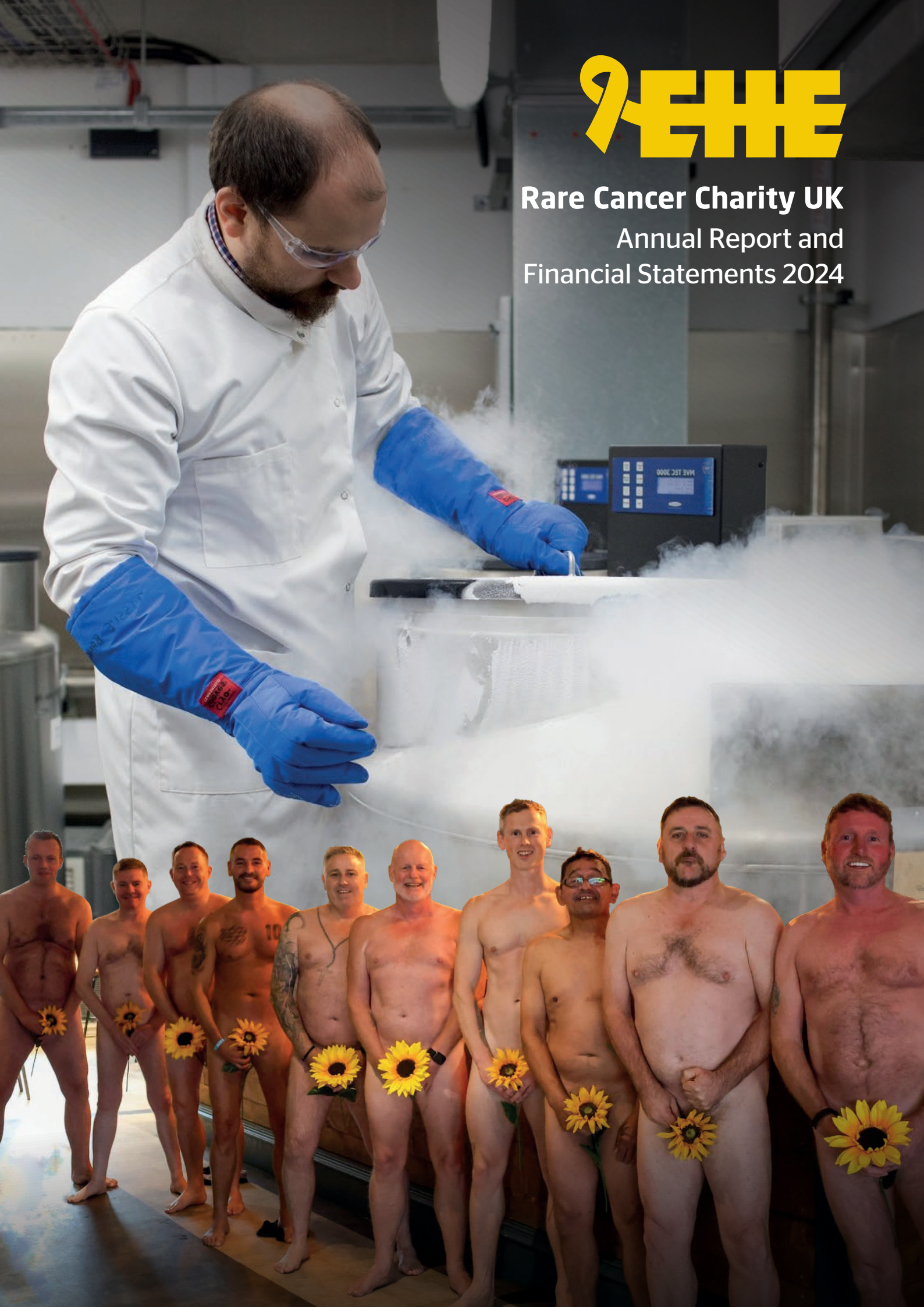




Rare Cancer Charity UK
Annual Report and
Financial Statements 2024



Epithelioid Haemangioendothelioma (EHE)

EHE Facts

May turn **aggressive** at **any time** without warning

Clinical signal that onset may be tied to **pregnancy** and **puberty** in young women

A **destructive vascular sarcoma** that is found in the walls of blood vessels

Commonly appears in **liver and lungs** but can appear anywhere

Living with EHE causes **enormous psychological stress**

Affects both males and females but is **more prevalent in women**

Can present as **indolent** (passive) or **aggressive**

Typically more aggressive in **young people**

One of the **world's rarest cancers**, with approximately 20 patients per year in the UK

Can re-present after long period with no disease

Often presents with **multiple tumours** called 'multifocal'

No recognised treatment, so treatment is by trial and error

No known cure. Aggressive disease is **normally fatal**

Find out more at: www.ehercc.org.uk

Message from the Trustees

In 2024, we once again enjoyed great support from our patient community, saw excellent progress in the research we are supporting, and made advances in important areas of advocacy for better EHE treatments. These outcomes resulted in 2024 being another excellent year for the EHE Rare Cancer Charity (EHERCC or 'Charity').

The EHE research activities we are funding continued to produce exciting results. The European collaborative research involving Istituto Nazionale dei Tumori (INT) in Milan, Italy and the Institute of Cancer Research (ICR) and the Royal Marsden Hospital (RMH) in London continues to study different aspects of the disease, and the researchers believe they have identified a biomarker for EHE, one of the key objectives. The UK National EHE Biobank, funded by the Charity, continues to grow, capturing critical EHE biosamples for use in research. The international EHE research programme we support has also continued to grow and produce important results.

Our patient support and advocacy activities have continued to grow. Patient support and information sharing has been provided on a patient-to-patient basis through the group's global EHE Patient Support Facebook page. At the same time, every opportunity has been taken to engage with the general public, regulators, hospitals and other key stake holders to ensure that awareness of our EHE global community and the Charity continues to grow.

We are also excited by the Charity's engagement with the European Medicines Agency, seeking a label extension of the drug sirolimus for the treatment of EHE, an important goal for the EHE patient community. This has been led by the European Organisation for Research and Treatment of Cancer (EORTC). The Charity is also a participant and Executive Committee member of the PUSH project (Pushing Ultra-rare Sarcomas beyond Hope), a new global platform designed to promote, support and run ultra-rare sarcoma studies, trials and other forms of disease investigation.

2024 was also another excellent year on the fundraising front, with grass-roots fundraising by our patients and their supporters. As we prepare this annual report, we want to again acknowledge that all the activities we are proud to report on have only been possible because of the extraordinary commitment of our patient community and their supporters. This is even more extraordinary when we recognise that EHE patient numbers will always be tiny due to the extreme rarity of the disease. That makes their achievements all the more amazing.

We will also never forget the amazing contribution made by the sarcoma clinical and research communities that we work so closely with. They strive every day to discover and deliver better treatment options for EHE patients. We could not be more grateful. As always, therefore, we want to recognise every single person who provided their support, in whatever form, to the Charity and the global EHE community in 2024 by saying a simple but profound "Thank you"!

Thank you!

Left to right:
Hugh Leonard (Chair of Trustees),
Jeff Collins (Trustee),
Kate Hooper (Trustee),
Sally Baker (Trustee),
Dr Oliver Pearce (Trustee).



01 Patient Support and Advocacy

In 2024 the Charity, working side by side with the other internationally-based EHE foundations and our EHE patient community, continued to engage with all stake holders with the core objective of increasing understanding and awareness of EHE.

In early January, Hugh Leonard, Chair of Trustees, participated in a very positive workshop hosted by the European Medicines Agency (EMA) and the European Organisation for Research and Treatment of Cancer (EORTC), to discuss the challenges faced by ultra-rare sarcomas in securing approvals for new or repurposed drugs. Hugh presented the experience of EHE patients as a case study for ultra-rare sarcomas in general. Senior clinicians from Europe, the UK and USA also participated, addressing a number of key areas.

In parallel with the EMA process above, the Charity is also a participant in the PUSH project, a new global platform with the aim of maximising the knowledge that is gained from interacting with, and treating, every single patient affected by an ultra-rare sarcoma and to support the development of new treatments to improve outcomes and quality of life. Ultimately it is hoped that a substantial number of institutes and academic centres across the globe will join the project, forming the PUSH Consortium, giving the PUSH Platform real momentum and an ability to change the landscape for ultra-rare sarcomas.

Collaboration is also a key feature of the Charity's advocacy initiatives. Global collaboration between the different national EHE foundations has continued to provide a common approach across international EHE programmes and initiatives. The Charity is working closely with 'EHE Italia Non Solo Laura' (Italy), particularly on European-focused matters, and with the EHE foundations in the USA, Australia and Canada, seeking to promote and expand the international EHE research and clinical collaborations for EHE.

Ensuring engagement with the UK EHE patient community is also a core objective. The UK EHE patient WhatsApp group continues to be an active and hugely positive component of our activities, although we continue to regularly communicate with the patient community through multiple social media and other communication routes.

02 Research

A key focus for our funded research was again the ongoing European collaboration involving Istituto Nazionale dei Tumori (INT), Milan, the Institute of Cancer Research (UK), and the Royal Marsden Hospital (RMH), London. This multi-faceted programme is researching a number of key aspects of EHE, one of the key objectives being to try and find a biomarker for EHE. We were therefore thrilled when the research team identified Growth and Differentiation Factor-15 (GDF15) as a strong likely biomarker for the disease.

A core component of this international collaboration is the ongoing non-interventional observational study of EHE patients. The study cohort was approaching 50 patients by the end of 2024, providing a unique data set for EHE. At the same time, the Charity continues to fund the first ever EHE-dedicated, prospective observational registry, a pan-European initiative, which is part of the European STARTER project, and is managed and coordinated by a dedicated team also based at INT in Milan.

The National EHE Biobank, funded by the Charity and based at The Royal Marsden Hospital in London, continues to gather critical bio-samples. The Charity and the Royal Marsden are continuing to promote awareness of the biobank with hospitals across the UK, supported by enthusiastic support from the UK patient community.

The above is all very exciting, but we do have to accept that not all research will be successful. One such case was the continued work we were doing with the Bateson Centre in Sheffield, where the team were seeking to generate a zebrafish model of EHE. Ultimately the team were unable to generate research-useful fish, and this, coupled with the ongoing successful development of PDX, mouse and human EHE cells lines globally, meant that the difficult decision to halt this research project had to be made.

03 Fundraising

Fundraising is the life blood of the Charity, providing the very foundations for all that we do, and in particular our ability to fund key research. Once again, our fundraising through the year was a combination of grass-roots activity driven by our patient community and their supporters, and charity-driven events.

The largest of our two charity-driven events is the London Landmarks Half Marathon, and 2024 saw great turnout from our supporters, with more than 50 runners registered for the run. It was another fantastic day with the runners enjoying near perfect conditions.

The RideLondon-Essex 100-mile-long cycling sportive was also well supported, with 14 riders taking on this major challenge. Hugh Leonard, Chair of Trustees, again felt that he needed to participate, having spent several years encouraging others to get involved, his third time riding in the event.

Fundraising can be both inspiring and deeply emotional as it is often organised in memory or support of a loved one. The Charity received great support from so many different groups and individuals. Every single one of these acts, which come in so many different forms, is priceless. We are so grateful for every single one, and so seldom single out any particular event. We did, however, love the group of men who decided to bare nearly all to create an EHE calendar to raise funds for EHE research. We loved the use of the sarcoma symbol of sun flowers to maintain a minimum level of modesty, and hope you don't mind us using it on our front cover.

More information about these and other events can be found in this report.

