Impact Report 2019

Alkaptonuria Society

An international patient at our International workshop in 2018

"When we were introduced to the AKU Society, we had a huge weight lifted off our shoulders. It's not just a charity, it's a community, where people can reach out to each other."

AKU

Alkaptonuria Society

Our Mission

Transforming the lives of AKU patients

through patient support,

community building and

medical research.

The AKU Society is a patient-led support group. We work to improve the lives of those with AKU and those who support them while researching for a cure.

On the cover: An AKU patient asks a question at our Patient workshop

We exist to find a cure for AKU

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> “DevelopAKUre was a monumental undertaking. It brought together 13 partner organisations from across Europe

staff: Ciarán Scott, who was Clinical Trials Coordinator in 2018 (and is now Communications Manager), for providing excellent support to European patients during DevelopAKUre; Lesley Harrison, Patient Support Manager, for always being there for our UK patients at the National AKU Centre; and Juliet Rowe, Fundraising Officer, for driving our fundraising plans as we continue to scale up AKU research and support patients.

There is still so much to be done to help AKU patients as they fight this ultra-rare disease. Please continuestaff: Ciarán Scott, who was Clinical Trials Coordinator in 2018 (and is now Communications Manager), for providing excellent support to European patients during DevelopAKUre; Lesley Harrison, Patient Support Manager, for always being there for our UK patients at the National AKU Centre; and Juliet Rowe, Fundraising Officer, for driving our fundraising plans as we continue to scale up AKU research and support patients.staff: Ciarán Scott, who was Clinical Trials Coordinator in 2018 (and is now Communications Manager), for providing excellent support to European patients during DevelopAKUre; Lesley Harrison, Patient Support Manager, for always being there for our UK patients at the National AKU Centre; and Juliet Rowe, Fundraising Officer, for driving our fundraising plans as we continue to scale

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Nick Sireau, Chair and CEO

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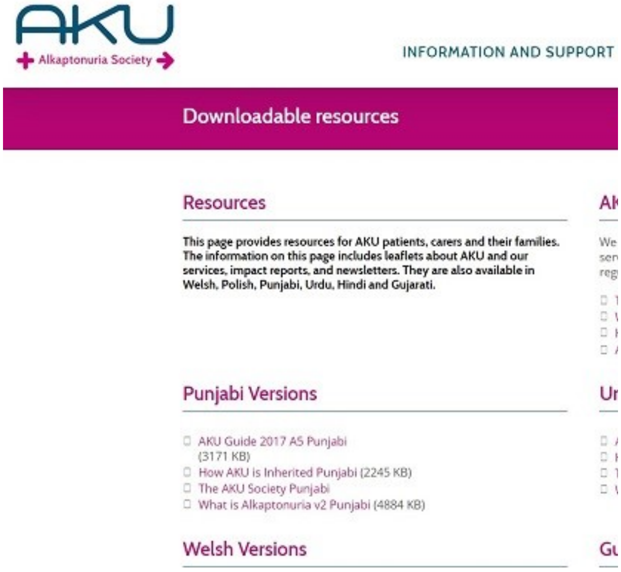

AKU
Alkaptonuria Society

INFORMATION AND SUPPORT

Downloadable resources

Resources

This page provides resources for AKU patients, carers and their families. The information on this page includes leaflets about AKU and our services, impact reports, and newsletters. They are also available in Welsh, Polish, Punjabi, Urdu, Hindi and Gujarati.

Punjabi Versions

- ☐ AKU Guide 2017 A5 Punjabi (3171 KB)
- ☐ How AKU is Inherited Punjabi (2245 KB)
- ☐ The AKU Society Punjabi
- ☐ What is Alkaptonuria v2 Punjabi (4884 KB)

Welsh Versions

New website

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DevelopAKUre

Ann Kerrigan

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8th Patient Workshop

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Tony Esmond
Richard Smith
Richard Smith
Barbara Smith
Barbara Smith
Barbara Smith
John Wright
Jennifer Wright
Sian Williams

Meet Tony

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Alkaptonuria Society
ciety Patient Workshop
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The Bluecoat

Patient Support

The National Alkaptonuria Centre (NAC) is based at the Royal Liverpool University Hospital and was established by the Department of Health (Highly Specialised Services Commissioning Group). It was set up to provide assessments and treatment for anyone over the age of 16 who has been diagnosed with AKU.

