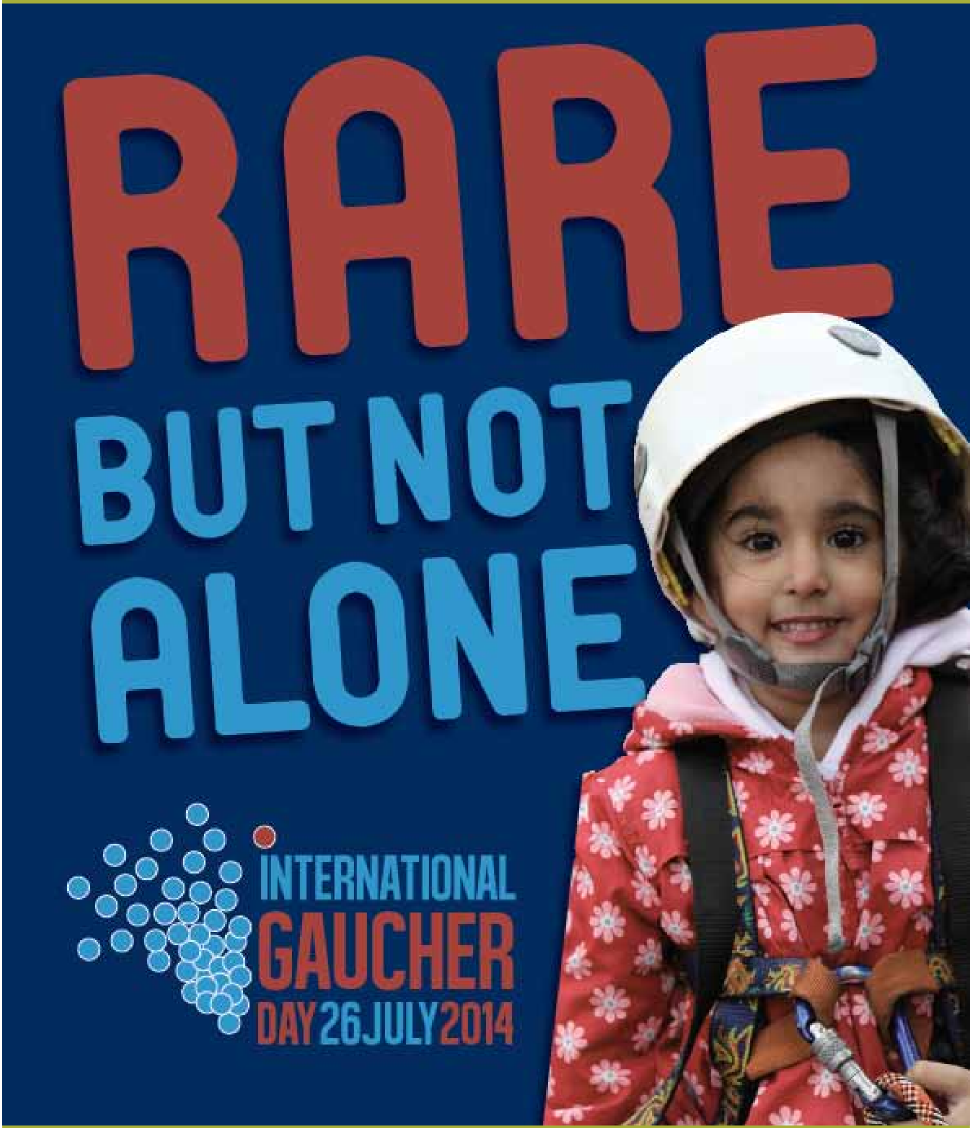
GauchersNEWS

December 2014 **Gauchers ASSOCIATION**



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Chairman's Chat

Dear Friends,

Welcome to the December 2014 edition of the Gauchers News. As usual this edition is packed full of personal stories, updates on the activities of the Association and its members and informative articles on research developments and scientific and medical news not only from the UK but from around the world.

In this edition's Chairman's Chat, I thought I would take some time to make specific mention of the incredible range of fundraising activities undertaken by patients, their families and friends and many others with a connection to the Gaucher community. We have always been incredibly fortunate to benefit from fundraising support from many quarters which provides the lifeblood for the Association's activities.

In this newsletter the fantastic variety of fundraising activities undertaken by our members, their friends and families has never been better illustrated – from bike rides to BBQs, from 10ks, half-marathons and marathons to tea parties and from ballet dancing to head shaving! The efforts people go to in order to raise money for the Association is simply amazing and every penny counts and I'd like to say a huge thank you to all of our fundraisers. There are always great opportunities to get involved with fundraising if you fancy some exercise! Not only do we continue to have Golden Bond places for the London Marathon (places for 2015 are filled but 2016 places are already up for grabs) but we have also now obtained places for the Great North Run (13th September 2015), the Vitality British 10k London Run (12th July 2015) and the Prudential 100 Mile Road London (2nd August 2015). If you are interested in participating in any of these events and raising money for the Association then please get in touch.

A special mention must be made for the Annual Gauchers Association London to Cambridge Bike Ride. Now in its 6th year, the five bike rides to date have raised almost £250,000 for the Association which is simply incredible. A huge vote of thanks must go to the organising committee and especially our very own Sarah Allard who works tirelessly every year to ensure that the day runs without a hitch. If you haven't yet had the opportunity to take part then start training now – next year's ride will be on Sunday 6th September and it's never too early to reserve your place!

Finally I want to make you all aware of a new initiative we are launching to bring together young patients with Gaucher disease. The 'Go with Gaucher' programme which brings together young Gaucher patients from around Europe has been a great success and we are looking to replicate that success with a UK-based programme. We are planning an activity weekend for young Type I and Type III patients between the ages of 16 and 25 which will be a great opportunity to meet other young patients, exchange experiences and ideas and hopefully have a lot of fun. Look out for updates on our web site and Facebook page and contact Helen in the office if you would like further information.

Enjoy the Xmas break and I wish everyone a happy and healthy New Year.

Dan

Personal stories – moving on...

The Gauchers Association are delighted to include these inspiring stories from two of our young members who have made the giant leap to move away from home to attend university and college. Both Maddie and James (and their parents!) tell the tale of their journeys:

Maddie:


Maddie Collin with Tanya Collin-Histed

'I'm Maddie. Living in a small market town has been a challenge for me to gain my independence as I've become a young adult. With limited public transport and not being able to drive, I've watched all my friends learn to drive whilst I rely on others to get around.