Objectives and Achievements for 2024

We believe that an honest assessment of our year’s activities and achievements against the goals we set ourselves is important for all our supporters and stakeholders. Below is the 2024 assessment; on the opposite page you will see our objectives for 2025 and beyond.

| | What we said we would do... | ...and what we achieved | |
|--|--|---|--|
| 01 Patient Support and Advocacy | Continue sirolimus repurposing | Dialogue ongoing with EMA with two Scientific Advice procedures completed |   |
| | Support PUSH platform | The Charity is a consortium member with Hugh Leonard a member of the Executive Committee | |
| | Revitalise other social media | Social media relaunched. Delivery of news and messaging ongoing | |
| | Engage with European patient community | Limited progress made. Exploring UK-Italy collaboration | |
| 02 EHE Research | Support existing projects | All projects supported |   |
| | Develop and promote coherent EHE research strategy | Limited progress made with this complex matter. Currently reviewing best way to develop the concept | |
| | Organise additional research review meetings | Further online meetings held | |
| | Coordinate with EHE Group entities | Close and proactive ties maintained with US, Canadian, Italian and Australian organisations | |
| 03 Fundraising | Support and encourage grassroots fundraising | Continued to work with our brilliant supporters |   |
| | Coordinate key charity events (LLHM and RideLondon100) | Both organised and delivered with great engagement | |
| | Continue to review fundraising strategy with NOVA fundraising | Limited progress as we worked on the broader research strategy | |
| | Coordinate review of research strategy and develop draft fundraising programme | Limited progress as requires completion of actual research strategy first | |
| Charity Organisation | Consider appropriate support for research strategy | Dr Paul Huang joins Research and Medical Advisory Board |   |
| | Implement fundraising strategy | Grass-roots ongoing, but new broad strategy delayed | |
| | Establish structures for European engagement | In progress and under review with EHE Italia | |
| | Evaluate need for additional structures and personnel for the above | In progress | |

Our Forward Focus for 2025

Because rare cancers are not rare to those who have them!

| | |
|--|---|
| 01 Patient Support and Advocacy <ul style="list-style-type: none">• Continue to promote sirolimus repurposing• Engage with other repurposing groups and platforms• Remain engaged with PUSH• Support UK patient community, and international patients where possible• Attempt to define European strategy | 02 EHE Research <ul style="list-style-type: none">• Support existing research• Execute research extensions where possible• Continue to search for new research opportunities• Identify joint funding opportunities within the EHE Group |
|--|---|

2025 AND BEYOND

| | |
|---|--|
| 03 Fundraising <ul style="list-style-type: none">• Support and encourage grassroots fundraising• Promote 10 for 10 fundraising campaign across 2025• Re-evaluate timing of fundraising strategy with NOVA fundraising• Evaluate ways to systemise fundraising engagement ideas through website modifications | Charity Organisation <ul style="list-style-type: none">• Consider appropriate support for research strategy• Implement fundraising strategy• Establish structures for European engagement• Evaluate need for additional structures and personnel for the above |
|---|--|

Foreword from Associate Professor John M. Lamar

I am honored to write this foreword for the UK EHE Rare Cancer Charity (EHERCC) Annual Report—particularly because it was the EHERCC and its sister foundations in the United States and Australia that provided the initial funding that enabled my laboratory to begin our work on epithelioid hemangioendothelioma (EHE).

I am a cancer biologist who has spent much of my career studying YAP and TAZ—two proteins that, although vital for normal cellular function, can drive cancer development and progression when not properly controlled. Like many of my colleagues, I was initially unaware of EHE, despite the fact that virtually all cases of this rare cancer are caused by mutant forms of YAP or TAZ. That changed when Dr. Guy Weinberg reached out to ask if I would help organize a scientific workshop. His goal was to bring together researchers studying YAP and TAZ with those focused on EHE, believing that this connection could accelerate progress for EHE patients.

At our inaugural YAP/TAZ-TEAD at the Crossroads of Cancer workshop, I met Drs. Brian Rubin and Munir Tanas and learned of their pioneering work on EHE. At the time, the field was still in its infancy. There were no reliable research models, and many fundamental questions about the disease remained unanswered. Yet I was immediately captivated by EHE and by the fusion protein TAZ-CAMTA1, which is present in more than 90% of all cases. In 2018, with seed funding from the EHERCC and the EHE foundations in the United States and Australia, my laboratory began our own EHE research in collaboration with Dr. Rubin’s group.

The seven years since have brought extraordinary progress. Through the collective efforts of EHE researchers and clinicians worldwide, we now understand much more about how the TAZ-CAMTA1 and YAP-TFE3 fusion proteins drive EHE formation and progression. Robust mouse models, human and mouse

EHE cell lines, and patient-derived xenograft models have been developed, providing essential tools to study this disease and identify therapeutic opportunities. EHE patients have been enrolled in clinical trials, and promising new drug targets are emerging from preclinical studies. The first biomarker has been identified, and the establishment of EHE biobanks and a global EHE patient registry is providing vital resources for researchers. A consensus paper authored by more than 80 experts now guides evidence-based best practices for managing primary and metastatic EHE.

Equally transformative has been the growth of the EHE research community. Awareness of EHE among cancer scientists has expanded dramatically, as has the number of laboratories working on the disease. Today, EHE is discussed regularly at international conferences, and pharmaceutical companies are increasingly interested in testing compounds that target YAP and TAZ in EHE models and including EHE patients in clinical trials.

Much of this progress has been made possible through the vision and dedication of the EHERCC and its sister foundations in the United States and Australia, and more recently Italy, together with dynamic patient groups in countries such as Canada and Germany. These organizations have been instrumental not only in funding critical research and supporting patients, but also in fostering collaboration between scientists and clinicians, a partnership that is absolutely essential to translating new discoveries into life-saving therapies.

Despite these remarkable advances, our work is far from done. There are still no approved treatments for aggressive EHE, and many questions remain about its biology and vulnerabilities. Continued progress will depend on expanding our research tools, developing new models, and sustaining the support of donors committed to this mission.

I am inspired by how far the EHE community has come in such a short time. With the continued leadership of the EHE foundations, I am confident that the coming years will bring even more significant breakthroughs and, ultimately, new hope for patients living with EHE.



The Lamar Lab team (from left to right): Jessica Scarchilli, Emily Norton, John Lamar, Seth Lecuyer and Sarah McMullan

John M. Lamar Ph.D. is an Associate Professor at the Molecular and Cellular Physiology Department at the Albany Medical College in Albany, New York. He obtained his Bachelor of Science in Biochemistry at SUNY Geneseo, Geneseo, NY in 2000; his Master’s Degree in Biology at Albany Medical College in 2006; and his Ph.D. in Biology at Albany Medical College in 2008. After completing spells first as a Postdoctoral Researcher and then a Research Scientist at Koch Institute at MIT, he returned to Albany Medical College where he has established his own laboratory focused on the study of cancer metastasis, which is responsible for greater than 90% of all cancer mortality. His work is heavily focused on the Hippo Pathway and its two effectors, Yes-associated protein (YAP) and Transcriptional coactivator with PDZ-binding motif (TAZ), and in particular, their involvement in EHE.

01 Patient Support and Advocacy

Our patient support and advocacy activities lie at the core of the Charity's activities and comprise two simple objectives. The first, inward focused, supports the whole patient community, while the second, outward looking, is about spreading awareness of EHE and the impact that an EHE diagnosis has on patients.

Support for EHE patients is largely provided through EHE social media platforms, both local and global, by the patient community itself. These are the people with real EHE understanding, allowing them to share experiences and provide valuable information on a range of topics.

Our outward-looking advocacy objectives are to take every opportunity to bring these facts to people's attention. We therefore take every opportunity to engage in dialogue as we continue to believe that there is no better way to spread awareness than by speaking to actual audiences.

We also share news about the Charity and its activities, in the EHE Group quarterly global newsletter, *The Pledge*, edited and produced here in the UK. We will also share this Charity Annual Report with as many people as we can as part of our awareness and patient support programme. We hope that people find the content of these different publications inspiring and share them as widely as possible.

Review by Sally Baker

"2024 has been another good year with regard to our patient support and advocacy activities. We were delighted with the continued engagement with the European Medicines Agency as we sought approval for the drug sirolimus for the treatment of EHE. We also continued through the year as a member of the Push Consortium, with Hugh Leonard our Chair of Trustees being a member of the PUSH Executive Committee. We were also delighted in 2024 when Dr Paul Huang from the Institute of Cancer Research agreed to join the Charity's Research and Medical Advisory Board. More information is provided below concerning our Patient Support and Advocacy activities through the year".

EMA engagement and sirolimus approval

In early January, Hugh Leonard was delighted to participate in a workshop hosted by the European Medicines Agency (EMA) and the European Organisation for Research and Treatment of Cancer (EORTC), to discuss the challenges faced by patients with ultra-rare sarcomas and those treating them. Hugh Leonard presented the perspectives of patients with these ultra-rare diseases, using EHE as a case study.



The core objective of the workshop was to consider how the regulatory pathway for approval of new treatments for ultra-rare sarcomas can be improved. Senior clinicians from Europe, the UK and USA also participated, addressing a number of key areas. In addition, more than 300 people participated online, including clinicians, health care professionals, researchers, patient advocates and members of the public.

A second such workshop was held in late May, also at the EMA offices in the Hague. Hugh Leonard again had the chance to participate, presenting the EHE Group's experience with mobilising and accessing patients and their information. Denise Robinson from the EHE Foundation in the USA updated the meeting on the history of sirolimus involvement in treating EHE, including some of the data from the EHE Group's sirolimus survey which was the subject of a scientific paper published in February 2024 (see the Research section of this Report).



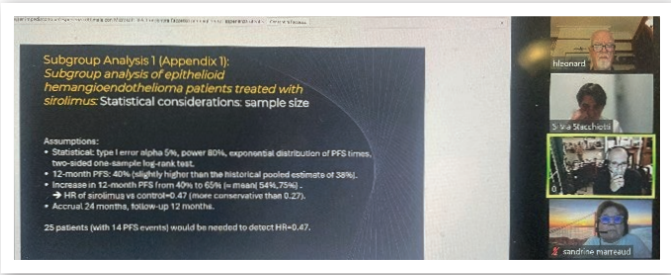
Both workshops were positive, with strong alignment between the clinicians, patient groups and the regulators, all keen to continue the dialogue and to find improved ways to approve new drugs or the repurposing of existing drugs for ultra-rare cancers.

The focus on repurposing is of particular importance for EHE as the EHE Group continues to seek approval of sirolimus for the treatment of EHE. Hugh Leonard explained:

"Getting a marketing authorisation approval for sirolimus will allow more EHE patients to have access to this important drug. This is necessary because, without formal regulatory approval, sirolimus is only available to patients if their doctor, hospital, and health system, and/or insurance companies will all allow off-label use. The only alternative may be to self-fund. As a result therefore, because of these off-label limitations, many of our patient community cannot access sirolimus".

01 Patient Support and Advocacy - continued

In addition, because EHE is so rare, many of the standard practices that regulators would expect an applicant to use in support of a drug approval, new or repurposed, are just not possible. The clinical team, led by Dr Stacchiotti, had therefore worked hard to develop a prospective observational study protocol which would generate data about the disease and the effect of sirolimus, in a manner that is as close as possible to those standard procedures.



In the technical exchange with the EMA, known as Scientific Advice, the application team provided a further set of detailed questions to the regulator, seeking feedback as to whether their latest study proposal would be likely to meet regulatory requirements.

The Scientific Advice response letter was received in mid-December 2024. Although it included a number of observations and identified concerns, the EMA did recognise the difficulties presented by such a rare disease and indicated that with some further small changes, the study protocol could be acceptable.

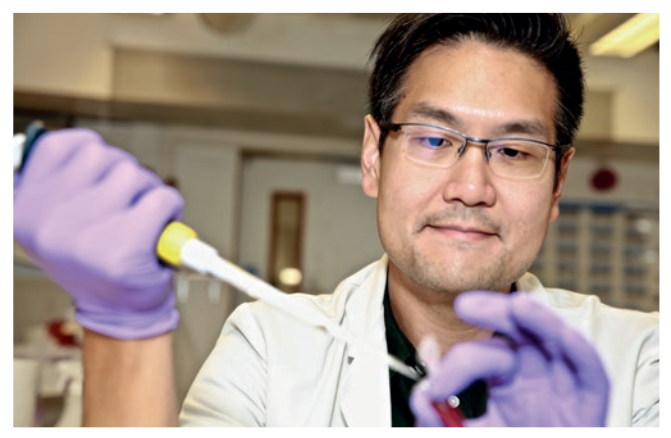


The Charity believes that this is a positive response, and hopes that it will soon be possible to start to actually gather data under the study protocol. Success of the study is not guaranteed but will of course be dependent on the actual results achieved, which must in fact meet or exceed the target results that are included within the protocol itself and which define the outcomes that will be necessary to demonstrate an acceptable level of efficacy.

We look forward to further EMA engagement and progress in 2025.

New member joins the Charity’s Advisory Board

In mid-January, the Charity was excited to be able to confirm that Dr Paul Huang had joined the Charity’s Research and Medical Advisory Board. Dr Paul Huang is Head of the Molecular and Systems Oncology Laboratory at the Institute of Cancer Research in London, UK. He received his PhD in Biological Engineering from Massachusetts Institute of Technology in 2008. His laboratory focuses on understanding aberrant signalling networks and drug resistance in sarcomas, with the goal of developing biomarkers and new therapies for these diseases. Dr Huang is the Deputy Director of the Joint Royal Marsden-ICR Sarcoma Research Centre, one of the largest sarcoma research centres in Europe. He serves as Vice Chair of the Pathology & Translational Research Committee of the EORTC Soft Tissue and Bone Sarcoma Group and on the Board of Directors of the Connective Tissue Oncology Society. He was elected a Fellow of the Royal Society of Biology in 2020.



