

## **NOTES ON THE SECOND MEETING OF THE aHUS ALLIANCE, HELD ON 1 NOVEMBER 2014, IN HOPITAL EUROPEAN GEORGES POMPIDOU IN PARIS.**

**Present:** Vim Altena, Gema Ariceta, Veronique Fremeaux -Bacchi, Carratala Rios Mireya, Francisco Montfort, Nadege Mullier, Nicolas Mullier, Robert Pleticha ,Marjolein Storm , Anastasiya Tatarnikova, Anne Sophie van Turendoudt ,Emma Woodward ,Len Woodward, Nacho Núñez Zorriquetta.

**Present By teleconference-** Linda Burke, Paulo Chiandotto , Margriet Eyegenraam ,Michael Eyegenraam , Kamal Shah, and Sala Stefano

**Nicolas** welcomed delegates to the Second Meeting of the aHUS Alliance , which was being held in the location where the concept of an international aHUS group was first discussed.

**Member Updates** (This should be read in conjunction with Annex A and with the adobe connect meeting recording of members' presentations)

**Belgium:** Although there has been an improvement in access to eculizumab, and there is a health policy that now supports its use for aHUS, the wording of the approval means it is still not being reimbursed for those without a current episode of aHUS. Therefore, those stable on dialysis, without current aHUS activity, are not being funded and would not be allowed a transplant. AIRG Belgium is campaigning vigorously in the media, and with politicians, to get the ruling changed.

**Canada:** aHUS Canada was formed almost two years ago . No universal access to eculizumab is yet available .Although drugs are evaluated by Health Canada, provision is organised on a Province and Territory basis, and funding has not been approved by any ;but some individuals are receiving treatment on a case by case basis. Ontario provision is looked to for a lead which others might follow. Like AIRG Belgium, aHUS Canada is also campaigning in the media and lobbying politicians to make eculizumab available.

**France :** Access continues to be made available under a well defined protocol.

**Germany:** A patient organisation ( including MPGN)has only recently been formed and has 26 members so far, there is very little patient information in

Germany about aHUS. Eculizumab is free to all in Germany who need it but the number receiving it is unknown. Home infusion is available .

**Netherlands:** Eculizumab has been made available for new onset and to support transplant for nearly 20 patients. The policy is about to be reviewed as treatment is paid for out of hospital budgets and some hospital budgets are now very stretched ; it may mean that some patients have to exit the drug.

**India:** No access yet, nor envisioned soon. Indian patients have to pay for treatment themselves and as yet can only access PEX in some cases and dialysis in no more than 10% of all cases, so for the majority aHUS leads to death, although the number is unknown as very few are diagnosed. Of those who are, there is a known aHUS incidence of 1 per month. A concern is that poor protection of IP rights may cause western Pharma not to market their product in the sub continent .

**Italy:** aHUS patients continue to receive access to eculizumab and doses are tailored to each patient to make use more affordable ,but in a safe way with self monitoring. In some cases the drug has been withdrawn safely, or returned to in a small number of relapses. There have been 50 known new patients.

**Russia :** A remarkable turnaround has taken place on access in Russia since the first alliance meeting and now 35 Russian patients are accessing eculizumab. Treatment for rare diseases is framed in legislation and although some patients may need legal advocate support, more patients are receiving eculizumab because of support by their clinicians.

**Spain** Access continues, with some disparity between regions still and with less certainty due to the economic situation, and there has not been a significant increase in numbers treated in recent years.

**UK:** Decisions on drug access are devolved to the national health services in England, Northern Ireland, Scotland and Wales. Under interim arrangements, there is full access to eculizumab in England and Northern Ireland, and substantial access through individual patient treatment requests in Scotland. Welsh patients can only access via individual funding and it is not known if any have. An organisation called NICE has been reviewing eculizumab for over a year, and on 20<sup>th</sup> of this month will be issuing their final guidance on use of

eculizumab to NHS England to implement. NICE proposed guidance is to recommend eculizumab for aHUS for all who need it. The guidance comes with conditions that there should be an aHUS expert centre, a patient registry, protocols for accessing and tailoring doses, and robust research carried out on when and how it will be suitable to exit treatment.

