negative affect on QUALITY at Work/School 28%
  - Issues with ANXIETY or DEPRESSION 27%

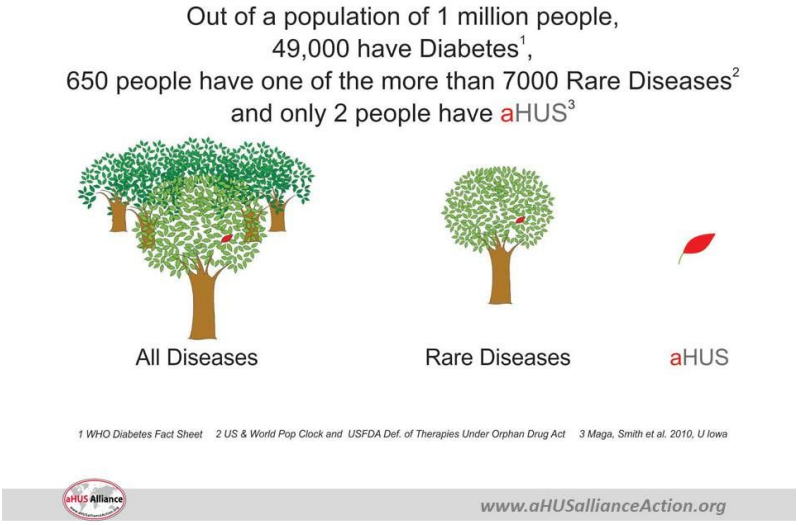
- aHUS Research - Participation 50% of Respondents have already done so, and 36% more would like participate but don't know how to engage.

- Inequality in Treatment Options among Nations - Access to eculizumab for treatment of aHUS patients worldwide plummets from 77% to only 37% for poll respondents in nations outside of the US & EU. (White Paper at http://ow.ly/Dbzb303ZqhU)

- COST Impact - 7 out of 10 state their specialist or medical team mention COST of aHUS treatment in discussing patient care options. 16% state cost concerns affect their treatment options or medical care.

- COST Treatment Access - 24% of respondents state aHUS medical care or treatment is limited by their National or Heath Ministry policies. 29% note that cost of medical care and treatment concern them and their family.

- Advancements in aHUS treatment or drug therapies- Factors or key considerations for use:
  - Cost of new drugs would likely affect our usage - 33%
  - Recommendation of our medical team - 28%
  - Type of drug delivery/Ease & Convenience of New Treatment - 24%



2016 aHUS Global Poll, RESULTS & Graphs: http://ow.ly/1DA7303FoJx

DIRECTORY of aHUS Patient Organizations: http://ow.ly/TlLw303QQGn

Access to aHUS Treatment: 2016 aHUS Global Poll White Paper – click here to view
aHUS and Dialysis Insights: 2016 aHUS Global Poll White Paper – click here to view

Press Kit: aHUS Alliance - click here to view

Resources – More about aHUS

*In ENGLISH: Disease OVERVIEW with definitions & research links

NCBI GeneReviews©, affiliated with the National Institutes of Health (NIH)
http://www.ncbi.nlm.nih.gov/books/NBK1367/

*In ENGLISH: OVERVIEW with detailed Info & Tables on aHUS triggers, genetics, extra-renal involvement (aHUS affecting other organs), and other topics

Kavanagh D, Goodship T H, and Richards A. Atypical Hemolytic Uremic Syndrome. Semin Nephrol 2013 Nov; 33(6): 508–530. doi: 10.1016/j.semnephrol.2013.08.003 http://ow.ly/QjUD303Tqlp

*In ENGLISH: Pediatric Focus

An international consensus approach to the management of atypical hemolytic uremic syndrome in children, Abstract: http://www.ncbi.nlm.nih.gov/pubmed/25859752

*In ENGLISH and Multiple Languages: KDIGO GLOBAL CONSENSUS

An international consensus approach to the diagnosis and management of patients with complement-mediated kidney disease, such as aHUS. *Atypical hemolytic uremic syndrome and C3 glomerulopathy: conclusions from a "Kidney Disease: Improving Global Outcomes" (KDIGO) Controversies Conference* (Goodship, THJ et al, 2017)

*aHUS Clinical & Diagnostic Checklist, Courtesy of www.RareRenal.org (UK): http://ow.ly/BuOR303SaLv

*Atypical HUS Clinical Channel - YouTube: http://ow.ly/mSyT303ZDch

Atypical HUS Patient Voice - YouTube

Rare Diseases – Fast Facts

- There are approximately 7,000 diseases and conditions designated as a rare disease, each affecting fewer than 200,000 Americans. In Europe, a disease is considered rare if it affects fewer than 1 in 2,000 people.
- Rare diseases as a group affect an estimated 25 to 30 million Americans, 1 out of 10 people. Eighty percent of rare diseases are genetic in origin, and it is estimated that about half of all rare diseases affect children.