The NAC provides one-stop care to assess and detect disease complications and formulate a shared care management plan with local health care providers. Anyone living in England and Scotland diagnosed with AKU is offered an annual review at the NAC and off-label access to the drug nitisinone, as well as advice and support on managing and living with AKU on a daily basis. People living in Wales and Northern Ireland can also access the NAC once the relevant NHS service has agreed funding.

In 2018 we saw 58 patients attend the NAC, 15 returning for their 7th visit. As awareness of AKU and the NAC increases amongst health care professionals, more patients are being diagnosed and referred to the service. 2018 was no exception, with five new patients at the NAC. For many, this is the only time they meet other people in a similar situation to themselves and have the opportunity to discuss their condition with experts in the field. All our patient group are asked for feedback on the service and changes where appropriate are made to improve and develop the service for the future. A patient attending the NAC during 2018 said; 'The best part is knowing we are being looked after and monitored by specialists in AKU. They understand what is happening to our bodies and provide us with lots of information and updates on the condition. It gives me peace of mind when the test results come through and I can discuss them with the team at the NAC.'

2018 saw our 2nd year working as part of the Breaking Down Barriers project (BDB). This project allows us to work with other organisations and support groups to come together and share examples of best practice. This project is helping us strengthen our ability to develop inclusive information and services for individuals and families affected by AKU. During the two years on the project, we have worked with our service users to review and change our patient information leaflets. These have now been translated into several languages ensuring we are reaching out to and supporting diverse communities. Funding from the project has enabled us to develop an educational video for the Envisage GP waiting room system. This short health education video raises awareness of AKU and the NAC and targets a broader audience than we might typically reach. Statistics received are positive with the video receiving a high number of plays on the system during 2018, as many as 873 in September. BDB has funded the addition of 'Browsealoud' to the AKU Society website, making it more accessible and reducing the barriers between the content and all our audiences. We hope to continue working with the BDB project for a third year, developing information and services for the extended families of people living with AKU.

In 2013 we started to offer home support visits as an extension of the NAC in Liverpool. These face to face visits provide an opportunity to discuss anything about the NAC, future visits, nitisinone, diet, blood and urine tests. It can be whatever the patient needs it to be. The value of these home visits cannot be underestimated and can make a huge difference to the life of one of our AKU patients. In 2013 we started to offer home support visits as an extension of the NAC in Liverpool. These face to face visits provide an opportunity to discuss anything about the NAC, to

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Graphic

"When we were introduced to the AKU Society, we had a huge weight lifted off our shoulders. It's not just a charity, it's a community, where people can reach out to each other."

Community Building

Over two days 89 people – AKU patients, supporters, researchers and staff – from around the world travelled to Liverpool for our 2nd international patient workshop. They came from as far off as Canada, the United States, Belarus, Jordan, Brazil and La Réunion.

Lesley Harrison, our Patient Support Manager, organised everything. In each hotel room were eight branded AKU chocolates, presented in elegant bags. On arrival, Lesley greeted each delegate and handed them their delegate pack, which was full of extra information for the event. Our three newly designed leaflets ('What is Alkaptonuria', 'How Alkaptonuria is Inherited' and 'The Alkaptonuria Society') were very popular.

From genetics to diet and exercise to cutting-edge research, patients left the event with up to date information and a rounded insight into their disease. Two of the most popular talks from the days were Prof Ranganath's explanation of the progress of the DevelopAKUre clinical trials and Dr Andrew Jone's experience treating AKU patients' pain and how patients can help themselves alleviate as much as possible.

Day one ended with a fantastic three-course meal, served in the Bluecoat's restaurant. While eating delicious food, delegates got to hear some personal stories. Jim Fish, from Canada, and Flavia Mayrink, from the AKU Society in Brazil, talked about growing up with AKU. Nick Sireau, our CEO, reminded everyone how he threw himself into the AKU Society when both his sons were diagnosed with the disease.

Nick said that we are living through a golden age of AKU research. Never have the medical and scientific worlds been so interested in AKU. At the heart of what we do, though, are still the patients. As they left, many said they felt less isolated. They were not alone with their condition, and there was hope for the future.