In pursuit of my independence and aspirations, I decided to go to college and then on to university to study health and social work and I took the huge step of moving away from my family and friends to live in Manchester at the start of September.

It hasn't been without its challenges and the sheer size of the city and navigating the buses and trams has been scary and at times overwhelming. However with the support of my aunt and her partner (who I live with), I am getting there.

I have however decided that when I choose the university I would like to go to next year, I will chose somewhere smaller. I also intend to take the big step and live in halls. I am excited about my future.

Growing up with Type III Gaucher disease has meant that my future has always been unknown but with the support of my family and my strong character I truly believe that I can reach my full potential and do what I want to do. I want to help people advocate for themselves, to empower them to reach their full potential and to create a life for themselves that's what they want and not what others feel is best for them.'

Maddie's mum Tanya says – 'On the day Maddie left, my heart was broken in two but we are so proud of her. She is a true inspiration and supporting her to achieve her goals, however scary that is, is what a parent should do.

It would have been easy to wrap her up in cotton wool but she is a rounded, articulate, beautiful young woman who can offer so much to the people around her. As such, we have pushed her to go outside of her comfort zone and pursue her dreams. Like all parents in our position, we miss her terribly and worry about her all the time but that's parenting!

We've had tears, but with lots of tough love, cuddles and so much love we are supporting her on her journey because we know that if she doesn't do this we believe she will always say 'what if?'

James:

Hi, my name is James Cox. I've recently moved from my family home in London to the south coast to start a degree in Forensic Computing at Portsmouth University.

Initially, I was worried about such a change because I have my treatment infusions every two weeks. After looking at various options for my medication, especially regarding a safe and clean environment and security for the drugs, I decided the best way is for me to go home by train every fortnight. Thanks to my university timetable I have been able to work my infusions around my studies and the train station is close to my halls of residence.

I'm still able to enjoy student life while also receiving vital medication. I'm having a great time and I'm meeting lots of new people and now I'm settled in, travelling home has just become a routine in my everyday life. It's also worth noting how good it is to be able to take my dirty washing home every two weeks!

James Cox

Mum & dad say – As parents we are glad James has his independence and is able to enjoy life at university and all the experiences it offers. We are still able to do his infusions for him and we miss him loads but before we know it he's back, wrecking his room and singing!

We always include a personal story in each edition of Gauchers News. If you would like to send your story to be included please contact Sarah at sarah@gaucher.org.uk or 01453 549231.

Patient & family support

I reported about my role in the last edition of Gauchers News and I have now been in the role for nine months and it has been extremely varied and busy.

Helen Whitehead

It has been great to meet so many of you on my travels around the country, speaking to you on the phone, via email or on Facebook. Amongst many different types, examples of my support includes helping people with benefits applications, dealing with issues at school and college, liaising with medical teams, helping with CVs and attending hospital appointments.

In June I worked with Genzyme to organise work experience for two young people to spend a week at Genzyme in Oxford (see page 9). It was a successful week and we hope to repeat it in the future. If you might be interested in work experience, please get in touch with me and we can talk through your options.

I attended a Lysosomal Storage Disorder (LSD) day and had the opportunity to speak to clinicians about Gaucher disease and hand out our booklets (the transition booklet ‘Stepping Out’ proved popular!) and it was a great event for raising awareness within the medical community. I also continue to work on the ‘Empowering Young Type III GD Patients to Shape the Future’ project which you can read more about in the update.

In October I attended an advocacy training day , this was an opportunity for advocacy workers and patient and family support workers from charities working with patients with lysosommal Storage Disorders to get together to compare services provided.

Amongst the other things that has kept me busy include attending courses on ‘Benefits and Mental Health’ and ‘Personal Independence Payment (PIP)’ which gave me up-to-date knowledge of benefits so if you have any questions or need help completing forms, please get in touch.

I have been working in collaboration with Dr Annupam Chakrapani, Paediatric Consultant at Great Ormond Street hospital to rewrite our type II Gaucher Disease booklet which will be available soon on our website and in leaflet form. In addition, I am in the process of supporting the rewriting of some of our information sheets, such as travel insurance, life insurance and benefits. Once they’re completed they will be available on our website. If you have any suggestions I would love to hear from you, from topics you would like to see covered and helpful tips for others to insurance companies that you would recommend to others. The only way I know is if you tell me! Lastly, I now have a ‘work only’ Facebook page. Search for Helen Whitehead and you’ll see my profile with the Gauchers Association logo.

You can of course email me at helen@gauchers.org.uk, call me in the office on 01453 549231 or call or text my mobile on 07795 192311. I’ll look forward to hearing from you and seeing how I can help you.

All-Ireland Advocacy Worker

Alison Wilson

The All-Ireland Advocacy & Support Service is a needs-led service that supports patients and their families living with Gaucher disease in both Northern Ireland and Eire.

Alison Wilson is a genetic counsellor based at Belfast Hospital in Northern Ireland and she has been working as an advocacy worker for the MPS Society. She is also working to support patients and families with Gaucher disease. If you live in Ireland and you have unmet support needs you'd like to discuss, please don't hesitate to contact Alison on +44 (0)7786 258336 or +44(0)2895 047779. You can also email her at alison@gaucher.org.uk.

A more detailed update from Alison on her work in Northern Ireland and Eire will be in the next edition of Gaucher News!

Velaglucerase alfa (VPRIV®) approved in Wales

The Association is very pleased to announce that velaglucerase alfa (marketed under the brand name VPRIV® by Shire) has been approved as a recommended treatment for Gaucher disease for use within NHS Wales and will be routinely available in the NHS across Wales by the end of 2014. Prescription will be subject to clinical assessment in conjunction with patients on a case-by-case basis.

The Gauchers Association supported this process by submitting a patient submission paper outlining the value of the treatment to the Gaucher community and we are delighted with the outcome.

Empowering young type III Gaucher patients

Working with our Type III young adult community has been extremely successful, they have worked extremely hard, stepping out of their comfort zones and are becoming great ambassadors for the Gaucher community and improving their confidence and skills, writes Tanya Collin-Histed, Chief Executive:

Patient Ambassador Training

Irma Shah has recently become a Patient Ambassador for Type III Gaucher disease. Here, she writes about her trip to Germany in August:

Irma Shah

‘I travelled to Berlin for two days in August to complete my Patient Ambassador training and it was a great experience. When I arrived I met the other Ambassadors as well as the two companies, Shire and Snow, who came to train us. I really enjoyed the training and working with them and I feel like I built a great relationship with them.

