

Together we will find a cure

BECAUSE

No-one should die of Histiocytosis

Histio UK Strategic Review

Executive summary of strategic recommendations

The overall mission of Histio UK was described in a new statement: *'No-one should die of histiocytosis'*

The specific goals of Histio UK were reformulated into three principles:

- Reduce confusion
- Reduce mortality
- Reduce permanent sequelae

The disbursement of grant funding was considered to comprise two strands:

- 1) strategic activities that specifically promote the goals of Histio UK within short term measurable outcomes;
- 2) research funding given in response to competitive peer-reviewed scientific applications aiming to increase knowledge and bring longer term benefits to patients.

Strategic recommendations to achieve the specific goals

- 1) To pilot a Patient Helpline email
- 2) To provide financial support for the UK Histiocytosis Registry, now open to recruitment. Specifically, to provide 0.2 FTE to a 'Person Designate' legally required by the Human Tissue Authority who will also assist with site opening, telephone consenting, recruitment, and management of the Access Committee.
- 3) To provide financial support for the Specialist Advisory Service for LCH-type histiocytosis. Specifically, to provide 0.2 FTE to an MDT coordinator to prepare cases for discussion, record outcomes and communicate recommendations by letter to referring physicians.
- 4) To provide financial support for an HLH MDT structure. Specifically, to provide 0.2 FTE to an MDT coordinator to prepare cases for discussion, record outcomes and communicate recommendations by letter to referring physicians.

Research funding in response mode

To continue to offer an annual call for proposals for £50,000 grants to fund scientific projects subject to peer review by the Scientific Review Board. The aim is to fund an equal number of projects in LCH-type histiocytosis and HLH, subject to the availability of funds.

Grant Project Funding Award



Histio UK are pleased to announce that the following research and strategy projects have been approved for funding in 2019.

Dr A Vaglio: Genome-Wide and Epigenome-Wide Association study In patients with Erdheim-Chester Disease. £50,000.

Aims of the project: Erdheim-Chester disease (ECD) is a rare histiocytosis characterised by proliferation of blood cells called histiocytes, which infiltrate several organs (such as the bone, the heart, the lungs and the brain) and cause irreversible organ damage. The cause of ECD is incompletely understood. Although mutations of genes controlling cell proliferation contribute to disease development, other factors may be involved.

The susceptibility to develop rare diseases such as ECD is usually related to genetic factors, including gene variants called polymorphisms, together with other inherited variations in the so-called epigenetic regulation of gene expression.

The present study will analyse the whole genome (the whole genetic information contained in the cells) of a large ECD patient cohort and of healthy controls, in order to explore whether gene polymorphisms and epigenetic variants are associated with ECD susceptibility.

The results of this study may clarify the genetic predisposition to ECD, and provide information regarding the mechanisms used by histiocytes to infiltrate and damage the target organs; these findings may ultimately help identify proteins or cellular mechanisms that may be targeted by specific treatments. This approach can also be extended to other histiocytoses such as LCH, whose genetic landscape is poorly investigated.

Prof. J West: Maximising the use of national electronic health data sources in England to ascertain, register and undertake epidemiologic research in Langerhans cell histiocytosis and Haemophagocytic Lymphohistiocytosis. £49,9356.53.

Aims of the project: The Histiocytic diseases are rare but have potentially very serious consequences in affected individuals. Researching them is challenging because of:

- A lack of accurate information on how new cases are recorded, diagnosed and registered across England;
- Incomplete information about the occurrence and natural course of the disease;
- Poor understanding of the reasons why these diseases occur; and
- The difficulty in identifying large enough numbers of cases to meaningfully answer research questions.

