

**ACTION
FOR A-T**

**FUNDING
RESEARCH,
FINDING HOPE**



Annual Report & Financial Statements

for the year ended 31st December 2020

Action for A-T - Charity registered number 1145303

www.ActionforAT.org

Legal And Reference Information

Patrons

Jonny Wilkinson CBE
Roger Black MBE
Jeremy Guscott MBE
Simon Shaw MBE
Rachel Morris MBE
Naga Munchetty
Leon Haslam

Chief Executive

Sean Kelly

Trustees at the date of approval

Toby Read (Chairman)
Emily Read
William Rowberry (Treasurer)
Tomos Shillingford
Maria Leonard
Chris Askew
Dr Mark Toms
Joseph Frost

Appointed during the year or since year end

None

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Neonatologist and Research Lead

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Professor Keith Caldecott, Professor of Biochemistry 'Royal
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University of Sussex

Dr Richard Kay, Medical Statistician

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Professor Grant Stewart, Professor of Cancer Genetics,
School of Cancer Sciences, University of Birmingham

Dr Alastair Wilkins, Reader in Neurology, University of Bristol
and Consultant Neurologist at Frenchay Hospital, Bristol

Professor Matthew Wood, Professor of Neuroscience
and Deputy Head of the Medical Sciences Division at the
University of Oxford

Patient Representatives

Mr Amandeep Sharma
Mrs Clare Gallagher
Mrs Natasha Schneider

Charity Registration Number

United Kingdom Registered Charity number: 1145303

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Chairman's Report

Action for A-T is the UK's leading A-T medical research charity. We exist to harness world leading medical science to help improve the lives of those affected by Ataxia-Telangiectasia.

To all of our supporters

2020 was a year like no other due to the impact of the COVID-19 crisis upon so many aspects of our daily lives. In common with many organisations, the ambitious plans and goals we set ourselves at the start of the year were completely overtaken by events and we immediately recognised that we would need to adapt quickly to overcome the unprecedented changes the pandemic would force upon all of us.

Many of Action for A-T's traditional fund-raising activities had to be cancelled, including all face-to-face challenge and social events. This huge limitation on how we could fundraise meant the team at Action for A-T had to show enormous resilience, flexibility, creativity, and sheer hard work to find new and innovative ways to raise money. It is to their enormous credit that whilst fundraising was of course impacted, our team of talented and dedicated staff and volunteers still managed to generate significant funds for medical research in A-T. I am humbled and so grateful to have received so much continuing support from our supporters and donors who contributed both their time and money so generously, despite everyone having to cope with so many changes to their lives.

Everyone involved in Action for A-T hopes that the world will begin to return to normal before too much longer. So many people are raring to get back involved in running marathons, having cake sales, attending charity fundraising events and all the multitude of other in-person activities our supporters participate in. We look forward to turning all that positive activity into funding the A-T medical research agenda we have successfully supported for so long.

Thank you to everyone who helped to ensure that during the most difficult times in 2020, Action for A-T was able to continue to support vital research into this condition and I look forward to hopefully seeing many of you face to face during 2021.

Warm wishes



Toby Read
Chairman, Action for A-T



Emily and Toby Read

Trustees' Report

The Trustees of Action for A-T present their annual report and the financial statements of Action for A-T for the year ended 31 December 2020.

The Trustees confirm that the annual report and financial statements of the Charity comply with Charities Act 2011, The Charities (Accounts and reports) Regulations 2008 and have been prepared in accordance with the Statement of Recommended Practice on Accounting and Reporting by Charities (Charities SORP FRS102).

Reference and Administrative Details

Action for A-T is a charity registered in England and Wales (No. 1145303). The Trustees listed on page 2 have overall responsibility for the strategic direction and effective governance of the Charity. The Trustees met in February, June, September, and November in 2020. The Charity is governed by the terms of its Trust Deed adopted on 6 January 2012 and as amended on 2 February 2012.

Public Benefit

Action for A-T's charitable objectives are set out in its governing Trust Deed, summarised in the "About Us" section on our website and include undertaking rigorously evaluated and properly conducted medical research into finding a cure or new treatment for the rare genetic condition Ataxia Telangiectasia (A-T).

During the year, the charity has continued to manage its ongoing research portfolio as well as committing funds to a variety of new A-T related research projects. Details of this work can be seen on pages 6 to 11.

Our Trustees have considered how our work may most effectively further our charitable objectives for the benefit of the public, in particular all those affected by Ataxia Telangiectasia, and have had regard to the Charity Commission's guidance on public benefit when reviewing activities against objectives set and in planning future activities.

Objects, Achievements & Performance

Action for A-T's mission is:

"To speed up the process of identifying a cure for A-T or treatments that delay or prevent the disabling effects of this childhood condition"

To further that mission, the Charity focused on the following key areas in 2020:

- Identifying & funding research into A-T
- Ensuring that our research is high quality, peer reviewed and has clear and achievable aims
- Strengthening our fundraising capabilities
- Building collaborative partnerships
- Raising public awareness about A-T

Identifying High-Quality Research into A-T

Our Aim

Action for A-T aim to build a community of A-T research leaders and increase the amount of research that is taking place here in the UK. Although we will always fund the highest quality applications wherever they are from; our key focus is to invest in projects and people based in the UK wherever possible.

Our Performance

Throughout 2020, our Chief Executive staged online meetings with key figures from the A-T research community and representatives from other medical research charities and patient groups to explore possible funding opportunities and further understand which areas of A-T research may provide the most promising outcomes.

Non-A-T researchers who specialise in related conditions were also contacted to drive future research projects and create greater interest in the condition. This strategy is repeated from year to year and once again proved very successful, enabling us to significantly increase the number and demographic of 'who' we advertise our grant rounds to.

Funding High-Quality Research into A-T

In addition to supporting and funding research aimed at improving the understanding of A-T, there are many innovative and exciting medical research opportunities that raise the prospect of real progress in combating the effects of genetic conditions such as A-T.

Our Aim

Action for A-T's aim is to be able to make grants to hospitals and universities throughout the world for the purpose of conducting research across all therapeutic areas related to A-T, with an emphasis on research that is likely to have a clear clinical application within the foreseeable future and/or which evaluate innovation in medical techniques which may have a potential benefit for children with A-T.

Our Performance

Since Action for A-T was established in 2012, we have invested **£2,946,528** in 40 global research projects under six main research headings.

Underpinning Research - Developing new tools and techniques for conducting research

Aetiology - Understanding the causes of A-T

Detection, Screening and Diagnosis - Tests for diagnosing and monitoring patients with A-T

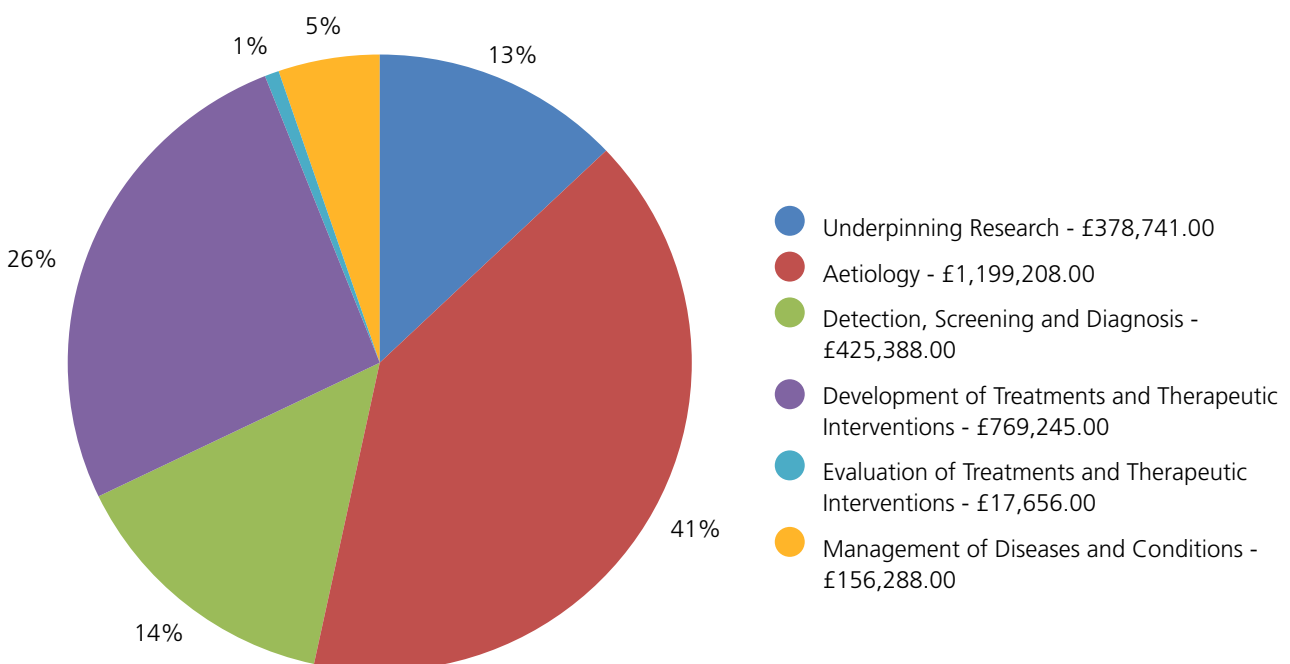
Development of Treatments and Therapeutic Interventions - Creating drugs and therapies to alleviate the symptoms of A-T

Evaluation of Treatments and Therapeutic Interventions - Studies to prove how new treatments work and understand how to use them

Management of Diseases and Conditions - Managing the symptoms of A-T

The chart below shows the historic allocation of our research investment portfolio and our total investment in each of these six research categories listed above.

Total Research Spend Since 2012



The following five grants were awarded by the Trustees in February 2020:

Using new immunotherapy methods to treat lymphoma and leukaemia in A-T

Principal Researcher: Dr Elad Jacoby
Institute: Sheba Medical Centre, Israel
Cost: £143,400 over 18 months
Start Date: March 2020

Project Overview



Dr Elad Jacoby

In recent years, immunotherapy has revolutionised the field of blood cancer therapy. Immunotherapy is a type of treatment which prompts the body's own immune system to identify and destroy cancerous cells. CAR T-cell therapy is a form of immunotherapy that uses specially altered T-cells, which are a type of white blood cell that plays a central role in the immune response.

CAR T-cells have been shown to be extremely effective in the therapy of blood cancers, without producing the usual side-effects of chemotherapy. Currently, there is no available data regarding the feasibility and safety of CAR T-cell production in A-T patients and given high morbidity with traditional therapies, the proposed study could pave the way for safer treatments of lymphoma and leukaemia in patients with A-T.

Dr Jacoby and his team at the Sheba Medical Center believe that CAR T-cell immunotherapy for lymphoid malignancies in A-T patients is feasible and may lead to acceptable clinical results in the absence of adverse genotoxicity. The team plan to activate, transduce and expand T-cells from patients with A-T and study the feasibility of this process in-vitro, as well as analysing the CAR integration sites in the genome as part of the safety assessment. They will test this hypothesis using blood immune cells taken from 15 A-T patients and 15 healthy participants to study the viral integration of the CAR in products of CAR T-cells.

As the study is preclinical there will be no direct benefit for participating patients. However, as cellular immunotherapy is available in many countries, should the study findings prove the feasibility and safety of this method, A-T patients will be able to receive treatment either with commercial products or on clinical trials in the future.

Recurrent pulmonary infections and their impact on neurological decline in A-T

Principal Researcher: Professor Margot Mayer-Proschel

Institute: University of Rochester, New York

Cost: £246,691 over 36 months

Start Date: July 2020

Project Overview



Professor Margot Mayer-Proschel

Professors Mayer-Proschel and O'Reilly have previously generated a novel mouse model to study the early pathologies identified in A-T patients and established that their model recapitulates brain pathologies that mark the onset of neurological decline as seen in A-T patients. The models are also highly susceptible to recurrent respiratory viral infections and display a defective immune response that is associated with dysfunctional repair of the lung. While these defects have thus far been studied in isolation, the team propose that the vulnerability of the lung in A-T patients is an important contributor to neurological decline. They will now study whether respiratory viral infections and lung disease can affect the neurological status of patients and whether it can contribute to disease progression.