The training consisted of a variety of activities including letter writing, group sessions and one-to-ones so we could get to know each other a little better and buildup our confidence before the second day where I had to present my story. We were divided up into groups and each group was assigned a writer to help us to prepare and edit our stories for the presentation. We worked with our writers for about two hours and then we were sent off to a quiet area to practise. I was very shy and a little scared because I’d never done anything like this before and it was soon my turn to present my story to everyone.

It was a relief to sit back down again but at the same time I felt proud of myself that I had finally presented my story to a small audience which I thought I could never do. This experience was a big achievement for me because not only have I become an Ambassador but it was the first time I had travelled abroad without my family. There is nothing at all that I didn’t enjoy about my experience in Berlin. I loved every moment of it and wish to carry on as a Type III Ambassador’.

My Work Experience at Genzyme Oxford

Gaucher patient Sara Khan writes about her experience doing work experience at Genzyme Therapeutics in Oxford:

Sara Khan & Sarah Pickton

'In June this year I was offered the opportunity to do some work experience for a week at Genzyme in Oxford. I was nervous since I'd never visited Genzyme, nor had I even been to Oxford so it was a big challenge for me!

I had done work experience in places such as the tourist office in Ilkley and in Spain a few years ago based around my college course which was Travel & Tourism, however I hadn't done any work experience in an office-based environment so I was eager to know more.

When I arrived in Oxford, I was greeted by Helen Whitehead, the Gauchers Association Patient and Family Support Worker who had handed me a set timetable of what I could expect in the week ahead.

Over the week I spent at Genzyme, I learnt something new every day chatting to people from all the different departments of the company like Reception, Finance, Treatment Areas and Products and Services. It was very interesting to learn about all the different treatments areas that Genzyme are involved in, where and how the treatments are made and how many countries around the world they are used in.

All the staff at Genzyme were very friendly and helpful towards me. Overall I felt I gained more confidence as the week came to end and I had a fantastic opportunity to see what it's like to work in an office-based environment at a company so close to the Gaucher community.'

If you are a young adult with Type I or III Gaucher disease and interested in work experience please contact Helen Whitehead, the Gauchers Association Patient and Family Support Worker at helen@gaucher.org.uk who is involved with this ever growing project.

Maddie Collin visits Haifa for the EGA Members’ Meeting

In her new role as a board member of the UK Gauchers Association, Maddie Collin visited Haifa in Israel in June 2014 for the 11th European Working Group on Gaucher Disease (EWGGD). She summarises the trip here:

Maddie Collin

The purpose of this meeting was to bring members from the 41 countries that are members of the European Gaucher Alliance (EGA) together to discuss issues, challenges and best practices; build friendships; listen to up-to-date information on therapies and research and review the work that the EGA board have been doing for the past two years.

Tuesday

Anne-Grethe Laurdisen, a Director of the EGA spoke about her involvement with the ‘Go with Gaucher’ project and what ideas had come from the recent meeting of young adults with Gaucher disease that took place in Munich earlier this year. Ideas included a pack for Gaucher patients who want to go travelling that included maps, medical information and information about pre-travel tests and how sharing best practice benefits patients globally.

Tanya Collin-Histed, Chief Executive Officer of the EGA spoke about requests for help to get treatment coming in from the Middle East, India, Pakistan, Zimbabwe and Ukraine. She talked about the challenges that Shire had experienced in early 2013 which had led to a delay in their globally charitable access scheme being launched. Their global programme finally started in 2014 and a total of 20 patients with Gaucher disease, Fabry and MPS II were approved for treatment. Genzyme aren’t accepting any new patients and Pfizer don’t have a humanitarian aid programme (but are willing to work with the EGA). Other presentations included ‘best practice’ and International Gaucher Day.

In the afternoon, we heard from representatives of the individual countries who attended. We discussed the challenges they faced regarding access to treatment as well as new ideas and projects. It was interesting to discover how each country differed and also how advanced some countries were over others (especially across Western Europe), because patient groups have been established for longer and are run by paid staff rather than volunteers.

Wednesday

We travelled to Protalix to see the Tali enzyme being produced. Our guide told us Tali had been tested on pigs and rats that were given a daily dose of the enzyme. There was six months of data into the dosage of the drug and in the last four years Protalix have worked with Pfizer on marketing and distribution outside of Israel. Protalix are looking to get Tali into more counties where it's currently unavailable. During the presentation, the CEO also talked about a clinical trial for an oral enzyme.

After we visited Protalix, we had a group workshop where we discussed important issues, including how to engage better with patients and members, social networking and days out although many of the ideas are dependent on funding.

In the afternoon, Genzyme and Shire told us about their humanitarian projects, current research and areas of interest to the Gaucher community. Genzyme discussed pricing, the production of Cerezyme and the hope that Cerdelga, an oral therapy for Type I adults, will be approved in Europe by the end of 2014. Shire talked about spreading awareness, educating medical professionals, hosting expert summits, working with local physicians and participating at local Gaucher centres as well as sponsorship and support for events and initiatives. Shire was also hoping to open their humanitarian aid programme for new applications in November 2014.

I think it's important for young patients in the UK to know more about where our enzyme treatment comes from and learn more about the drug company's involvement with Gaucher. This could be done by more young people with Gaucher attending key meetings or having an information day. I think we do need more communication between patients in the UK so people feel included and part of the Gaucher group if they want to be. It is important to be there for people even if they don't want to be involved at the moment and I also think there needs to be more activity on the Facebook page set up by the young patients.

The trip to Haifa was thoroughly enjoyable and informative. It was an excellent opportunity which I'm really thankful to have had.

Representatives from the European Gaucher Alliance 41 member countries in Haifa, Israel

Quantifying the Burden of Disease for Patients & Families with nGD

On 10th June, Tanya Collin-Histed, Chief Executive of the Gauchers Association attended a small patient advisory board meeting in Boston, USA hosted by Genzyme Sanofi to discuss the burden of disease for patients and their families with Type III Gaucher disease (GD). She writes:

'There were four other patient representatives at the meeting, from the patient groups representing Gaucher families in the US; Greg and Deborah Macres from the Children's Gaucher Research Fund and Carrie Ostrea and Theresa Sweeney from GT 2/3.

As well as being patient advocates, each of us either had children who had passed away from Type III or had a Type III child living today and were specifically invited to the meeting because of our personal and professional experiences with Type III.

The other attendees were from Genzyme Sanofi's Research & Development team, their Regulatory board and their Patient Advocacy team.