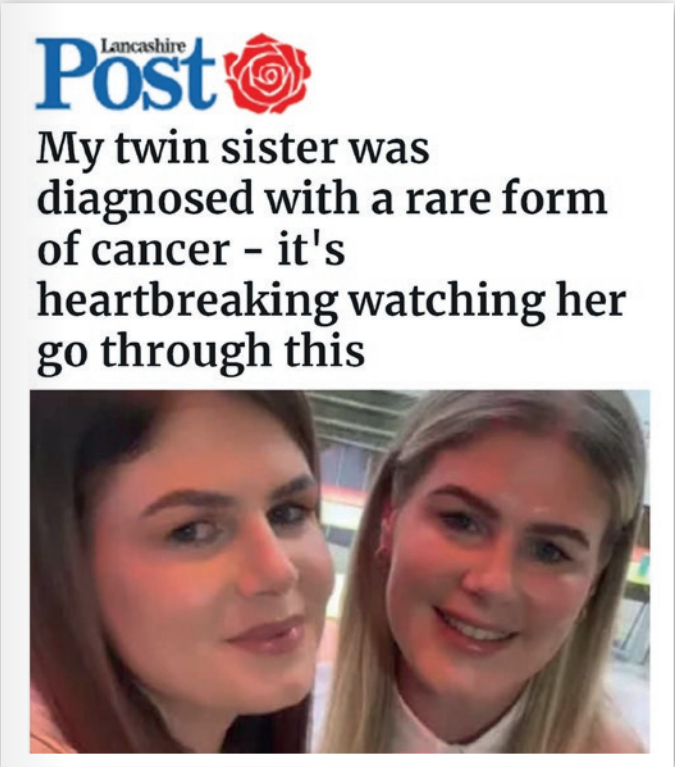
Hugh Leonard, Chair of Trustees, was thrilled to be able to welcome Dr Huang:

“We are really delighted to be able to confirm that Dr Paul Huang has joined the Charity’s Research and Medical Advisory Board, and welcome him to the team. Dr Huang works at the Institute of Cancer Research, which is a sister organisation of the Royal Marsden Hospital, where he continues to be a great champion of the EHE cause. He is a key collaborator with Dr Stacchiotti and her team in Milan on the research that the EHE Group continues to fund, including research to identify possible EHE biomarkers. It is hard for us to overstate how delighted we are to have Dr Huang join our Advisory Board”.

Twins tell their story to spread awareness

One of the objectives of the EHE Group globally is to spread awareness of the disease. This can be done in several different ways, but it is generally recognised that one of the most powerful formats is when EHE patients tell their stories. That sounds easy but, to open yourself to the public in such personal circumstances is very tough and requires considerable bravery and determination from those who do it.

One such case is Kym and Emma, twins from the UK, who wanted to share their story. As twins, the sisters are facing and dealing with the disease from a particularly unique viewpoint. Their story was first picked up by local press, and then nationally by the Mirror Group.



Emma was diagnosed with EHE in 2024, and Kym spoke openly about how heart-breaking it is to see her sister suffering and how hard she is working to support her sister and striving to make sure she is getting the best care possible. Kym said:

“With her being my twin, it’s been so hard for me not being able to control this situation and make her better as she is literally my other half. Over the last six months Emma has shown more strength and resilience than I have ever seen in a person. Some days she is fine and others she will wake up in agony. She’s on a lot of pain relief and anti-sickness drugs. She’s fighting against this disease everyday whilst remaining the world’s best mummy to her two boys and partner”.

Emma wanted to thank Kym for her brilliant support at such a tough time.

“I don’t know what I’d have done without Kym. From day one after getting my diagnosis she’s been full into research, fundraising, gathering all sorts of different contacts to help me, whilst having her own young family to care for. She’s the real superwoman”.



At the Charity, we could not be more grateful to Kym and Emma for doing so much to spread awareness at such an incredibly difficult time. Once again we are inspired and amazed by the commitment and compassion that patients and their families, like Kym and Emma, have when faced with an EHE diagnosis.

01 Patient Support and Advocacy - continued

Quality of Life study

To better support drug development and improve overall outcomes for people diagnosed with EHE, the European Organization for Research and Treatment of Cancer (EORTC) has been conducting an international study on Quality of Life (QoL) in people with rare cancers, including EHE. The EHE Foundation, on behalf of the EHE Group, engaged with EORTC to enrol EHE patients in this study, as Denise Robinson explained:

“The EHE Foundation has been working to enrol people with EHE in this quality-of-life study. This is an easy way for patients to help with EHE research from the comfort of their own homes! The overall objective of the study is to develop a document that can be used to help measure the quality of life in EHE patients, and which can then be used also to assess the impact of a specific drug on such patients. While QoL assessment will never be sufficient on its own to justify drug approvals, the reality with ultra-rare diseases is that all evidence is important, and for this reason, this study is important, and one that the Foundation wanted to support. As always, the EHE patient community was keen to help, and were able to provide EORTC with the EHE participants they needed, another excellent example of how patient involvement is so important to our overall EHE objectives. We want therefore to thank all those who took part in this important study”.

This is an important collaboration of the EHE Group, aligning with ongoing efforts to work with regulators to look at repurposing drugs, specifically sirolimus for the treatment of EHE, as well as novel drugs for ultra-rare sarcomas. In repurposing drugs or to get a new investigational drug into review, the issue of establishing the efficacy of a drug has arisen. Regulators rely on a standard called RECTIST v1.1 to measure efficacy, but recognise that this does not work well in the case of some ultra-rare diseases such as EHE.

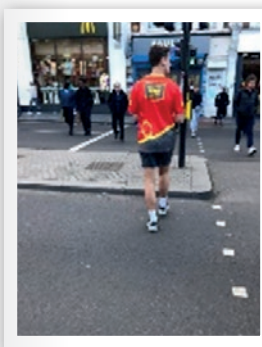
We are grateful to EORTC for undertaking the development of a standardised instrument that can be used to objectively assess the change in symptoms experienced by the patient, measuring the effect on the QoL of those taking a drug, or during active surveillance. QoL assessment is an established and recognised process in the clinical and research world. The EHE Group completed such a survey of QoL for EHE patients in 2019, led by Dr. Marije Weidema from the Reboud University in Holland, but this assessment was of the disease generally, and not undertaken with specific reference to treatment outcomes or a specific drug. (The published paper for this assessment can be found in the EHE Library on The EHE Foundation website or at <https://www.tandfonline.com/doi/full/10.1080/0284186X.2020.1766696>).



Quality of Life Study
Volunteers Needed



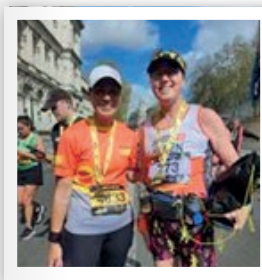
Spreading EHE awareness



Spreading awareness of EHE takes different forms, one of which is people wearing EHE merchandise with our EHE logos on them. Jake Leonard, Hugh Leonard’s and Sally Baker’s son, who lives in London posted a photo of somebody out jogging at lunch time wearing an EHE Rare Cancer Charity running shirt. Sally Baker noted:

“It’s great to see our running shirts out and about. We hope one or more people might have asked what the charity does!”

Michelle Hughes was also thrilled when her running shirt led to an approach from another runner at the finishing line of the London Landmarks Half Marathon:



“When I came across the finish line a fellow runner who had seen my shirt came up to me and said she is a nurse who treats EHE patients. She proceeded to tell me about her hope for our cancer. It was incredible to hug her knowing how much she cared”.

We love these impromptu stories of EHE awareness and engagement.

Remembering those who have left us

One of the things that our global community always wants to do is to remember those who left us far too early, of whom there are far too many. Kim Alexander-Bird posted news of just such an opportunity when she visited the Royal Marsden Cancer Charity’s ‘Ever After Garden’ in Grosvenor Square in London.



The Ever After Garden is a tranquil haven where anyone can come to remember treasured friends and family they have lost, offering a space to pause and reflect over the Christmas period. Visitors are invited to dedicate a rose in memory of a loved one, whilst making a donation to The Royal Marsden Cancer Charity, helping to fund groundbreaking research which improves the lives of cancer patients everywhere.

02 EHE Research

The Charity, and indeed the global EHE Group, is both excited and inspired by the levels of EHE research engagement we see in the UK and globally. Thanks to our wonderful supporters we are also delighted that we have been able to expand and extend our existing research while also considering new research projects, together with our sister EHE Group entities.

In 2024, the collaborative EHE research being led by Istituto Nazionale dei Tumori in Milan, and the Institute of Cancer Research and the Royal Marsden Hospital in London has delivered important results together with a number of new and exciting research ideas. The observational study and the prospective registry of EHE patients has also continued to grow, both also managed by INT in Milan.

We are also delighted at the excellent support for, and progress of, the National UK EHE Biobank. This important infrastructure is maintained at the Royal Marsden Hospital, collecting critical EHE bio-samples for future research, funded by the Charity.

Summaries of these research programmes can be found on the following pages.

Review by Dr Oliver Pearce and Dr Kate Hooper

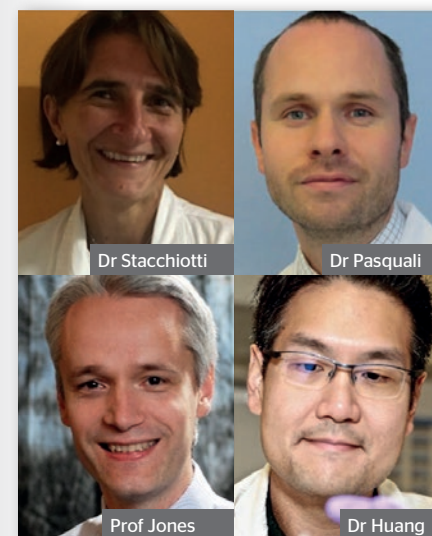
European collaboration

The Charity, together with the EHE Foundation (USA), is funding this major European collaboration involving the Istituto Nazionale dei Tumori (INT), Milan, the Institute of Cancer Research (ICR) in London, and the Royal Marsden Hospital (RMH) in London.

The overall research program entitled 'Evaluation of Cytokines and Hormones as Biomarkers for EHE' was initiated at the end of 2020 and extended and expanded at the end of 2022. The resultant overall research comprises a number of different objectives or projects as follows:

- the identification and validation of novel circulating and tissue biomarkers to inform patient management (prognosticators and predictors of response to medical agents) as well as potential therapeutic targets (**Project 1**)
- the development of patient-derived xenograft (PDX) models (**Project 2**)
- the assessment of the activity of drugs relevant for the disease (**Project 3**)
- the generation of PDX-derived cell lines to investigate the cellular and molecular determinants of drug activity and to be submitted to a CRISPR whole-genome screen to identify new therapeutic targets (**Project 4**)
- the identification and evaluation of miRNAs (**Project 5**)
- the description of the radiologic characteristics of EHE and their correlation with the clinical outcome (**Project 6**)

Dr Silvia Stacchiotti, one of Europe's leading sarcoma clinicians, and Dr Sandro Pasquali, one of our leading sarcoma researchers, are joint Principle Investigators (PIs) of the project at INT; Dr Paul Huang is the PI at the ICR, and Professor Robin Jones is the PI at the RMH.



Biomarker focus

A key focus of this research continues to be the assessment of circulating cytokines, hormones (and miRNAs), and estrogen receptor expression, etc, and the identification of a novel biomarker for EHE.

Using a protein array able to simultaneously detect the expression of a hundred different cytokines in plasma samples of EHE patients (n=15) and healthy individuals (n=6), a small panel of inflammatory cytokines was found to be differentially expressed. Among them the researchers focused on Growth and Differentiation Factor-15 (GDF-15), a member of the TGF- β super-family, which has multiple roles in a wide variety of cellular processes. Using a specific ELISA assay, they looked at the concentration of circulating GDF-15 in a retrospective series of 23 EHE patients and observed a statistically significant association of GDF-15 levels with EHE aggressiveness. These results were confirmed in a second cohort of 21 EHE patients prospectively collected within the currently ongoing observational study.



Dr Pasquali and Dr Zaffaroni reviewing research results with the EHE Group

The research team also used their models to test the results by measuring GDF15 levels before and after treatment with drugs that were seen to clinically benefit patients.

Following further validation, the Charity was delighted to see the research paper, entitled 'GDF-15 predicts epithelioid hemangioendothelioma aggressiveness and is down-regulated by sirolimus through ATF4/ATF5 suppression', published in *Clinical Cancer Research* at the end of the year.