Many A-T patients suffer from chronic and reoccurring respiratory infections. While neural and lung pathologies have been studied in isolation, emerging evidence suggests that poorly controlled respiratory infections are associated with neurological decline. The team hypothesize that the neurologic decline seen in A-T individuals might be driven or exacerbated by systemic inflammatory responses that develop when the lung fails to effectively defend against infections. Subsequent studies in their developed animal model showed that these display an impaired ability to respond to recurrent respiratory viral infections and develop dysfunctional airway pathology reminiscent of those found in lung biopsies of A-T individuals.

The team have found no other reports that formally test a lung-brain connection in A-T neurologic disease and saw a unique opportunity to fill this knowledge gap. They will use state of the art techniques to analyse central nervous system and lung pathology using a combination of lab and animal-based experiments. They will work to manipulate and characterise lung and brain function in A-T and hope to yield new insights into the progression of pathology in A-T. Individual iPSCs will be grown and differentiated into cerebellar organoids using a defined protocol which makes use of molecules that reproduce development of the cerebellum in vitro (in a dish). Organoids will be validated using a variety of biochemical and immunohistochemical techniques. This involves testing of genetic material and cutting/staining sections of the mini brains to assess they have made the right cell types to learn about the cerebellum. Organoids will be prepared for single-cell sequencing, in collaboration with the core facility at the Wellcome Trust Centre for Human Genetics. This means the team can examine one cell at a time, from thousands of replicates, from large numbers of organoids, giving them the best resolution to dissect out the genetic basis of dysfunctional ATM in Purkinje neurons. This data will then be analysed both in-house and with collaborators.

Deciphering the molecular mechanism linking Ataxia-Telangiectasia and Ataxia-Telangiectasia-Like Disorder

Principal researchers: Dr John Reynolds

Institute: University of Birmingham

Cost: £105,183 over 36 months

Start Date: October 2020

Project Overview



Dr John Reynolds and Grant Stewart

Dr John Reynolds and his team at the University of Birmingham have recently identified a unique ATLD patient who exhibits all the clinical symptoms associated with ATLD, but in contrast cells from this patient can activate ATM efficiently and can repair IR-induced DNA damage normally. This suggests that a global inability to repair DNA double strand breaks may not be the underlying cause of the ataxia in ATLD and A-T patients as previously thought.

The team have discovered that the MRE11 mutation present in this unique ATLD patient specifically causes a defect in repairing DNA damage induced by the activity of an enzyme called Topoisomerase 2 (TOP2). TOP2 is a protein that resides in the cell and helps to relieve tangles in the DNA, called

supercoils, by cutting the DNA and allowing it to unwind, before resealing the break. This activity of TOP2 is very important for regulating the expression of specific genes, particularly genes associated with brain development and function. However, in certain situations TOP2 is unable to reseal the DNA break, and as such the cell requires the activity of other proteins e.g. ATM and MRE11, to repair the damage. Therefore, it is possible that mutations in MRE11 that cause an inability to repair DNA breaks caused by the activity of TOP2, will block the expression of genes that are required for brain development and function, leading to a progressive decline in neuronal fitness.

Based on these preliminary observations, the team will study how this unique mutant MRE11 protein causes a specific defect in the repair of TOP2-associated DNA double strand breaks and how this can be linked to the neurodegeneration exhibited by patients with A-T and ATLD.

Dr Reynolds and team believe that the results from this study will benefit clinicians involved in diagnosing patients with ATLD and atypical A-T. Clinically, patients with ATLD and A-T are nearly indistinguishable, and one of the diagnostic tests for ATLD, like A-T, is a failure to activate ATM following exposure to ionising radiation. Since the implications from this work is that patients with clinical ATLD may not exhibit an inability to activate ATM, this could affect the ability of the clinician to diagnose these patients properly. Results from this study will identify which DNA damage inducing agents should be used instead of, or in addition to, ionising radiation when carrying out diagnostic research to confirm a genetic diagnosis of ATLD.

*The David Peake Study to test the feasibility of whole-body MRI for cancer surveillance in children and young people with Ataxia Telangiectasia

Principal Researcher: Dr Rob Dineen

Institute: University of Nottingham

Cost: £100,557 over 24 months

Start Date: October 2020

Project Overview



Dr Rob Dineen

At present, there are no guidelines on how children and young people with A-T should be screened for cancer. Scans that use x-rays, like CT scans, can be particularly harmful in people with A-T so the current practice at the UK Paediatric A-T clinic is to perform blood tests. These tests can detect most types of leukaemia but are not effective at detecting solid tumours. As highlighted in a recent international consensus statement, effective cancer surveillance strategies for A-T patients are urgently required.

Dr Dineen and his team at the Queens Medical Centre will carry out a feasibility study of cancer surveillance using modified blood tests and whole-body MRI. They will recruit 20 participants with A-T who along with their parents and carers, will be invited to participate in workshops and focus groups to fully understand the impact of the tests and any findings.

The team will use advanced MRI scanning techniques to search for signs of cancer and to establish the technical feasibility of whole-body MRI. They will also carry out radiological analysis of the spectrum of findings taking appropriate action where needed. Each study participant will be scanned, and a Band 7 NHS Research Radiographer, Band 7 NHS Paediatric nurse and health psychologist research assistant will also be appointed to coordinate the various elements of the study.

This feasibility study will equip the research team with the vital information needed to develop a larger trial to test whether cancer surveillance using whole body MRI scanning and blood tests is effective in children and young people with A-T. If successful, the proposed cancer surveillance programme could provide an effective way to identify the cancers in children and young people earlier, allowing earlier referral to the specialist centres. Because A-T is such a rare disease, and because a trial of cancer surveillance would be very difficult for other groups to deliver, it is highly likely that any guidelines developed from this UK-led trial would be adopted in many other countries.

**This research study was part funded thanks to a generous donation from the David Peake Appeal. David lost his battle with Pancreatic Cancer in 2016 and his family chose to commit the funds raised by his work colleagues at Grange Aston Martin and Cambria Automobiles PLC towards this project.*

A new function for ATM and a new theory for the clinical abnormalities in A-T

Principal Researcher: Professor Malcolm Taylor

Institute: University of Birmingham

Cost: £53,619 over 24 months

Start Date: March 2021

Project Overview



Professor Malcolm Taylor

Building on their initial pilot study in 2011, Professor Taylor and his team at the Institute of Cancer and Genomic Sciences will perform a detailed survey of cancer incidence in a cohort of ~300 UK individuals with A-T to gain a more accurate assessment of the tumour types that individuals develop.

The cohort of patients are registered with the national A-T clinic in Nottingham where Professor Taylor is well known having diagnosed the ATM mutations in virtually all 300 patients. Approximately 200 are still alive, some having already been treated for a cancer and of the ~100 that are deceased, 50% were due to cancer.

The data on cancers in this population will be analysed in a standard manner, in terms of the types of tumour and incidence rates of the different cancers compared to the normal population. Where tumour material is available, further molecular characterisation of the tumours using techniques such as whole genome sequencing and RNA-Seq will be performed. These will give a detailed assessment of both the mutational and transcriptional spectrum of the tumours and provide insight into the cancer phenotype within A-T, identifying the genetic drivers and biochemical pathways that promote and maintain this phenotype.

The results of the survey will enable the team to compute the risk of A-T patients developing cancer plus the full range of cancers they are predisposed to. They also hope to determine how the tumour type is related to the particular ATM mutations carried by the individuals and if there is any impact of the different types of mutation on clinical outcome.

COVID-19

Shortly after our 2020 grant awards were made, the COVID-19 pandemic became a worldwide problem and operational restrictions were enforced on virtually all research institutions. As a result, the proposed start dates of the above studies were delayed and many existing grant recipients were forced to put their work on hold and subsequently request no costs extensions. At the time of writing this report, the pandemic continues to impact our daily lives and we expect there to be further delays to a large proportion of our research portfolio. We are monitoring the situation and working closely with our grant holders to help minimise any disruption to their work.



Ensuring that our research is of the highest quality, is well managed and has clear and achievable aims

Research is at the heart of what we do, and our research strategy outlines our intentions on achieving our mission.

Our Aim

Through our award-winning research process we aim to only fund projects of the highest-quality that have the greatest chance of advancing our understanding of A-T, improving treatment and management, and accelerating benefits to patients.

Our Performance

The Trustees generally stage one grant round per year. Applications are peer reviewed by independent external reviewers before they are passed on to our Research Advisory Committee for further review. Projects which pass the peer review process and are deemed high enough quality are passed on to the Trustees to make the final decision based on several key criteria including available funds, strategic alignment, and existing research commitments.

Action for A-T administered its own full grant round at the end of July 2019 and a record number of applications were received from a variety of global research institutions. These applications were subject to our rigorous award-winning peer review process to ensure that the money generously raised by our donors was put to the best possible use and provide assurance to our Trustees.

Our Research Advisory Committee (RAC) are part of this process and made up of independent scientific experts from fields related to A-T, including clinicians, non-clinicians, and statistical expertise from a wide range of UK institutions. Lay members are also on the committee to incorporate the views of parents and patients living with A-T, bringing a valuable and unique perspective over what research should be funded. The RAC met in January 2020 to formally review all the applications and their funding recommendations were passed on to the Trustees.

Project outcomes and sharing knowledge

We believe that sharing project outcomes and knowledge is a fundamental requirement for the development of new research strategies which will ultimately benefit those living with the condition. Progress, annual and final reports are mandatory for all our research grants to ensure that they are reaching the agreed milestones. These reports are formally reviewed by our scientific advisor to ensure that each project is in line with its stated aims and the outcomes are then converted into lay summaries to share with our supporters.

In addition, we also ask researchers to use the Global Research Impact Assessment Platform called Researchfish to report on their projects periodically and several years after their grant has ended.

Researchers are also encouraged to publish their findings in medical journals and where possible, make the findings freely available. The outputs of research such as publications usually occur sometime after a research project ends and it can take many years to translate research ideas into new treatments or therapies to improve quality of life.

The outcomes of all the concluded research we have funded are displayed on our website under our 'Completed Research' section. Where applicable, these posts also include link to any online publications and we also share project information via our social media channels and in various communications to our supporters and potential donors.

Evaluating the outcomes of our research

As with all charities, we are passionate about demonstrating the impact of our work and understand the importance of monitoring the progress and outcomes of our research. Throughout the year we continued to work with our scientific advisor and other members of our Research Advisory Committee to oversee and evaluate the effectiveness of our ongoing research portfolio. Between them, they monitored the progress of ongoing projects and signed off on the projects which were completed to help ensure that the original research aims stated in the application were fulfilled.

The following studies concluded in 2020 and the initial project outcomes are as follows:

Identifying new therapies for blood cancers in patients with A-T

Principal Researcher: Professor Malcolm Taylor

Institute: University of Birmingham

Project Completion Date: January 2020

This research was funded in partnership with BrAshA-T

Project Overview



Professor Malcolm Taylor and team

Patients with Ataxia Telangiectasia (A-T) have an increased risk of developing blood cancers. These patients do not have a protein called ATM which is important for response to chemotherapy treatment. Consequently, when receiving chemotherapy for their blood cancers A-T patients experience substantial toxic effects. Therefore, new less toxic, blood cancer-specific therapies are highly needed.

Previously, Professor Taylor and his team developed a mouse model in which new treatments could be tested. This model lacked the ATM protein and had reduced development of T-cells, a combination which allows them to develop a variety of blood cancers that occur in human A-T patients. Upon investigation of these blood cancers the team noted that a

protein called c-Myc is particularly active in cancer cells. In this study the team aimed to explore different ways of suppressing the action of c-Myc. They also wanted to test whether the addition of small molecules that inhibit c-Myc can stop cancer progression, and if these molecules could be used in combination with low doses of chemotherapy.

