

aHUS alliance Global Action Financial Report for the year ending 31 December 2017

Income and Expenditure Report for year ending 31 December 2017

Income	Unrestricted funds	Restricted Funds	Other Funds	Total Funds	Last Year	
Donations	-	-	-	-	15000.00	
Grants	5903.60	-	-	5903.00	0.00	
Fundraising	0.00	-	-	0.00	0.00	
Other	0.00	-	-	0.00	0.00	
Sub Total	5903.60	0.00	0.00	5903.60	15000.00	

Total Receipts	5903.60	0.00	0.00	5903.60	15000.00	
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Expenditure						
Management meetings	0.00	-	-	0.00	0.00	
Website	514.50	-	-	514.50	0.00	
Travelling	2442.00	-	-	2442.00	0.00	
Promotional Materials	171.24	-	-	171.24	0.00	
Conference	11840.98	-	-	11840.98	0.00	
Printing	39.60	-	-	39.60	0.00	
Subsistence	227.67	-	-	227.67	0.00	
Bank Fees	103.00	-	-	103.00	0.00	
Sub Total	(15338.99)	-	-	(15338.99)	0.00	

Total Payments	(15338.99)	-	-	(15338.99)	0.00	
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Net Receipts/(Payments)	(9435.39)	0.00	0.00	(9435.39)	15000.00	5564.61
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Cash Flow and Balance Statement as at 31 December 2017

	Cash	Total
Opening balance	15000.00	15000.00
Receipts	5903.60	5903.60
Expenses	15338.99	15338.99
Balance as at 31 December 2017	5564.61	5564.61

Annual Report

Trustees Annual Report for the period 1 January 2017 to 31 December 2017

Introduction

aHUS alliance Global Action is a Registered Charity in England and Wales (No 1167904). It held its second Annual General Meeting on 3 April 2018 by Skype.

Governing Document

The aHUS alliance 's governing document is the Foundation Model for Incorporated Charitable Organisations and under its constitution clause 15 (3)(a) a quorum for a general meeting is 2 trustees entitled to vote. 3 trustees were in attendance for the Annual General Meeting.

Objects

aHUS alliance Global Action's objects are to:

- promote the relief and support of persons affected by aHUS,
- advance the education of the public and the medical profession about aHUS.

Note: atypical Haemolytic Uremic Syndrome (aHUS) is an ultra-rare disease affecting about 15000 to 40000 people globally. It is serious, progressive, debilitating, life-threatening and incurable. Largely genetic in origin, it can affect people at any age. Caused by unregulated activity in the normally well-controlled complement system, if unchecked the disease causes kidney and other organ failure.

The aHUS Alliance Global Action exists to support the collaborative work of the aHUS alliance. The aHUS alliance has existed as an informal organisation of international aHUS patient organisations since Rare Disease Day (28 February) 2013. The aHUS alliance Global Action has 3 Trustees and is run entirely by volunteers.

Complementing the objects above its operational objectives are to:

- promote global awareness of aHUS
- work with International aHUS researchers

- support newly emerging national aHUS patient groups

by connecting, informing and collaborating with interested groups.

Trustees in post in the period 1 January 2017 to 31 December 2017.

Trustee/ Director/ Secretary	Linda Burke
Trustee /Director/Treasurer	Len Woodward
Trustee/ Director/Chair	Kamal Shah

As no Trustee had completed their term of office, and no letters of resignations, nor nominations for new Trustees been received, the Board of Trustees remained unchanged.

Activity in the period 1 January 2017 to 31 December 2017

The alliance set out at the beginning of the year to focus specifically on:

- access to complement inhibitor treatments
- encouraging aHUS patient engagement
- producing an aHUS patient research agenda

During the year another collaboration was included:

- raising awareness of the diagnosis challenge of aHUS in the context of TMAs.

Access to Complement Inhibitor treatments

Throughout the year the alliance drew attention to access difficulties in developing countries like South Korea, South Africa and India and where called upon supported those who were suffering denied access.

Representatives from the alliance met with pharmaceutical companies, Alnylam and Chemocentryx to find out about their developing technologies for the treatment of aHUS; and to advocate for their trials to take place in countries with an unmet need for treatment for aHUS. Updates and outreach emails were sent throughout by the alliance regarding therapeutic drug discovery to industry as well, which yielded phone or email contact with multiple stakeholders. The alliance developed its network of interested clinicians and researchers, which expanded throughout 2017 due to in part to international interest in drug access issues. As of December 2017, this

resource lists 24 physician/researchers from 19 nations as our alliance 'partners in advocacy'.

The alliance began a series of news blogs "aHUS trials watch" to draw attention to trials which involved, and would be of potential benefit to aHUS patients, including new drug options, as well as developments in the use of eculizumab. This new feature is in addition to two pharma pipeline overviews, the main one in January 2017 and an updated version added to the aHUS Key Info & Fact Sheet for the 24 September annual campaign.

Encouraging patient engagement.

The alliance facilitated an awareness video for Rare Disease Day on its theme of "research". 75 patients from 17 countries joined in and collectively voiced what mattered to them from aHUS Research (see also Research Agenda below).

The alliance continued the engagement of patients in advocacy in the Third Ahus Awareness Day programme of events under the banner of "Rising above aHUS". Another video was produced in which patients and/or carers expressed what they felt Doctors should know about living with aHUS.

Patients were engaged in a symposium about TMA and aHUS (see below) and videos of their participation were released as part of the alliance's aHUS Awareness Day programme of events.