02 EHE Research Continued

Results of this groundbreaking research show that:

- a) In the two models used, a patient-derived xenograft (PDX) and paired EHE cell line, EHE cells produce and release GDF-15. GDF-15 is a cytokine that cancer cells produce and is used as a biomarker for the severity of many diseases.
- b) These results indicate that GDF-15 could be a useful novel biomarker to predict EHE aggressiveness and disease progression.
- c) In the EHE models used, sirolimus was shown to reduce GDF-15 expression.
- d) Additionally, sirolimus (not yet approved for EHE treatment) had a higher anti-tumor activity than doxorubicin (approved for soft tissue sarcoma) when compared in the model systems.

For EHE patients, these findings give hope for further preclinical drug development using human-derived EHE models, better-informed treatment decisions, and the ability to monitor disease progression, all with the goal of improving patient outcomes.

The paper can be found at:
<https://pubmed.ncbi.nlm.nih.gov/39283723/>

Interesting results relating to other potential biomarkers such as miRNAs have also been seen and remain under investigation.

Critically, in addition to the lab-based work, a key component of this research was the creation of a clinical observational study in to which INT and RHM had enrolled more than 50 EHE patients by the end of 2024. Each patient is being treated under a standard protocol, creating a significant, high quality study of the disease which will inform ongoing thinking regarding EHE research and treatment.

Research extended

The original proposal from the research group was for a three-year research project. It was agreed at the outset that the EHE Group would commit to fund the initial two years and review funding of the third year based on the results of the first two years. The research work and results achieved and the importance of the observational study, as shown by its application, with appropriate amendments, to the sirolimus approval programme, were summarised by the research team at the end of the second year in July 2024. That detailed summary was at the core of the EHE Group's assessment, leading to The EHE Foundation and The EHE Rare Cancer Charity agreeing to jointly fund the third year of the project.

In addition to the third year of the project, the research team also submitted a proposed work scope and budget amendment to enable continuation and expansion of the observational study, increasing its target cohort from 50 to 100+ patients, and its duration to the end of 2027, allowing data to be collected for a larger group over a longer period, leading to a better and more accurate understanding of the disease. This observational study has now also become a fundamental component of the ongoing work with regulators to have sirolimus approved for the treatment of EHE. With agreed modifications to the original protocol and increased verification of protocol compliance, the study will now become a prospective observational study. It is also hoped that this new study may be joined by other institutions from around the globe.

Dr Pearce commented:

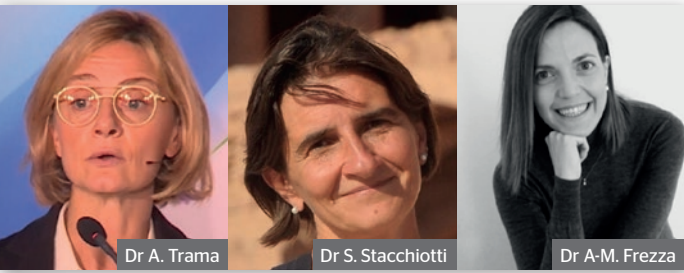
"The work and progress made has been exceptional and we are so grateful to the whole research team for their ongoing dedication and focus on EHE. After a thorough review, we were pleased to approve the third year funding for this excellent project, and remain excited by its ongoing potential".

Dr Hooper was also excited by the growing observational study:

"We are excited by the development of the observational study into a prospective observational study with the result that a sub-set of the patients who are prescribed sirolimus can be used to support the work we are doing to seek approval for this drug from regulators. Canada joining the study is also an excellent development, and we will be working hard to introduce the study to other institutions".

EHE Prospective Observational Registry

The need for pan-European registries for rare cancers was recognised by the European Reference Network in Rare Adult Solid Cancers (EURACAN). The STARTER project was therefore established in 2022 on the basis that gathering data on rare cancers will support research, help to develop and/or improve clinical practice guidelines, support multidisciplinary discussion and consultations for patients with rare cancers, and ultimately improve quality of care across Europe.



The Charity was therefore delighted when it was informed that EHE had been selected to be the first rare cancer within this important new data-gathering initiative, and immediately committed to funding the setting up of the first such prospective observational registry for the disease. The registry Principle Investigators (PIs) are Dr Annalisa Trama and Dr Silvia Stacchiotti, from INT in Milan, with Dr Anna Maria Frezza providing clinical support. The registry involves more than 22 hospitals across Europe. Patient enrolment into the registry started in 2023, with 20 patients enrolled at the end of 2024.



Dr Hooper was delighted to see the number of hospitals that engaged and the number of patients being enrolled into the registry:

"Joining the registry is not compulsory so we were delighted to see that 22 hospitals had joined the project. The registry team hope to add more hospitals in the future, including international centres who have expressed interest. It is also encouraging to see 20 patients already enrolled by the end of 2024. Only new patients recently diagnosed can be included, so with the ultra-rare nature of EHE, building patient numbers will always be slow. But, capturing this data is so important and over time the study will provide high-quality valuable information that will be important for generating hypotheses and further interrogation of disease treatments. This was why we did not hesitate to provide funding to INT for the registry when we were approached".

PUSH Platform continues to grow

2024 also saw important progress on new infrastructure called the PUSH Platform (Pushing Ultra-rare Sarcomas beyond Hope). This platform is being developed to help address the significant challenges that patients, clinicians and researchers of ultra-rare sarcomas face, such as:

- Having minimal data on the natural history of their disease;
- An acute lack of research funding to comprehensively study ultra-rare diseases;
- Few if any defined treatment options (in EHE there are no disease-specific approved systemic treatments);
- Major hurdles to, and in most cases the impossibility of, designing and performing large, randomised clinical trials within a reasonable timeframe and cost, due to the extreme rarity of patient populations; and
- Difficulties in drug development and access due to no or minimal interest from commercial drug developers and their perception of high costs and risk of failure when engaging with regulators.



02 EHE Research Continued

It is ultimately hoped that a significant number of institutes and academic centres across the globe will join the project, forming the PUSH Consortium, giving the PUSH Platform real momentum and an ability to change the landscape for ultra-rare sarcomas. The first two sarcoma studies to be included under the PUSH Platform were EHE and Chordoma. Two additional multi-centred disease studies were also added in 2024 for: Low Grade Fibromyxoid Sarcoma/Sclerosing Epithelioid Fibrosarcoma (LGFMS/SEF); and Adult-type Rhabdomyosarcoma (RMS).

The PUSH Platform will include collating relevant prospective and retrospective data from substantial numbers of patients from around the world, including real-world data from patient registries such as the EHE Global Patient Registry managed by the EHE Foundation in the USA. PUSH will incorporate data from a large range of different sources that will in turn be used, with regulatory involvement and input, to define and undertake appropriate new prospective studies and associated analyses to help deliver new treatments for patients in faster, lower-risk, well understood and defined processes. PUSH is designed to also include prospective clinical trials. PUSH is particularly important for patients with ultra-rare diseases such as EHE, who face significant unmet needs in terms of the availability of effective treatments.

The PUSH Platform is also a component of the ongoing discussions with the EMA and the USA Food and Drug Administration (FDA) concerning the processes and procedures relating to drug approvals for ultra-rare sarcomas, as reported in the Patient Support and Advocacy section of this report. There is recognition that changes need to be made to reduce the time, risk and costs of such processes, and so encourage pharma involvement in the development of such drugs.

The Charity is actively supporting the PUSH Platform, with Hugh Leonard, and Denise Robinson from the EHE Foundation in the USA, both being members of the PUSH Executive Committee. There is a huge amount of work in progress, as Hugh explained:

“Establishing major new infrastructure like PUSH takes a huge amount of work, focus and determination. Multiple working groups have been established looking at a wide range of relevant issues, such as data collection, legal issues, operational management, trial protocols, translational research and regulatory/pharma liaison. Right at the moment a key area of focus is how we resource and run PUSH to ensure that the daily operation and administration of the platform is fully covered, effective and efficient. Exciting discussions are underway and we hope we can report further progress over the coming months”.

Drug Repurposing for EHE

‘Drug repurposing’ is the process whereby researchers and clinicians test drugs that are already approved for human use for other diseases to see if they show sufficient activity against another disease (that it is not approved for), such as EHE. This is a growing area of drug development research and is a hugely important part of any cancer research programme. It is also a critical pathway to produce approved drugs for ultra-rare diseases, as developing truly novel compounds is almost certainly uncommercial in these cases. Repurposing is therefore a core part of the EHE Group’s ongoing research programme and future EHE strategy.

The EHE Group is funding different drug screening projects and has already identified a number of good candidates. At the same time, the Charity continues to work hard to secure an approval from the EMA for a label extension of sirolimus for the treatment of EHE. This process was started in 2022 when the AntiCancer Fund in Belgium submitted an application, ‘sirolimus-for-the-treatment-of-EHE’ for the EMA’s new repurposing pilot process. The application was successful and the AntiCancer Fund then reached out to Dr Stacchiotti in Milan and to the Charity to seek support in taking the application forward.

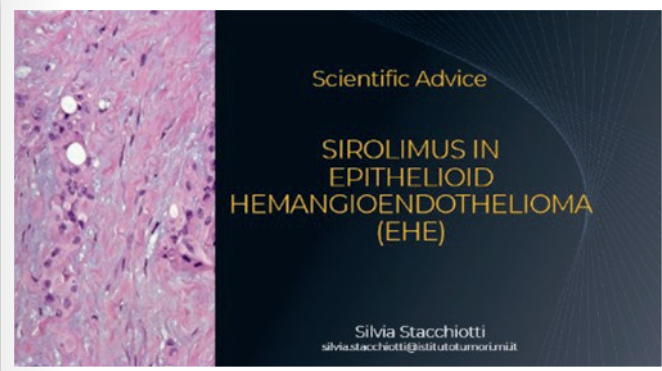


EHE Repurposing Group videoconference

In January 2024, Hugh Leonard participated in an EMA public workshop in Amsterdam, to discuss ways that the drug repurposing process could be improved, which used EHE as a case study. Hugh’s presentation addressed the challenges faced by ultra-rare diseases and why efficient repurposing was so essential. At the end of March an additional informal meeting was held with the EMA to review two new study proposals submitted by the EHE repurposing group, and to answer questions the group had with regard to the Scientific Advice being sought. Scientific Advice is the EMA process where applicants can provide and pose questions relating to their supporting data and whether it is sufficient to support the repurposing of the drug.

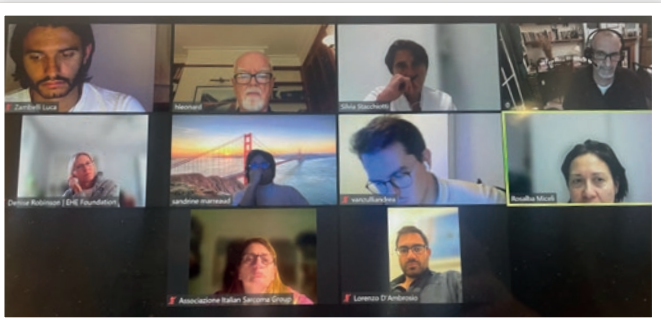


The EHE Group continued through the second quarter to engage with the EMA regarding the approval of sirolimus for the treatment of EHE.



The Scientific Advice we received clarified that some additional data would be required. It also appeared to clarify for the regulator that their standard approach with its prescriptive and rigid structure was not suitable when considering ultra-rare diseases.

One of the key issues under discussion with the regulators is how efficacy of the drug is measured, because the traditional methodology, using a process called RECIST v1.1, does not work well for EHE.



The EHE repurposing group was therefore focused on addressing the points raised by the EMA, including the development of disease-specific criteria for evaluating whether a drug is working. This is necessary because the traditional criteria for assessing a drug are based on common cancers where tumour shrinkage is expected. As the EHE patient community understands, a key objective with EHE is to re-establish disease stability once EHE has become progressive, but which is likely not to include shrinkage. The group were therefore pleased that the EMA appeared to understand these challenges of testing the activity of a drug in such rare diseases and were actively engaging to help find solutions. The team at INT in Milan in particular completed some amazing work looking at different assessment criteria, culminating in acceptance of their presentation ‘Redefining Radiological Responses in Epithelioid Hemangioendothelioma: Beyond RECIST?’ for oral presentation at the prestigious CTOS 2024 Annual Meeting.

The EHE patient community has also been able to add important data to these discussions, from its survey of EHE patients globally, helping to provide further evidence of the beneficial effects of sirolimus. The results of the survey and the resultant published paper are described in the article below (‘Sirolimus patient perspectives survey results published’).

The discourse with the EMA continued with a further round of Scientific Advice submitted in late summer 2024, including revised data generation plans. At the end of the third quarter, the EMA provided a list of further questions that needed to be addressed. These were answered and discussions were ongoing at the end of the year.

As the year closed, it looked likely that further data would need to be collated on the disease and its treatment with sirolimus, through a new prospective observational study, building off the existing EHE observational study that was started by INT in 2022. The Charity hopes that further progress can be made in 2025, helping to bring forward approval of sirolimus.

