

## **Global aHUS Patient Voice Survey- Research and Registries**

The agenda for what research is carried out on aHUS has been largely set by clinical researchers based on personal interests/challenges/curiosities, and what can be targeted and sustained by the research funds raised. Although, just what those are overall, and how visible the global agenda is, remains unclear.

The traditional role of patients in research is to provide blood and other clinical samples, along with personal data, including their social and psychological wellbeing.

Registries are an important source of information about patients for research, whether at institutional, national or global level.

Recruitment of patients into the registries is an expensive and time consuming activity; but the importance of doing so, particularly when rare disease patients by definition are so few, is rewarding.

In recent years there has been a noticeable trend towards including patients in the discussion about priorities for research into the disease that affects them, not because they are fundraisers, but just to give those setting the agenda, a different perspective.

The 2016 Global aHUS Patient Voice (233 responses from 23 countries) for Research, and related Registries, has provided the following insights, which are compared in some cases to the findings from similar questions in 2014 Global Voice (217 responses).

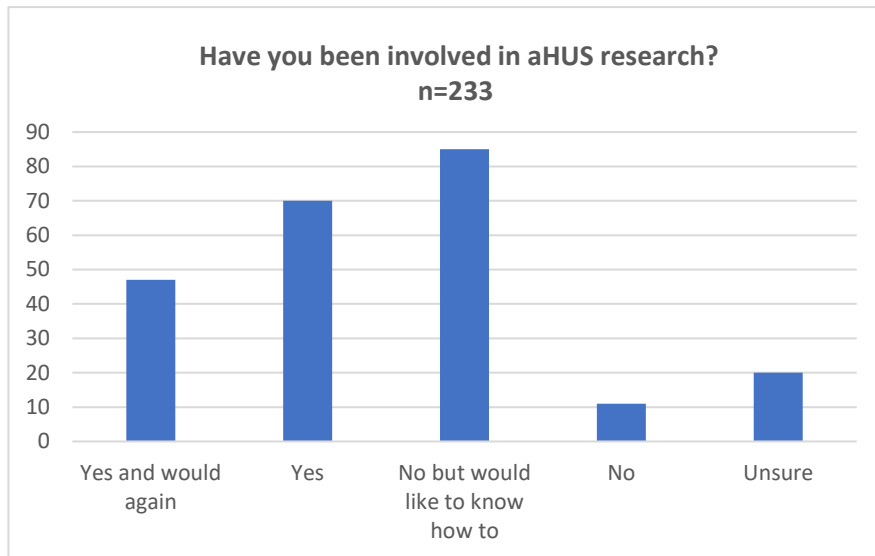
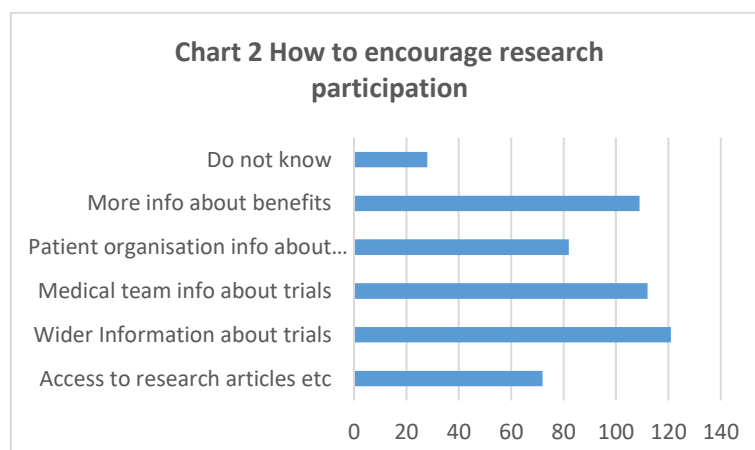