Project Outcome

The first objective, using a technique 'in-vitro' (experiments performed outside of a living organism such as in a test tube/petri dish), looking at inhibition of tumour cell lines (by inhibiting c-Myc) was successful. The team found inhibiting several specific pathways within the c-Myc protein particularly effective, including inhibiting Aurora kinase B, BET and mTOR. The team were also able to look at combinations of inhibitors that might have a synergistic effect.

For the second phase of testing 'in-vivo', modifications of the project had to be made as there were insufficient mice with blood cancer to test all the treatments identified in-vitro.

They were therefore able to test certain inhibitors and able to use a slightly different mouse model in bigger numbers. BET and mTOR inhibition showed particularly effective tumour inhibition. The research team recognise the need to carry out further inhibitor tests in more mouse models to make further final conclusions. In the medium term, this work provides the basis to potentially develop more treatment options for one of the commonest malignancies seen in A-T patients.

Publications

At the time of writing this report, a publication is currently being prepared.

A Zebrafish model of Ataxia Telangiectasia (A-T)

Principal Researcher: Dr Andrew Grierson

Institute: Sheffield Institute for Translational Neuroscience (SITraN) & The Bateson Centre, University of Sheffield

Project Completion Date: June 2020

Project Overview



Dr Andrew Grierson

Almost all human disease genes (approx. 70%) have an equivalent in zebrafish. While humans and zebrafish may not look alike, they are similar. Zebrafish are vertebrates (have a spinal cord) which is critical for modelling disorders of the nervous system such as A-T. This research aimed to genetically engineer zebrafish carrying mutations in the gene, called ATM, that causes A-T in human patients. The team

aimed to explore whether zebrafish could provide an adequate animal model for A-T. There is a need for adequate models as currently there are limitations with conventional animal models of A-T. They do not develop the neurological characteristics (cerebella degeneration) of the disease that an A-T human patient does. Once the research team created the zebrafish, they planned to look at the brain abnormalities seen in A-T and look at potential therapeutic drugs that may improve such symptoms.

Project Outcome

The team were able to generate the ATM mutant zebrafish as planned using standard CRISPR techniques. For the second part of the project, they moved on to characterizing the phenotype of the ATM mutant zebrafish, particularly in the context of the neurological characteristics seen in human A-T patients. The cerebella of 12-month-old zebrafish were examined (both A-T & non A-T types). Unfortunately, in the A-T phenotype zebrafish there was no evidence of cerebella degeneration; both pathologically and functionally (using swimming behaviour). But there were some interesting findings which may be worth further exploration. Like patients, these fish were infertile, however, while A-T patients are sensitive to radiation, these fish are not. A-T patients also have a high incidence of cancer – uncontrolled cellular growth, and the team found that the male mutant zebrafish also exhibit uncontrolled cellular growth in their testicular tissue. It was unclear whether this was due to neoplastic (cancer) changes or not. This project will not influence further research in this area but has answered the original question as to whether zebrafish generated with new CRISPR technologies can be used as a model for the ataxia seen in A-T.

Publications

The work will now be written up as a PhD thesis. Then a paper will be written to submit for publication.

Unravelling the antibody deficiency in Ataxia Telangiectasia

Principal Researcher: Associate Professor Mirjam Van der Burg

Institute: Leiden University Medical Centre (LUMC), The Netherlands

Project Completion Date: November 2020

Project Overview



Professor Mirjam Van der Burg

A-T is characterised by progressive cerebellar ataxia, chromosomal instability, immunodeficiency and increased cancer susceptibility. Patients' lives are shortened, usually from cancer or chronic lung failure, of which the latter is partly due to recurrent infections. Patients have an increased risk of severe infections because of a defect in antibody production by B-cells. The team aimed to explore and understand the immunoglobulin/antibody defect seen in some patients with A-T, recognising that the defect is seen variably in patients. Those patients with severe immunoglobulin defects are known to have an earlier onset disease and a higher risk of malignancy. Once the defect was better understood the team planned to use this information to explore potential therapeutic targets and hoped to identify which patients were at risk of severe antibody deficiency.

Project Outcome

The findings of this study have the potential to be clinically useful in the short term given that A-T can be detected on Newborn Blood Spot (NBS) screening. The NBS is the heel prick test taken on all babies in the UK by the midwife on Day 5 of life. Currently in the UK SCID (and hence A-T) is not one of the 9 conditions screened for on the blood spot. Decision making on which rare conditions should be screened for is influenced by a variety of factors; however, the fundamental premise is that early diagnosis should improve outcome.

It could be argued that this is currently not the case in A-T, a situation that may change as new treatments are discovered. The team from the Netherlands discussed the findings with families who universally stated despite there not being specific treatments to improve outcome in A-T they would like to know of the diagnosis early.

The impacts of the CSR and T-cell defects need to be further explored and once better understood the research team acknowledge may lead to new clues for treatments.

One of the discoveries has the potential to have clinical impact in the short term (screening). The defects in the immune system that the team discovered during this research project need further work but do in the longer term have the potential to have clinical impact via the discovery of new potential therapeutic strategies.

Publications

The team have published 2 papers, have a third which has been accepted with some minor revisions and are preparing a 4th manuscript.

Schoenaker MHD, Blom M, de Vries MC, Weemaes CMR, van der Burg M, Willemsen M. Early diagnosis of ataxia telangiectasia in the neonatal phase: a parents' perspective. *Eur J Pediatr.* 2019.

Blom M, Schoenaker MHD, Hulst M, de Vries MC, Weemaes CMR, Willemsen M, Henneman L, van der Burg M. Dilemma of Reporting Incidental Findings in Newborn Screening Programs for SCID: Parents' Perspective on Ataxia Telangiectasia. *Front Immunol.* 2019;10:2438.

Weitering T, Takada S, Weemaes CMR, van Schouwenburg PA, Van der Burg M. ATM: Translating DSB response to adaptive Immunity. *Trends in Immunology* (accepted with minor revisions)

Takada S et al. Unraveling antibody deficiency in patients with Ataxia Telangiectasia (manuscript in preparation)

Longitudinal brain changes in childhood A-T

Principal Researcher: Dr Rob Dineen

Institute: University of Nottingham

Project Completion Date: November 2020

This research was funded in partnership with BrAshA-T

Project Overview



Dr Rob Dineen

Dr Dineen and his team at the Nottingham University Hospital wanted to understand longitudinal brain changes in children and young people with A-T. They wanted to know if the progression in movement and other nervous system problems is closely matched by changes in the structure of the back part of the brain that controls coordinated movements (the cerebellum) as measured on MRI scans of the brain. The cerebellum is known to be the main part of the brain to be affected by A-T, but knowing that changes over time are accompanied by changes in the movement and other nervous system problems could be very useful for trials of new treatments for A-T. In such trials, being able to show that treatments slow or stop the damage to the cerebellum will be extra evidence of a positive effect of the treatment, and that could be helpful in reducing the movement and other nervous system problems. Lastly, the team wanted to trial whether MRI scans could be carried out in very young children (aged 3-6 years) with A-T.

Project Outcome

The team showed that young people with A-T demonstrate a greater reduction in the size of the cerebellum than older children and young adults with A-T. They found that this early reduction in the size of the cerebellum matched the time at which children with A-T experienced the most rapid increase in problems with movement and other nervous system degeneration. Their data shows that the cerebellar volume in A-T patients decreases up to the age of 12 years but then stabilises. The team replicated previous data showing the decline in clinical status occurs to a greater extent in early rather than later childhood. They were also able to show that there was a correlation between changes in cerebellar volume and neurological status.

These findings proved the original research hypothesis that changes in brain MRI metrics reflecting pathophysiological processes in A-T will predict changes in neurological status. There is potential clinical impact given that the changes on MRI and associated neurodegeneration appear to occur early in childhood (i.e. before 12 years) and therefore certain potential treatments should be targeted at this age group.

Unfortunately, the team struggled to recruit very young children to the study (not helped by recruitment having to end due to the COVID-19 pandemic), and while they found that some young children were happy to have the scan, others did not want to. This approach may work for future research studies, but clearly clinicians will need to consider each child individually and work closely with the parents / carers to make sure the child is likely to respond well to the preparation for the MRI scan.

Publications

R.A. Dineen, C.V. Blanchard, S. Pszczolkowski, S. Paine, M. Prasad, G. Chow, W.P. Whitehouse and D.P. Auer (2021) Accumulation of Brain Hypointense Foci on Susceptibility-Weighted Imaging in Childhood Ataxia Telangiectasia. American Journal of Neuroradiology DOI: <https://doi.org/10.3174/ajnr.A7107>

The use of patient stem cell-derived mini-brains to study A-T

Principal Researcher: Dr Samuel Nayler

Institute: University of Oxford

Project Completion Date: December 2020

This research was funded in partnership with BrAshA-T

Project Overview



Dr Esther Becker and Dr Samuel Nayler

What makes a person with A-T different from you or I? Since the ATM gene was first identified in 1995, numerous teams worldwide have committed themselves to investigating this question. The most debilitating aspect of this disease, the neurological implications, arises from degeneration of the cerebellum. The cerebellum sits at the back of the brain and communicates to most other brain regions, it can be thought of as the control centre to the brain. Why though, does mutation of this gene, affect this brain region so profoundly? Dr Nayler and his team set out to make cerebellar organoids or ‘mini-brains’ from induced pluripotent stem cells (iPSCs) to help answer these questions.

Project Outcome

The team were successful in creating ‘mini-brains’ from induced pluripotent stem cells (iPSCs) as per their aims. This is the first step in trying to find drugs which will ultimately make these differences disappear. Thanks to the data generated with these mini-brains, it will be possible to use sophisticated computational methodology to predict if there are drugs which will be effective to treat these differences. The direct clinical impact of this work is some way off as any potential treatments will take many years to research and validate, however there is a clear plan from the team going forward as to how this could happen.

Publications

Nayler SP, Becker EBE. (2018) The Use of Stem Cell-Derived Neurons for Understanding Development and Disease of the Cerebellum. *Frontiers in Cellular Neuroscience*. 12:646. doi: 10.3389/fnins.2018.00646.

[Van Essen M, Nayler SP], Becker EBE, Jacob J. (2019) Deconstructing cerebellar development cell by cell. *PLOS Genetics*. *joint first-authors. <https://doi.org/10.1371/journal.pgen.1008630>

[Van Essen M, Nayler SP], Jacob J, Becker EBE (2020, In press). Modelling cerebellar development using induced pluripotent stem cells. *Neuromethods*.

Nayler SP, Agarwal D, Curion F, Bowden R, Becker EB (bioRxiv, 2020, & In preparation). Singlecell sequencing of human iPSC-derived cerebellar organoids shows recapitulation of cerebellar development. [co-first authors]

To view all of our current and complete research projects, please visit <https://actionforat.org/category/research-projects-funded/>

Strengthening and Increasing our Fundraising Activities

Our Aim

Action for A-T funds high quality peer reviewed medical research to speed up the process of identifying a cure for Ataxia Telangiectasia (A-T). We endeavor to raise funds from a variety of activities which appeal to new and existing supporters. Since 2012 we have invested just under £3,000,000 in 40 A-T related research projects around the world and this has only been possible thanks to our generous supporters.