02 EHE Research Continued

Sirolimus patient perspectives survey results published

The EHE Group were delighted to report that a paper addressing the important issue of patient access to the drug sirolimus (one of a class of drugs known as mTOR inhibitors) had been published in *Frontiers in Oncology*, a prestigious, peer-reviewed scientific journal, on 26 February. This paper, entitled ‘The patient perspective on sirolimus for epithelioid hemangioendothelioma (EHE): results of a community survey highlighting the importance of equitable access to treatments’ presented the results of a survey of the global EHE patient community, conducted in 2023, about their access to, and results of treatment with, sirolimus.

The patient perspective on sirolimus for epithelioid hemangioendothelioma (EHE): results of a community survey highlighting the importance of equitable access to treatments

 Denise Robinson¹

 Hugh Leonard²

 Giacomo Giulio Baldi³

 William D. Tap⁴

 Robin L. Jones⁵

 Silvia Stacchiotti⁶

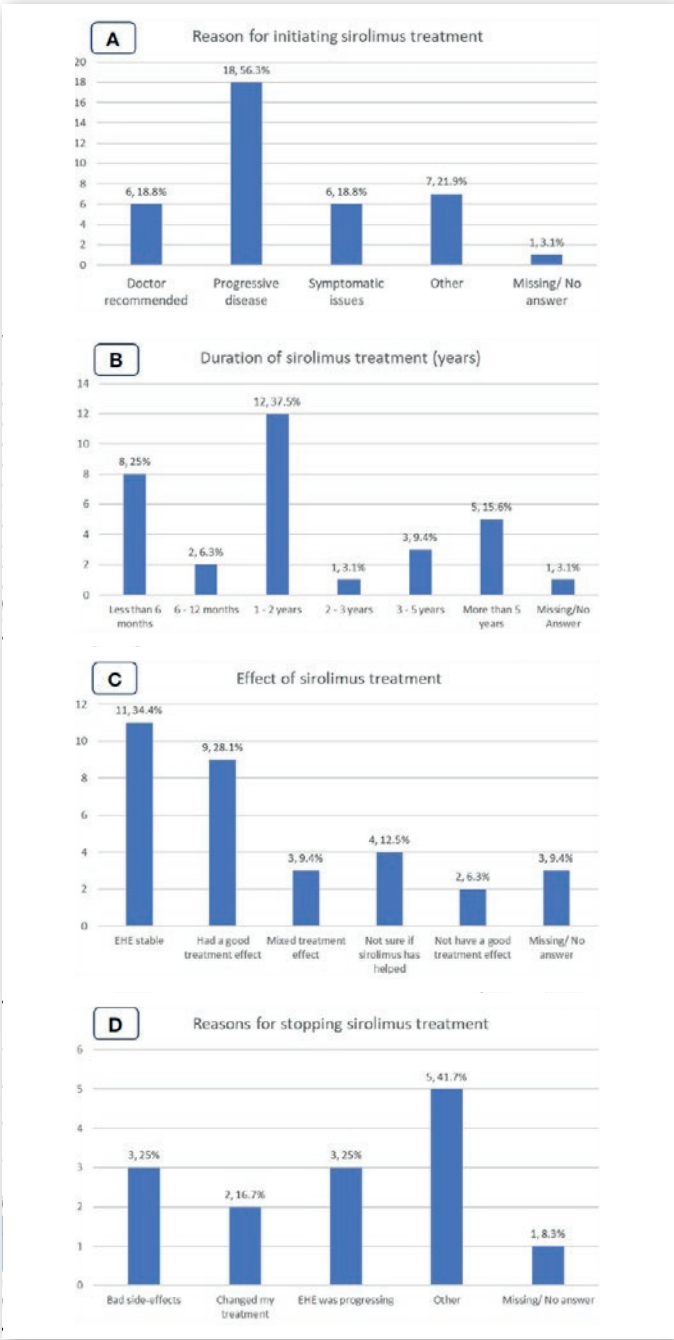
 Pan Pantziarka^{7,8*}

The survey, coordinated by Denise Robinson, Director of Research at The EHE Foundation, and Hugh Leonard, Chair of Trustees of The EHE Rare Cancer Charity UK, was undertaken to support the ongoing application to the European Medicines Agency (EMA) for a label extension of sirolimus (also called rapamycin and marketed by Pfizer under the tradename Rapamune®) to include the treatment of EHE. Although sirolimus has been globally approved and widely used for many years, mainly as an immunosuppressant, it can still only be prescribed to EHE patients ‘off label’ as it has not been formally approved for the treatment of EHE. This means sirolimus is only available to patients who access the drug off-label, resulting in inequality of access to this important treatment.

There is a growing body of significant evidence, including the experience of our patient community, that sirolimus is now the leading front-line drug for the systemic treatment of EHE. In 2021 the ESMO paper ‘Epithelioid hemangioendothelioma, an ultra-rare cancer: a consensus paper from the community of experts’ recognised, when comparing different drugs used to treat EHE, that **“the highest clinical activity has been reported for mTOR inhibitors”** and confirmed that **“the panel [of experts] agrees that these [mTOR inhibitors] represent the preferred treatment options for patients with advanced and moderately progressive disease”**.

The EHE Group survey collated the experiences from 130 patients, 32 of whom were non-liver-transplant patients that had experience of treatment with sirolimus. A high percentage of these patients had experienced good results, with some having been on the drug for more than 5 years.

The results of the sirolimus survey are an excellent example of why patient data is so important. The data, summarised in the graphs shown here, supports the position expressed in the ESMO paper described above, namely that mTOR inhibitors like sirolimus are being successfully used in the treatment of EHE. Its responses also confirmed that these drugs are not equally accessible for all patients because they are only available off-label.



Patient experiences of the Group S (sirolimus) cohort. (A) Reasons for initiating sirolimus treatment. (B) Duration, in years, of sirolimus treatment. (C) Patient perspective on the effect of sirolimus treatment. (D) Reasons for cessation of sirolimus treatment.

Hugh Leonard expanded on why the published paper is important to the regulatory process, not only with the EMA in Europe, but also with other regulators like the FDA in the USA:

“Regulators want to hear and understand the experience and perspective of the patient community. However, in presenting such information, we need to be able to show that the data was collated and analysed in a rigorous manner, in line with accepted scientific practise, and that the results and conclusions drawn are supported by the underlying data. The publication of the results in a peer-reviewed paper confirms all these requirements have been met and allows the survey to be accepted and considered by regulatory bodies in their deliberations”.

The Charity wants to thank the other authors of the paper, Giacomo Giulio Baldi, MD; Pan Pantziarka, PhD; Silvia Stacchiotti, MD; Robin Jones, MD; and William Tap, MD, for their contribution, guidance, and commitment in helping to produce the paper. Huge gratitude is also extended to The EHE Rare Cancer Foundation Australia, EHE Canada, and EHE Italia Associazione Non Solo Laura ODV, all of whom were instrumental in mobilising support and participation of EHE patients. But the biggest thanks, without doubt, goes to each and every one of the 130 EHE patients who took part in the survey. Without their fantastic support, there would have been no data to work with.

If you wish to read the paper, it can be found at:
<https://www.frontiersin.org/journals/oncology/articles/10.3389/fonc.2024.1367237/full>

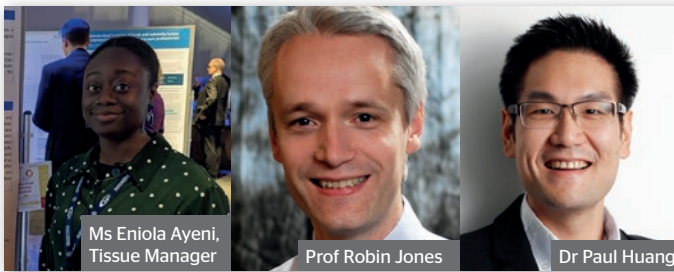
The importance of biobanking

The collecting of biological samples is critical for future research into any disease. With common cancers, tissue and fluid samples are abundant and collecting these samples is relatively easy. For a disease as rare as EHE, however, banking biological samples is absolutely critical. With so few patients, many of whom do not undergo surgery, tissue samples in particular are ultra-rare. It is hard to over-state how important tissue donation is for moving EHE research forward. For these reasons the Charity funded the establishment of the National UK EHE Biobank at the Royal Marsden Hospital, and continues to fund the annual costs for collecting the samples.



Progress has been very good. During 2024 a total of 17 new patients enrolled in the biobank, taking the total to 53 patients. At the end of 2024, 32 patient blood samples, 37 FFPE samples; and 3 live tissue samples were held within the biobank:

This is encouraging progress, but capturing and storing the samples is of little value if they are not used for research. Access for researchers is now critical, but has to be very tightly controlled, both under the ethics approval granted for the biobank, and under the relevant UK legislation relating to human tissue use. Professor Robin Jones, Principle Investigator for the biobank, Dr Paul Huang, and Eniola Ayeni, the Biobank Tissue Manager, were therefore instrumental in establishing the Biobank Access Committee which we hope will meet in 2025.

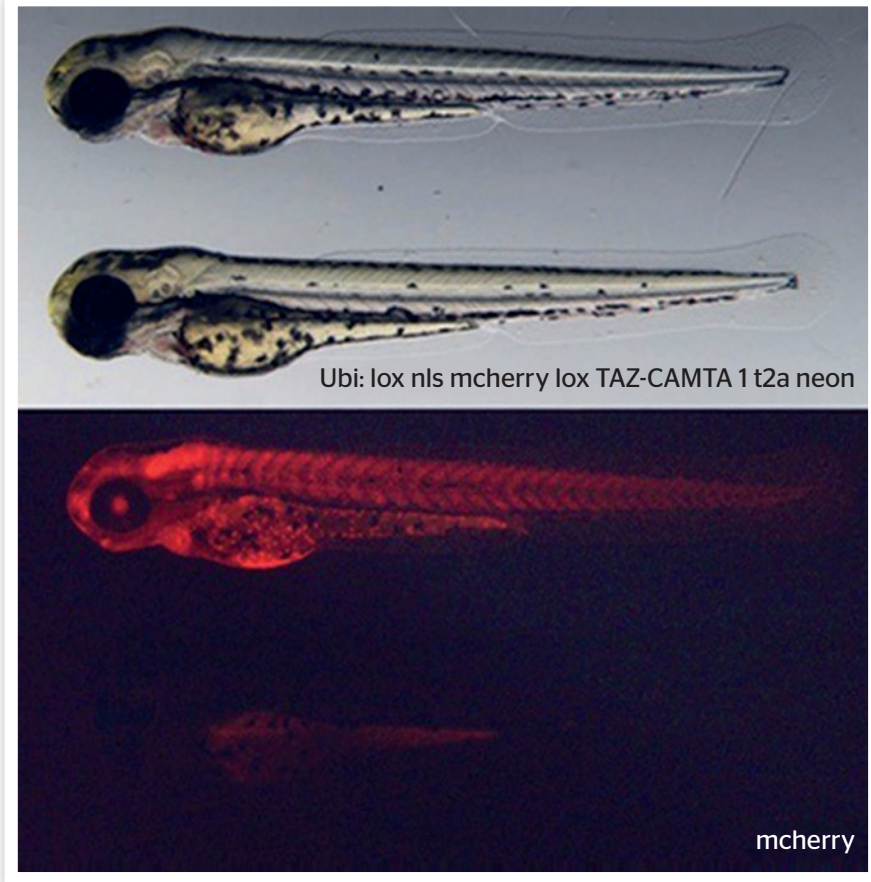


02 EHE Research Continued

Zebrafish research suspended

In 2018, the Charity initiated research at the Bateson Centre at the University of Sheffield. The Bateson Centre is one of the largest and oldest zebrafish facilities in Europe, and researchers there are experts in developing models of human diseases in zebrafish. Zebrafish are used because they are cheap and quick to produce, if you are successful in creating the model you want. They are also largely transparent, making it easy to see morphological changes as the fish develops.

The primary reason for initiating this research project, seeking to develop a zebrafish model of EHE, was the complete lack of any viable EHE models of any kind. In 2018 there were no PDX mouse models, no PDX-related cell lines, no genetically-engineered mouse (GEM) models, and no human cell lines. Work was underway in these different areas, but with no guarantees of success with any specific model; the continuation of work on all models was essential. The Charity understood that creating a zebrafish model would be a challenge, but all EHE models were proving to be very difficult to establish, so the zebrafish model could not be ignored.



The team at the Bateson Centre was led by Dr Fredericus van Eeden, with Eleanor Markham undertaking most of the painstaking technical work. Over the following five years, the team introduced the gene translocation constructs into the fish, requiring an unbelievable attention to detail to ensure the constructs were correctly located. Many challenges were faced, but each time the team found solutions that allowed them to move on. This included introducing a switching mechanism into the fish so that the gene translocation constructs could be turned on, as they seemed to be fatal at the embryonic stage of the fish. Multiple variations of the construct were tried, with minute adjustments made to its structure and positioning in the fish.