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25th November 201
The Bluecoat
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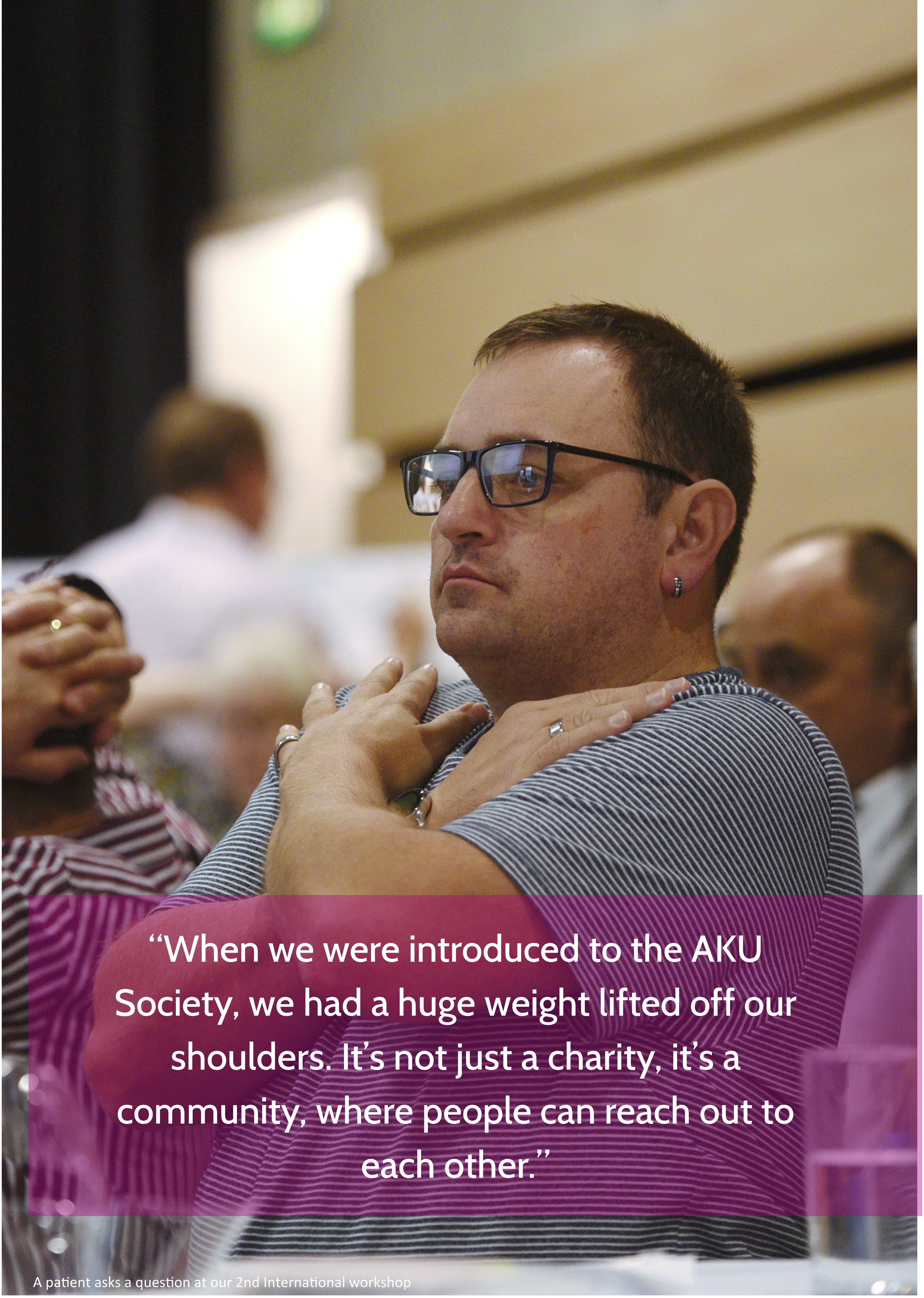
AKU
Alkaptonuria Society
Transforming the lives of AKU patients
lack Bone Disease



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A patient asks a question at our 2nd International workshop

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Graphic

High Frequency of AKU in S

- AKU prevalence in general (1:100,0
250,000)
- In Slovakia prevalence 1:19,00
- The common European AKU chromo
have had marginal contributions to t
AKU gene pool
- Most of the AKU chromosomes have
origins in a single small region in th
Carpathian mountains.
- – Andrea Zatkova et.al Am. J. Hum G
67:1222-1339,2000

Medical Research

2018 was a busy year for research at the AKU Society. It was the last year of the SONIA 2 clinical trial (Suitability of Nitisinone In Alkaptonuria 2) and the end of the DevelopAKUre programme. DevelopAKUre was a series of trials run by a consortium of 13 European partners all with one ambition, to prove that nitisinone works in treating AKU.