**Scientific Advisory Board:** Veronique explained the nature of the aHUS registry and the longitudinal database it is creating; and how it benefits patients who participate, including news and updates and a soon to be created website.

Open since April 2012, it contains as at 21/10/14 details of 587 patients from 17 countries. Patient medical details are updated every 6 months, including information from the patients themselves.

The governance of the database is provided by a Scientific Advisory Board, which includes very well known aHUS experts from several countries, who are responsible for advising Alexion, and setting out questions of interest for analysis and publication, including the part that a patient representative can play in shaping the how the registry is managed and used for the good of patients.

Len then led a discussion in which it was decided that the aHUS Alliance should link with the SAB, and nominate someone to represent them ( the nomination to be decided outside of the meeting), and also to ask the SAB to agree to two deputy representatives to provide for contingency and continuity due to the long term ( up to 2025) commitment. It was also agreed that the patient representative should serve for a two year term and then be replaced by a deputy. Veronique would report our decision to the SAB at its next meeting in November. The first likely SAB meeting for the Alliance's representative to attend would be in Barcelona in February 2015.

**Current aHUS research in France:** Veronique gave a comprehensive overview of the work of the teams at HEGP and the steps in genetic screening were explained including the way in which new variants are identified and recorded. The work of the research team INSEN was shown as well as the French Study Group for aHUS led by Chantal Loirat and with over 100 clinicians participating. There are now over 450 patients in the French aHUS Registry.

Veronique mentioned a number of French aHUS research projects including the work of Marie Durey on AI-HUS and Lubka Roumenu on Hyperactive c3 convertase. Veronique reminded the Alliance that genetic causes are only known in 50-60% of cases where there is no co existing disease, of the importance of early screening for AI-HUS mutations, that most cases were sporadic with incomplete penetrance and that the recently identified DKGE mutation is a cause mechanism of aHUS.

Veronique concluded her talk by giving recognition to the trust and support that aHUS patients offer to aHUS Researchers.

**An over view of aHUS causes, treatment and transitional issues:** Gema provided an over view of the causes , diagnosis( differentiation from HUS) and disease mechanism of aHUS as well as its treatment options in new onset and transplants in children and adults and their outcomes ( and graft survival rates), including some details of eculizumab, and how it works, as well as some trial data on measures of effectiveness, especially on native kidney function retention and eculizumab supported transplants . The effectiveness and availability of eculizumab also raises the issues of transition from paediatric to adult and Gema provided a considerable list of factors/ good practices that needed to be in place to make the transition work better, including a list of self testing “readiness “questions . A case for an aHUS expert centre was outlined with the benefits to patients and links with patient organisations.

**aHUS advocacy in the USA** Linda talked about the recently renamed Foundation for aHUS( previously Foundation for children with atypical HUS) and explained the connectivity principles that underpinned the Foundation’s advocacy work. It is a leading fundraiser for aHUS research and has established an information hub through its sponsored website, and in doing so is keen to forge links with international organisations, whilst decentralising its outreach to the regions in the States. It is also supportive of Rare Disease organisations and their campaigns for rare disease awareness. Linda outlined a programme of current projects, and concluded with an update on Omeros’ OMS721 progress and gave a vision of how the Alliance might deliver one of its key aspirations to hold an international patients event.

**aHUS Day:** The Alliance members agreed to support an international aHUS day to be held in week commencing 20<sup>th</sup> September 2015. A task group will be set up to look at ways of promoting the day and integrating events in all member countries to maximise awareness.

**FEDERG:** Francisco provided a brief summary of what FEDERG had done, which basically so far was to create its legal identity.