Our Performance

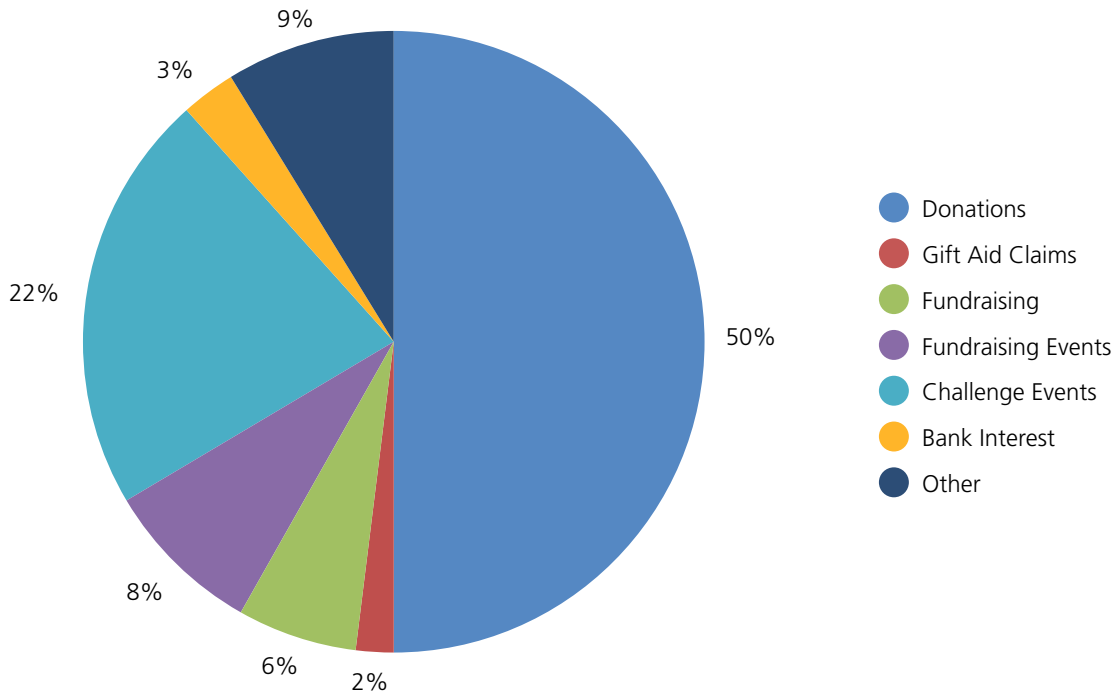
Action for A-T are registered with the Fundraising Regulator (previously the Fundraising Standards Board) and are committed to upholding the code of fundraising practice. We receive all our funding from voluntary sources, including individuals, companies, trusts and foundations. We also purchase places in several physical challenges as well as staging and benefiting from a series of other social and sporting events.

2020 was an incredibly difficult year for fundraising as we were forced to cease most of our events activities due to the ever-changing situation relating to the COVID-19 outbreak. Events usually account for approximately 60% of our total income and normally provide a steady and reliable source of funds, but restrictions on group gatherings meant they were unable to take place. We adapted to the new fundraising environment quickly, and where possible staged and benefited from virtual style events. We also significantly increased our trust and foundation applications and applied to various emergency funding schemes for support. As well as raising funds, we also reduced our overheads by taking advantage of the government led furlough scheme and making significant reductions to our general and administrative expenses.

In 2019 places were purchased for the 2020 Virgin London Marathon and 80 unused places were carried forward for the 2020 Prudential Ride 100 event. The Virgin London Marathon (along with virtually all other mass participation events) was cancelled, and the unused places were carried forward to 2021. The Prudential Ride 100 was also cancelled and due to the uncertainty about its future, we were provided with several Virgin London Marathon places for use in future years. Both event organisers staged virtual versions of their events to placate entrants and provide fundraising opportunities for participating charities. These activities enabled us to raise £56,000 allowing us to make a 360% return on our original investment, as well as providing us with several free places for future London Marathons. The Trustees also agreed a material investment in an advertising package for the 2021 London Marathon which includes a further 9 places.

In line with our fundraising strategy and as a direct result of the pandemic, we diversified our income base to ensure that we were not solely dependent on any one source of funding. Ongoing COVID-19 restrictions meant that Challenge and Social events performed far worse than in previous years, accounting for only 30% of our income (2019 - 59%). Whilst other fundraising initiatives, such as donations, funding partnerships and trust and foundation applications increased to 56% (2019 - 29%). Very few new fundraising activities took place throughout the year but some of our existing supporters were able to stage their activities in a virtual format. We are especially grateful to the staff and clients of BCA yet again for choosing to stage a virtual one-day cycle event (instead of their planned overseas ride) which raised over £30,000. We are also extremely grateful to Simon Toon and his friends for their multiple fundraising efforts throughout the year and our patron Naga Munchetty who secured a £32,000 donation after appearing on the "Who Wants to Be a Millionaire" gameshow. In addition, we would like to thank Quest Professional, Psigma Investment Management and St James's Place for their generous corporate support.

The pie chart below shows the percentage splits of each of our areas of fundraising in 2020.



Fundraising for Research

We would also like to record our thanks to all our volunteer committee members for their time and commitment in helping us achieve our mission, as well as everyone who donated their time, auction prizes and/or supported or participated in our events.

In addition to the above, we are also extremely grateful to the trusts and foundations who provided emergency funding grants throughout the year and restricted funding for various research projects (these are listed on page 50). We are always keen to work with other trusts, foundations and corporate funders who might be interested in supporting our work.

At the time of writing these accounts, the COVID-19 pandemic continues to significantly impact the way in which every organisation operates around the world. As a result, we have been forced to cancel many of our larger scale social events and delay many of our planned activities until much later in the year. We hope that the vaccination programme will be completed towards the end of the summer, allowing us to stage and benefit from many of our planned activities. It is hard to predict the potential impact that the ongoing pandemic will have on our overall income, but it seems unlikely at this stage that we will be able to deliver the fundraising budget which was signed off by the Trustees in January. We continue to react to the crisis by further diversifying our fundraising activities and reducing our costs to ensure that there are no going concern issues.



Events since the end of the year

Information relating to events since the end of the year is provided in the notes to the financial statements.

Building Collaborative Partnerships

Our Aim

Action for A-T strongly believes that there are many opportunities to further the search for a treatment or a cure for A-T through working with others that may have overlapping aims or objectives to our own. To this aim, we actively seek out like minded organisations and where possible, create collaborative partnerships.

Our Performance

Co-funding A-T Research Projects with the UK based Children's Medical Research Charity Sparks

Action for A-T continued to work closely with UK Children's Medical Research Charity Sparks which joined forces with Great Ormond Street Hospital Charity as a subsidiary in February 2017. Sparks are currently managing the following jointly funded research project which is due to end in the first quarter of 2021:

- "Optimising lung imaging in people with A-T" - Dr Peter Merkus at the Radboud University Medical Centre, Netherlands

Co-funding A-T Research Projects with the US Charity the A-T Children's Project

Action for A-T and the US based A-T Children's Project joined forces at the end of 2018 to fund two new research projects. Both studies are being managed by the A-TCP and Action for A-T has contributed towards each project. The studies are as follows:

- "Non-viral gene therapy for Ataxia Telangiectasia" – Dr James Dixon at the University of Nottingham, United Kingdom
- "Genetically tailored splice-modulating therapy for A-T" – Dr Timothy Yu at the Harvard and Boston Children's Hospital, USA

Action for A-T have committed £50,000 towards the "Non-viral gene therapy" project (The final £25,000 was paid at the end of 2020) and £17,656 (\$23,000) towards the "Genetically tailored splice-modulating therapy" (this was paid in full at the end of 2018).

Co-funding A-T Research Projects with the Australian charity BrAshA-T Ataxia Telangiectasia

Action for A-T have an ongoing collaborative funding partnership with BrAshA-T who are based in Queensland, Australia. BrAshA-T agreed to support the patient stem cell-derived mini-brains study being carried out by Dr Sam Nayler at Oxford University and their agreed contribution totalling £29,460 was paid in full in December 2019. This project concluded in October 2020 and the project outcomes are shown on page 18.

Co-funding A-T Research Projects with the Australian charity BrAshA-T Ataxia Telangiectasia and the UK based A-T Society

At the start of 2019, Action for A-T established a new collaborative funding partnership with BrAshA-T and the A-T Society in connection with the Natural Histories Study being carried out Dr William Whitehouse and Dr Emily Petley at the University of Nottingham. Throughout 2020, each organisation contributed £15,230.91 towards the cost of this study. As the project is scheduled to conclude in 2022, the remaining payments are likely to be made throughout 2021 and 2022.

As the COVID-19 pandemic will impact our ability to fund new projects in 2021, Action for A-T will continue to explore additional joint funding opportunities with likeminded organisations throughout the year to maintain momentum and maximise the investment into A-T research.

Raising Public Awareness about A-T

Our Aim

As an ultra-rare disease, most people are unaware of A-T or the devastating effects of the condition. Action for A-T believes that raising awareness of the impact of A-T on children and their families is an important part of increasing the potential funds available to further improve the understanding of the condition and to progress research into possible treatments or a cure.

Our Performance

Action for A-T has significantly increased the public awareness of the condition over the past 12 months and will continue to explore all options to promote wider understanding of A-T and why research to find a cure or treatment is so important.

The Charity's main achievements in this area to date include:

Celebrity Support

Our celebrity patrons have continued to help promote Action for A-T and the need for more research into the condition by championing campaigns and taking part in various activities throughout the year. As well as participating in a virtual challenge, Naga Munchetty represented Action for A-T on the game shows "Who wants to be a millionaire" and "Celebrity Catchphrase". As well as securing substantial donations, the shows provided an opportunity to highlight the charity's work to a huge TV audience. Sir Clive Woodward was the only celebrity to attend an actual event (as this took place before the COVID-19 restrictions were enforced) but several other celebrities including Chris Sheasby, Leon Haslam, Jonny Wilkinson, Simon Shaw and Mike Tindall helped generate awareness and support by taking part in virtual events and campaigns or starring in promotional social media videos.



Visibility at Research Institutions, Conferences and Medical Meetings

All Action for A-T funded researchers are provided with promotional materials to display at their research facility and required to acknowledge our financial support by including our logo on any posters or presentations. Throughout 2020, all in-person conferences and meetings were cancelled due to the pandemic, but researchers funded by Action for A-T were able to present their findings at various webinars and online events. These presentations showcased our latest projects and provided the research community with a comprehensive view of the progress made to date.

Digital Communications

The Charity website actionforAT.org remains the focal point for event sign-ups, electronic donations, and research news. We update and enhance the site on an ongoing basis to provide the best possible experience for our stakeholders. In 2020, further upgrades were made to enhance the user experience and assist with navigation including the introduction of interactive pictogram to explain the symptoms of A-T and a complete redesign of our home page.

Our monthly e-newsletter continues to be a successful tool to communicate our latest news to supporters. Using Mailchimp for this proves to be cheaper than a traditional printed newsletter and has the functionality to track usage and monitor engagement. Our e-newsletter is sent to all our consenting supporters, has a clear unsubscribe function and inactive supporters are removed from the mailing list periodically. As promotion of events and activities was limited in 2020, the e-newsletter was predominantly used to inform supporters about our work. These updates were well received and enabled us to secure online donations, including one for £2,000 at the end of the year.

Social media remains a key communication tool for the Charity as it is free, easily accessible and can reach wide audiences. It also allows us to engage with our supporters on a more strategic level and build additional support from their own contacts. Posts are carefully managed to ensure that the content is engaging and not repetitive and this more targeted and planned approach helped us increase our followers throughout the year. We created and participated in several large social media campaigns during 2020 including #26in26, #26steps4AT, #2.6challenge and the "What is not named does not exist" campaign. These posts were viewed and shared thousands of times around the world. We will continue to develop our social media presence in 2021 using all available technological advancements and popular platforms to raise further awareness.

National and Local News Media Coverage

We continued to raise awareness of A-T through a series of articles and editorials in the local, national and industry press and various news websites. These helped us highlight the condition to new audiences, recruit volunteers and secure additional support and donations.

Film

In April 2020, Action for A-T created a new awareness-raising film featuring a grandfather telling the tragic story of his granddaughter (Jenni Gallagher) who lost her battle with A-T aged 12 years old. The film includes an interview with Dr William Whitehouse who is a Clinical Associate Professor at the Queens Medical Centre, Nottingham as he and his team are carrying out a study to plot the Natural History of A-T. The film was produced to raise awareness about the devastating effects of A-T as well as raising funds for our research programme. Throughout the year, it was shared widely on various social media channels as well as being included on our website. It will also be used to raise awareness and funds at various events when the COVID-19 restrictions are eased.