Ultimately the team were able to create EHE fish with switchable mechanisms for both the TAZ-CAMTA1 and YAP-TFE3 variations of the disease. But although the gene translocations could be turned on, and low levels of the fusion proteins seemed to be present, they were in very small quantities, making the fish unviable as EHE models.

But while the zebrafish remained stubbornly resistant to producing a viable EHE model, model developments were progressing in other areas. Dr Brian Rubin was finally able to get his GEM model to work, creating an unbelievably robust model for all presentations of the disease. Cell lines soon followed. At the same time, Dr Silvia Stacchiotti and the team at INT in Milan created the first viable EHE PDX mouse model from a patient tissue sample. This also led to a further cell line. More recently there has been exciting progress with human cell lines in the USA. With all this excellent model progress elsewhere, the Charity could not justify further expenditure on this project.

The decision was therefore taken in August 2024 to suspend the research project. All the work completed has been carefully documented, and any important constructs stored. In the final 6 months the team also investigated if EHE cells could be injected into zebrafish and kept alive, as a xenotransplantation model. These xenotransplant fish provide a life support system for the EHE cells which might then be exposed to treatment with drugs. This will be a possible future development, should the use of such xenotransplant fish become important.



03 Fundraising and Finance

2024 was another year in which we have enjoyed unbelievable support from our patient members and their supporters. Together, bringing their energy, enthusiasm and determination to the role of fundraising, they have raised substantial funding for critical EHE research.

In 2024 we received more than £132,000 from all sources. Of this sum, £15,000 was received from a single donor, specifically to fund our charity running costs. That means that 100% of the remaining sum of £117,000, received from all other sources, is available for EHE research. These funds are critical to a charity that is dealing with a disease as rare as EHE, as without this we would not be able to support the research that continues to deliver such exciting results. In the previous section we provided an overview of our current research, none of which would have been possible without the amazing support we continue to receive.

And it is not just the UK that is enjoying such progress. Our sister foundations in the USA, Australia, Italy and Canada are also making great advances. We cannot thank our supporters across the globe enough for their dedication and wonderful support.



Review by Jeff Collins

Every year, the EHE patient community and their supporters provide us with amazing support and raise significant funds for EHE research. 2024 was no different, with the Charity receiving more than £132,000 in donations. That support means that we can again not only support our ongoing research, but look at ways to expand it, as well as consider new projects.

In 2024, our grassroots fundraising was strong, with our patient community and their supporters again supporting the charity. We also enjoyed wonderful support in our two major charity-organised events, where we had more than 50 runners in the London Landmarks Half Marathon, and 14 riders in the RideLondon-Essex 100 event. Particular mention goes to Sam who rode the 100 miles on a stationary bike in his lounge due to having broken his wrist during training!

We saw huge support for Emma, a newly diagnosed patient, championed by her twin sister, Kym, and all her family. Collectively, they threw themselves into fundraising for EHE research with amazing support from so many people. Their 2025 calendar, '10 men with sunflowers', seen on the front cover of this report, was a particularly imaginative fundraising idea. We also received substantial donations from Luke Harrison, also diagnosed with EHE in 2024. Team Dean, supporting Paul, were again amazing, with a large contingent not only running, but also hosting fundraising events in Birmingham.

Kelly Denton held her annual EHE Quiz Night at the start of the year and her bottle tombola at the Penge Festival in the summer. Lorraine Faulds and her daughter, Louise, walked 96 miles from Glasgow to Fort William in Scotland in support of Lorraine's mother, and Katy ran the London Marathon in support of Nicola Henderson. Nicola and the Charity also enjoyed yet more wonderful support from Darran and Kerry Marks as they both took on the Sodbury Slog.

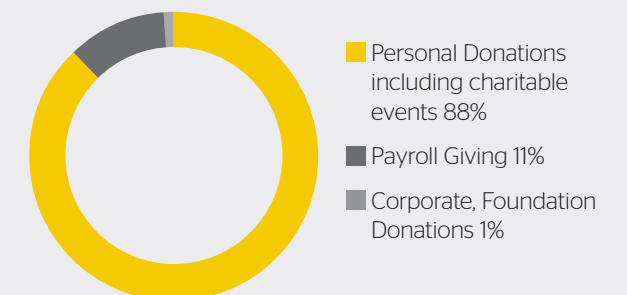
Judith Gordon and friends were on the fundraising trail in Scotland, participating in both the Edinburgh Shine and Super Nova events. James and Kirsty, organised a raffle in support of Issy Payne, their mother. Issy's colleagues at the Royal Mail also wanted to support her and Issy was delighted to report that they had collected a substantial sum for the Charity. Steph Scott and Adam Patrick held raffles to add to their fundraising page.

Key objectives

In the 2024 calendar year our supporters raised a total of £132,868 for the charity's key objectives. The sources of these funds are summarised on the pie chart above.

During 2024 we provided funding for our UK research project at the University of Sheffield. We also funded the EHE National Biobank and Tissue Manager at the Royal Marsden. We jointly funded the collaborative research project between INT in Milan and the ICR in London.

Source of funds



Administration/business running costs

In 2024, we once again received the generous support of a single donor who funded 100% of the charity running costs. As in all previous years, we segregated these funds, donated for administration purposes, from those received from all other sources, which can therefore be allocated to fund our key objectives, in particular, EHE research. It is the intention of the Trustees that the same will be true in 2025.

The charity received funding of £15,000 for its administration and business running costs account. During 2024, £9,900 of administrative expenses had been incurred, which included an accrual of £1,200 for the independent examiner's fee.

The remaining spend of £7,700 (excluding the £1,200 independent examiner's fee) included the fees associated with fundraising events conducted in 2024, and publishing costs, in addition to ongoing website maintenance.

"We are once again so grateful to our patient community, their supporters, and the corporate groups and foundations who supported us through the year. A particular and huge thanks must go to Luke Harrison for his wonderfully generous donation. We are also grateful to all those who helped us and volunteered so that we could keep our charity running costs as low as possible. I also want to thank all the companies and service providers who have supported us with their services, often provided at no or reduced cost."

"In 2023 we noted that the prevailing financial climate meant that 2023 and years beyond would continue to be financially challenging. It is thanks to our amazing patient community and their wonderful supporters that we have in fact been able to continue to raise substantial funds for the Charity through this period including 2024, for which we are hugely grateful. We will however continue to ensure that we already hold the funds for any funding commitments we enter into. We will also continue to manage all funds with the utmost care to ensure that we get the maximum benefit from every pound that we have so generously received."

Jeff Collins

Wonderful support for EHE in 2024

The following pages highlight some of the amazing fundraising events that raised funds for Charity in 2024.

Quiz nights are booming!

The start of the year saw two great quiz nights taking place to support the EHE Rare Cancer Charity.

The first was the annual EHE quiz night organised by Kelly Denton and the members of Laptops and Lipstick, a women's business network based in South London. Laptops and Lipstick adopt a charity each year to support, and selected the Charity for 2022/2023. They then extended their support for a further year, holding their annual charity quiz night on 12 February 2024 at the brilliantly supportive Bridgehouse Pub in Peckham.

The event was a huge success, as Kelly explained:

"Last year's quiz was fantastic, but this year's was even better. We again filled the pub, who did an excellent job hosting the event. We also had a lot of brilliant raffle prizes. Hugh Leonard was present and gave a brief update on the research that was taking place, thanks to the generosity and support for evenings like ours. It was also special having four actual EHE patients there on the night, bringing home the reality of EHE. And to top it all, we raised £1,960 during the evening for EHE research. I was so proud of everybody who organised and participated in the event. Without them we would not have raised those critical funds".

The second quiz night was organised by Jo and Phil Lane in support of Paul Dean, EHE patient. Jo organised a similar event in 2023 in the build up to the London Landmarks Half Marathon, and decided to do the same for the 2024 event. Jo and Phil have been wonderful supporters of Paul and the Charity and the three together form a formidable team.



Paul Dean, EHE patient, runner and cyclist, with Jo and Phil Lane, quiz night organisers and hosts.

Easter raffles raising research funds

People find many different ways to raise funds to support EHE research. Steph Scott and Adam Patrick were running the London Landmarks Half Marathon in April, and received great support on their fundraising page. But they wanted to do more and came up with the idea of holding small raffles with limited numbers of tickets through social media to raise research funds.

Their first raffle was very easter-focused, with a tantalising array of chocolates. Selling 60 tickets only at £3 each, they raised £180 for research. Not content with this, they then launched a second raffle for a prize comprising several different spirits. We want to thank them for such a great idea.



Runners turn out in force for EHE



Each year the Charity coordinates a team of runners to run in the London Landmarks Half Marathon. And 2024 was no exception, with more than 50 people running to raise funds for EHE research. This year we had three EHE patients taking up the challenge, including Michelle Hughes and her friend from Canada. Hugh Leonard posted a pre-run photo of many of those taking part with a very simple message – *"Love these guys!!!"*

Hugh also posted news of the build up to the run:

"Sally and I are on our way up to meet our EHE runners pre-race. It's looking like the weather will be lovely. And we have the wonderful Michelle Hughes running with the team this year. Huge thanks to every single one of them for running, and to everybody who has supported their fundraising efforts. They are an awesome band. Just live! Just run!"



03 Fundraising and Finance Continued

In addition to Michelle Hughes, patient Paul Dean also ran, supported by 19 other members of his fantastic support network.



Paul was the fastest of the EHE running cohort and wanted to thank so many for helping:

“Today was London Landmarks Half Marathon 2024! No PBs for me this year, as expected as I’d had some non EHE related ailments, but pleased with how it went! Firstly a huge well done to all the runners that ran for EHE Rare Cancer Charity today, so far my dream team is just shy of raising £10,000! An incredible achievement for such a small charity. Some great performances out there today and some real battles of will going on. Really proud of everyone. All in all it was a great day. It was a pleasure to meet up with some of you and our Canadian friends. well done everyone”.

People also wanted to post photos of their runners post-race:

Darran Marks, who ran in support of Nicola Henderson, was enjoying a beer while proudly showing off his race medal!

Tracey Betts posted a photo of her two sons, Josh and Connor, who ran in support of their mother and to raise research funding.



Riders join the cause

The EHE community in the UK not only find runners every year to help their cause, but also put up a group of riders who take on the ‘Ride London 100’ event, a 100-mile cycling sportive on closed roads in Essex, north-east of London. Sally Baker said:

“This is the UK’s biggest cycling event and sees more than 20,000 riders riding 100 miles to raise funds for so many fantastic causes. We were thrilled to have 14 riders riding for us this year”.

Four brilliant women rode in support of a dear friend, Sarah, who was diagnosed with EHE late in 2023. Her brilliant friends were determined to raise money and awareness for EHE on the day. One of them even had to buy her first bike for the event as she’d never really cycled before.



Kelly, whose daughter has EHE, also had great support from her nephew Sam, and several other riders recruited to the cause by Sam. Unfortunately Sam in the end had to take part from his lounge, as he had recently broken his wrist and could not take part in the actual Ride London 100 event. Sam rode a 100 mile virtual route based on a ride in France!



Kelly also recruited several other riders who did a great job and raised significant funding for EHE Research. She wanted to thank Jeremy Adams particularly for his great support.

Kelly’s at the Peckham Show

Fundraising is not just about big organised events. A significant amount of the funding raised by the EHE community comes through the accumulation of many small events and donations. There is no amount of money that is too small to matter, particularly for ultra-rare diseases, so we are always delighted when we see somebody raising funds for us, regardless of the amount.

One champion of our cause is Kelly Denton who every year runs a bottle tombola at the Penge Festival in South London, always supported by her daughter Neve and their dog Max! Here is a photo of Kelly’s stall at the 2024 Peckham Show where she raised an amazing £230. Thank you so much Kelly for another great effort. And please say thank you to Neve and Max too!



Lorraine and Louise are on the march!

Lorraine’s mother was an EHE patient. Like many EHE patient family members, Lorraine and her daughter Louise wanted to help, and so decided to get walking! Sadly, Lorraine’s mother passed away before they could make the walk, but they were even more determined to complete the 96 miles

“Hi everyone, Louise and I set off this morning on our 96-mile trek from Milngavie, Glasgow to Fort William. Raising awareness and money for EHE was something we felt we could and wanted to do to help combat this horrible disease. I know that my wonderful mother would want us to do this, and she would have been cheering us on”.