Dr C Booth: Evaluation of quercetin as a novel therapeutic in a murine model of XIAP deficiency. £22382.36

Aims of the project: XIAP Deficiency is one of the genetic causes of haemophagocytic lymphohistiocytosis (HLH), which is a serious life-threatening inflammatory sickness. Patients with XIAP deficiency get sick because their immune system causes too much inflammation. Certain immune system cells over-produce IL-18 and IL-1beta, which are inflammatory proteins. These proteins make patients sick with fevers, low blood counts, HLH, and other problems like inflammatory bowel disease. Patients can die from these problems. Current treatments for XIAP deficiency include steroids and other immune suppression, chemotherapy, or bone marrow transplant, all of which pose serious side effects or risk of death. New treatments are needed for patients with XIAP Deficiency that don't have serious side effects or risk of death. We have discovered that quercetin can prevent XIAP deficient cells from making too much IL-18 and IL-1beta. Quercetin is a natural anti-oxidant found in many fruits and vegetables, so it has very few side effects or serious risks. Quercetin may be a good treatment for patients. The aim of this project is to test the ability of quercetin to prevent inflammation and disease in mice with XIAP deficiency to see if a human trial should be done.

Prof. M Collin: UKHR -Registry Research Associate £10,159.73



UK Histiocytosis Registry Opens

The UK network of clinicians with a special interest in histiocytosis received final approval to open the UK Histiocytosis Registry on 14th June 2019. The impetus for a UK Registry originally came from patients and relatives wanting to do something immediate and tangible to raise awareness of histiocytosis and provide data for a range of activities that will ultimately improve lives of patients. Review of the documentation by patients and carers affiliated with Histo UK was a critical step in gaining final approval from the Research Ethics Committee. From the professional perspective, registries are critical to describing the case of need for services and to form the basis of clinical and scientific studies. A website is being built at <https://research.ncl.ac.uk/ukhr/> hosted by Newcastle University. The database itself will be contained in a secure web application used by medical research centres throughout the world, known as REDCap (Research Electronic Data Capture, <https://www.project-redcap.org>). This structure will allow doctors at UK centres to enter data on their patients remotely and for anonymous data to be shared with international histiocytosis registries such as that initiated by the Erdheim Chester Disease Global Alliance. The database will also contain dedicated sections for ECD, LCH and Haemophagocytic Lympho-Histiocytosis (HLH). It is anticipated that the Hospital Across Specialty Collaboration (HASC), will provide specialist guidance for the data to be captured from patients with HLH. Patients who have provided their contact details to Histo UK for 'advance registration' will be formally asked for their consent to join the registry by the end of 2019 by their local doctor or by telephone. All of the documentation will soon be available to read at <https://research.ncl.ac.uk/ukhr/>.



5th Histo UK Conference & Forum

The 5th UK Histiocytosis Forum will be held in November 2019. The meeting will include:

Session I. HLH

- a) Late presentations of primary HLH
- b) UK Hyperferritinaemia audits
- c) The UK HLH Across Specialty Collaboration (HASC) initiative
- d) Case-based discussion

Session II LCH, ECD and the inflammatory myeloid neoplasms

- a) Pathogenesis of inflammatory myeloid neoplasms
- b) ECD in the UK
- c) Keynote address 'Management of histiocytosis in adults'
- d) Case-based discussion

If you would like to attend, please contact the office.



Partnership Projects

Histo UK has teamed with the UK Collaboration Haemophagocytosis across specialty collaboration (HASC) initiative.

In June 2019 over 40 Clinicians drawn from multi-disciplinary backgrounds and from across the UK were hosted in London by Histo UK in discussions on HLH in Adults as well as Case discussion Included in the agenda were:

1. Active cases for joint learning
2. Establishing minimal dataset for investigations
3. Linking MDTs
4. Anakinra use and access
5. Access to bone marrow transplant for rare diseases
6. Mechanics of biobank and registry

Share Your Voice



I know all parents think their child is special, but Layla really was. Always ahead of her time. She was walking at just nine months, out of nappies by her second birthday and at 2 years old, you could pretty much have a full conversation with her. She had something about her. Wise beyond her years almost like she'd been here before. She had a very loving, caring nature and always shown empathy towards others even at such a young age. She was popular at school and loved her friends. She'd put her arm around them and talk to them like she was the grown up and they were the child. She had an old soul for a little girl and often people would say she was like a 'little old woman'.