All of the charity's films are available to view on our Vimeo and YouTube channels.

<https://vimeo.com/actionforat/videos>

<https://www.youtube.com/channel/c/ActionforatOrg>



Our Future Plans: Areas of Focus for 2021

The Trustees believe that Action for A-T is well placed to meet its charitable objectives during and after the COVID-19 pandemic but have also identified the need for further development to strengthen its research activities. During 2021 we will consolidate and diversify our fundraising activities whilst actively seeking other funding partnerships to meet the demands of the A-T research community. We will also continue to promote A-T research to engage new audiences and maintain interest from the research community.

We will implement a number of measures throughout the year to ensure that the Charity continues to be fit for future purpose and to help us achieve our aim of moving closer to tangible therapies or a cure for A-T.

Adapting to the New Environment

As a small charity, we have limited resources and must therefore think very carefully before choosing to invest in new activities or personnel to help us deliver our charitable objectives. Our size and structure enabled us to adapt quickly to the ever-changing environment in 2020 but as the COVID-19 pandemic will continue to impact everything we do in 2021, we will:

- Review and prioritise projects based on available resources
- Seek out new opportunities to raise funds in a safe and sustainable manner whilst reviewing existing fundraising Initiatives, prioritising those which are most likely to be deliverable
- Utilise new and existing technology to disseminate key information to the wider A-T community
- Work closely with our grantees to minimise delays and ensure projects are progressing in line with their stated aims
- Ensure that we have the necessary skills and people to deliver our organisation wide objectives
- Keep up to date with current trends and remain flexible so that change can be implemented quickly if required

Continuing our Investment in Medical Research in the UK and Abroad

We will continue to seek out new research opportunities from the wider research community as well as focussing on key research themes related to A-T. Where possible, priority will be given to UK based projects whilst still calling for international applications where funds will allow. We will also continue to collaborate with other research charities whilst actively seeking new funding partners with similar objects to our own.

We will maintain our close relationship with the Association of Medical Research Charities (AMRC) and work in conjunction with our research advisory committee (RAC) to develop and improve our peer review process and ensure that the research we fund is of the highest quality. This will include constant evaluation of our systems and processes as well as enlisting the help and support of various A-T experts around the globe.

Membership of the RAC will be continually reviewed to ensure that we have a broad range of scientific expertise in areas relating to A-T as well as lay representation from various A-T families as Action for A-T strongly supports public and patient engagement in its research processes.

Increasing Awareness of our Work and the Condition

Raising awareness of A-T and the work of the charity is one of our key charitable objectives. To date, we have implemented various strategies including the use of celebrities, digital and printed media and the creation of emotive films featuring researchers and A-T families. We will continue to seek out new ways in which we can highlight A-T whilst focusing on two key projects in 2021. Firstly, we will work alongside other A-T organisations to create a global A-T directory platform containing key information to assist the wider A-T community. We will also deliver a family focused interactive webinar (as face-to-face meetings and conferences are not permissible) showcasing some of the research which is currently being undertaken around the world.

Working Closely with Others to Meet our Objectives

Ongoing research funding is critical to ensure that existing knowledge is expanded and interest in such a rare and complex condition is retained. Whilst COVID-19 restrictions continue to impact our fundraising capabilities, we are less able to meet the increasing demands of the research community. We will therefore continue to develop existing, and create new partnerships with major stakeholders across the globe including patients, their families/supporters, researchers, clinicians, and A-T patient groups and charities, to help maintain a funding pipeline and speed up the process of finding a therapy or cure for A-T.

Diversifying and Consolidating our Fundraising Activities

As the COVID-19 pandemic is likely to impact our fundraising capabilities for the foreseeable future, we will work hard to retain key relationships and deliverable activities whilst exploring new and innovative ways to engage safely with new and existing supporters. Our events calendar will be scaled down to comply with ongoing restrictions and wherever possible, we will embrace virtual event opportunities. We will continue to develop and expand our charitable trusts and foundations programme with a key focus on emergency funding opportunities whilst actively seeking new investment and support from individuals, corporates, and major donors. We will also work closely with our key supporters and volunteer groups to raise funds in a safe and sustainable manner and only invest in new activities which are low risk with a very good chance of successful delivery. As well as seeking new opportunities to diversify our income, we will look to reduce costs where possible and stay poised to reintroduce many of our postponed events and activities as soon as restrictions are eased.

Structure, Governance & Management

Governing Document

Action for A-T is a charity registered in England and Wales (No. 1145303). The Charity is governed by the terms of its Trust Deed adopted on 6 January 2012 and as amended on 2 February 2012.

The Trustees have adopted the following policies and procedures for the recruitment, appointment, induction and training of new Trustees:

Trustee Recruitment and Appointment

The Trustees listed on page 2 have overall responsibility for the strategic direction and effective governance of the Charity. Trustees are either elected or co-opted under the terms of the Trust deed and the total number of Trustees may not be fewer than five. If a vacancy occurs or a skills need is identified, new Trustees are recommended by the existing Trustees and interviewed by the Chairman and at least one other member of the board. Election is by majority vote and potential candidates must be over the age of eighteen and eligible to act. The Trustees will continue to evaluate the composition of the Board and will consider further appointments to broaden and strengthen the Board's capabilities to facilitate the growth of the Charity.

Trustee Induction and Training

New Trustees receive an induction pack containing a copy of the trust deed, strategy, relevant information about the Charity and its work; and also, a copy of the Charity Commission literature about the role and responsibilities of being a Trustee. New Trustees are also invited to meet the Chief Executive and team members to learn first-hand how the Charity operates on a day-to-day basis.

During the induction process, new Trustees are also told about the connected parties' rule, given a copy of the Charity's conflict of interest policy, and asked to sign a Trustee declaration form. Where the Trustees have identified that there is a connection between the charity, or its Trustees and any third party with whom the Charity has dealt with, the Trustees will identify the relationship and the amounts involved within the notes to the financial statements.

Organisational Structure

The Trustees meet formally four times per year and no business shall be conducted unless at least one-third of the total number of Trustees at the time, or two Trustees (whichever is greater) are present throughout the meeting. There are two meetings where the focus is on awarding grants for medical research and two meetings where Trustees review strategy and set operating plans and budgets. There is a review of operating and financial performance at every meeting. The Chief Executive is invited to attend all meetings of the Trustees and the Head of Fundraising is invited to report on plans and progress at specific meetings.

While most of the business of the Charity is conducted at the scheduled Trustee meetings, there are occasional ad-hoc meetings to deal with matters of special interest and regular electronic meetings are held to review ongoing governance objectives.

The Board of Trustees delegates the exercise of certain powers in connection with the management and administration of the Charity as set out below. This is controlled by regular reporting back to the Board of Trustees so that all decisions made under delegated powers can be ratified by the full Board of Trustees in due course.

The Research Advisory Committee (RAC) is chaired by a member of the Board of Trustees. Members of the RAC have a broad range of scientific expertise in areas relating to A-T but are not generally active researchers in the field of A-T in order to minimise bias and conflicts of interest. There is also lay representation on the committee as Action for A-T supports public and patient engagement in its research processes. The Trustees are very grateful to all of the members of the RAC for providing expert guidance and advice on a pro bono basis.

Research Advisory Committee

The Research Advisory Committee (RAC) is chaired by a member of the Board of Trustees. Members of the RAC have a broad range of scientific expertise in areas relating to A-T but are not generally active researchers in the field of A-T to minimise bias and conflicts of

interest. There is also lay representation on the committee as Action for A-T supports public and patient engagement in its research processes. The Trustees are very grateful to all the members of the RAC for providing expert guidance and advice on a pro bono basis.

Chief Executive

The Chief Executive is responsible for the day-to-day management of the Charity's affairs and is assisted by a small executive team who act under his direction. As well as overseeing the operations of the Charity, the Chief Executive is also responsible for implementing policies as agreed by the Board of Trustees.

Employees, Volunteers and Contractors

The Charity aims to be an organisation where employees, volunteers and contractors enjoy a sense of fulfilment and where they feel supported and developed. All stakeholders are kept fully informed about the Charity's strategy and objectives, as well as day-to-day news and events. Individuals are encouraged to give their suggestions and views on performance and strategy.

The Charity supports equal opportunities and a policy of recruitment and promotion based on aptitude and ability without discrimination is followed. Action for A-T is committed to the training, career development and promotion of all its employees. An individual's career development is assessed through annual appraisal and supervision. Training programmes are provided to meet any on-going needs, with the aim of developing individuals for both their current and their future roles.

Risk Management

The Trustees have introduced a formal risk management process to assess business risks and implement risk management strategies. This has involved identifying the types of risks the Charity faces, prioritising them in terms of potential impact and likelihood of occurrence, and identifying means of mitigating the risks.

As part of this process the Trustees have reviewed and are satisfied with the adequacy of the Charity's current internal controls and the costs of operating controls relative to the benefits obtained. Procedures have been established for reporting failings immediately to the Chief Executive and to the Board of Trustees.

It is recognised that internal controls can only provide reasonable but not absolute assurance that major risks have been adequately managed. In the opinion of the Trustees the key risks are:

- The loss of reputation due to error, or fraud.
- The loss of income due to error, or fraud.
- Insufficient numbers of Trustees to allow the Charity to continue.
- Insufficient funds to allow the Charity to meet its objectives.
- Excessive reserves without justification deterring future donors and fundraisers.

In the opinion of the Trustees the policies and procedures are currently adequate to mitigate financial and reputation loss due to error or fraud whilst maintaining a viable future financially.

Reserves Policy

The Trustees review the reserves policy each year to ensure that all relevant risk areas are included in accordance with guidance issued by the Charity Commission. Risks included are the impact of unexpected, reduced income, and the potential impact of restructuring costs and liabilities required to downsize the organisation in an orderly manner, if a permanent income reduction was anticipated.

The review concluded that the existing reserve target level of Six months of operating costs (approx. £90,000) was sufficient to cover the risks identified in the review. This allows sufficient time for Trustees and management to take appropriate mitigating actions, if required.

The Trustees continue their commitment to develop and grow the level of investment in research into A-T, whilst maintaining a focus on managing the overall costs of the charity.

Investment Policy

The overall policy of the Charity is to maintain a series of designated funds deposit accounts to ring-fence any funds which the Trustees have identified as being made available for specific research projects, or other charitable activities.

The Trustees recognise the need to review this policy on an annual basis and to look at other potential opportunities whilst appropriately monitoring the available funds and being proactive in their management, to ensure the best interests of the Charity's objectives are maintained

Funds committed for research are paid out in arrears over the duration of the project. The Trustees reviewed and agreed to deposit funds in timed deposit accounts in order to obtain the best return with minimal managed risk.

During the year the Trustees transfer any funds surplus to general running costs of the Charity into an interest-bearing account which will offer a higher rate of return than holding it in the main Charity bank account. The aim is to ensure that the fund is not diminished over time due to inflation and to ensure that these funds are invested in future research projects.

Pay and Remuneration of Key Management Personnel

The Trustee's principles are to pay staff a fair salary that is competitive within the charity sector, proportionate to the complexity of each role, it's related funding and responsible in line with the charitable objectives. Pay levels for all employees are reviewed annually and any overall percentage increases are authorised by the Board of Trustees.

Remuneration for the Chief Executive is agreed by the appropriate remuneration committee. Salaries are benchmarked using external data available from pay surveys (for the voluntary sector and charities located in and around London) and market conditions for the specific role and the target is the market median averages of these.

Ultimately however, salary increases relate to the funding available for each role and whether the increase will be affordable in the long term. To ensure pay is fair for all roles we are committed to paying all our employees at or above the London living wage as determined by the Living Wage Foundation.

