

Annual Report

Trustees Annual Report for the period 1 January 2018 to 31 December 2018

Introduction

aHUS alliance Global Action is a Registered Charity in England and Wales (No 1167904). It held its third Annual General Meeting on 19 March 2019 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 2018 to 31 December 2018.

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

Len Woodward had completed his term of office and had formally expressed his willingness to continue to serve for three more years. He was duly re-elected to the Board of Trustees. No nominations for new Trustees been received. The Board of Trustees remained unchanged.

Activity in the period 1 January 2018 to 31 December 2018

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 importance and role of aHUS Expert Centres.

Access to Complement Inhibitor treatments

Throughout the year the alliance continued to draw attention to access difficulties to complement inhibitors in many countries, particularly in the developing world.

At the outset of the year, the alliance published an open letter to Alexion Pharmaceuticals to provide a “patient dividend” so that more patients could afford to be treated. This appeal was augmented when a recommendation was made by some eminent aHUS Clinicians in South Africa, via the aHUS alliance, for a new model for compassionate access in countries where complement inhibitors are unavailable. The alliance continued to develop its network of interested clinicians and researchers, which expanded throughout 2018 due to in part to international interest in drug access issues.

The alliance continued to monitor complement inhibitor trials of interest to aHUS patients through its “aHUS trials watch” series. Additionally, the alliance issued its extensive annual overview of complement inhibitor news, drug pipelines, knowledge advancements for similar diseases and market factors which may affect development of new aHUS treatment or disease management.

Encouraging patient engagement.

The alliance again facilitated awareness videos for both for Rare Disease Day and aHUS Awareness Day. It added more patient-made videos to its aHUS Patient Voices channel on YouTube. The alliance continued to reach out throughout the year on social media to engage and involve aHUS patients, caregivers, and national patient organisations.

Several alliance articles on its website were devoted to the importance of international patient involvement in all areas of research, industry and global aHUS advocacy. During the alliance’s whistle-stop tour in

July, a meeting was held with Alexion Pharmaceuticals to encourage them to reactivate its 2011 international patient advisory panel.

aHUS Patients Research Agenda

The alliance approved the Global aHUS Patients' Research Agenda at the Fourth Meeting of the aHUS Alliance affiliates which was held in Nijmegen in The Netherlands on 9 November 2018. It was agreed that it will be officially launched and adopted on Rare Disease Day 2019.

The alliance also decided at the Fourth Meeting of its affiliates to continue 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 (SAB).

The SAB concluded its study on the research topic provided by the alliance in November 2015 on whether there are better outcomes if a complement inhibitor is prescribed prophylactically before a kidney transplant or as a rescue therapy if the transplant triggers another episode of aHUS. The Research Report was published in Kidney International on 12 December 2018.

aHUS Expert Centres

Disease expert centres, or centres of excellence, are important for the understanding and management of rare diseases. During the year the alliance began a study of the role and key characteristics of aHUS Expert Centres. This led to a visit to four European aHUS Expert Centres in France, Italy and the UK in what was termed a "whistle top tour" in the reports of these visits. The alliance found that core to the development of such centres was decades of important research into the cause of and treatment of aHUS led by outstanding researchers who had developed teams to provide a succession and continuity for the benefit of aHUS patients. The alliance has established strong links

with these centres and continues to shine a spotlight on why expert centres contribute to the health of those affected by aHUS.

The alliance website articles continued to stress the importance of such centres and to highlight that their research efforts often included multiple valuable components to their programmes, advising medical personnel, working collaboratively with teams in other nations, presenting at conferences, and inclusion of aHUS advocates as valuable partners in their efforts

aHUS Alliance Affiliates Meeting

The Fourth Meeting of the aHUS Alliance affiliate organisations was held in Nijmegen, Netherlands on 9 November 2018. It followed an excellent Dutch aHUS Patient Conference hosted by Nierpatienen Vereniging Nederland on 8 November 2018, which featured talks by the team from the aHUS Expert Centre at Radboud University Medical Centre about its research and experience of the effective and economic use of the complement inhibitor, eculizumab.

Representatives of ten affiliated organisations from Canada, France, Germany, India, Italy, Rest of World, Rumania, Russia, The Netherlands and UK attended. The meeting covered news about advocacy activity in individual countries. Talks were then given about the aHUS Registry and its study of the treatment of aHUS kidney transplant patients, and about ERKNet, a European initiative to share expertise for the treatment of aHUS under its thematic Thrombotic Microangiopathy (TMA) Work Group. Discussions followed about common interests like affordable access to treatment and action needed. A decision was made to adopt the draft aHUS Patients Research Agenda as the alliance's statement on what research matters to aHUS patients

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, which has also expanded its online footprint and impact. During 2018 there were 38,456 views of the website recorded, which is an increase of 8326 (27.5%) on 2017. The website has now had over 78000 views cumulatively since its launch in 2016.

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Commentary on the accounts for the period 1 January 2018 to 31 December 2018 (see Annex A)

The alliance received no income in the financial year. An amount is due from its participation in the Scientific Advisory Board of the aHUS Registry for the purpose of providing expert advice. Payment is expected in the next reporting period.

The main expenditure was for travelling as part of the Expert Centre Study. Although again there has been no expenditure on management meetings, which are held by Skype at no cost, some postage costs are incurred for signed documents and use of Google forms for administration. Bank charges are incurred for international payments and standing charges for the charity bank account with CAF Bank.

It is estimated that between 1500 and 2000 unpaid hours per annum are donated by volunteers to maintain the operational work of the alliance.

With receipt of the SAB payment due, it is believed that enough reserves are held for two more years of operating.

As the charity's income is less than £25,000 an independent examination is not required.

Serious Incidents in the year.

There were no serious incidents in the year.

Linda Burke

Secretary

31 March 2019

Annex A

aHUS alliance Global Action Financial Report for the year ending 31 December 2018

Income and Expenditure Report for year ending 31 December 2017

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

Total Receipts	0.00	0.00	0.00	0.00	5903.60	
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Expenditure						
Management meetings	0.00	0.00	0.00	0.00	0.00	
Website	193.22	0.00	0.00	193.22	514.50	
Travelling	2229.52	0.00	0.00	2229.52	2442.00	
Promotional Materials	-	0.00	0.00	-	171.24	
Conference	-	0.00	0.00	-	11840.98	
Printing & Postage	40.80	0.00	0.00	40.80	39.60	
Subsistence	-	0.00	0.00	-	227.67	
Bank Fees	94.00	0.00	0.00	94.00	103.00	
Sub Total	2557.54	0.00	0.00	2557.54	(15338.99)	

Total Payments	2557.54	0.00	0.00	2557.54	(15338.99)	
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Net Receipts/(Payments)	(2557.54)	0.00	0.00	(2557.54)	(9435.39)	
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Cash Flow and Balance Statement as at 31 December 2018

	Cash	Bank	Total
Opening balance	5564,61	0.00	5564.61
Transfers	(5475.91)	5475.91	0.00
Receipts	0.00	0.00	0.00
Expenses	0.00	2,557.54	2557.54
Balance as at 31 December 2018	88.70	2918.37	3007.07

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