EURORDIS: Founders of Global Rare Disease Day: Info & Resources www.eurordis.org
NORD: Rare Disease Day Info & Resources, specific to the USA www.rarediseases.org
RareConnect : Disease-Specific Webpages, sponsored by NORD and EURORDIS www.rareconnect.org

These organizations provide information, services, resources, and support to the rare disease community. Their Rare Disease Day resources include press kits, social media tools, Rare Disease Day graphics and more.

World Rare Disease Day, recognized annually on the last day of February, encourages patients and their families, medical professionals, researchers, government officials, and companies developing treatments for rare diseases to join together to focus attention on rare diseases as a public health issue.

Rare Disease Day

aHUS Awareness Day is marked annually on 24 September

Created by the aHUS Alliance in 2015, and marked in various nations around the world, aHUS Awareness Day provides an opportunity for individuals and organizations around the world to join together in support of people living with aHUS. An opportunity to provide aHUS insights, information and outreach, we encourage participation of all stakeholders who seek to provide advancement for patients globally. Learn More about

aHUS Awareness Day 2017 - aHUS Alliance

CITATIONS

[1] Genetics Home Reference. Atypical hemolytic-uremic syndrome. http://ghr.nlm.nih.gov/condition/atypical-hemolytic-uremic-syndrome

[2] Noris M, Caprioli J,Bresin E, et al. Relative Role of Genetic Complement Abnormalities in Sporadic and Familial aHUS and Their Impact on Clinical Phenotype. Clin J Am Soc Nephrol. 2010;5:1844-1859.

[3] Benz K and Amann K. Thrombotic microangiopathy: new insights. Curr Opin Nephrol Hypertens. 2010;19(3):242-247.

[4] Tsai HM. The molecular biology of thrombotic microangiopathy. Kidney Int 2006 Jul;70(1):16-23.

[5] Noris M and Remuzzi G. Review Article: Atypical Hemolytic–Uremic Syndrome. N Engl J Med [1212]2009;361:1676-87.

[6] Caprioli J, Noris M, Brioschi S, et al. Genetics of HUS: the impact of MCP, CFH, and IF mutations on clinical presentation, response to treatment, and outcome. Blood. 2006;108:1267-1279.

[7] Kavanagh D and Goodship T. Atypical Hemolytic Uremic Syndrome, Genetic Basis, and Clinical Manifestations. Acquired Hematopoietic Disorders: Complement-Mediated Blood Disorders. 2011:15-20.

[8] Frémeaux-Bacchi, V. Treatment of atypical uraemic syndrome in the era of eculizumab. Clin Kidney J. 2012;5:4–6.

[9] Bresin E, Daina E, Noris M, et al. Outcome of renal transplantation in patients with non–Shiga toxin-associated hemolytic uremic syndrome: prognostic significance of genetic background. Clin J Am Soc Nephrol. 2006;1:88-99.

[10] aHUS Action. Atypical Haemolytic Uremic Syndrome (aHUS). Accessed March 6, 2013. Available at: http://www.ahus-action.org/wp-content/uploads/2011/10/aHUS-Action-Briefing-for-Parliamentarians-FINAL.pdf.

[11] Burke L, et al. The Atypical HUS Foundation (USA). A Parent's Perspective - aHUS Bootcamp. April 23, 2013. Available at: https://www.rareconnect.org/uploads/documents/a-parent-s-perspective-ahus-bootcamp.pdf

[12] Loirat C, Frémeaux-Bacchi V. Atypical hemolytic uremic syndrome. Orphanet J Rare Dis. 2011 Sep 8; 6:60.

[13] Tschumi S, Gugger M, Bucher B, Riedl M, Simonetti G. Eculizumab in atypical hemolytic uremic syndrome: long-term clinical course and histological findings. Pediatric Nephrology. November 2011;26(11):2085-2088.