Financial Review

The Trustees are satisfied with the performance of the Charity and consider that the Charity is in an excellent position to continue its activities during the coming year, and the Charity's reserves are adequate to fulfil its current obligations.

Income

At present, research into A-T is heavily dependent on charitable donations and by extension, Action for A-T's ability to instigate, organise and facilitate the fundraising efforts of our supporters and donors. The principle source of funding is donations from individuals, companies, and proceeds of fundraising events.

Total income raised in 2020 was **£458,645 (2019 - £768,819)**.

Charitable Expenditure

The cost spent on Charitable Activities Was **£839,221 (2019 - £910,844)** of which **£677,077 (2019 - £673,985)** was for grants provided for funding of research projects.

The cost of generating voluntary income was **£97,468 (2019 - £55,576)** whilst the cost of generating income from charitable events was **£45,090 (2019 - £163,705)**

Governance costs of **£19,586 (2019 - £17,578)** were incurred.

General Funds

The Trustees will aim to make best use of any unrestricted funds, however they reserve the right to retain general reserves in hand until they can be used in the most efficient manner to further the objectives of the Charity. The Trustees are conscious that it should not provide funding for research where they have not been able to demonstrate that its use will offer the best opportunity to further the knowledge and treatment into A-T. Please refer to the investment policy with regard to those times where the Trustees believe general funds are in excess of the anticipated running costs of the Charity.

Designated Funds

Designated funds of £nil were brought forward from 2019 In 2020, **£nil** of funds were passed on to the research projects detailed in note 8 on page 40.

As at 31 December 2020 designated funds carried forward are £nil.

Trustees' Responsibilities

The Trustees are responsible for preparing the Trustees' Annual Report and the financial statements in accordance with applicable law and United Kingdom Accounting Standards (United Kingdom Generally Accepted Accounting Practice).

The law applicable to charities in England & Wales requires the Trustees to prepare financial statements for each financial year which give a true and fair view of the state of affairs of the Charity and of the incoming resources and application of resources of the Charity for that period. In preparing these financial statements, the Trustees are required to:

- Select suitable accounting policies and then apply them consistently;
- Observe the methods and principles in the Charities SORP 2015 (FRS 102);
- Make judgements and estimates that are reasonable and prudent;
- State whether applicable accounting standards have been followed, subject to any material departures disclosed and explained in the financial statements;
- Prepare the financial statements on the going concern basis unless it is inappropriate to presume that the Charity will continue in operation.

The Trustees are responsible for keeping proper accounting records that disclose with reasonable accuracy at any time the financial position of the Charity and enable them to ensure that the financial statements comply with the Charities Act 2011, the Charities (Accounts and Reports) Regulations 2008 and the provisions of the trust deed. They are also responsible for safeguarding the assets of the Charity and hence for taking reasonable steps for the prevention and detection of fraud and other irregularities.

The Trustees are responsible for the maintenance and integrity of the Charity and financial information included on the Charity's website. Legislation in the United Kingdom governing the preparation and dissemination of financial statements may differ from legislation in other jurisdictions.

The Trustees declare that they have approved the Trustees report included in the previous pages

Signed on behalf of the charity's Trustees



Toby Read (Chairman)



William Rowberry (Treasurer)

Dated: 24th May 2021

Dated: 24th May 2021

Auditors Report

Independent Auditor's Report to the Trustees of Action for A-T

Opinion

We have audited the financial statements of Action for A-T Registered Charity Number: 1145303 (the 'charity') for the year ended 31 December 2020 which comprise the Statement of Financial Activities, the Statement of Financial Position, the Statement of Cash Flows and notes to the financial statements, including a summary of significant accounting policies. The financial reporting framework that has been applied in their preparation is applicable law and United Kingdom Accounting Standards (United Kingdom Generally Accepted Accounting Practice).

In our opinion the financial statements:

- give a true and fair view of the state of the charity's affairs as at 31 December 2020 and of its incoming resources and application of resources, for the year then ended;
- have been properly prepared in accordance with United Kingdom Generally Accepted Accounting Practice; and
- have been prepared in accordance with the requirements of the Charities Act 2011.

Basis for opinion

We conducted our audit in accordance with International Standards on Auditing (UK) (ISAs (UK)) and applicable law. Our responsibilities under those standards are further described in the Auditors' responsibilities for the audit of the financial statements section of our report. We are independent of the charity in accordance with the ethical requirements that are relevant to our audit of the financial statements in the UK, including the FRC's Ethical Standard, and we have fulfilled our other ethical responsibilities in accordance with these requirements. We believe that the audit evidence we have obtained is sufficient and appropriate to provide a basis for our opinion.

Conclusions relating to going concern:

In auditing the financial statements, we have concluded that the trustees' use of the going concern basis of accounting in the preparation of the financial statements is appropriate.

Based on the work we have performed, we have not identified any material uncertainties relating to events or conditions that, individually or collectively, may cast significant doubt on the charity's ability to continue as a going concern for a period of at least twelve months from when the financial statements are authorised for issue.

Our responsibilities and the responsibilities of the trustees with respect to going concern are described in the relevant sections of this report.

Other information

The trustees are responsible for the other information. The other information comprises the information included in the Annual Report, other than the financial statements and our Report of the Independent Auditors thereon.

Our opinion on the financial statements does not cover the other information and, except to the extent otherwise explicitly stated in our report, we do not express any form of assurance conclusion thereon.

In connection with our audit of the financial statements, our responsibility is to read the other information and, in doing so, consider whether the other information is materially inconsistent with the financial statements or our knowledge obtained in the audit or otherwise appears to be materially misstated. If we identify such material inconsistencies or apparent material misstatements, we are required to determine whether this gives rise to a material misstatement in the financial statements themselves. If, based on the work we have performed, we conclude that there is a material misstatement of this other information, we are required to report that fact. We have nothing to report in this regard.

Matters on which we are required to report by exception

We have nothing to report in respect of the following matters where the Charities (Accounts and Reports) Regulations 2008 requires us to report to you if, in our opinion:

- the information given in the Report of the Trustees is inconsistent in any material respect with the financial statements; or
- sufficient accounting records have not been kept; or
- the financial statements are not in agreement with the accounting records and returns; or
- we have not received all the information and explanations we require for our audit.

Responsibilities of Trustees

As explained more fully in the Statement of Trustees' Responsibilities, the trustees are responsible for the preparation of the financial statements which give a true and fair view, and for such internal control as the trustees determine is necessary to enable the preparation of financial statements that are free from material misstatement, whether due to fraud or error.

In preparing the financial statements, the trustees are responsible for assessing the charity's ability to continue as a going concern, disclosing, as applicable, matters related to going concern and using the going concern basis of accounting unless the trustees either intend to liquidate the charity or to cease operations, or have no realistic alternative but to do so.

Our responsibilities for the audit of the financial statements

We have been appointed as auditors under Section 144 of the Charities Act 2011 and report in accordance with the Act and relevant regulations made or having effect thereunder.

Our objectives are to obtain reasonable assurance about whether the financial statements as a whole are free from material misstatement, whether due to fraud or error, and to issue a Report of the Independent Auditors that includes our opinion. Reasonable assurance is a high level of assurance, but is not a guarantee that an audit conducted in accordance with ISAs (UK) will always detect a material misstatement when it exists. Misstatements can arise from fraud or error and are considered material if, individually or in the aggregate, they could reasonably be expected to influence the economic decisions of users taken on the basis of these financial statements.

Irregularities, including fraud, are instances of non-compliance with laws and regulations. We design procedures in line with our responsibilities, outlined above, to detect material misstatements in respect of irregularities, including fraud. The extent to which our procedures are capable of detecting irregularities, including fraud is detailed below:

- Identify and assess the risks of material misstatement of the financial statements, whether due to fraud or error, design and perform audit procedures responsive to those risks, and obtain audit evidence that is sufficient and appropriate to provide a basis for our opinion. The risk of not detecting a material misstatement resulting from fraud is higher than for one resulting from error, as fraud may involve collusion, forgery, intentional omissions, misrepresentations, or the override of internal control.
- Obtain an understanding of internal control relevant to the audit in order to design audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the charity's internal control.
- Evaluate the appropriateness of accounting policies used and the reasonableness of accounting estimates and related disclosures made by the trustees.
- Conclude on the appropriateness of the trustee's use of the going concern basis of accounting and, based on the audit evidence obtained, whether a material uncertainty exists related to events or conditions that may cast significant doubt on the charity's ability to continue as a going concern. If we conclude that a material uncertainty exists, we are required to draw attention in our auditor's report to the related disclosures in the financial statements or, if such disclosures are inadequate, to modify our opinion. Our conclusions are based on the audit evidence obtained up to the date of our auditor's report. However, future events or conditions may cause the charity to cease to continue as a going concern.
- Evaluate the overall presentation, structure, and content of the financial statements, including the disclosures, and whether the financial statements represent the underlying transactions and events in a manner that achieves fair presentation.

Because of the inherent limitations of an audit, there is a risk that we will not detect all irregularities, including those leading to a material misstatement in the financial statements or non-compliance with regulation. This risk increases the more that compliance with a law or regulation is removed from the events and transactions reflected in the financial statements, as we will be less likely to become aware of instances of non-compliance. The risk is also greater regarding irregularities occurring due to fraud rather than error, as fraud involves intentional concealment, forgery, collusion, omission or misrepresentation.

A further description of our responsibilities for the audit of the financial statements is located on the Financial Reporting Council's website at www.frc.org.uk/auditorsresponsibilities. This description forms part of our Report of the Independent Auditors.

Use of our report

This report is made solely to the charity's trustees, as a body, in accordance with Part 4 of the Charities (Accounts and Reports) Regulations 2008. Our audit work has been undertaken so that we might state to the charity's trustees those matters we are required to state to them in an auditors' report and for no other purpose. To the fullest extent permitted by law, we do not accept or assume responsibility to anyone other than the charity and the charity's trustees as a body, for our audit work, for this report, or for the opinions we have formed.

(Continued)



A & N (Haslemere) Limited Statutory Auditor
Aruna House
2 Kings Road
Haslemere
Surrey
GU27 2QA

Dated: 24th May 2021

A & N (Haslemere) Limited is eligible to act as an auditor in terms of section 1212 of the Companies Act 2006.

Statement Of Financial Activities

for the year ended 31 December 2020

	Notes	Unrestricted Funds £	Restricted Funds £	31.12.20 Total Funds £	31.12.19 Total Funds £
Income and Endowments from					
Donations and legacies	3	158,623	107,279	265,902	359,826
Charitable Activities					
Fundraising & challenge events	5	139,407	-	139,407	394,885
Investment income	4	12,130	-	12,130	11,108
Other income		41,206	-	41,206	3,000
Total		351,366	107,279	458,645	768,819
Expenditure on					
Raising funds	6	97,468	-	97,468	55,576
Charitable Activities					
Fundraising & challenge events	7	45,090	-	45,090	163,705
Grants		612,260	64,817	677,077	673,985
Governance costs		19,586	-	19,586	17,578
Total		774,404	64,817	839,221	910,844
Net Income / (Expenditure)		(423,038)	42,462	(380,576)	(142,025)
Reconciliation of Funds					
Total funds brought forward		610,461	-	610,461	752,486
Total Funds Carried Forward		187,423	42,462	229,885	610,461

The notes form part of these financial statements

Statement Of Financial Position

for the year ended 31 December 2020

	Notes	Unrestricted Funds £	Restricted Funds £	31.12.20 Total Funds £	31.12.19 Total Funds £
Fixed Assets					
Tangible assets	17	324	-	324	486
Current Assets					
Stocks	18	14,251	-	14,251	10,811
Debtors	19	51,849	-	51,849	79,868
Cash at bank	20	1,455,142	42,462	1,497,604	1,539,632
		1,521,242	42,462	1,563,704	1,630,311
Creditors					
Amounts falling due within one year	21	606,965)	-	606,965)	(422,303)
Net Current Assets		914,277	42,462	956,739	1,208,008
Total Assets Less Current Liabilities		914,601	42,462	957,063	1,208,494
Creditors					
Amounts falling due after more than one year	22	(727,178)	-	(727,178)	(598,033)
Net Assets		187,423	42,462	229,885	610,461
Funds					
Unrestricted funds	23			187,423	610,461
Restricted funds				42,462	-
Total Funds				229,885	610,461

The notes form part of these financial statements

The Trustees declare that they have approved the financial statements.