In describing their walk, Lorraine also explained how they had used technology to help raise funds:

“We wanted to try and collect funds during our walk, as we felt we would have the chance to meet many other walkers and with our Charity t-shirts on, we felt there might be interest in, and an opportunity to talk about, EHE. We also are aware that a lot of people don’t carry cash around these days, so we decided to set up and carry a QR code so that if people asked we could show them the code and link them straight to our fundraising page. And it did work, helping us raise £2,772 for EHE research!”



03 Fundraising and Finance Continued

Judith organises participation at Edinburgh events

Judith Gordon and friends participated in the Edinburgh Shine night in September, and were joined by Lorraine Foulds, as Judith explained:

“Here we are having a ball doing the Edinburgh Shine night, raising funds and attention for EHE and enjoying the clear night and good vibes while doing that”.



Lorraine echoed Judith's comments:

“Our attendance was all instigated by Judith. It was good to catch up with her and hear her good news”.

Then on Saturday 2 November, Judith and David Gordon, and Judith's two best friends, Lynda Harvie and Mary Hughes, all joined the Edinburgh Super Nova 10km run/walk. Judith again wanted to use the event to raise both awareness of EHE and funds for EHE research. Both Judith and David were wearing their EHE bibs in support of the charity, and the four together raised close to £2,000 on the night.



Katy runs the London Marathon

Nicola Henderson was delighted to be able to share news of a special person taking on the London marathon:

“We have EHE representation at the marathon today! My childhood friend Katy. Rooting for her as she has a terrible virus as well poor thing! She's raising funds for EHE and Fertility UK”.



Massive support for Emma

In the Patient Support and Advocacy section of this Annual Report, we reported on how twin sisters, Kym and Emma, had used Emma's story to raise awareness of EHE following Emma's diagnosis with the disease. But raising awareness was not all Kym was determined to do. In addition to all she was doing to help Emma, Kym also set about driving a dynamic fundraising programme, including taking on different challenges herself.

Kym started with the Lancashire Loop Trail Run in late November. Kym ran together with her and Emma's brother, Ben Snape, her running partner Jessica Daggers, and Emma's Auntie-in-law, Ginny. Emma was also pleased that the Charity was able to provide two of their charity running shirts to help spread awareness of EHE.

All four set off on 24 November to complete the event in atrocious conditions, and complete what Kym described as “the hardest thing I have ever done”. Kym cannot thank her fellow runners enough for getting her through the run.



Baring all for EHE research

Emma's family and friends quickly became champions of the EHE cause. So we were not surprised when Emma's step-father, Kevin, posted news of a new and brilliant fund-raising campaign. Kevin explained:

“As a lot of you are now aware, my step-daughter Emma was diagnosed with a very rare form of cancer in July. Her twin sister Kym and Brother Ben (amongst others) have already started to raise money to donate to the EHE Rare Cancer Charity here in the UK to help fund research of, and treatments for, this sarcoma. Well.....to that end we've done 'a thing'!!! A couple of weeks ago a few brave lads bared all and a very kind (if not traumatised) photographer took some pictures and we made a calendar!! We're all very proud of how it's turned out and they are now on sale for £10. If you would like to buy one, please comment below and I'll send you a private message on how to pay etc... thank you. Oh... and please feel free to share the heck out of this post!”

We loved the idea; we loved the 'naked ambition' and commitment; we loved the sunflowers; and we loved the compassion of the 10 lads who did indeed bare all, and in so doing have raised over £200 for EHE research.



03 Fundraising and Finance Continued

Micro pub with mega performance

Following on from their superb EHE calendar, the Albion Ale House micro pub next organised a festive 5km run and walk to help raise funds for EHE research. The take-up and turn-out was again brilliant, helping to raise more than £2,500. Emma and Kym were both there to say thank you and to join the festivities.



Next up was a festive carol singing session at The Albion which Beccy shared:

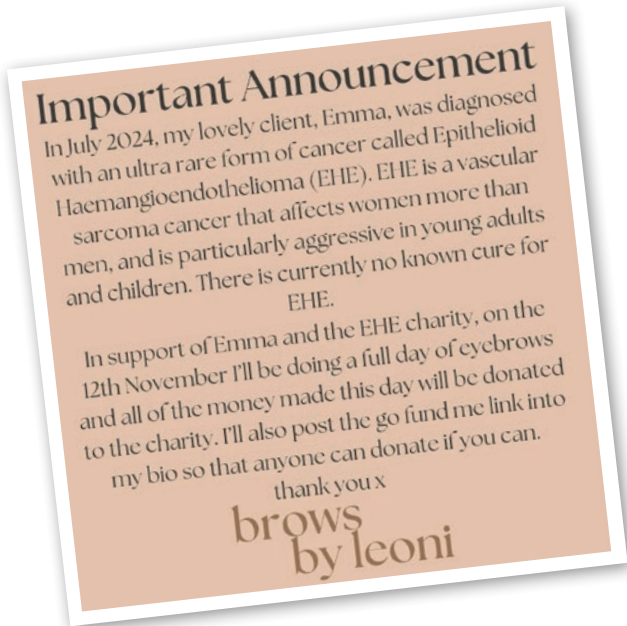
“Good Afternoon and Merry Christmas to you and all your loved ones. This is a little clip of our Christmas Carols Afternoon, which we held for the charity on behalf of Emma and Kym. It was a happy and fun afternoon and we made a nice sum of £1,255 for the charity. Not bad for a little micropub. All the very best to you all. Becs (Emma’s mum-in-law.) Xx”.

Sadly we cannot include the video posted by Beccy here, but we can tell you that the energy and volume of the carols was fantastic. We love the spirit and determination to **Just Live!**



Support from many directions

Emma and Kym’s story caught the attention and compassion of so many people who want to help. Kym shared news of one such supporter, Leoni, who also wanted to do something to help Emma. This young woman who does Kym’s and Emma’s eye brows, held a charity day on 12 November for EHE and raised £380. Emma and Kym were so grateful for her wonderful support.

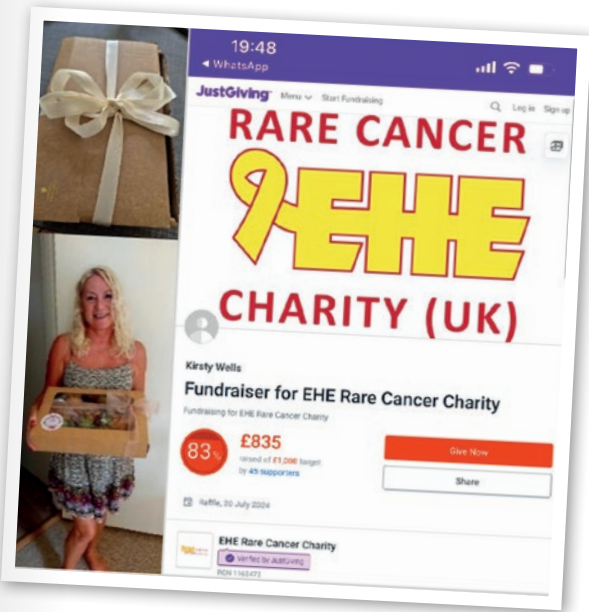


The Payne family raffle

Isolde (Issy) Payne was an EHE patient who lived in the UK. Issy and her two children, James and Kirsty, wanted to help raise funds for EHE research, and came up with the idea of holding an online auction. As is often the case, the family were amazed at the generosity of so many people, both donating the prizes and buying raffle tickets. Issy said:

“We were so grateful that people helped us raise over £800. Prize donations were fabulous and we could not have hoped for more in terms of people buying the raffle tickets. We also got to say thank you personally to those who picked up their prizes, as you can see in this photo”.

Sadly, Issy passed away in early 2025, but in that short period she had seen the amazing love and support for her from so many people.



The Sodbury Slog!

One of the Charity’s most supportive groups, from our inception, has been and continues to be the amazing family and friends of Nicola Henderson. 2024 was no exception with many people again supporting us. Kerry and Darran Marks continued their year-on-year support, not only with Darran running in the London Landmarks Half Marathon at the start of the year, but with both Kerry and Darran taking on the Sodbury Slog, an aptly named run deliberately through muddy fields. It is a slog indeed, but Darran and Kerry took up the challenge with their usual good humour and enthusiasm as we think can be seen in these photos.



Our sincere thanks

The Charity could not be more grateful for the wonderful support received from so many people over the year, as shown by the inspiring stories above. Sally Baker, herself an EHE patient, wanted to register that gratitude on behalf of all the Trustees:

“Once again, I am humbled by these wonderful acts of kindness and support. The stories above of course only capture a small number of the contributions made. There are literally hundreds and hundreds of donations that make up the total sum raised, and it is impossible for us to include them all. We do, however, want to say thank you to every person who organised or participated in an event, or shared details, or made a donation, no matter what size, for having the compassion to support our cause and join the battle to defeat EHE. On behalf of the global patient community, thank you”.

EHE Rare Cancer Charity UK

Financial

Accounts

for 2024

| | |
|--|----|
| Independent Examiner's Report to the Trustees of EHE Rare Cancer Charity (UK)..... | 37 |
| Statement of Financial Activities for the period ended 31 December 2024 | 38 |
| Balance Sheet as at 31 December 2024 | 39 |
| Notes to the Accounts for the period ended 31 December 2024 | 40 |

EHE Rare Cancer Charity (UK)

Independent examiner's report

Independent examiner's report to the trustees of EHE Rare Cancer Charity (UK)

I report to the charity trustees on my examination of the accounts of EHE Rare Cancer Charity (UK) (the Charity) for the year ended 31 December 2024.

Responsibilities and basis of report

As the trustees of the Charity you are responsible for the preparation of the accounts in accordance with the requirements of the Charities Act 2011 ('the Act').

I report in respect of my examination of the Charity's accounts carried out under section 145 of the Act and in carrying out my examination I have followed all applicable Directions given by the Charity Commission under section 145(5)(b) of the Act.

Independent examiner's statement

I have completed my examination. I confirm that no material matters have come to my attention in connection with the examination giving me cause to believe that in any material respect:

- 1 accounting records were not kept in respect of the Charity required by section 130 of the Act; or
- 2 the accounts do not accord with those records; or
- 3 the accounts have not been prepared in accordance with the methods and principles of the Statement of Recommended Practice for accounting and reporting by charities (applicable to charities preparing their accounts in accordance with the Financial Reporting Standard applicable in the UK and Republic of Ireland (FRS 102)).

I have no concerns and have come across no other matters in connection with the examination to which attention should be drawn in this report in order to enable a proper understanding of the accounts to be reached.

(signed) "RJP LLP"

Michael Blay FCCA
RJP LLP
Ground Floor
Egerton House
68 Baker Street
Weybridge
Surrey
KT13 8AL

Date: 30 October 2025

EHE Rare Cancer Charity (UK)

Statement of Financial Activities

for the period ended 31 December 2024

| | Notes | Unrestricted funds £ | Restricted funds £ | Total 2024 £ | Total 2023 £ |
|---------------------------------|-------|-------------------------|-----------------------|--------------------|--------------------|
| Incoming resources: | | | | | |
| Donations | 2 | 117,868 | 15,000 | 132,868 | 73,837 |
| Total incoming resources | | 117,868 | 15,000 | 132,868 | 73,837 |
| Resources expended: | | | | | |
| Costs of generating donations | | 1,931 | 3,150 | 5,081 | 10,468 |
| Charitable activities | | 41,580 | - | 41,580 | 146,463 |
| Governance costs | | - | 1,200 | 1,200 | 1,200 |
| Other administrative costs | | 65 | 5,584 | 5,649 | 14,839 |
| Total resources expended | 3 | 43,576 | 9,934 | 53,510 | 172,970 |
| Net surplus for the year | | 74,292 | 5,066 | 79,358 | (99,133) |
| Transfer between funds | | - | - | - | - |
| Balance brought forward | | 167,735 | 4,021 | 171,756 | 270,889 |
| Funds carried forward | 8 | 242,027 | 9,087 | 251,114 | 171,756 |

Funds carried forward for 2024
£251,114

EHE Rare Cancer Charity (UK)

Balance Sheet

as at 31 December 2024

| | Notes | Unrestricted funds £ | Restricted funds £ | Total 2024 £ | Total 2023 £ |
|---|-------|-------------------------|-----------------------|--------------------|--------------------|
| Current assets | | | | | |
| Debtors | 5,9 | 86,723 | - | 86,723 | 128,246 |
| Cash at bank and in hand | | 211,504 | 11,837 | 223,341 | 201,410 |
| Total current assets | | 298,227 | 11,837 | 310,064 | 329,656 |
| Creditors: amounts falling due within one year | 6,9 | (50,860) | (2,750) | (53,610) | (120,518) |
| Net current assets (liabilities) | | 247,367 | 9,087 | 256,454 | 209,138 |
| Creditors: amounts falling due after one year | | | | | |
| | 7,9 | (5,340) | - | (5,340) | (37,382) |
| Net assets (liabilities) | | 242,027 | 9,087 | 251,114 | 171,756 |
| Funds carried forward | | | | | |
| Unrestricted funds | | 242,027 | - | 243,707 | 167,735 |
| Restricted funds | | - | 9,087 | 7,407 | 4,021 |
| Total funds | 8 | 242,027 | 9,087 | 251,114 | 171,756 |

The Charities Act 2011 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 at the year end and of the surplus or deficit for the year then ended.

