# NOTES ON A MEETING HELD ON 16 FEBRARY 2013 IN BARCELONA, BETWEEN aHUS PATIENT SUPPORT GROUP REPRESENTATIVES

Attending Francisco Monfort, Carratala Rios Mireya, aSHUA ; Nicolas Muller, Nadiege Muller AIRG France(aHUS): Anne-Sophie van Turenhoudt AIRG Belgium (aHUS) Paolo Chiandotto , Sala Stefano, Progettoalice-SEU, Anastasiya Tatarnikova , Another Life Russia, Kath Woodward , Len Woodward aHUSUK.

1. It was decided that Francisco Montfort of the host organisation ASHUA would chair the meeting.

2. Each organisation described their formation and objects (file cmk ,m,.opies of the presentations are attached). Of particular note from each presentation was:

- AIRG Belgium (aHUS) because of the small number of patients, support is provided by combining with other renal genetic patients;
- AIRG France (aHUS) has emerged from a long standing association of patients with renal genetic conditions
- aHUSUK formed after the first gathering of aHUS patients in the UK in 2011 and has focussed on raising awareness and understanding of aHUS in the UK and provided help and support to patients and carers
- Alice Progretto of Italy – was formed by family and friend of Alice a little girl who had aHUS in 2004. The group has done much to establish and equip an aHUS centre of excellence;
- ASHUA of Spain was created by the parents of a little boy who had aHUS in and who established a considerable clinical contact network
- Another Life in Russia was formed by bringing together support for all patients who suffer from over active complement activity, including aHUS and PNH patients.

3. Representatives briefly outlined the current treatment of aHUS in their countries (full details are given in the presentation files attached) but of particular note was:

- Belgium – the patient number are unknown but estimated at around 40, there is no general accessibility to eculizumab except under a social security rule which entitles all patients to six months worth of treatment. Four children have access to eculizumab but once the six months expired no further attempt will be made to treat any aHUS recurrence and so transplants successfully supported with eculizumab would be allowed to fail.
- France – Patient numbers exceed 400 and are very well recorded; all patients who need it have full and free entitlement to eculizumab in a well defined treatment protocol.
- Italy- around 40 known patients and have full access to eculizumab when needed ,but protocol monitoring and controls have been developed to permit variable dosage levels and treatment intervals and have facilitated exit ,if complement activity has settled.

- Russia - patient numbers are very uncertain because of diagnosis difficulties with many patients dying of renal failure before genetic cause can be determined. Legal entitlement to treatment is set out by statute laws, but implementation is inconsistent with reluctance by individual clinicians to comply with the law. Legal challenges are in progress. A heart warming story of how 5000 Moscow citizens have collectively funded one little girl's treatment with eculizumab for 1 year; but great uncertainty surrounds what will happen to her once the twelve months expire.
- Spain has around 200 patients with 85 accessing eculizumab, but some constraints are now taking place due the economic situation.
- UK around 200patients with no general entitlement to eculizumab from the NHS yet; but it is available in some part of the country for some patients on individual request. Different arrangements exist in each of the devolved NHS organisations for England Wales Scotland and Northern Ireland. aHUSUK are currently engaged with NHS organisations to fully explore the "cost effectiveness" across the patient cohort.

Some 900 patients are represented by the six organisations combined, and common themes of diagnosis problems and justifying cost effectiveness were evident.

4. A list was created of the kinds of things that patient groups could do while working together. Although no commitment to doing them was made at the meeting, it was agreed that where something could be done quickly, and everyone was willing, it would be done before any overall plan can be agreed.

5. A presentation was due to be given by Drs Roman and Otiose, but they were unable to attend. A letter was read out from the doctors (copy follows) which welcome the initiative being taken by patient organisations and how it would strengthen the links with the clinicians for the benefit of all.

6. EURORDIS representative Marta described the role and purpose of EURODIS and its link to NORD through the Rare Connect Community. Martha gave advice and help on developing a group of patient's organisations, including financial and communication support, using the Rareconnect website

7. Patients Group representatives then discussed and agreed the following that:

- an alliance of patients groups is needed;
- the alliance needs to be formed as a legal entity and that it should be formed using Spanish regulations;
- the organisation should be called **aHUS Alliance** and would be open to any aHUS patient organisation throughout the world;
- he aHUS Alliance should be announced as a Rare Disease Day event.

8 .The date of the next meeting of the newly created aHUS Alliance is to be decided, but it was proposed it should be held in Milan.

Things an alliance of national aHUS patients organisations can do together.

- Maintaining a database of key comparative data about aHUS
- Sharing information about good ideas( e.g. the Italy controlled dosage study)
- Make patient numbers visible
- A compendium of triggering events
- Encourage Doc to Doc in Europe
- Database of mutation distribution comparisons
- Find and support other countries in set up( globally)
- Participation in a scientific council in Europe
- Develop liaison between country clinicians
- Create a foundation for emergency treatment from Corporate sponsorship
- Contrasting and encouraging uniformity of access across countries
- Addressing common problems Diagnosis and Cost Effectiveness
- Representation to and raise awareness to MEPs
- Clarifying Human Rights issues e.g. right to family life
- Encouraging Registries of Patients and Others
- Legal Challenges
- Using Rareconnect to spread global knowledge
- Contact and consultation with EMA
- I Books on aHUS multi language
- A psychologist support package for new aHUS onset children's parents
- Share literature between countries
- Patient experience research
- A database of general aHUS and its treatment research topics for undergraduates and post graduate thesis ( iPAD reward)
- An aHUS "APP"
- Extending the DNA school across borders
- Electing an aHUS representative on FEDERG Board speaking for alliance
- A global patients event

Valencia on February 15, 2013
for the attention of Mr. and Mrs. Francis Monfort Mireia Carratalá

*We are sorry that for personal reasons we cannot attend the meeting organized and we had so kindly been invited. It would have been our desire to attend it because we consider it an important initiative and thus to contribute to the purposes they serve.*

*Physicians have a responsibility to patients with aHUS we feel attached to family associations in their efforts and concerns. We are convinced of the importance of integrating into the Public Administration Committee to ensure appropriate and rational use of treatments that can be used in patients.*

*We also believe in the importance of the formation of expert groups in aHUS disease, existing group in our country and we belong, with various missions:*

- *to promote uniform treatment protocols*
- *Conduct multicenter studies and share individual experiences to enrich the collective knowledge.*
- *Ensure equal access to treatment for all patients.*
- *Participate in patient records to a better understanding of the disease.*
- *To spread knowledge to other professionals to refer and consult expert groups with more experience in the process.*

*Doctors, patients and family associations are the reason for the pharmaceutical industry and we must establish a cordial relationship and mutual cooperation for the benefit of patients and unrelated to economic issues.*

*Rare diseases should not have borders. Time and resources are scarce but we must strive and not hesitate to ask for input from both industry and public organizations to promote communication among professionals in our country and other countries for the benefit of patients.*

*We realize that there is much to learn and improve the treatment of patients and the difficulty in disseminating the knowledge and solutions to these diseases.*

*Serve this letter to get ASHUa available in everything deemed of interest and should you wish to serve as consultants on questions or issues that may arise during illness to individuals and to the association itself.*

*Attentively*

*Dr. Elena Santiago Román Ortiz & Dr. Mendizabal Oteiza*