GOOD WILL COME TOGETHER

NOTES OF THE THIRD MEETING OF THE aHUS alliance HELD IN THE SENATE HOUSE UNIVERSITY OF LONDON On 28th JUNE 2015.

Members present:

Kerri Grey	Australia	Mireya Carratala	Spain	
Anne- Sophie VanTurenhoudt	Belgium	Maria Bang	Spain	
		Francisco Montfort	Spain	
Nicolas Mullier	France	Stefan Persson	Sweden	(Part day only)
Nadage Mullier	France	Inge-marie Lantz	Sweden	(Part day only)
Nacho Nunez Zorriqueta	Germany	Debbie Thelwell	UK	
Kamal Shah	India	Christine Western	UK	
Paolo Chiandotto	Italy	Kim Balsdon	UK	
Liliana Chiandotto	Italy	Jeremy Appleton	UK	
Marjolein Storm	Netherlands	Emma Woodward	UK	Chair
Joyce Cransfeld	Netherlands	Len Woodward	UK	
Wim Altena	Netherlands	Linda Burke	USA	
Irina Anastasia Tartankova(SKYPE)	Russia	Prof Tim Goodship	Guest speaker	

Apologies were received from aHUS Canada (Margriet and Michael Eygenraam)

Introductions

The meeting was chaired by the host nation and Emma Woodward welcomed delegates to London. Members and guests then introduced themselves including those from Australia, India and USA who were attending their first meeting in person.

International aHUS Awareness Day

Linda and Francisco gave an informed and motivating introductory talk (copy of PowerPoint slides attached) about the aims of the International aHUS Awareness Day and the ideas suggested so far for 24th September 2015. Members then suggested further creative and imaginative ways for patient organisations collectively, and aHUS patients individually, to promote, and be active on, the day. A working group led by Linda and Francisco would be set up to coordinate an alliance programme deploying action decided upon.

aHUS alliance website

Linda, Francisco and Kamal presented some design concepts and potential content for the development of the alliance's website (copy of PowerPoint slides attached). Members then fedback some further ideas and ways for the site to be become more visible (currently on second page of Google search results). Again a working group with Linda, Francisco and Kamal's leadership was set up to take the ideas forward and see whether there is an ability and resource to deliver.

Complement Inhibitors Development

Dr Wynne Western-Davies gave an illustrated talk (copy of PowerPoint attached) about complement and aHUS, and what inhibitors are in advanced stages of development for the treatment of complement dysregulation, including aHUS. Members asked informed questions about these developments and provided a collective patient voice about what patients thought about different modes of treatment.

aHUS treatment in UK

Prof Tim Goodship gave a presentation about the treatment of aHUS in the UK from when he initiated research in the late 1990s to the current day. He outlines the application processes gone through for the NHS to provide universal free access to Eculizumab in UK. Tim explained the setup, a national expert centre (genetic screening, decision protocols for Eculizumab access and cessation) to deliver a highly specialised service from Newcastle upon Tyne to England, and other parts of the UK if required. He then shared some statistics about English patients and what had happened to them in the first two years of that service. His talk also included how devolution of health care had impacted on aHUS patients in Wales Scotland and Northern Ireland. (A copy of his presentation is attached).

Access to complement inhibitors

Kamal gave a very thought provoking introduction to a key topic for the alliance how to access complement inhibitor therapy in countries in which is not, or not entirely, available. Using the comparative data sheets that some countries had supplied (see appendix B) he summarised current status. He then showed countries whose stratus had changed the most successfully since the inaugural meeting of the alliance in February 2013 i.e. Belgium, Russia and UK to stimulate a discussion about what makes a difference. From the feedback he got, Kamal would produce a paper to propose a model of access “levers “ which could be adapted and used by any country patient organisation seeking access to therapy currently denied.

SAB Report and ideas for research.

Len, as the alliances representative on the aHUS Registry Scientific Advisory Board, reported back to the alliance what had happened at the two meeting since the alliance decided to accept an invitation to the alliance join the SAB. Two written reports had already been sent to members so the focus was on collecting member’s ideas of what aHUS patients regard as priorities for research and several ideas were collected. These will be put into a report by *Len*, which will be circulated to members before being sent to the SAB. A presentation to the SAB on the outcome will be made by Len at the SAB’s next meeting in San Diego in November this year.

aHUS alliance Mission

Len gave presentation (PowerPoint slides attached) to show how the original aspirations for collaborative work had led to a Mission Statement and Vision which he proposed for the alliance. The alliance discussed the proposal which then was agreed as the initial working mission statement and vision for the alliance:

Mission:

The *aHUS alliance*, through the collaboration of its members, will promote global awareness of aHUS, will work with international aHUS researchers and, by supporting newly emerging national aHUS patient groups, will bring relief and support to those affected by aHUS to save , and improve the quality of, more lives

Vision:

GOOD WILL COME TOGETHER

aHUS alliance Organisation

Len gave an overview of the work done so far in establishing a formal organisation of the alliance (see PowerPoint slides attached). The working group had looked at various options and recommended a charitable incorporated organisation. Len outlined the advantages and disadvantages of registering and running such an organisation and asked whether the alliance was as yet **ready willing and able** to move from the loose affiliation, it currently is, to a formal entity. From the discussion that followed it was agreed we needed to work on this in manageable stages as it was too soon to commit. As a first step a group including Len and Anastasia would work on writing a “constitution” for the alliance members to discuss, agree and use.