In preparing these financial statements, the trustees are required to select suitable accounting policies, and then apply them on a consistent basis, making judgements and estimates that are prudent and reasonable. The trustees must also prepare the financial statements on the going concern basis unless it is inappropriate to presume that the charity will continue in business.

The trustees are responsible for keeping proper accounting records which disclose with reasonable accuracy at any time the financial position of the charity and to enable them to ensure the financial statements comply with the Charities Act 2011. The trustees are also responsible for safeguarding the assets of the charity and hence taking reasonable steps for the prevention and detection of fraud and other irregularities.

These accounts were approved by the trustees committee on 29 October 2025 and signed on its behalf by:

(signed) "Hugh Leonard"

Hugh Leonard
Chairperson

EHE Rare Cancer Charity (UK)

Notes to the Accounts

for the period ended 31 December 2024

1 Accounting Policies

Basis of preparation: The financial statements of the charity, which is a public benefit entity under FRS 102, have been prepared in accordance with the Charities SORP (FRS 102) '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 (FRS 102) (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.

Incoming resources

Recognition of incoming resources: These are included in the Statement of Financial Activities when:

- The charity becomes entitled to the resources;
- The trustees are virtually certain that they will receive the resources; and
- The monetary value can be measured with sufficient reliability.

Deferred income: Where grants are received in advance and specified by the donor as relating to specific accounting periods, these are deferred on an accruals basis to the period to which they relate.

Tax reclaims on donations and gifts: Incoming resources from tax reclaims are included in the Statement of Financial Activities at the same time as the gift to which they relate.

Incoming resources with related expenditure: Where incoming resources have related expenditure, the incoming resources and related expenditure are reported gross in the Statement of Financial Activities.

Volunteer help: The value of any volunteer help is not included in the accounts.

Investment income: Investment income is included in the accounts when receivable.

Expenditure and liabilities

Resources expended are inclusive of VAT where applicable which cannot be recovered.

Liability recognition: Liabilities are recognised as soon as there is a legal or constructive obligation committing the charity to pay out resources.

Costs of charitable activities: A research grant is recognised when the Charity formally notifies the recipient of the award following scientific review. The liability is measured as the total of expected payments for the award. Grant payments that are contingent on a successful outcome of and payable after a future scientific review are disclosed as commitments. Liabilities for awards payable more than one year after the balance sheet date are recorded at the value the Charity expects to settle the grant or award.

Governance costs: These include the costs of preparation and examination of statutory accounts, the costs of any general meetings and the costs of any legal advice to trustees on governance or constitutional matters.

Administrative fund

This fund has been established by the trustees to fund all governance and administrative costs and is funded by a single donor for these restricted purposes.

2 Analysis of incoming resources

Donation income:

| | 2024 £ | 2023 £ |
|---|-----------|-----------|
| Unrestricted funds: | | |
| Personal donations including fundraising events | 116,634 | 50,949 |
| Corporate and Foundation donations | 1,234 | 2,438 |
| | 117,868 | 53,387 |
| Restricted funds: | | |
| Administration fund | 15,000 | 20,450 |
| | 15,000 | 20,450 |

3 Analysis of resources expended

| | Unrestricted funds £ | Restricted funds £ | Total 2024 £ | Total 2023 £ |
|--|----------------------------|--------------------------|--------------------|--------------------|
| Costs of generating donations: | | | | |
| Just giving fees | 560 | - | 560 | 956 |
| Fundraising event entry and other fundraising fees | - | 3,150 | 3,150 | 8,580 |
| Credit card and other processing fees | 1,371 | - | 1,371 | 932 |
| | 1,931 | 3,150 | 5,081 | 10,468 |
| Costs of charitable activities: | | | | |
| University of Manchester PhD Study | - | - | - | 4,314 |
| Royal Marsden Biobank | 18,089 | - | 18,089 | 19,719 |
| Zebrafish Study University of Sheffield | (14,970) | - | (14,970) | 58,097 |
| Fondazione IRCCS and ICR Biomarkers | 13,595 | - | 13,595 | 36,538 |
| Fondazione Prospective Study | 24,866 | - | 24,866 | 27,795 |
| | 41,580 | - | 41,580 | 146,463 |
| Governance costs: | | | | |
| Independent examiners' fee | - | 1,200 | 1,200 | 1,200 |
| | - | 1,200 | 1,200 | 1,200 |
| Other administrative costs: | | | | |
| Design and publishing | - | 4,400 | 4,400 | 4,675 |
| Website maintenance | - | 1,164 | 1,164 | 10,020 |
| Bank fees | 65 | 20 | 85 | 144 |
| | 65 | 5,584 | 5,649 | 14,839 |

Trustees' expenses
£4,269 of expenses were incurred by trustees during the period, primarily associated with fundraising event entry fees and reimbursed by the Charity (2023 – £Nil).

4 Taxation

The Charity is exempt from Corporation Tax on its charitable activities.

EHE Rare Cancer Charity (UK)

Notes to the Accounts (continued)

for the period ended 31 December 2024

| 5 Debtors | Unrestricted funds £ | Restricted funds £ | Total 2024 £ | Total 2023 £ |
|--|-------------------------|-----------------------|--------------------|--------------------|
| Prepayment to Royal Marsden | 25,847 | - | 25,847 | 42,442 |
| Prepayment to Fondazione IRCSS Istituto Nazionale Dei Tumori | 12,395 | - | 12,395 | 52,654 |
| Prepayment to the University of Sheffield | - | - | - | 29,382 |
| Refund due from the University of Sheffield | 44,351 | - | 44,351 | - |
| Other debtors | 4,130 | - | 4,130 | 3,768 |
| | 86,723 | - | 86,723 | 128,246 |

Included within the debtors figures above are amounts of £5,340 (2023 - £37,382) due after more than one year.

| 6 Creditors: amounts falling due within one year | Unrestricted funds £ | Restricted funds £ | Total 2024 £ | Total 2023 £ |
|--|-------------------------|-----------------------|--------------------|--------------------|
| University of Manchester | - | - | - | - |
| Royal Marsden | 26,107 | - | 26,107 | 23,717 |
| Fondazione IRCSS Istituto Nazionale Dei Tumori | 24,753 | - | 24,753 | 51,457 |
| University of Sheffield | - | - | - | 29,382 |
| Accrued independent examiners' | - | 2,400 | 2,400 | 2,400 |
| Other creditors | - | 350 | 350 | 13,562 |
| | 50,860 | 2,750 | 53,610 | 120,518 |

| 7 Creditors: amounts falling due after one year | Unrestricted funds £ | Restricted funds £ | Total 2024 £ | Total 2023 £ |
|---|-------------------------|-----------------------|--------------------|--------------------|
| Fondazione IRCSS Istituto Nazionale Dei Tumori | - | - | - | 12,712 |
| Royal Marsden | 5,340 | - | 5,340 | 24,670 |
| | 5,340 | - | 5,340 | 37,382 |

8 Details of funds

Administrative Fund

This fund has been established by the Trustees to fund all governance and administrative costs and is funded by a single donor for these restricted purposes.

9 Commitments and contingencies

In July 2018, the Charity contracted with the **Bateson Centre at the University of Sheffield** to develop an EHE zebrafish model. In August 2019 and July 2021, a second and third phase of the project was agreed and in July 2022, the Charity contracted with the Bateson Centre to fund an additional one-year, full-time MPhil student to further assess the EHE zebrafish model that the Charity had funded in 2018 through 2021. The July 2022 contract was further extended in July 2023 to provide one additional year of funding to support the ongoing assessment of the EHE zebrafish model. The total cost of the additional year of assessment is £58,763 and commenced in July 2023. In 2024, £29,381 of payments were made and recorded as charitable activity cost (2023 - £58,097). The project completed during 2024 and as all elements of the agreed work program had not been completed, a refund of contributions made of £44,351 was recorded in debtors and as a reduction of expenditures incurred. The net expense recorded was therefore negative £14,970 during 2024.

In March 2021, the Charity agreed to fund costs associated with the establishment and administration of an EHE Biobank and the provision of a Tissue Manager with the **Royal Marsden Cancer Charity** and the **Royal Marsden NHS Foundation Trust** with an estimated total cost of £85,150 over five years commencing in April 2021 (four years commencing April 2022 regarding the Tissue manager). In 2024, £18,255 of payments were made (2023 - £27,567) and £18,089 recorded as charitable activity cost (2023 - £19,719). The amounts committed to for future years have been included within Debtors and Creditors as appropriate.

In June 2022, the Charity agreed to expand the project contracted with the **Fondazione IRCCS Istituto Nazionale Dei Tumori in Italy** and the **Institute of Cancer Research: Royal Cancer Hospital, UK** to look in greater detail of the mRNA analysis and to develop additional PDX models of different variants of EHE. Total costs of the expansion over the two years of the project are estimated at £117,396 with costs funded 50% by the Charity and 50% by the EHE Foundation in the United States. In 2024, payments of £20,760 were made and £13,595 was recorded as charitable activity cost (2023 - payments of £37,401 were made and £29,247 was recorded as charitable activity cost). The remaining amounts payable by the Charity have been included in Debtors and Creditors as appropriate.

In December 2022, the Charity contracted with the **Fondazione IRCCS Istituto Nazionale Dei Tumori** in Italy to fund costs associated with a study aiming to provide a description of the population affected by EHE, giving an insight into the natural history of the disease and its variants, leading to the possible identification of clinical and biochemical prognostic and predictive factors. Total costs of the project are €91,500. In 2024, payments of £17,200 were made and £24,866 was recorded as charitable activity cost (2023 - payments of £35,903 were made and £26,654 was recorded as charitable activity cost). The remaining amounts payable by the Charity have been included in Debtors and Creditors as appropriate..

Trustees' Declaration

As Trustees of the EHE Rare Cancer Charity (UK), the undersigned have fully reviewed the content of this Report of the Trustees and confirm that they each consider it to be a true and fair reflection of the Charity's activities and operations for the year ending 31st December 2024. They each confirm that there are, to the best of their knowledge, no exceptional or special events that have occurred or that should be reported.

The Trustees also confirm that they have undertaken their respective roles and responsibilities with due regard to the public benefit requirements of the charity, and have taken into account the Charity Commission's public benefit guidance when making any decision and producing any reports relating to the Charity's charitable objects and its associated activities.

Signed this 30th day of October, 2025

(signed) "Hugh Leonard"
Hugh Leonard Chair of Trustees

(signed) "Jeff Collins"
Jeff Collins Trustee

(signed) "Kate Hooper"
Kate Hooper Trustee

(signed) "Oliver Pearce"
Oliver Pearce Trustee

(signed) "Sally Baker"
Sally Baker Trustee

Charity Information

Charity Name:
The EHE Rare Cancer Charity (UK),
A Charitable Incorporated Organisation (CIO)

Also known as:
Also known by its acronym, EHERCC

Charity number:
1162472

Web address:
www.ehercc.org.uk

Registered address:
23 Geneva Road, Kingston Upon Thames,
Surrey, KT1 2TW

Charity Trustees:
Mr Hugh Leonard (Chair)
Mr Jeffery Collins
Dr Katharine Hooper
Dr Oliver Pearce
Ms Sally Baker

Established in 2015.
Also working closely with EHE foundations in USA,
Australia, Canada and Italy.
Managed and run by volunteers.
All running costs funded by single donor.
100% of all donations received therefore available
to deliver core objectives.

The Pledge

The Pledge is the quarterly newsletter of the EHE group of foundations. It is produced in London and provides details of the group's worldwide activities in their key areas of advocacy and patient support, research, fundraising, and any other stories of interest. If you would like to be added to the distribution list to receive a copy of **The Pledge** each quarter, please contact the Charity.





EHE Rare Cancer Charity (UK)

23 Geneva Road
Kingston-Upon-Thames
Surrey
KT1 2TW

www.ehercc.org.uk

contactus@ehercc.co.uk

Registered charity: 1162472



A huge thank you to eightcreate.co.uk for the graphics and design work in producing this report and all editions of *The Pledge*



We would like to also thank Bennett Brookes Chartered Accountants for providing bookkeeping services to EHERCC



Printed by Optichrome Limited

Front cover images

Tissue handling at the Royal Marsden
biobank facility.
and
Brilliant calendar campaign raises
EHE research funding.