Layla also loved to sing and dance. She was a great little entertainer, always doing shows for us and she was funny with it too without even trying to be. She oozed confidence and charisma and would definitely stand out in a crowd. You'd always remember our Layla. With her wild ginger curls, infectious smile and her big personality, she stole the hearts of all who knew her. What we didn't realise was, she was only borrowed to us for 6 short years and was needed in a better life. She was needed in Heaven.

On November 24th, 2017, I had a call from school to pick Layla up as she wasn't feeling very well. When I arrived, she was lying down with temperature. I thought a bit of Calpol and cuddles from Mummy would do the trick and had no doubt she'd be back at school after the weekend. Unfortunately, that wasn't the case. By the Sunday evening, she'd become so lethargic and spiked a temperature of 40.9. We also noticed she had come out with an unusual looking rash. We made our way to A&E. After her first blood test, her CRP levels (which detect inflammation in the body) were 300 plus. Which suggested an infection and a very nasty one at that. We thought at that stage we were dealing with Sepsis or Meningitis. She was transferred to Bolton Royal where she was treated for over a week with antibiotics, but Layla wasn't showing any signs of improving. They'd ruled out Sepsis and Meningitis after a series of tests and Layla's illness was becoming more and more of a Mystery. The Doctors at Bolton told us there was nothing more they could do for Layla and the specialist's teams at Manchester Royal Infirmary wanted Layla there. I could see in the doctors and nurses faces that even they were starting to worry and the fact they called us an emergency ambulance to MRI made me think we were dealing with something life-threatening.

The team at Manchester were brilliant and reassured us that she was now in the best care and they would make her better. But Layla's health continued to deteriorate, and she was placed in a high dependency unit while doctors continued to run more tests as they still didn't have a confirmed diagnosis.

On Saturday 9th December, Layla took a turn for the worst and was rushed into intensive care and put into an induced coma. The docs warned us then, she may not survive but against the odds, she stayed strong and continued to fight. Whilst in a coma, the docs took a sample of Layla's bone marrow and shortly after that they were able to diagnose Layla with an extremely rare blood disease called HLH which is short for [hemophagocytic lymphohistiocytosis](#). Of course, we'd never heard of it but as we started to research, it didn't make sense. How could this happen to our Layla? She was singing and dancing at a Little Mix concert only two days before. She was never sick!! she was everything a normal happy healthy little girl should be!

We were told to expect a long road to recovery for Layla. At least an eight-week hospital stay and months of chemotherapy. We prepared ourselves for this and was prepared to do whatever it took to make our girl better. We just wanted her to wake up.

On the 15th Dec, her 6th Birthday, she opened her eyes. We put up balloons by her bed and her brother and sister and grandparents all came to see her. It was a happy moment but also very sad that she was spending her bday in the hospital and so poorly. Not quite the birthday we had promised her. However, the docs were really pleased with Layla's progress and moved her into an oncology ward which would become our home until Layla was well enough to come home.

Unfortunately, the very next day, Layla's health deteriorated quickly and drastically, and she ended up back in Intensive Care and put back in an induced Coma. Layla was bleeding internally, and they sadly couldn't stop the bleed. The machine by this point was the only thing keeping her alive. It was the worst two days of our life. On the Sunday night, while we were sleeping, the hospital called us to tell us that Layla was unstable and basically prepared us for the worst. We called our parents who arrived shortly afterward and we all stood round Layla while they fought through the night to save her. It was not meant to be, she was too poorly. It was time to say goodbye.

Our beautiful daughter passed away at 5.05am on the 18th December and our lives changed forever.

There's a chance Layla may have survived had they diagnosed her sooner. Therefore, I am very passionate about raising money for this charity to help fund the research needed to uncover the causes of HLH, diagnose early and hopefully finding a cure. We hope that no other parent has to go through what we've been through.