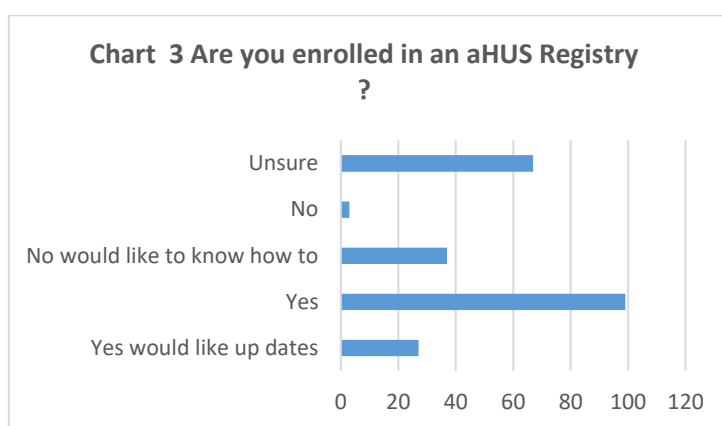
Chart 1 – Question 29 of Survey

Around 50% of patients have been involved in some aHUS research, up considerably from the 35% responding in 2014. Of those who have not, 37% would like to find out and know more about participating, whilst around 1 in 10 say they are not sure about whether they have participated or not (down slightly to 9% from 11% in 2014); and only a small minority 5% say they have not, and would appear not to want learn more about doing so. So 95% of patients would be interested in participating in research.

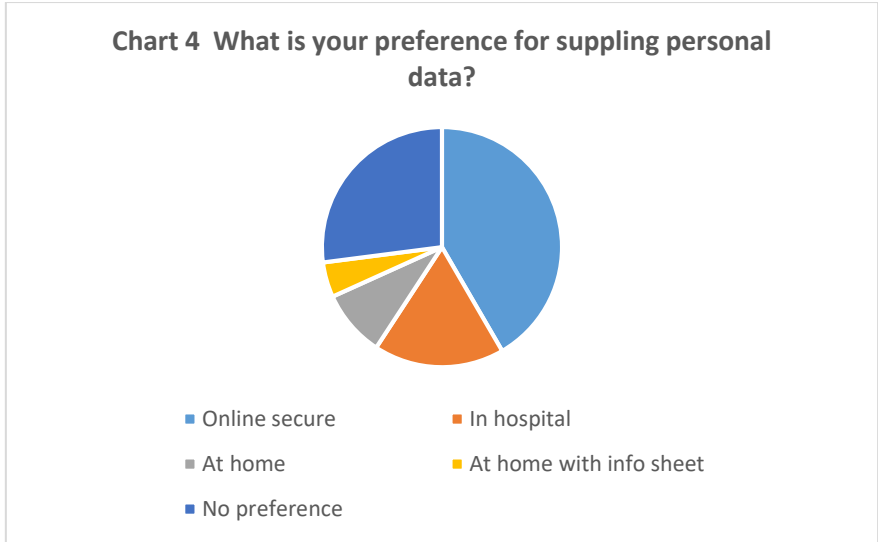
Frequently in rare diseases it is difficult to find sufficient patients to make research valid. It is clear for aHUS that patients are saying that potentially more of them here, ready and interested, even if we have already been involved previously.



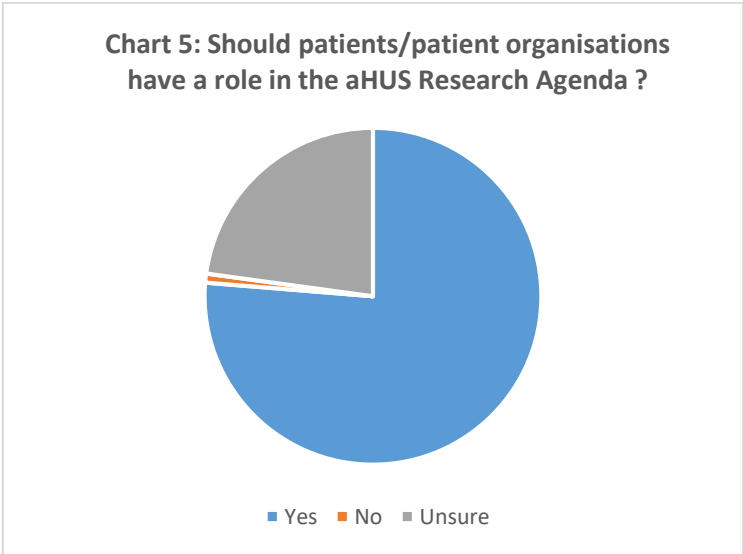
121 respondents thought more information about trials is needed to encourage patients to participate, and that information would be better coming from patients' clinical contacts (118) rather than patient organisations, although some (92) thought such organisations had a part to play. Information about the benefits to patients and carers for taking part was seen as an important way to encourage them. Only a minority thought having more access to research articles and publications would help them become involved.