SONIA 2 was designed to test the effectiveness and safety of nitisinone in treating AKU. We have to wait until later in 2019 for final results; however, the one-year report and a paper published by the team at the NAC have left us feeling positive. Once we receive the results, and if they are positive, we will approach the European Medicines Agency (EMA) for a license to use the drug for AKU across Europe. We hope that if this is successful, it will then start to become available across the world.

With the help of SOBI (Swedish Orphan Biovitrum), we also surveyed 120 AKU patients from across the world. The answers will be used to understand how patients receive a diagnosis and how many doctors they see before they get one, along with how AKU symptoms affect patients. The findings will help us if and when we ask the EMA for a license for the use of the drug in AKU. We are also very close to starting a study, called the SOFIA Paediatric study. This will look at what age the symptoms in AKU begin in children with the disease. This will be used to work out at what age nitisinone treatment could start in children. We hope this will lead to a generation of children growing up without any of the symptoms of AKU.

We also continue to support other research projects and PhDs that continue to advance our knowledge of AKU and the way it affects patients. These studies are not just academic; it continues at the National Alkaptonuria Centre in Liverpool.

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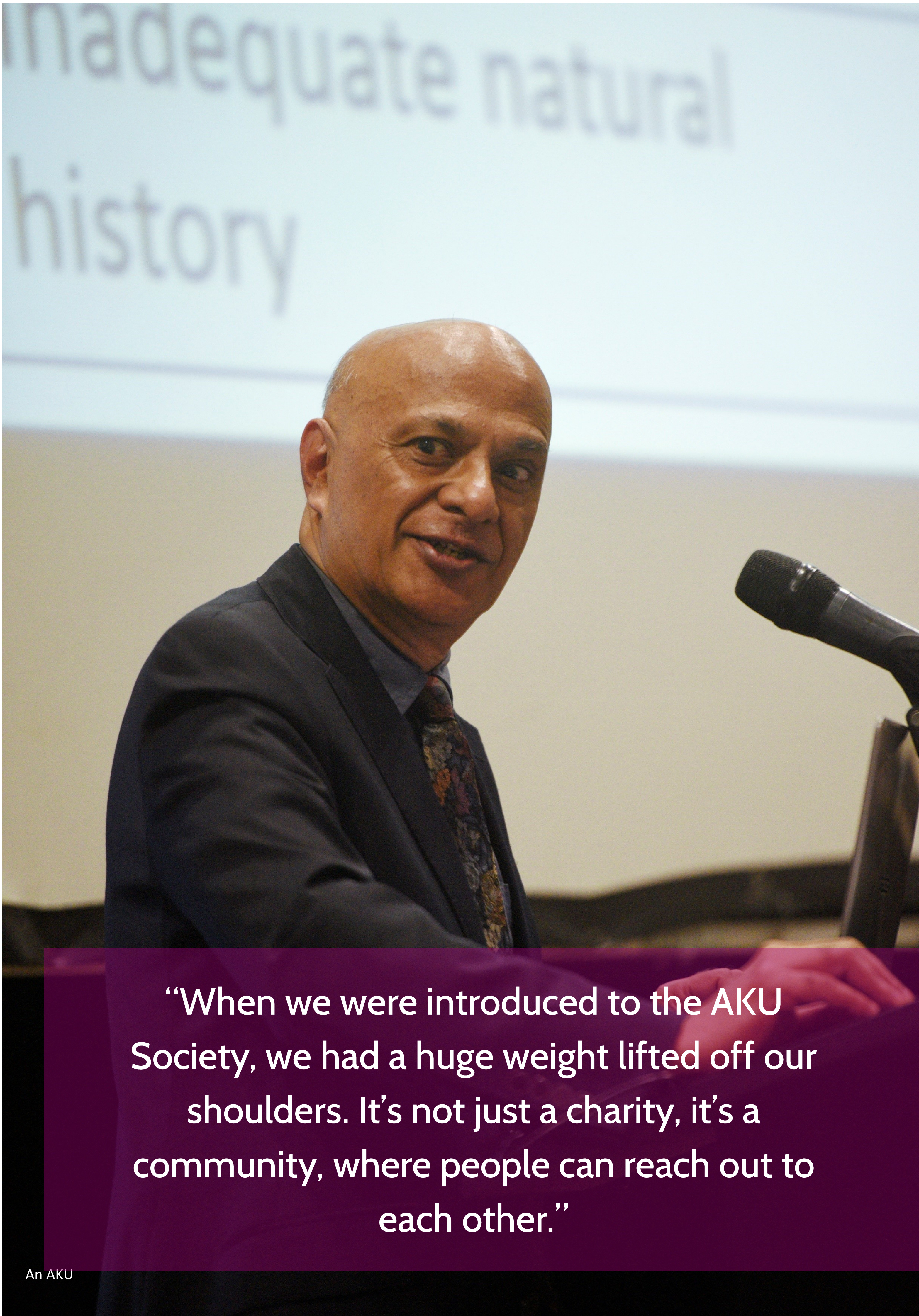