We introduced ourselves and then each of the patient advocates told their personal story and spoke about their work in Type III and the challenges and issues that face the Gaucher community.

As a patient advocate for the UK and in my role as Chief Executive Officer for the European Gaucher Alliance (EGA), I had carried out some research amongst the global Type III community prior to the meeting to ensure that my presentation was representative of the diverse Type III population.

I undertook this through a survey directly to Type III families in the UK and through the EGA membership globally. I would like to thank everyone that fed into this survey as the information was invaluable and highlighted the heterogeneity of Type III patients.

A Potential Therapy for Type III Patients

Genzyme has a small molecule that they are exploring for Type III GD, however it is a very heterogeneous disease even within the same genotype i.e. L444p/L444P. Also there are very severe Type III patients, milder Type II's and then milder Type III patients.

This poses a great challenge to Genzyme. It is important when planning a clinical trial to consider the requirements of the regulators from the onset. In order to get marketing authorisation for a treatment they would need to be able to demonstrate a functionality that improved and had a meaning to the patient. A clinical biomarker would also be used but the FDA and EMA require something that has meaning to the patient and this is the challenge with Type III patients.

Time is also an issue as historically, the length of a clinical trial is very short and with the milder chronic neurological patients an improvement may take a time to capture.

Dr Raffi Schiffmann, originally from National Institute of Health (NIH), now at the Institute of Metabolic Disease at the Baylor Research Institute in Dallas was also at the meeting. He has devised a draft questionnaire for patients and parents to identify the burden of disease. The patient advocacy representatives at the meeting along with clinicians around the world will help shape this questionnaire and then help distribute it throughout the world to as many Type III families as possible. This work will be vital in looking at what endpoints should be in the clinical trial.

At the meeting it was agreed that next year there will be a Type II/III family meeting in the US, the first one ever and during this meeting the families will be asked to complete the questionnaire.

I have been asked and have agreed to help organise this meeting and also to be there to present the work we've done in the UK with the Type III community. I'm also going to bring a few of the Type III girls with me to the meeting which will be a great honour.

I have waited 18 years for this meeting and although there are challenges ahead, this is a very exciting step forward for the entire Gaucher community.'

2015 nGD Family Conference

To be held in Manchester on 13th-15th November 2015.
Watch this space for more details. If you have any queries please contact Sarah at sarah@gaucher.org.uk.

6th nGD family conference 2013

Gene Therapy for Gaucher Disease – Moving in the right direction

Enzyme Replacement Therapy (ERT) has revolutionised the treatment of patients with Type I Gaucher disease (GD) however Types II and III that affect the brain remain a significant challenge with conventional medicine offering no route to therapy. There is clearly an overwhelming need to develop a treatment.

Simon, Giulia & Ahad

Drs. Simon Waddington and Ahad Rahim of University College London (UCL) have been developing gene therapy to treat a mouse model that mimics the devastating brain pathology associated to aggressive Type II GD.

They have been using viruses that are genetically engineered to remove the 'bad bits', rendering them safe, and replacing them with a fully functional and therapeutic glucocerebrosidase (GBA) gene. These viral vectors were injected into the brains of pre-natal or new-born mice where they deliver the therapeutic GBA gene into the cells. A significant increase in the lifespan of the mice was observed together with an amelioration of brain pathology and subsequent improvements in behavioural studies and biochemical markers. This is highly encouraging given the severity of the mouse model – one of the most challenging among neurodegenerative lysosomal storage disorders – and the study is now being prepared for publication.

On the basis of this work, the Gauchers Association and UCL have co-funded an exciting PhD studentship to begin investigating the viral vector administered to the mice via a minimally invasive intravenous route with two fundamental questions requiring answers: Is this minimally invasive route of administration as effective in extending the lifespan of these mice and is the brain rescued from the lethal neurodegeneration? Intriguingly, given the systemic nature of delivery, does this also address the visceral symptoms?

The PhD studentship was awarded to Miss Giulia Massaro, who recently joined Dr. Rahim's laboratory and will be working in close collaboration with Dr. Waddington's group. Giulia began her scientific training in neuroscience as an undergraduate at the University of Padova and postgraduate studies at the University of Trieste where she studied neurodegenerative disorders. The ascendency of gene therapy over recent years means that this studentship is a timely opportunity to develop this technology further for potential application for GD.

Gaucherite Research Study

In May 2013, Professor Timothy Cox of Addenbrooke's Hospital in Cambridge gave a WebEx seminar organised by the Gaucher Association on the reasons why the Gaucherite research study should be conducted.

Kathy Page

We are very pleased to say that the team that was working on securing a grant from the Medical Research Council have been successful in their bid.

The study protocol has now been submitted to Ethics and the teams at all eight specialist Lysosomal Storage Disorder centres around the country are preparing to recruit and accept patients.

Amongst many others, Kathy Page has been working hard in the background with colleagues to progress this study to the point of recruitment and she is the new Lead Research Nurse for the study. She is experienced in working on rare disease studies and she joined Professor Cox's team at Addenbrooke's specifically to work on the Gaucherite study.

Kathy said 'this is an exciting time and a great opportunity to be able to see how we can improve treatment for Gaucher patients'.

She continues, 'To achieve our aims we need to recruit 250 patients. If you are five years or over with a diagnosis of either Type I or Type III Gaucher disease, you may be suitable for the Gaucherite study. Our team will be focusing on the two major areas of disability – skeleton (bone) and nervous tissue (brain) and data will be gathered from your routine clinical tests your doctor has requested and from what you report back to them at your clinic appointments.

It's also our intention to explore the added risk factors and clinical characteristics for Parkinsonism in adults. Some patients who have Gaucher disease may also be carriers of Parkinson's disease or they may develop the condition and by looking more closely at this neurological disability could help us to predict who may develop symptoms and what therapies might be beneficial in their treatment.'

If you are interested in participating in the research study or you'd like more information, please speak to your clinical team at your specialist centre. Alternatively, call Sarah in the office on 01453 549231 and she will give you the information you need.

1st International Gaucher Day

The 1st International Gaucher Day raised almost £1,700!

The first International Gaucher Day (IGD) was held on Saturday 26th July 2014. It is the brainchild of the European Gaucher Alliance (EGA) based on an idea from the participants at 'Go with Gaucher 2012', a seminar for young adult Gaucher patients held in Germany and was celebrated in each of the 41 member countries with a host of special events.