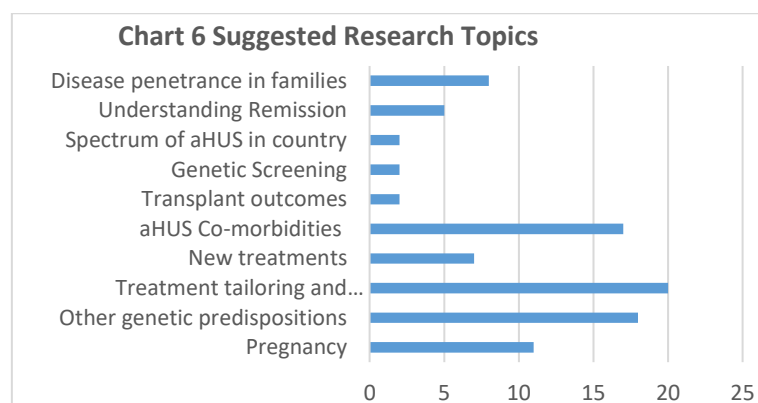
42% of respondents said they were included in registries (up 9% on 2014). 17% say they definitely are not, although only 1% within that group would prefer not to know how to become registered. A surprising number, 29%, said they were unsure whether they have participated in a Registry or not, slightly less than in 2014 when 31% were unsure. The need for Registry enrolment to be a “noticeable event” by patients remains to be addressed. Potentially 99% of patients would be interested in being enrolled in a Registry.



As far as supplying data personally to the Registry at regular intervals is concerned, a substantial number 42% would prefer to do it online, and perhaps another 27%, currently having no preference, may add to that number. 17% prefer to do it at clinic while 14% would wish to supply data at home in writing, of whom some, (5%) would wish to do it with the help of an information sheet reminder about filling in the data.



76% of respondents said that patients, or patient organisations, should play a role in setting the aHUS research Agenda. Just 1% said definitely not. 23% were unsure whether they should be or not. Despite the high response to play a role only 90 out of the 233 respondents suggested a research topic. Although that was from just 39% of respondents, it is a significantly high response given the traditional reticence of the aHUS community to discuss and suggest research topics in the social media.



92 topics were suggested which have been categorised into common themes. The highest suggested common theme for research was “treatment tailoring and withdrawal (of Eculizumab)”. Finding “other genetic predisposition factors/triggers” was not far behind along with knowing about “co-morbidities and side effects of aHUS and its treatments”.

Reflecting the special interest in the aHUS community, the case of the pregnancy trigger was thought important by some as requiring specific research and surprisingly that was followed by wanting to “understand what affected disease penetration within families”.

Some want to know about “new ways of controlling complement” and others to “understand why some go into remission while others do not”.

Very few suggested more research on transplant outcomes, given that dialysis patients are frequently the patient group left outside of the scope of funding of Eculizumab.

A small number wanted more “effective genetic screening” and others to “know more about aHUS patients in the own country”.

So that is what Global aHUS Patients have said about Research and Registries in the aHUS alliance survey.

aHUS Patients have said they

- have participated in increasing numbers in research and some would like to do get the opportunity to do so, and others to do even more;
- think more information about trials would be an encouragement, particularly coming from their clinicians;
- want to be in research registries;
- want to supply their data to Registries in the way they prefer, and a substantial number want to do that online
- think that patients/patient organisations should have a role in research agenda setting
- have ideas about what are the priorities for research.

At present there is no visible research agenda for aHUS. To find it, all the current aHUS investigators research objectives would have to be aggregated, categorised and shared.

Now the aHUS patient voice says there is for them.

Until now patients have played no part in shaping those research objectives, but the Global Voice of aHUS patients in 2016 asks for the chance for patients to contribute, and add more value to the research partnership between Clinicians, Academia, Pharma and Patients.

The task now for aHUS patients is for their voice to be heard by their partners in research.