NPUK NEWS

Summer 2018

IN THIS ISSUE

Annual Family Conference / Research and Clinical Trials / Fundraising News & much more!

www.npuk.org / 0191 415 0693

Contact Us:

Address: Niemann-Pick UK (NPUK), Suite 2, Vermont House, Concord, Washington, Tyne & Wear, United Kingdom, NE37 2SQ

Helpline: 0191 415 0693 / Email: info@npuk.org

@NiemannPickUK @NiemannPickUK @niemannpickuk @NPUK

Contents

Chairman's Chat

Dr William Evans, NPUK Chair

Welcome to our summer newsletter, I hope you will find the articles and updates contained within these pages interesting and informative.

There is much happening within the field of Niemann-Pick, at a national and international level, and as always, NPUK aims to bring you relevant and current information that shows the high level of work and progress across care and support, research and clinical trials.

As I am sure you are aware, new data protection legislation came into force in May 2018, the 'General Data Protection Regulation, or GDPR - means that we need your consent to get in touch with you, or to hold or process information about you. If you would like to stay in touch with NPUK to receive our information or use our services, please ensure you let us know by completing the form on our website or contacting a member of our staff team. You can find more information about GDPR on pages 18-19 of this newsletter and contact details for our staff team on page 51.

We are busy planning our 25th Annual Family Conference and 9th Interactive Workshop on Niemann-Pick disease, which will take place 21st-23rd September 2018 at Wyboston Lakes, Bedfordshire. The event will bring together families, clinicians and researchers from all over the world to hear the latest updates and advances in the Niemann-Pick field, including a broad range of clinical, research and practical topics, and provide much-needed social time to catch up with friends old and new. For a rare disease community such as ours, time together is precious - wherever you are in the world, the issues and challenges of living with Niemann-Pick are shared. Getting together inspires and encourages each of us – whether an affected individual or family, a researcher or clinician, and helps to drive progress.

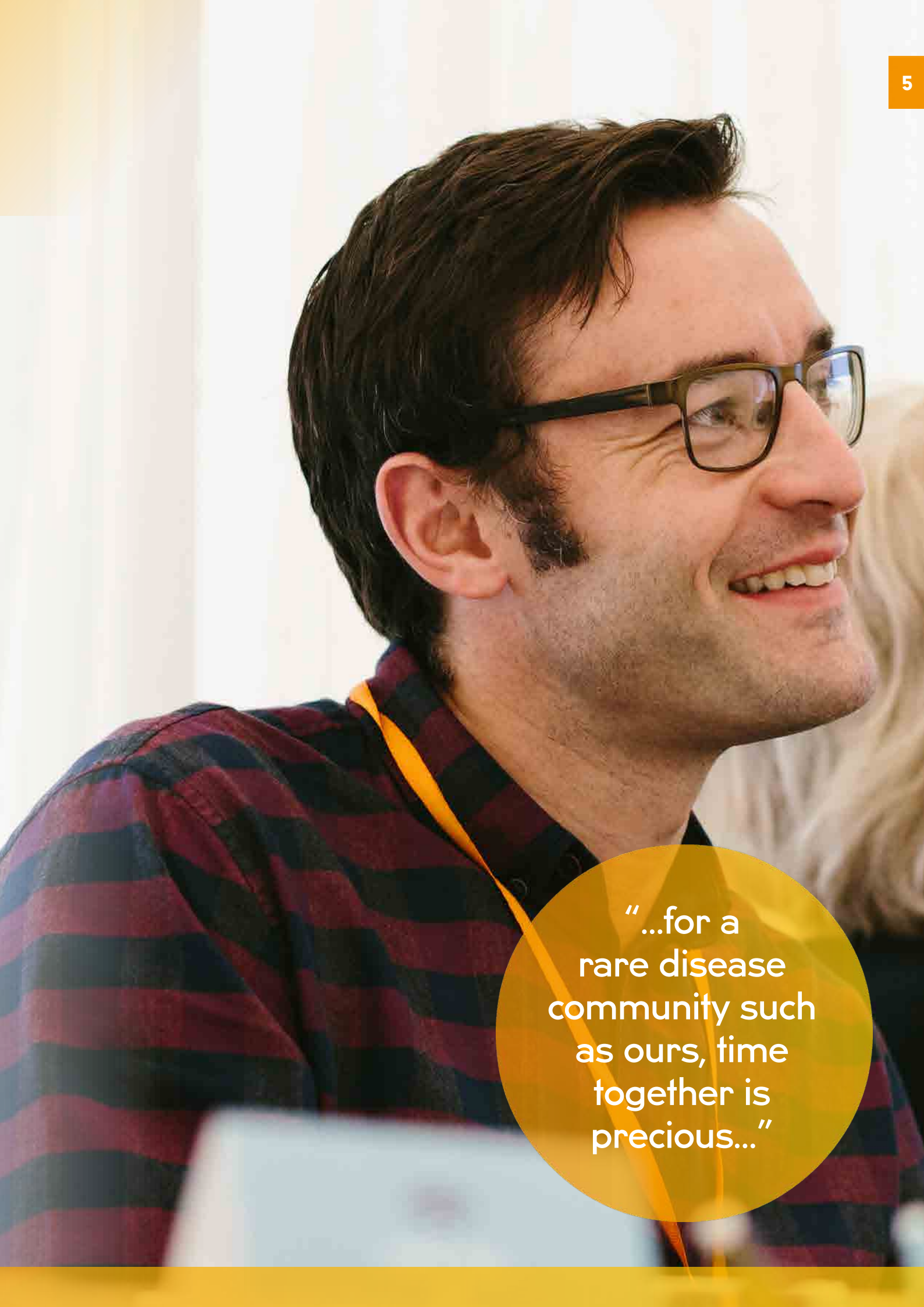
This is an important year for our community, with four active clinical trials in progress and others in the offing. We are carefully watching the outcomes of trials in other similar conditions and following their journey to achieving approval and funding of therapies. Our work here in the UK and on the international stage, means we are well placed to influence and lobby in order to achieve optimum care and access to treatments as they become available.