INTERNATIONAL
GAUCHER
DAY 26 JULY 2014
European Gaucher Alliance

The aim of IGD is to raise international awareness of Gaucher disease and the slogan for the day is 'Rare but Not Alone.'

Here in the UK we asked our members to celebrate IGD by organising or hosting a coffee morning at home or at work with friends, family and colleagues. To help our members celebrate this very special day we also sent them a Bag for Life, goodies branded with the new IGD logo, stickers to give to friends and family and a poster to display at home or at work to help us to raise awareness of Gaucher disease.

This is how some of our members celebrated IGD across the UK:

Katrina Yates: 'After the arrival of the goodie bag (a great idea), I planned a fundraising Strawberry Tea. It seemed to be a most positive, enjoyable way to celebrate Phillipe Gaucher's birthday – make the most of the sunshine and raise funds at the same time! It also gave me an opportunity to share information about the impact and treatment of Gaucher disease in an informal manner.

All went well – despite a horrendous storm arriving as the event started, but with support and encouragement of friends we raised £100 for the Gauchers Association. It may be that the Strawberry Tea becomes an annual event!'

Mandy Rose: 'I decided to do something completely different to raise money and awareness for the Gaucher Association so I had my head shaved! I'm 35 and I was diagnosed with Gaucher disease in 2001.

I arranged a gathering of my friends in the motor-biking community and we all rode to Wellington in Telford where the head-shaving took place. It was a great turnout and a fantastic night and while at the time of writing money is still coming in, the total as it stands is £690.

I have never gone public about my Gaucher disease and this seemed like the perfect time to say 'look world, this is what I have, but this is who I am and I will not let this stop me from being me!' I am a Gaucher patient, rare but not alone.'

Our very own **Maddie Collin** hosted a BBQ for her friends and they raised £128.

Mia Bardoe hosted a coffee morning for friends and family and raised £715.

On the night of IGD, Jeremy Manuel, the current EGA Chairman who has served the Gaucher community so passionately for well over 20 years addressed over 40 people at a dinner held at the Shomrei Hadath synagogue in North West London.

He explained Gaucher disease, its prevalence, manifestations and treatment possibilities and spoke about the work of the UK Gauchers Association and the EGA, referencing their tireless work helping patients access treatment around the world. His talk was enthusiastically received by members who were excited that similar activities were taking place that day in our member countries across the globe.

On behalf of everyone at the Association, we would like to say a big 'thank you' to everyone – our members, their friends, guests and colleagues – for their efforts in raising awareness and money, money that will be directed to where it's most needed.

We really do hope this will be an annual event so start thinking about what you'd like to do for the 2nd International Gaucher Day in 2015!

*A gallery of photos showing how IGD was celebrated around the world can be viewed at **www.eurogaucher.org**. We would value your feedback on how to improve this event for 2015 please contact Sarah at sarah@gaucher.org.uk.*

The Susan Lewis Memorial Award

The Susan Lewis Memorial Award was initially set up to provide bursaries to doctors and healthcare professionals from developing countries to travel to the UK Centres of Excellence for mentoring and educational programmes.

Following a review, the programme was extended to support a wider range of initiatives and to encourage the education of doctors in Gaucher disease. Since the last edition of Gauchers News the Association has supported both Dr Ashok Vellodi from Great Ormond Street Hospital in London to travel to Jordan to speak at a patient meeting and attend a Gaucher clinic to meet doctors. We also supported Professor Timothy Cox from Addenbrooke's in Cambridge to travel to Skopje in Macedonia to attend the Macedonia Academy of Sciences and Arts (MASA) Conference on Rare Diseases on 15th November 2014. Dr Ashok Vellodi reports:

Professor Timothy Cox

In June (supported by the Susan Lewis Memorial Fund) I went to Jordan to attend a clinic with Dr Mohammed Antaki of the Jordanian Gaucher Society.

In the clinic I saw three adults and eight children patients. Two of the adults were sisters, one of whom had two affected children. They and the third adult had Type I, the rest had Type III, and only three were on treatment.

- There is free healthcare in Jordan but for various reasons most people attend private clinics.
- There are a total of 34 patients in Jordan, with 14 on ERT and all but one (funded by the King) has treatment provided through Genzyme's International Cerezyme Access Programme (ICAP).
- There is little awareness of Gaucher Disease in Jordan.
- There are no Gaucher specialists, and specialists such as orthopaedic surgeons know very little about the condition, so raising awareness is urgent, as is establishing at least one Gaucher centre.
- Patients have to go overseas to see a specialist and while some go to Israel, it's hard to get a visa since many Jordanians are of Palestinian origin.

- Most patients who attended the clinic had Type III, but no genotyping data was available for any patient.
- Applications for humanitarian programmes are complex and prohibitively expensive, and forms have to be completed by Jordanian doctors who charge high fees. Outpatient appointments are Jordanian Dinar (JOD) 20 (£17.50) and filling in applications costs JOD100 (£87) and according to Genzyme, the quality of the applications is poor. Fresh applications often have to be made incurring further fees and some doctors have even refused to complete them. Additionally further approvals are required from the Health Minister and the King before an application for compassionate treatment can be submitted.


Dr Ashok Vellodi

A central advisory committee has now been created by the government to decide on the criteria by which patients are prioritised. An agreement reached with Genzyme meant that the government will pay for five patients which Genzyme will provide treatment for five more patients on compassionate grounds match.

Some parents were interested and engaged, but there is reluctance to form a support group. There are a number of reasons why this might be but we need to work hard to further awareness and to increase the availability of medication and access to specialists.

The European Gaucher Alliance (EGA) will work with Dr Mohammed Antaki of the Jordanian Gaucher Society over the coming year to support the patients and families in Jordan.

Full details of the funding opportunities within the Susan Lewis Memorial Fund can be found on our website at www.gaucher.org.uk. They are also available as a hard copy or PDF email sarah@gaucher.org.uk

Find us on Facebook

Don't forget to visit and like our Facebook page at ***www.facebook.com/TheGauchersAssociation****. As well as keeping you up to date with news and events we hope to encourage greater awareness of the Association.*

Activity Weekend for Types I & III Young Adults

Following the success of the 'Empowering Young Type III GD Patients to Shape the Future' project, the Association is keen to expand our work with young people and explore how to engage young Type I patients in the work of the Association.