The alliance was joined by affiliate patient groups from Romania and Poland during the year. It also accepted an affiliate organisation for "aHUS Rest of the World" which would represent countries which had emerging patient advocacy but were not yet able to create their own organisation. Patients from Brazil, Pakistan, Poland, Israel, South Africa, the United Arab Emirates, and New Zealand decided to participate which has resulted in an expansion of the networking list of aHUS advocacy to include patient voices for 28 nations.

As a direct result, the alliance has created resources to amplify the patient voice to include: expansion of our global Atypical HUS Patient Voice channel on YouTube, "*aHUS Fact Sheets*" (fully cited and a brief printable version), graphics about the rarity of the disease ("*Red Leaf*" image in French, English and Spanish), and the "*Know aHUS*" education materials (in French, English and Spanish)

aHUS Patients Research Agenda

The collective list of research questions asked in the Rare Disease Day video were added to the topics raised in the 2016 aHUS global poll as well as those decided upon by alliance affiliates at the Third General Meeting of the alliance to produce an interim aHUS patients research agenda:

1. Is there a diagnosis sweet spot which can be found before a developing thrombotic microangiopathy turns into a catastrophic episode of aHUS?
2. Is there an optimal way in which a complement inhibitor can be delivered to suit an individual's need?
3. Can a clinically effective therapy be developed that is affordable for all aHUS patients?
4. Can a complement inhibitor be stopped safely when not needed by some aHUS patients and what makes them different?
5. Are there long-term studies of outcomes of those in remission whether treated by a complement inhibitor or not?
6. Is there a significant difference in outcome between having a complement inhibitor before or after a kidney transplant?
7. Can the side effects of treatment using a complement inhibitor be distinguished from those temporary and permanent ongoing ailments which follow initial onset?
8. Does the anxiety and self-esteem of aHUS patients vary significantly between treatment types and what can be done reduce and boost them respectively?
9. How does living with aHUS impact on education and work?
10. Do aHUS families have all the correct information to make informed family planning decisions?
11. When it comes to genetic testing of aHUS family members what is best - to know or not to know and what can be done with the knowledge?
12. How many aHUS patients are there in my local area, my country and the world and how do they differ?
13. Are the triggers of aHUS fully catalogued and understood and will it help to know how variable are the risks of these between individuals?
14. Is it more cost effective, as well as clinically effective, for the management of an aHUS patient's treatment to undertake genetic testing?
15. Will there be a cure eventually?

The alliance continued its partnership with the aHUS Registry (US National Institutes of Health ClinicalTrials.gov Identifier: NCT01522183) and provide a patient representative on the Registry's Scientific Advisory Board. The dialogue model that was set out in the manuscript written by the SAB and published in the Orphanet Journal of Rare Diseases in November 2016, continued to be embedded in the Registry's way of working to bring about patients included in research.

Awareness of Thrombotic Microangiopathy and aHUS diagnosis

Following a meeting initiated by (with) Dr. Joseph V. Bonventre, professor at Harvard Medical School and Chief of the Division of Renal Medicine of the Brigham and Women's Hospital, the alliance partnered in a conference with the title "Thrombotic Microangiopathy Symposium: Through the Lens of aHUS". The aim of the symposium was to demonstrate the difficulty of diagnosing aHUS which is very rare among all causes of TMA. The alliance was supportive of the need to approach TMA diagnosis in a multidisciplinary team work approach as the Boston Hospitals and others have done.

In a unique departure from traditional 'MedEd' events the alliance patient voice was incorporated throughout all decisions and phases of TMA Boston, from initial concept through implementation. Each clinical presentation was prefaced by aHUS patients from 3 nations (UK, CA, USA) who provided their experiences with diagnosis, treatment, and the sweeping impact aHUS has had on their lives. All presentations were filmed (creating a YouTube playlist of 9 videos), resulting in the ability of a global audience to learn and to better understand the complexities involved with treating or living with aHUS.

Website

The website www.ahusallianceaction.org continues to be the alliance's main communication channel with the aHUS community and is linked to the social media. By the end of 2017 30,000 views of the website content had been recorded, an increase of 200% on 2016 views.

Commentary on the accounts for the period 1 January 2017 to 31 December 2017 (see Annex A)

All transactions continued to be in cash through the funds held by Kidney Research UK on behalf of the alliance; while the alliance sought its own bank account. A bank account was secured and will become operative in 2018.

Some costs were incurred in developing, particularly SEO recognition to improve on a listing for an "aHUS" search and maintaining the website during the year.

Representatives of the alliance travelled and met with Dr Andrew Seidlecki of the Brigham and Women's Hospital in Boston to explore the opportunities for collaborations between aHUS Registry National Coordinators and patient advocates.

The Conference “TMA through the lens of aHUS” resulting from the discussions and was held in Boston on 24 August 2017. Costs for a video of the Symposium, accommodation and fares for patient speakers, as well as conference room facilities, catering and related conference items e.g. badges are included in the Conference expenses.

Unrestricted and unconditional educational grants were received from pharmaceutical companies Alnylam and Chemocentryx to partially defray the cost of the TMA Symposium held at the Harvard Medical School Conference Centre in August.

The sum held at the end of the year is sufficient for another year of alliance operations, excluding major projects. The Trustees are exploring other funding sources.

It was estimated that 2000 hours of unpaid voluntary time was donated by Trustees in the period.

As the income of the alliance in the year was less than £25000, no independent examination is needed.

Serious Incidents in the year.

There were no serious incidents in the year.

Linda Burke

Secretary

31 March 2018

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