**aHUS Global Poll 2014 :** Robert outlined what had taken place in creating the poll and the subsequent use of the data about aHUS provided on Rare Connect. Anastasia would like to collate similar information about aHUS in Russia.

**aHUS Alliance Organisation Development:** Len outlined the development of the Alliance and how there is now a need to consider a move from a loose informal association of individual members to a formal legal entity with its own governance. It was agreed that a sub-committee should be set up to look at options and what could be implemented by the next meeting .

**Sharing out aHUS Alliance responsibilities :** a brief discussion took place about sharing out the tasks among Alliance members. For one task about setting up a website it was agreed a subgroup would be created to work on the purpose and design for such a website. Other identified tasks would be assigned to groups of members to work on.

**Alliance Aspirations :** As there was too little time left to neither look at the current list of aspirations, nor collate new aspirations ,it was agreed that this should be done post conference ;and that a subcommittee should be set up to collect new ideas and also turn the aspirations list into a mission statement and vision for the Alliance.

**Next Meeting:** Members agreed that the next meeting of the Alliance will be held in London on Sunday 28 June 2015(preceded on 27<sup>th</sup> by the 2<sup>nd</sup> UK aHUS Patients' Conference).

## Annex A

Country	Belgium	India	Russia	Spain
Population (millions)	11 million	1200m	142.7 m	47m
aHUS Patient Organisation Name	AIRG Belgique	The Atypical HUS India Foundation	Another Life	ASHUA
Type of organisation	Registered Charity	Registered Charity	Interregional Public Organization	Registered Charity
Organisation website	<a href="http://www.airg-belgique.org">www.airg-belgique.org</a>	<a href="http://www.ahus.in">www.ahus.in</a>	<a href="http://www.life-complement.com">www.life-complement.com</a>	<a href="http://www.ashua.es">www.ashua.es</a>
Dedicated to aHUS only (Yes/No)	No	Yes	No	Yes
Number of members (or if no just aHUS Members)	157	5	33	47
Prevalence ( total surviving) of patients	Not known	Not known	70	150
Is it an estimate or actual number	N/A	N/A	Estimate	Estimate
At what date?	N/A	N/A	01/11/2014	01/11/2014
Annual Incidence ( new on set)	11	Not known	142	15
Is it an estimate or actual number	estimate	N/A	Estimate	Actual
For which period	1 year	N/A	From 1/4/13 to 31/3/14	1/4/13 to 31/3/14
Number patients on dialysis	25 on dialysis	Not known	Not available	Not known
Estimate or Actual	actual	N/A	N/A	N/A
At what date?	30/08/2014	N/A	01/10/2014	01/10/2014
Number receiving eculizumab	Not known	0	35	80
At what date?	N/A	01/11/2014	01/10/2014	01/10/2014
Is eculizumab for all(yes/no)	No*	No	Yes*	No
Who decides?	National Insurance	N/A	Regional healthcare ministries*	Regional Health Administrations
Other comment	* Not for transplantation		*85 regions	

## Annex A (continued)

Country		UK
Population (millions)		62m
aHUS Patient Organisation Name		aHUSUK
Type of organisation		Registered Charity
Organisation website		<a href="http://www.ahusuk.org">www.ahusuk.org</a>
Dedicated to aHUS only (Yes/No)		Yes
Number of members (or if no just aHUS Members)		118
Prevalence ( total surviving) of patients		200
Is it an estimate or actual number		Estimate
At what date?		01/11/2014
Annual Incidence ( new on set)		24
Is it an estimate or actual number		Actual
For which period		1/4/13 to 31/3/14
Number patients on dialysis		20
Estimate or Actual		Estimate
At what date?		01/10/2014
Number receiving eculizumab		55
At what date?		01/10/2014
Is eculizumab for all(yes/no)		Yes*
Who decides?		NHS in devolved nations*
Other comment		* England/NI, (All)