Using the model of the European Gaucher Alliance – Go with Gaucher – we want to take the next generation projects forward in order to encourage young adult Gaucher patients to come together to exchange information and ideas; to help improve the quality of life of the participants; broaden their horizons and also support the work of finding and strengthening the future leaders of the Gaucher Association. Tanya Collin-Histed, Chief Executive writes:

Our plan is to hold a weekend camp to bring together a dozen young people aged 16-25, Types I and III males and females who will be selected by their treatment centres. The programme will be designed by our two young Type III girls who sit on the Association's Management committee with the support of our Patient & Family Support Worker Helen Whitehead and myself.

The aim of this project will be:

- To give young people with GD the opportunity to meet other people with GD, often Type 1 patients have not had this opportunity.
- To listen to their own personal challenges and journeys
- To identify what, if any support they would like from the Association and/or other providers.
- To explore how they could become further engaged in the work of the Association

For further information and to discuss ideas for the weekend please contact Helen Whitehead by phone on 01453 549231 or by email at helen@gauchers.org.uk.

Events coming up in 2015

- **New Families meeting** – we will be in contact with Paediatric centres for this
- **nGD 2015 Family Conference**
- **New patients meeting** – we will be in contact with all UK LSD centres for this

Introducing the Patient Charter

In the June 2013 edition of Gauchers News we outlined that the Lysosomal Storage Disorders (LSD) Centres were to introduce a Patient Charter for all Gaucher patients starting this year.

The Charter is there as a guide for everyone in the Gaucher community, including patients, their families and the LSD centres. The Charter also gives providers of the service important benchmarks that the LSD centres as gatekeepers of NHS funding must meet. Equally, there are crucial benchmarks which much be achieved by patients and carers in return, including attendance at appointments, keeping on track with infusions and managing home care deliveries and fridges.

Everyone knows there are ongoing pressures on the NHS budget and this in turn will mean that together the patients, representatives and doctors and nurses at the LSD centres must work together to protect and shape the LSD service going forward to ensure that we meet patients' needs and at the same ensure that the service is as efficient and effective as possible.

Each year the service is asked to save at least 5% and each year new patients are diagnosed and new treatments for previously untreated patients are being developed, putting yet more pressure on funding. Therefore it is imperative that all stakeholders do everything they can to ensure that the current funding is put to the best use possible and that resources aren't wasted.

The Patient Charter will be discussed with you in clinic by your doctor. Please read the Charter and discuss any concerns you have with your doctor.

If you would like a copy, please call Sarah in the office on 01453 549231 or email sarah@gaucher.org.uk

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__The Gauchers Association__ would like to take this opportunity to wish all our members, families, friends and all our collaborative partners __Seasonal Greetings__ and __best wishes for the New Year__ and to thank everybody for their support throughout 2014. We look forward to an exciting and challenging 2015.

If you Hear Hooves it May be a Zebra...

PAL
AWARDS
Patient Advocacy
Leadership Awards

We are delighted to say that the Students 4 Rare Diseases (www.students4rarediseases.org) project is going from strength to strength! A big 'congratulations' to the team for winning the 'Best Poster' at the International Society for Neonatal Screening (ISNS) Conference in Birmingham in October and the £250 prize will go towards funding further projects.

Also, working in collaboration with the LSD Collaborative, the Students 4 Rare Diseases team have secured funds from Genzyme Sanofi, Shire Pharmaceuticals, Actelion Pharmaceuticals and BioMarin to launch a Rare Disease Society for medical students in Northern Ireland.

Recognising & Supporting Mental Health, Emotional Health and Wellbeing Issues in Young People with LSDs

Following on from the roundtable meeting that took place in March this year with 30 healthcare professionals representing all eight LSD centres in England and Wales, the LSD Collaborative have been awarded a grant from Genzyme Sanofi to take forward one of the meeting outcomes.

The grant will be used to fund a one year project to work with a group of young adults with Type III Gaucher disease and a number of other LSDs patients with Mucopolysaccharide Diseases I, II, IVA and VI to identify the emotional health and wellbeing issues they have experienced. This will be used to work with families of younger patients to overcome stigma and possible reluctance to seek help.

Genzyme's New Type I Oral Therapy

In June's edition of Gauchers News, we reported that Eliglustat (marketed under the brand name Cerdelga™), Genzyme's new oral therapy for Type I Gaucher disease will be the subject of a technical appraisal by the National Institute of Health and Care Excellence's (NICE) new Highly Specialised Technologies Appraisal Committee.

Genzyme has made the decision to put the appraisal on hold whilst they continue to develop their pricing model for Eliglustat in the UK which will ensure that patients are able to be prescribed a treatment based on their clinical needs rather than based on cost. Whilst the Association is disappointed by this decision we recognise that pricing treatments for ultra-orphan drugs is a complex issue and is different in every country and that is it important that Genzyme take the time to get this right before the NICE appraisal can proceed further.

The timeline to restart the NICE appraisal process is not yet known however the Association will liaise closely with Genzyme and NICE and will communicate any updates through our website and Facebook page.

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2015 Golf Day

__The Gauchers Association__ is pleased to announce the return of the ever popular __Golf Day__ to be held at Dyrham Park Golf Club, Galley Lane, Barnet, Hertfordshire on __Wednesday 20th May 2015__. This event is open to everyone with a handicap. Places are limited so please contact Sarah at sarah@gaucher.org.uk or on 01453 549231 if you would like to enter individually or as a team.

This edition of **GauchersNEWS**
has been very kindly sponsored by

Anna Ferguson and the *New Zealand Gauchers Association (NZGA)*

Anna writes: “This donation has come from money I raised through a large musical event for the NZGA.

“Unfortunately, the NZGA closed down a number of years ago because it became very hard to keep it running and to raise significant funds when some Gaucher patients were known to be receiving a higher dosage of Cerezyme than others. When we started, the NZGA had to lobby the New Zealand government to get the drug in the first place as there were so few people in our country with the disease as well as the drug being almost prohibitively expensive.

“I have a son with Type I Gaucher disease but despite his illness, he is New Zealand’s top sports photographer and I am incredibly proud of him. Another patient we knew at the NZGA is now a prominent TV and radio celebrity and one we originally had to fight for to get the drugs (along with only about 14 other patients at the time). There remains a very small number of Gaucher patients in New Zealand in comparison to other countries.

“We have truly appreciated the Gaucher Association sending us their newsletters over the years and it’s great to be kept updated on all research, the information about all the pharmaceutical companies’ efforts and the fantastic fundraising you all do. Thank you.”