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An AKU

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Fundraising

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The AKU Society would like to say a massive thank you to our funders, fundraisers and supporters. Trusts and foundations have made up a large portion of our income and have enabled us to host patient workshops and vital medical research. Below is the list of funders who have supported us over the year and what their grant was used for:

Childwick Trust – SOFIA Paediatric study

European Commission – FP7 DevelopAKUre project. Our clinical trials into the drug nitisinone

National AKU Centre – Patient support before, during, and after NAC clinics

Rosetrees Trust – Research funding at the University of Liverpool and Lancaster University

Sylvia Adams Charitable Trust – Breaking Down Barriers ethnic minority communities project

Swedish Orphan Biovitrum (SOBI) - 2nd International Patient Workshop

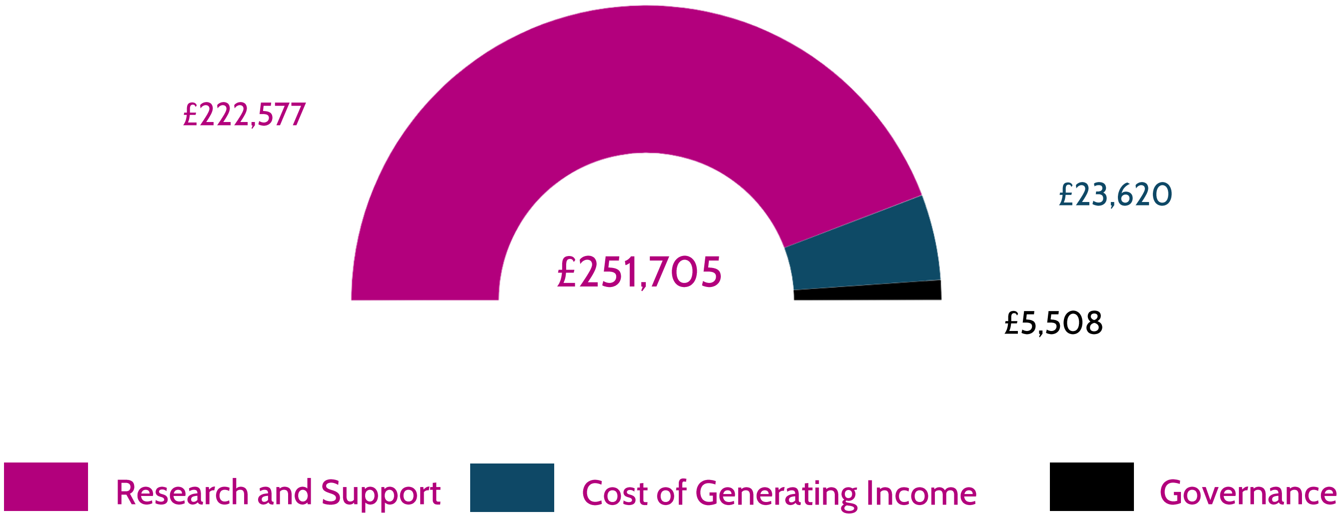
We are also very grateful to our community fundraisers this year; Jamie Rumbelow, who ran the Edinburgh half marathon; and Carol Day for hosting her fabulous Garrod Day Coffee Morning, raising £112.10. Well done!

A massive thank you to everyone who has donated, volunteered and everyone who has been involved in our events. The AKU Society is extremely grateful for your support.

Back cover: AKU Society CEO Nick Sireau : Findacure/Barbara Asboth

Income

Expenditure



	Unrestricted Funds	Restricted Funds	Total Funds
Incoming resources	£	£	£
Voluntary Income	9,903	92	19,218
Other	498	-	1,178
Research and Support	1,750	214.778	316,693
Total Incoming Resources	12,151	214,870	227,021
Resources Expended			
Costs of generating voluntary income	-	28,270	28,270
Research and support	20,041	165,286	185,327
Total Resources Expended	20,041	193,556	213,597

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AKU
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Registered Charity in England and Wales: 1101052