Signed on behalf of the charity's Trustees.



Toby Read (Chairman)

Dated: 24th May 2021



William Rowberry (Treasurer)

Dated: 24th May 2021

Statement of Cash Flows

for the year ended 31 December 2020

	Notes	31.12.20 £	31.12.19 £
Cash flows from operating activities:			
Cash generated from operations	1	(54,158)	156,378
Net cash provided by operating activities		(54,158)	156,378
Cash flows from investing activities:			
Purchase of tangible fixed assets		-	-
Interest received		12,130	11,108
Net cash provided by investing activities		12,130	11,108
Change in cash and cash equivalents in the reporting period		(42,028)	167,486
Cash and cash equivalents at the beginning of the reporting period		1,539,632	1,372,146
Cash and cash equivalents at the end of the reporting period		1,497,604	1,539,632

Notes to the Statement of Cash Flows

for the Year Ended 31 December 2020

1. Reconciliation of net (expenditure)/income to net cash flow from operating activities

	31.12.20 £	31.12.19 £
Net (expenditure)/income for the reporting period (as per the Statement of Financial Activities)	(380,576)	(142,025)
Adjustments for:		
Depreciation charges	162	786
Interest received	(12,130)	(11,108)
(Increase)/decrease in stocks	(3,440)	(1,806)
Decrease in debtors	28,019	53,389
Increase/(decrease) in creditors	313,807	257,142
Net cash provided by operations	(54,158)	156,378

2. Analysis of changes in net fund

	31.12.20 £	Cash Flow £	31.12.20 £
Net cash			
Cash at bank	1,539,632	(42,028)	1,497,604
	1,539,632	(42,028)	1,497,604
Total	1,539,632	(42,028)	1,497,604

Notes to the Financial Statements

for the Year Ended 31 December 2020

1. Accounting Policies

Basis of preparation of financial statements

The financial statements of the charity, which is a public benefit entity under FRS102, have been prepared in accordance with the Charities SORP (FRS102) 'Accounting and Reporting by Charities: Statement of Recommended Practice applicable to charities preparing their accounts in accordance with the Financial Reporting Standard applicable in the UK and Republic of Ireland (FRS102) (effective 1 January 2019)', Financial Reporting Standard 102 'The Financial Reporting Standard applicable in the UK and Republic of Ireland' and the Charities Act 2011.

The financial statements have been prepared under the historical cost convention. The accounts are prepared in £ sterling to the nearest £1.

Action for A-T meets the definition of a public benefit entity under FRS102. Assets and liabilities are initially recognised at historic cost or transaction value unless otherwise stated in the relevant accounting policy notes.

Accounting convention

The financial statements are prepared on a going concern basis, under the historic cost convention.

The Charity is entirely dependent on receiving income from fundraising and donations and as a consequence the going concern basis is also dependent on the continuation of such income.

Incoming resources

All incoming resources are included in the Statement of Financial Activities under FRS102 when that receipt is probable, whereas it was previously recognised when the Charity is legally entitled to the income and the amount can be quantified with reasonable accuracy. For legacies, entitlement is the earlier of the Charity being notified of an impending distribution or the legacy being received.

Gifts in kind, including donated professional services are recognised as income when the Charity has control over them, any conditions associated with the donated item have been met, the receipt of economic benefit from the use of by the Charity if the item is probable and that economic benefit can be measured reliably. In accordance with the Charities SORP (FRS102), the general volunteered time is not recognised and refer to the Trustees' Report for more information about this contribution.

On receipt, donated professional services are recognised on the basis of the value of the gift to the Charity which is the amount the Charity would have been willing to pay to obtain that service on the open market.

Gifts in kind donated for distribution are included at fair value upon receipt under FRS102 subject to the cost of recognition outweighing the benefit provided to Action for A-T. Previously they were included at a valuation and recognised as income when they are distributed. Any donated facilities are included at the value to the Charity where this can be quantified, and a third party is bearing the cost. No amounts are included in the financial statements for services donated by volunteers.

Resources expended

All expenditure is accounted for on an accruals basis and has been included under expense categories that aggregate all costs allocated to activities. Where costs cannot be directly attributed to particular activities, they have been allocated on a basis consistent with the use of the resources.

Overheads have been allocated on the basis of the activity income of the Charity.

Fundraising costs are those incurred in seeking voluntary contributions and do not include the costs of disseminating information in support of charitable activities. Support costs are those costs incurred directly in support of expenditure on the objects of the Charity and include project management carried out by the Trustees. Governance costs are those incurred in connection with the administration of the Charity and compliance with constitutional and statutory requirements.

Grants offered subject to conditions which have not been met at the year-end date are noted as a commitment but not accrued as expenditure.

Notes to the Financial Statements

for the Year Ended 31 December 2020

Governance costs

Governance costs comprise all costs involving the public accountability of the Charity and its compliance with regulations and good practice. These costs include costs related to the audit, legal fees, and apportionment of overheads.

Allocation and apportionment of costs

Fundraising Events & Challenge Events costs are allocated in their income ratio during the year.

Tangible fixed assets

Depreciation is provided at the following annual rates in order to write off each asset over its estimated useful life.

Computer equipment - 33% on cost.

Stocks

Stocks are valued at the lower of cost and net realisable value, after making due allowance for obsolete and slow moving items.

Taxation

The charity is exempt from tax on its charitable activities.

Fund accounting

General funds are unrestricted funds which are available for use at the discretion of the Trustees in furtherance of the general objectives of the Charity and which have not been designated for other purposes.

Restricted funds are funds which are to be used in accordance with specific restrictions imposed by donors which have been raised by the Charity for particular purposes. The cost of raising and administering such funds are charged against the specific fund. The aim and use of each restricted fund is set out in the notes to the financial statements. Any investment income, gains or losses are allocated to the appropriate fund.

Designated funds are funds set aside by the Trustees out of general reserves for a particular purpose. The aim and use of each designated fund is set out in the notes to the financial statements. The Trustees will review the funds on an ongoing basis. At the conclusion of the purpose for the fund any excess remaining funds will be transferred back into general funds. If a shortfall arises the Trustees will consider whether any additional general funds should be transferred to designated funds. Any investment income, gains or losses are allocated to the appropriate fund.

Foreign currencies

Assets and liabilities in foreign currencies are translated into sterling at the rate of exchange ruling at the balance sheet date. Transactions in foreign currencies are translated into sterling at the rate of exchange ruling at the date of the transaction. Exchange differences if applicable are taken into the Statement of Financial Activities.

Going Concern

The financial statements have been prepared on a going concern basis as the trustees believe that no material uncertainties exist. The trustees have considered the level of funds held and the expected level of income and expenditure for 12 months from authorising these financial statements. The budgeted income and expenditure is sufficient with the level of reserves for the charity to be able to continue as a going concern.

Notes to the Financial Statements

for the Year Ended 31 December 2020

2. Critical Accounting Judgements and Key Sources of Estimation Uncertainty

Critical accounting estimates and judgements

In the application of the charity's accounting policies, the Trustees are required to make judgements, estimates and assumptions about the carrying amount of assets and liabilities that are not readily apparent from other sources. The estimates and associated assumptions are based on historical experience and other factors that are considered to be relevant. Actual results may differ from these estimates.

The estimates and underlying assumptions are reviewed on an ongoing basis. Revisions to accounting estimates are recognised in the period in which the estimate is revised, if the revision affects only that period, or in the period of the revision and future periods if the revision affects both current and future periods.

Key sources of estimation uncertainty

The annual depreciation charge for tangible fixed assets is sensitive to changes in the estimated useful economic lives and residual value of fixed assets. The useful economic lives and residual values are reassessed on an annual basis. They are amended where deemed necessary to reflect current estimations based on physical condition, utilisation, and advancement of technologies. Note 17 discloses the carrying value if the tangible fixed assets and the depreciation policy is listed above in accounting policies.

3. Donations and Legacies

	Unrestricted Funds £	Restricted Funds £	31.12.20 £	31.12.19 £
Donations	68,506	107,279	175,785	232,188
Gift Aid	8,498	-	8,498	63,530
Fundraising	81,619	-	81,619	64,108
	158,623	107,279	265,902	359,826

4. Investment Income

	Unrestricted Funds £	Restricted Funds £	31.12.20 £	31.12.19 £
Bank interest	12,130	-	12,130	11,108

5. Income from Charitable Activities

	31.12.20 £	31.12.19 £
Fundraising events	43,820	178,591
Challenge events	95,587	216,294
	139,407	394,885

6. Raising Funds

	Unrestricted Funds £	Restricted Funds £	31.12.20 £	31.12.19 £
Raising donations and legacies				
Staff costs	60,010	-	60,010	46,821
Support costs	37,458	-	37,458	8,755
	97,468	-	97,468	55,576

7. Charitable Activities Costs

	Direct Costs £	Grant funding of activities (see note 8) £	Support Costs (see note 9) £	Totals £
Fundraising & challenge events	40,927	-	4,163	45,090
Grants	-	622,245	54,832	677,077
Governance costs	-	-	19,586	19,586
	40,927	622,245	78,581	741,753

8. Grants Payable

	31.12.20 £	31.12.19 £
Grants	622,245	598,034

The total grants paid to institutions during the year was as follows:

	31.12.20 £	31.12.19 £
Using new immunotherapy methods to treat lymphoma and leukaemia in A-T	143,400	-
Recurrent pulmonary infections and their impact on neurological decline in A-T	246,691	-
Deciphering the molecular mechanism linking Ataxia-Telangiectasia and Ataxia-Telangiectasia-Like Disorder	105,183	-
*The David Peake Study to test the feasibility of whole-body MRI for cancer surveillance in children and young people with Ataxia Telangiectasia	100,557	-
A new function for ATM and a new theory for the clinical abnormalities in A-T	53,619	-
Natural History of A-T (N-HAT)	-	177,866
De Villartay - Does irregular repair of DNA damage in Neurons and Lymphocytes cause cellular degeneration in Ataxia Telangiectasia patients?	-	65,000
Innovative methods for gene therapy in Ataxia-Telangiectasia	-	100,000
The use of patient stem cell-derived mini-brains to study Ataxia Telangiectasia	-	98,000
The ATeam: Producing healthcare guidance for children with Ataxia Telangiectasia including the exploration and design of a home-based exercise package	-	89,692
Development of CRISPR/Cas Genome Editing as a Treatment for Ataxia Telangiectasia	-	40,000
	649,449	570,828

9. Support Costs

	Management	Finance	Information Technology	Other	Wages	Governance Costs	Totals
	£	£	£	£	£	£	£
Raising donations and legacies	13,430	950	3,599	19,479	-	-	37,458
Fundraising & challenge events	1,492	106	400	2,165	-	-	4,163
Grants	-	-	-	-	54,832	-	54,832
Governance costs	-	-	-	-	-	19,586	19,586
	14,922	1,056	3,999	21,644	54,832	19,586	116,039

Activity	Basis of allocation
Management	90 % Raising Donation & legacies 10 % Fundraising & Challenge Events
Finance	90 % Raising Donation & legacies 10 % Fundraising & Challenge Events
Information technology	90 % Raising Donation & legacies 10 % Fundraising & Challenge Events
Human resources	90 % Raising Donation & legacies 10 % Fundraising & Challenge Events
Other	90 % Raising Donation & legacies 10 % Fundraising & Challenge Events
Governance costs	100% Governance cost

10. Trustees' Remuneration And Benefits

There were no Trustees' remuneration or other benefits for the year ended 31 December 2020 nor for the year ended 31 December 2019.