The Helen Manuel Foundation

In loving memory of

Helen Manuel

28 May 1924 – 23rd December 1984

2015 Virgin London Marathon

Once again, the Association successfully secured five Golden Bond places for the 2015 Virgin London Marathon on Sunday 26th April 2015.

This is one of our biggest and most important fundraising events of the year and five intrepid souls completed the 34th London Marathon for the Gauchers Association, raising over £9,600 thanks to the generous support of their friends and family as well as our members and we are hoping to exceed that figure next year! Alongside the other 36,000 runners who will take to the streets next spring passing more than three quarters of a million spectators along the route from Blackheath to The Mall will be Claudia Cataldo, Lizzie Strong, Carly Doltis, Professor Atul Mehta and his daughter Avani who will be running for the Association. From all of us here, we wish them the very best of luck with their training over the coming months and please try to support them as much as you can. Here are their stories:


Claudia Contaldo & Lizzie Strong

"Hi, I'm **Claudia Contaldo**, I'm 25 from Northamptonshire and I work in London. I live a fairly active lifestyle and feel that now is a good time to take on such a big challenge."

"I'm **Lizzie Strong**, I'm 28 from Kent and I also work in London. I enjoy running in my spare time both for exercise and to clear my head after a stressful day in the City! We both work together and we realise how fortunate we are to be young and healthy and in a position to give something back to the community. A colleague of ours suffers from Gaucher disease and we thought what better way to show our support than to run the Marathon and raise awareness for such a worthy cause like the Gauchers Association which isn't as well-known or publicised as other charities despite the excellent support it provides to the sufferers and their families."

"We are very excited to be given this opportunity so wish us luck!'


Carly Doltis with her twin girls

Carly Doltis writes: 'I am a 29 year old mother of three year-old twin girls and as you can imagine life is pretty hectic! I absolutely love being a mum but wanted to do something for 'me' that I would feel extremely proud of. My brother has Gaucher disease so I thought what better way to raise money and awareness than attempting to run a marathon! It will be just after I turn 30 so I think it will be a great way to kick off the next stage of my life! Running the London Marathon for the Gauchers Association is a fantastic opportunity to raise awareness of this rare disease and to improve funding for research and treatment.'

Professor Atul Mehta with daughter Avani

Professor Atul Mehta, a founder participant of our annual London to Cambridge Bike Ride is now taking things one step further and will be running the London Marathon with his daughter Avani. They will be raising money for the Gauchers Association in partnership with Myeloma UK, highlighting the extremely important link between these two diseases.

Atul specialises in Gaucher disease and Myeloma at the Royal Free Hospital in London and he knows first-hand the invaluable contribution that the Gauchers Association has made over many years in allowing access to life saving treatment for patients around the world. The Association has forged partnerships with universities and drug companies to fund research and enzyme treatment in developing countries and is embarking on a project with doctors and scientists to develop new treatment approaches independent of drug companies, e.g. stem cell transplantation, which may be much more appropriate in countries such as India. Research into this rare disease is providing valuable insights into common conditions such as Parkinson's disease and blood cancer including Myeloma, both of which are much more prevalent among Gaucher sufferers.

Avani's support and enthusiasm has been the seed around which this project has grown and without which it has no hope of success. She persuaded her dad to run a half-marathon a couple of years ago, and hasn't stopped since. Please support them as they undertake this much greater challenge.

You can help the Gauchers Association to continue to raise awareness of this important and completely treatable condition which is neglected in many parts of the world and you will also help Myeloma UK, a partner organisation in this project, to move us nearer to a cure. Professor Mehta's fundraising page is here: **www.justgiving.com/teams/MehtaMarathon**

We are nothing but forward-thinking and we already have five Golden Bond places for the London Marathon in 2016! If you think you're up for what will be one of the biggest challenges you've ever faced, please call Sarah in the office on 01453 549231 or email sarah@gauchers.org.uk but hurry, they always go very quickly!

The Great North Run 2015 – spaces available

The Association is pleased to announce it has secured 5 Golden Bond places for the 2015 Great North Run on Sunday 13th September. If you are interested in taking part in this iconic event and supporting the work of the Gauchers Association please contact Sarah at sarah@gaucher.org.uk or 01453 549231.

Fifth Annual London to Cambridge a great success!

On September 7th, 100 cyclists set off from University College School in Hampstead north London on route to Addenbrooke's Hospital in Cambridge for the 5th annual London to Cambridge Bike Ride.

The weather was warm and mostly dry and the few raindrops over Berkhampstead quickly passed so that riders arrived to a finisher's medal presented by Don Tendell in Cambridge in warm sunshine.

First three riders over the finish line

The route differed slightly from previous years but this allowed riders more time in the beautiful Cambridge countryside. Although it added two miles to the route, regular riders requested this as a permanent change as the views from the high ridge across the fields were spectacular. The route marshals made sure that the riders followed the correct route and gave enthusiastic support to spur them on.

As always the refreshment stops were a highlight with the various and delicious cakes on offer. Thanks to Sherrards Bakery in Hampstead Garden Suburb and Vivozoom for the croissants, cakes and pastries at the first stop and as ever to the ladies at the fourth stop who broke cake baking records with more than 12 varieties of cake on offer! As always, the Addenbrooke's team ran the last 10 miles from the fourth stop and their enthusiasm on those last miles always spurs on the tired riders for the last leg of the journey. Special thanks to all the marshals and especially to Liz Morris who not only organises the volunteers but is there at the end to check every rider in.

The Association really benefits from the time and goodwill of the volunteers who support the riders along the way. The day would be impossible without the team at the registration desks, the marshals who stay put until the last rider has passed, the hosts at the refreshment stops and the vehicles on the road, moving the refreshments along the route, being ready to find lost riders and to transport riders if necessary. The Association is lucky to have such a large group of enthusiastic and dedicated friends to provide this support.

The professionals involved are also invaluable and this year was no exception. CSS who organise the route, arrange all the signage and provide the sweep vehicle make it look so easy but much planning is required to ensure the day runs smoothly. This year they had to quickly arrange the re-route to get round unexpected road works.

We always hope that the kind and diligent St John's Ambulance crew will see little action but they did have to check out an enthusiastic teenager who took a high speed tumble, fortunately without serious injury.

We again thank Shire and Genzyme as major sponsors and pay tribute to Sarah Allard from the Association who worked tirelessly over the past six months pulling everything together. We receive many compliments from riders about the organisation of the ride and the friendly, almost carnival atmosphere of the day. This is down to the attention to detail Sarah shows to every task.