Thank you in advance, Michelle, Paul & family and of course Layla xxxxx

Billy's Mammoth Task



As some of you may be aware our beautiful boy Sam was diagnosed 12 months ago with Langerhans cell histiocytosis (LCH) this is a very rare condition that affects around only 50 children per year in the UK. To simplify the condition, it is a mutation of abnormal cells deriving from the bone marrow to skin, lymph nodes and bones. In September of 2017 Sam attended our local Hospital Salford Royal as he was suffering from headaches. After a scan of his skull an abnormality was located, and he was transferred and admitted to Manchester Children's Hospital under the care of the Neurologist team. After further scans and x rays the Neuro surgeon was 99% sure it was LCH. To be 100% sure Sam would have to undergo surgery where a biopsy of the growth on his skull was taken. After a few days anxious wait we were given the diagnosis that it was LCH and that the growth was benign. Sam is expected to make a full recovery but continues to be monitored under the Oncologist team at Manchester Children's Hospital for a further 5 years. For those of you who do not know Sam he is an exceptional sportsman and a caring boy. He is brave, courageous, my

hero and despite the trauma of his diagnosis and the operation continues to live his life to the full. Donning his head guard and enjoying his beloved Rugby. As parents we are so proud of him given what he has had to overcome and to bounce back and excel in his sport and life in general he will always be our little hero. 12 months have now passed and it is only now that we have started to fully understand the condition. And as a result of our research and the rarity of the condition I have decided to embark on a fundraising Journey throughout 2019. The money raised will go to <http://www.histiouk.org/what-is-histiocytosis/> who are helping fund the research into rare conditions.

I will be aiming to cycle and run in multiple events and climb several mountains. Sam will also accompany me on as many events as he can if he can fit it in his busy diary. Thank you for taking time to read this and hope you can donate to this worthy cause.

Thanks Billy

- 1) Mam-Tor 1695ft. 27th December 2018, 2) Mast 10k 20/01/2019 (R) -6 Miles, 3) Manchester Marathon 07/04/2019 (R) 23 miles
- 4) Go Tri Dunham Massey Spring Duathlon run 3 mile run 12 mile cycle 28/04/19, 5) Whitehaven to Tynemouth coast to coast cycle 140 miles, 6) Southport-Hornsea coast to coast cycle 210miles, 7) Eccles to RMCH and return 16 miles 23/12/2019 run.

Awareness & Fundraising **Our thanks to you all!**

Here are just some of the activities you, our Histo Champions have taken part in raising Awareness and Funds for us this year:

(Do not forget to post to your events and activities to our Facebook page).

Team TS Foods – Three Teams of Four run the Belfast Marathon

Mark Knox, Dan McMullan, Catherine Cleland, Jim Warnock, Pádraig Grant, Jayne Moore, Paul Holmes, Aoife McClean, Nigel Sloan, Claire Moore, Ronan Barr, Darragh Ward.



Team Astro Jack – 30 run the Mersey Tunnel 10k

Lee, Rachel, Liam, Leck, Rachel, Steph, Phil, Flick, JP, Jay, Adam, Sam, Jo, Mike, Sean, Kelly, Camilla, Becky, Dave, Bruce, Amelia, Andy, Grandma, Grandad, Dawn, Christine, Lisa, Nanny, Grandad, KDC, Martyn Katarina, Mark, Ross, Carol, Danny, Tom, Chelsea.

On Sunday 14th April, a team of 30 HistoUK fundraisers (and a small army of supporters!) joined forces to run the Mersey Tunnel 10k. The race starts at the mouth of the Kingsway Tunnel which links Liverpool to Wirral. The team met up at the start of the race and had time for a quick team photo. Spirits were high and the team proudly ran in their 'Team Astro Jack Daley' running vests which had been sponsored by The Soccer Dome.