Trustees' Expenses

There were no Trustees' expenses paid for the year ended 31 December 2020 nor for the year ended 31 December 2019.

11. Staff Costs

Only one employee received emoluments in excess of £60,000.

The Charity considers its key management personnel to be the Trustees, Chief Executive and senior management. The total employment benefits of the key management personnel were £121,049 (2019 - £121,049). Only one employee had employment benefits falling between £65,000 and £70,000 (2019 - 1).

12. Comparatives for the Statement of Financial Activities

	Unrestricted Funds £	Restricted Funds £	Total Funds £
Income and endowments from			
Donations and legacies	190,654	169,172	359,826
Charitable activities			
Fundraising & challenge events	394,885	-	394,885
Investment income	11,108	-	11,108
Other income	3,000	-	3,000
Total	599,647	169,172	768,819
	Unrestricted Funds £	Restricted Funds £	Total Funds £
Expenditure on			
Raising funds	55,576	-	55,576
Charitable activities			
Fundraising & challenge events	163,705	-	163,705
Grants	504,813	169,172	673,985
Governance costs	17,578	-	17,578
Total	741,672	169,172	910,844
Net income/(Expenditure)	(142,025)	-	(142,025)
Reconciliation of funds			
Total funds brought forward	752,486	-	752,486
TOTAL FUNDS CARRIED FORWARD	610,461	-	610,461

13. Employee Benefits

The cost of any unused holiday entitlement is recognised in the period in which the employee's services are received.

Termination benefits would be recognisable immediately as an expense when the Charity is demonstrably committed to terminate an employee's employment or to provide termination benefits.

14. Pension Costs

The pension costs charged in the financial statements represent the contributions payable by the Charity during the year to a multi-employer defined benefit pension scheme.

Notes to the Financial Statements - continued

for the Year Ended 31 December 2020

15. Financial Instruments

The charity only has financial assets and financial liabilities of a kind that qualify as basic financial instruments. Basic financial instruments are initially recognised at transaction value and are subsequently measured at their amortised cost.

16. Creditors and Provisions

Creditors and provisions are recognised where the charity has a present obligation resulting from a past event that will probably result in the transfer of funds to a third party and the amount due to settle the obligation can be measured or estimated reliably. Creditors and provisions are normally recognised at their settlement amount after allowing for any trade discounts due.

17. Tangible Fixed Assets

	Computer Equipment £
Cost	
At 1 January 2020 and 31 December 2020	<u>2,358</u>
Depreciation	
At 1 January 2020	1,872
Charge for year	<u>162</u>
At 31 December 2020	<u>2,034</u>
Net Book Value	
At 31 December 2020	<u>324</u>
At 31 December 2018	<u>486</u>

18. Stocks

	31.12.20 £	31.12.19 £
Stocks	<u>14,251</u>	<u>10,811</u>

Stocks are stated at the lower of cost and estimated selling price less costs to complete and sell. Cost includes all costs of purchase, costs of conversion and other costs incurred in bringing stock to its present location and condition. Due allowance is made for obsolete and slow-moving items.

Donated stocks are fair valued when their economic benefit is probable, it can be measured reliably, and the charity has control over the item.

Notes to the Financial Statements - continued

for the Year Ended 31 December 2020

19. Debtors: Amounts Falling Due Within One Year

	31.12.20 £	31.12.19 £
Deposit	925	925
Prepayments and accrued income	50,924	78,943
	51,849	79,868

Trade and other debtors are recognised at the settlement amount due after any trade discount offered. Prepayments are valued at the amount prepaid net of any trade discounts due

20. Cash at Bank

	Unrestricted Funds £	Restricted Funds £	31.12.20 Total Funds £	31.12.19 Total Funds £
Bank account no. 1	8,548	42,462	51,010	48,813
Bank account no. 2	15,484	-	15,484	16,017
Bank account no. 3	865,898	-	865,898	740,208
Bank account no. 4	565,212	-	565,212	734,594
Total	1,455,142	42,462	1,497,604	1,539,632

Cash and cash equivalents include cash in hand, deposits held at call with banks, other short-term liquid investments with original maturities of twelve months or less, and bank overdrafts. Should any bank overdrafts arise they would be shown within borrowings in current liabilities.

21. Creditors: Amounts Falling Due Within One Year

	31.12.20 £	31.12.19 £
Grant Payable	600,198	406,569
Accruals and deferred income	6,767	15,734
	606,965	422,303

22. Creditors: Amounts Falling Due After More Than One Year

	31.12.20 £	31.12.19 £
Other creditors	727,178	598,033

23. Movement in Funds

	At 1.1.20 £	Net Movement in Funds	At 31.12.20 £
Unrestricted Funds			
Unrestricted Funds	610,461	(423,038)	187,423
Restricted Funds			
Restricted Funds	-	42,462	42,462
Total Funds	610,461	(380,576)	229,885

Net movement in funds, included in the above are as follows

	Incoming Resources £	Resources Expended £	Movement in Funds £
Unrestricted Funds			
Unrestricted Funds	351,366	(774,404)	(423,038)
Restricted Funds			
Restricted Funds	107,279	(64,817)	42,462
Total Funds	458,645	(839,221)	(380,576)

Comparatives for movement in funds

	At 1.1.19 £	Net Movement in Funds	At 31.12.19 £
Unrestricted Funds			
Unrestricted Funds	752,486	(142,025)	610,461
Total Funds	752,486	(142,025)	610,461

Notes to the Financial Statements - continued

for the Year Ended 31 December 2020

23. Movement in Funds - Continued

Comparative net movement in funds, included in the above are as follows:

	Incoming Resources £	Resources Expended £	Movement in Funds £
Unrestricted Funds			
Unrestricted Funds	599,647	(741,672)	(142,025)
Restricted Funds			
Restricted Funds	169,172	(169,172)	-
Total Funds	768,819	(910,844)	(142,025)

A current year 12 months and prior year 12 months combined position is as follows:

	At 1.1.19 £	Net Movement in Funds	At 31.12.20 £
Unrestricted Funds			
Unrestricted Funds	752,486	(565,063)	187,423
Restricted Funds			
Restricted Funds	-	42,462	42,462
Total Funds	752,486	(522,601)	229,885

A current year 12 months and prior year 12 months combined net movement in funds, included in the above are as follows:

	Incoming Resources £	Resources Expended £	Movement in Funds £
Unrestricted Funds			
Unrestricted Funds	951,013	(1,516,076)	(565,063)
Restricted Funds			
Restricted Funds	276,451	(233,989)	42,462
Total Funds	1,227,464	(1,750,065)	(522,601)

24. Related Party Disclosures

The Trustees all give freely their time and expertise without any form of remuneration or other benefits in cash or kind (2019 - £nil). There are no expenses paid to the Trustees in the year. No trustee is deemed to have benefited as a result of a related party connection. All trustees have declared all such relationships to the Chairman.

25. Post Balance Sheet Events

Covid-19 Pandemic

At the time of writing these accounts, the Covid-19 pandemic has significantly impacted the way in which every organisation operates around the world. As a result, we have been forced to cease most fundraising activities and all our events due to the ever-changing situation relating to the outbreak. It is unclear how much of an impact this will have on our overall income for the year, but we are unlikely to meet the fundraising budget which was signed off by the trustees in January. We have reacted quickly to the crisis and further diversified our fundraising activities to ensure that there are no going concern issues. If the crisis continues until the end of the year (as predicted) we are unlikely to fund any new research projects until the end of 2021.

26. Ultimate Controlling Party

The Trustees control Action for A-T in accordance with the trust deed.

Detailed Statement of Financial Activities

for the Year Ended 31 December 2020

	Unrestricted Funds £	Restricted Funds £	31.12.20 £	31.12.19 £
Income and Endowments				
Donations	68,506	107,279	175,785	232,188
Gift aid	8,498	-	8,498	63,530
Fundraising	81,619	-	81,619	64,108
	158,623	107,279	265,902	359,826
Investment income				
Bank interest	12,130	-	12,130	11,108
Charitable activities				
Fundraising events	43,820	-	43,820	178,591
Challenge events	95,587	-	95,587	216,294
	139,407	-	139,407	394,885
Other income				
Other income	3,000	-	3,000	3,000
HMRC JRS Grant	38,206	-	38,206	-
	41,206	-	41,206	3,000
Total incoming resources	351,366	107,279	458,645	768,819
Expenditure				
Raising donations and legacies				
Wages	60,010	-	60,010	46,821
Charitable activities				
Event campaign costs	40,927	-	40,927	128,307
Event - fundraising & challenge	-	-	-	5,258
Grants to institutions	557,428	64,817	622,245	598,034
	598,355	64,817	663,172	731,599
Support costs				
Management				
Wages	14,922	-	14,922	15,393
Finance				
Bank charges	1,056	-	1,056	2,237
Information technology				
Repairs and renewals	3,999	-	3,999	2,060

Detailed Statement of Financial Activities

for the Year Ended 31 December 2020

	Unrestricted Funds £	Restricted Funds £	31.12.20 £	31.12.19 £
Information technology				
Other				
Rent, rates and water	13,089	-	13,089	13,523
Insurance	1,036	-	1,036	675
Telephone	758	-	758	883
Postage and stationery	809	-	809	905
Sundries	1,976	-	1,976	534
Advertising	2,042	-	2,042	1,366
Subscription	932	-	932	438
Donation collection fees	1,002	-	1,002	881
	21,644	-	21,644	19,205
Fundraising				
Wages	54,832	-	54,832	75,951
Governance costs				
Wages	13,802	-	13,802	11,355
Auditors' remuneration	3,300	-	3,300	3,300
Accountancy and legal fees	2,322	-	2,322	2,137
Computer equipment	162	-	162	786
	19,586	-	19,586	17,578
Total resources expended	774,404	64,817	839,221	910,844
Net (expenditure)/income	(423,038)	42,462	(380,576)	(142,025)

Acknowledgments

Action for A-T relies entirely on its donors and supporters who so generously give their time and money to support vitally needed research into A-T. With their support we believe we can make a difference to all people with A-T and their families.

We would like to give special thanks to the following organisations that worked alongside us in 2020:

Our Research Partners

BrAshA-T Ataxia Telangiectasia and their trustees

A-T Children's Project (USA) and their Medical Research Team

Ataxia Telangiectasia Society

Sparks Children's Medical Research Charity

Support from Companies, Trusts & Foundations

We are aware that Companies, Trusts & Foundations are often approached to support charities and are therefore extremely grateful to the following organisations for their generous support.

Aviva
 An Anonymous Trust
 Barclays
 Barton Comms
 BCA
 Bergqvist Charitable Trust
 Big Give
 Blyth Watson Charitable Trust
 Broyst Foundation
 Cast Media Group
 Chapman Charitable Trust
 Cinch
 Data IQ
 Ed Shanley
 Epsom & Ewell Borough Council
 Epsom Rotary Club
 Mr & Mrs Gary Shaughnessy
 Global Entertainment
 Hiscox Foundation
 ITV
 Jones 1986 Charitable Trust
 Kent Community Foundation
 Lumen Financial Ltd
 Michael and Anna Wix Charitable Trust

Midwood McTurk Trust
 Naga Munchetty
 National Lottery Community Fund
 Paul & Clare Rooney
 Psigma Foundation
 Quest Professional
 Rawson Digital
 Real Golf
 Reed Foundation
 Rest-Harrow Trust
 Robin Hewison
 Sir Samuel Scott of Yews Trust
 St. James' Place Foundation
 Steel Charitable Trust
 Syder Foundation
 Tesco
 Work for Good

Corporate or professional services provided by:

A & N Chartered Accountants
 Akin Gump
 Chris Stanton
 Gecko Graphic Design

Our Supporters

We have relied almost exclusively on our supporters, volunteers and Trustees for their time and commitment to raising money and spreading awareness about this devastating condition. Thank you to everyone who has helped us to push research into A-T forwards.



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