Finally, a big thanks goes to the riders and their sponsors without whom we wouldn't be achieving our goal of raising funds for the Association to continue its vital work on behalf of Gaucher patients. This newsletter details many of the current activities that the Association carries out on behalf of Gaucher patients in the UK and the impact of this work spreads to patients around the world and other patients suffering from rare conditions. Everybody who participates in the ride, the cyclists and their sponsors, the volunteers, the corporate sponsors and the professionals all are vital partners by enabling the Association to fulfil this vital role.

THANK YOU ALL.

***DIARY DATE** The 6th Annual London to Cambridge Bike Ride will take place on September 6th 2015. Please save the date and join us as a cyclist, a marshal or a volunteer. Please send details to Sarah.*

Vitality British 10k London Run

The Association is pleased to announce that we have secured six places for the Vitality British 10k London Run on Sunday 12th July 2015.

Dubbed 'the world's greatest road race route', the event takes runners through the heart of London passing some of the most iconic landmarks in the world.

Starting on Piccadilly outside The Guards & Calvary Club, runners will pass the new WW2 Bomber Command Memorial which was unveiled by HM The Queen in June 2012 as well as The Ritz, St. James's Palace, Trafalgar Square, St. Paul's, the London Eye, Big Ben and the Houses of Parliament and Westminster Abbey before finishing on Whitehall. Up to 25,000 runners take part every year and all abilities are represented, including experienced runners as well as those attempting a 10k for the very first time!

If you are interested in supporting the Association and you would like to take part in this most iconic of runs, please contact Sarah on 01453 549231 or email sarah@gaucher.org.uk.

The Prudential 100 Mile Ride London


Ashley Young & Alan Rosen

2014 was one of the warmest and driest summers for years and naturally the Prudential 100 Mile Ride London took place on the coldest and wettest day of the year! On August 10th Ashley Young, Stephen Slade and Alan Rosen together with 24,000 other cyclists set off from The Olympic Village in London at 6.30 am to cycle the 100 miles through leafy Surrey before finishing in The Mall, opposite Buckingham Palace.

Alan Rosen writes: It was a real shame that the weather was so awful and the seven and half hours in the saddle did take its toll, including but not limited to aching limbs, very sore bottoms and a thorough soaking!

Even allowing for discomfort we had a great day with a tremendous sense of achievement just to finish (with only 19,000 of our closest friends), knowing we had raised much needed funds for the Gauchers Association. We set minimum targets of £500 each and we raised £2,000 in total.

The ride was amazing and was brilliantly organised. The roads were closed to traffic which made it safe and crowds, braving the cold and wet, were at every corner cheering everyone on. The camaraderie amongst the cyclists was wonderful with everyone helping each other, whether it was to help fix a puncture or just to give moral support. Like the London Marathon there were some unusual sights, including people on tandems or town bikes. Young and old, everyone was determined to enjoy the day. The refreshment stops were every 15 – 20 miles and there were copious amounts of bananas and energy bars which were happily and frequently devoured by us all.

Riding along the Embankment was very exciting and seeing London with its splendid buildings and then turning into The Mall will be a memory that will stay with us for years. As we arrived at Buckingham Palace and received our medals, it filled us with tremendous pride and nicely set us up in preparation for next year's Ride London! Hopefully it will be warmer and less wet!

We are delighted to announce we have three Golden Bond places for the 2015 Prudential 100 Mile Ride London on Sunday 2nd August 2015. If you are interested in taking part and would like to support the Association, please contact Sarah in the office on 01453 549231 or email sarah@gaucher.org.uk.

In Memory of Emma Hall

Lisa Robson and Gareth Farrell are raising money for the Gauchers Association in loving memory of their beautiful baby sister Emma Hall who sadly died of Type II Gaucher disease in 1996 aged just two and a half.

This year Emma would have celebrated her 21st birthday. To raise money, Gareth cycled the 140 mile coast-to-coast route from Whitehaven to Tynemouth and Lisa completed the Great North Run, her first half marathon!

They both prepared and trained hard and their efforts most certainly paid off. They received fantastic support from family and friends and all of us at the Association send huge congratulations and an even bigger ‘thank you’ to Gareth and Lisa for raising well over £2,100.

Happy 21st Emma. You are forever in our thoughts.

Gareth Farrell

Lisa Robson

Member's fundraising

Here at the Association, we rely on our members, their families and their friends who generously support our work through subscriptions, donations and the organising of fundraising events. We are, as always, extremely grateful for this support and would like to remind you that we have various fundraising resources available to help you such as balloons, t-shirts, running vests, thunder sticks, ponchos, stickers, posters, leaflets and a raffle licence.

We would like to take this opportunity to thank all our members who generously donate additional funds with the payment of their annual subscription along with those who pay a regular monthly standing order. If you would like to set up a regular standing order or you'd like information and advice on fundraising and the types of things you can do, please contact Sarah in the office by email at **sarah@gaucher.org.uk** or call **01453 549231**.

Donations received from June 2014 – October 2014 totalled £4,619

Generous donations have been received from:

Jeff Hammerschlag, Mr & Mrs Beecham, Mr & Mrs Regen, Mrs K P Stewart, Pamela Gawthorpe, Robert Sloan, Susan Cowan, E G Ansell, Mrs P M Dawson, Dr A Sekhar, Dr A Gerhard, Mr B Mackinnon, Mrs S F Harris, Mrs J E Fowkes, Mrs N J Hennessy and **S Kriss**.

The Sally Prout School of Dancing kindly donated £420 from their proceeds received from their recent production of The Magic Castle. The pupils thoroughly enjoyed their four days of performances at The Beacon Theatre in Cheltenham.

Mrs Rona Troman very kindly requested donations totalling £585 to celebrate her 60th birthday.

The William Brake Charitable Trust very kindly donated £2,000 towards the neuronopathic fund on the recommendation of Debbie Isaac.

In Memory

John Tempest Funeral Directors kindly arranged a donation of £64 in memory of **Eunice Fitzpatrick**, the late mother of Mrs Susan Cowan.

Donate Online

We rely on your generous support to enable us to continue to meet the needs of those suffering from Gaucher disease and donations can be made online at **www.gaucher.org.uk**. Simply click on the 'Donate' link on the right and you will be directed to our Just Giving page.

Donate By Text

You can also donate to the Gauchers Association by text. Simply text **GAUK01 £2, GAUK01 £5** or **GAUK01 £10** to **70070** NOW!

Thanks so much. Every penny you donate means we can help fund research and do everything we want to do to further our understanding of Gaucher disease.