Any other business

- The Dutch members presented each alliance delegate with a book about aHUS written by a PHD research student in the Netherlands, and also reported on a four year study (2014 to 2017) at Radboud University on the optimisation of Eculizumab treatment.
- Paulo gave a short presentation of the Milan Italy and dosage tailoring experience to show the substantial experience in the past three years of the safety of the adjustment and withdrawal of Eculizumab treatment (copy of slides attached).
- As Len will be in San Diego at the time of the American Society of Nephrologists (ASN) meeting it was agreed that he would work with the aHUS Foundation in the USA to provide an information booth about the alliance for delegates to the conference.
- Robert Pleticha has invited someone from the European aHUS community to attend a webinar about the development of Rare Disease Reference Networks. It was agreed that a European alliance member should attend the webinar and report back.
- It was agreed that a European alliance member should attend and represent the alliance at the 5th International HUS/aHUS/TTP/C3 conference in Innsbruck on 12 to 14th July.
- Belgium ,Canada and France gave updates about their respective countries and these are attached, or for Canada at Appendix A
- More countries have provided or updated the key data sheets and these are attached in Appendix B. Others ,even if all data is not available should provide a response.

Next meeting

It was agreed that the next gathering of alliance in full committee would be in autumn 2016.

USA, India and Belgian representatives offered to look into hosting that, or subsequent meetings.

Appendix A: Update from aHUS Canada

- AHUS Canada is a not-for-profit organization formed in November 2012.

- The mission of the organization is to support patients and families living with atypical hemolytic uremic syndrome.

- connect those affected by the condition to establish a Canadian aHUS community
- build public awareness and understanding of this very rare and potentially fatal disease
- advocate for the best possible care and treatment for patients

- We estimate there are between 70-90 aHUS patients in Canada

- In March 2013 Health Canada approved the sale of Eculizumab but each province has to make their own funding decision.

- Currently only the province of Quebec has provided funding.

- aHUS Canada has been advocating actively in Ontario and in February 2015 Ontario provided interim funding for patients that qualify under the interim funding criteria on a case by case basis.

- With the current interim funding patients in need of a transplant are not eligible for Eculizumab.

- Delivered over 8000 petitions to the Ontario government.

- In May 2015 we had the privilege of having Prof. Goodship in Toronto and he met with Ontario Ministry of Health and patients.

- aHUS Canada hosted their 3rd annual patient conference on May 22&23.

Goals for 2015:

- Continue to raise awareness by securing media stories.

- Encourage other provinces to provide interim funding.

- Negotiate with Ontario Health Ministry to change current interim funding criteria to include patients in need of transplant.

- Add additional patient stories to aHUS Canada website.

- Recruit new board members for succession plan.

Appendix C: Comparative Country Data

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	www.airg-belgique.org	www.ahus.in	www.life-complement.com	www.ashua.es
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	

Country	**Netherlands**	**UK**	**France**
Population (millions)	17 m	62m	
aHUS Patient Organisation Name	Nierpatiënten Vereniging Nederland	aHUSUK	
Type of organisation	Patients Association	Registered Charity	
Organisation website	www.nvn.nl	www.ahusuk.org	
Dedicated to aHUS only (Yes/No)	No	Yes	
Number of members (or if no just aHUS Members)	40	118	
Prevalence (total surviving) of patients	Not known	200	
Is it an estimate or actual number	N/A	Estimate	
At what date?	N/A	01/11/2014	
Annual Incidence (new on set)	5 - 10 children, 10-15 adults every year	24	
Is it an estimate or actual number	Estimate	Actual	
For which period	Every year	1/4/13 to 31/3/14	
Number patients on dialysis	Not known	20	
Estimate or Actual	N/A	Estimate	
At what date?	N/A	01/10/2014	
Number receiving Eculizumab	About 17	55	
At what date?	Spring 2015	01/10/2014	
Is Eculizumab for all(yes/no)	No, no pre-emptive use after transplantation	Yes*	
Who decides?	Doctors and ultimately boards of directors at Academic hospitals, because they have to pay for it out of hospital budget.	NHS in devolved nations*	
Other comment	• Reimbursement is under discussion with the government. • In 2014 (till 2017) a study ‘Safe and cost-effective use of Eculizumab/Soliris in patients with aHUS in clinical practice’ has started.	* England/NI,Wales (All) Scotland individual case	

Country	Germany	Australia	Italy
Population (millions)			
aHUS Patient Organisation Name			
Type of organisation			
Organisation website			
Dedicated to aHUS only (Yes/No)			
Number of members (or if no just aHUS Members)			
Prevalence (total surviving) of patients			
Is it an estimate or actual number			
At what date?			
Annual Incidence (new on set)			
Is it an estimate or actual number			
For which period			
Number patients on dialysis			
Estimate or Actual			
At what date?			
Number receiving Eculizumab			
At what date?			
Is Eculizumab for all(yes/no)			
Who decides?			
Other comment	•		

Country	USA	Canada	Sweden
Population (millions)			
aHUS Patient Organisation Name			
Type of organisation			
Organisation website			
Dedicated to aHUS only (Yes/No)			
Number of members (or if no just aHUS Members)			
Prevalence (total surviving) of patients			
Is it an estimate or actual number			
At what date?			
Annual Incidence (new on set)			
Is it an estimate or actual number			
For which period			
Number patients on dialysis			
Estimate or Actual			
At what date?			
Number receiving Eculizumab			
At what date?			
Is Eculizumab for all(yes/no)			
Who decides?			
Other comment	•		