[14] Mache C, Acham-Roschitz B, Frémeaux-Bacchi V, et al. Complement Inhibitor Eculizumab in Atypical Hemolytic Uremic Syndrome. Clin J Am Soc Nephrol. 2009; 4:1312–1316.

[15] Loirat C, Garnier A, Sellier-Leclerc AL, Kwon T. Plasmatherapy in atypical hemolytic uremic syndrome. Semin Thromb Hemost 2010:36(6):673-681.
[16] Zuber J, Fakhouri F, Roumenina LT, Loirat C, Frémeaux-Bacchi V on behalf of the French Study Group for aHUS/C3G. Use of eculizumab for atypical haemolytic uraemic syndrome and C3 glomerulopathies. Nat Rev Nephrol 2012:8:643-657.
[17] Dialysis – Side Effects. National Health Service. NHS Choices. Available at: www.nhs.uk/Conditions/dialysis/Pages/side-effects.aspx. Accessed on January 10, 2014.
[18] Nester CM, Barbour T, Rodriquez de Cordoba S, Dragon-Durey MA, Fremeaux-Bacchi V, Goodship THJ, Kavanagh D, Noris M, Pickering M, Sanchez-Corral P, Skerka C, Zipfel P, Smith RJH. Atypical aHUS: state of the art. Mole Immunol 2015 Apr 3 [Epub ahead of print]; 67(1):31-42, 2015. http://www.sciencedirect.com/science/article/pii/S0161589015003508
[19] Fakhouri F, Frémeaux-Bacchi V, Loirat C. Atypical hemolytic uremic syndrome: From the rediscovery of complement to targeted therapy. European Journal of Internal Medicine 2013:24:492-495.
[20] Legendre C, Babu S, Furman R, et al. Safety and Efficacy of Eculizumab in aHUS Patients Resistant to Plasma Therapy: Interim Analysis from a Phase 2 Trial. Abstract presented at the 43rd annual meeting of the American Society of Nephrology, Denver, CO, USA, 16–21 November 2010.
[21] Muus P, Legendre C, Douglas K et al. Safety and Efficacy of Eculizumab in aHUS Patients Resistant to Plasma Therapy: Interim Analysis of a Phase 2 Trial. Abstract presented at the 43rd annual mtg. of the American Society of Nephrology, Denver, CO, USA, 16-21 November 2010.
[22] Legendre C.M., Licht, Muus P et al. Terminal Complement Inhibitor Eculizumab in Atypical hemolytic-Uremic Syndrome. N Engl J Med 2013;368:2169-81.
[23] Kim J, Waller S and Reid C. Clinical Report: Eculizumab in atypical haemolytic-uraemic syndrome allows cessation of plasma exchange and dialysis. Clin Kidney J. 2012,0:-3.
[24] Kim Maga, TK, Nishimura CJ, Weaver, AE, Frees KL, Smith RJ. Mutations in alternative pathway complement proteins in American patients with atypical hemolytic uremic syndrome. Available at: http://www.ncbi.nlm.nih.gov/pubmed/20513133
[25] Nester CM, Smith RJH. Factors influencing treatment of atypical hemolytic uremic syndrome. Clin J Am Soc Nephrol 2014 Aug 18 [Epub ahead of print]; 9(9):1516-8, 2014.
[26] 28 June 2015, aHUS Alliance mtg. of international aHUS patient organizations. London.
[27] Risitano, Antonio. The Future of Complement Treatment. At the European Society for Blood and Marrow Transplantation, Sept 29-Oct.1, 2014. Slide 4. Naples. http://www.ebmt.org/Contents/Resources/Library/Slidebank/AAIDcourse2014/Documents/22%20Risitano%20EBMT%20Naples%20PNH%20v3%20x%20Dan.pdf
[28] 2016 aHUS Global Poll. Conducted by the aHUS Alliance, 45 questions were offered in a poll for aHUS adult patients and pediatric caregivers, made available in 6 languages. (N=233, from 23 countries) Poll Overview: http://ow.ly/gSj8303GcdH Poll Questions & Results: http://ow.ly/1DA7303FoJx Poll Webinar, courtesy of RareConnect with commentary by Dr. C. Licht: http://ow.ly/ACiN303GajE
[29] Hofer J et al. Extra-Renal Manifestations of Complement-Mediated Thrombotic Microangiopathies. Front Pediatr. 2014; 2: 97.

www.aHUSallianceAction.org