Jack is an 8-year-old, originally from Manchester who was diagnosed with multi-system LCH in 2014. He completed a year of treatment and was disease free until 2018 when a routine scan identified that the disease has reactivated. Jack has been remarkably well on treatment and has inspired his team to raise over £45,000 for The Jack Daley Histo Fund which is a special named fund at HistoUK. Jack and his team hope to raise £50,000 to help with funding the Histo registry.



Sam & Billy Hill	Layla's Summer Ball	Sean's Great South Run
Lions-Wetherby	Romney Marsh Morris Charity	Seahan's House Day Nursery
Isabel - Gwent	Scott of Batley - Because of Blake	Ronan Belfast Marathon
Ismay's going for the chop		Simpson Millar -Leeds
RBS-Thamet Westcliff on Sea	Rachel- Sheffield Half Marathon	Chris - Great North Swim
Isobel – Cardiff Half Marathon	Rachel- Stafford Half Marathon	Ronan - TS Foods Team -Belfast Marathon /gallery/he-was-fading-in-front-of-us-838/
Michael - Link on The Cornish Pirates (RFU Championship team) web site	Layla's Pub Crawl	Rachel, Jamie -Sheffield Half Marathon.
Liam – Liverpool Half Marathon	Rebecca - Stafford	Zoe-Liverpool Half Marathon
Marie-London Marathon 	Team SOBI -Scarfel Pyke	Astro Jack – 38 run the Mersey Tunnel 10k
Anna – Edinburgh Marathon	Rita's – Line Dancing Team	Layla's Team – HMP Manchester
West Wakefield Methodist Church	Neil – Cycling Lands End to John O'Groats	Rachael – Sheffield Half
Tracy's -Tough Mudder 	Yvonne & Family - HLH Campaign	Laura's - Hackney Half
Ian's- Visitors Suppers	Hugo - Daisy's Fund	Charles - Southampton
Stephanie – Belfast City Marathon	Simpson Millar LLP	Olivia's Owletts
Our Match Funders	Alex Heaton Fundraising	McGregor's & DHL Match
In Lieu of Golden Wedding Gifts		Canada Life -Team
Our Corporate Donors	All our anonymous Donors	All our In-Memory Fundraisers
Individual Donors	Our Give As You Earn Donors	Regular Donors

Our thanks go to everyone raising awareness and funds.

Thank You All!

Support Services

contact

support families bring families together and help families take action for others.

We are here for all families, whoever they are and whatever their child's condition, helpline 0808 808 3555 www.contact.org.uk.

CancerCare (LCH Adults) North Lancaster & South Cumbria.

They do a variety of things 1:1 like counselling, reiki, aromatherapy massage, and Alexander technique, and also group things like Tai Chi, jewellery making and other creative stuff!. <https://cancercare.org.uk> 01524 381 820.

MacMillan in your area: <https://www.macmillan.org.uk/in-your-area/choose-location.html>

Carers UK: Offers advice and support for carers, including benefits checks and help with finances. www.carersuk.org 0808 8087777

Together for Short Lives: Charity for children & Young people. www.togetherforshortlives.org.uk 0808 8088100

Family Fund: Grants to parents of children who are disabled or ill. www.familyfund.org.uk 01904 550055

Disability living allowance helpline: 0345 712 3456

Fundraising Ideas & Tools

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We have a great new way to help us raise funds. Go Raise: <https://www.goraise.co.uk/histiocytosis-uk>

Whenever you buy online for yourself, family, friends or colleagues, if you are booking travel and hotels or are looking for business suppliers/services, stationery, office equipment, couriers, insurance hundreds of business suppliers and services will make a free donation to Histiocytosis UK as a thank you, and at no extra cost to you or your company or organisation!

You could help us raise £1000s in extra funding this year, just with your routine or business purchases.

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<https://www.easyfundraising.org.uk/causes/histiocytosis>



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@ "Histio Champions" celebrating patients, families and champions of all Histio Awareness

@ "Histiocytosis UK - LCH" community support

@ "Histiocytosis UK - HLH" - community support



Twitter: @histiouk.



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