The Research Report from our Trustee and Research Coordinator, Bill Owen, on pages 6-9, shows just how much activity and interest is taking place around the world to develop therapies and improve care for all those affected by Niemann-Pick disease.

All of NPUK's activities aim to bring tangible benefits for our community, to provide hope and to raise awareness of these diseases, and once again, it is important to recognise the amazing support of those who fundraise on our behalf. On pages 24-31 you can read about some of the wonderful fundraising efforts and events that have taken place recently. Without the support of our families, friends and grant providers, our work and services could not continue. Your support makes a vast and important difference, and is hugely appreciated by us all - thank you.

Warmest wishes,

Will


“...for a rare disease community such as ours, time together is precious...”

Research Report:

Bill Owen, NPUK Trustee & Research Coordinator gives an update on the latest in Niemann-Pick research:

Recent activity

We are well into another year and, as usual, we have high hopes that progress will be made to resolve rare inherited diseases such as the Niemann-Pick, lysosomal storage diseases and other similar inherited conditions that cause so much devastation to families. Our UK charity continues to engage with our research collaborators at universities and medical establishments to monitor research progress and to provide assistance where possible.

In recent weeks we have given grant assistance to the Universities of Oxford and Cardiff in relation to assisting overseas students to undertake studies in the UK and in collating evidence to allow applications for larger grants to be made to the UK organisations that support scientific research.

Clinical trials continue to progress, although it will be some time before formal assessment of trial results can be made, but perhaps many families reading this article will have their opinion on how effective the new medicines are. We can only hope for the best. Should results prove promising then we must overcome other difficulties such as obtaining marketing approval from the EMA for Europe and the FDA for the USA. Further hurdles to jump include the Health Technology Assessment by NICE in the UK, not an easy process and, agreement of the NHS Commissioners that the medicines are affordable within current financial provisions.

As a point of interest we were recently contacted by an Italian scientist working at the University of Istanbul who had just received a grant from the EU to research the delivery of cyclodextrins using nanoparticles that cross the blood/brain barrier. His work will be aimed at NPC in the first instance. Researchers now regularly contact our charity for information and we are happy to help where we are able.

Some issues with treatments

Anyone following this process for other medicines as reported in the press and online media will be acutely aware of the problems being experienced in the pricing of new drugs. A recent report was issued by NICE relating to a therapy for one of the forms of Batten's disease NCL2/CLN2. Although no details can be made available until the report is published, the report is very informative in that it provides an insight into the difficulties posed when trying to assess the balance of efficacy, extension to life, degree of morbidity and cost. We have seen from published evidence on medicines for other diseases that the price per patient per year can be hundreds of thousands of pounds. Treatments that are able to arrest the progress of a disease mean that the drug must continue to be prescribed for decades rather than a few years. Other healthcare costs are incurred over this prolonged period for the provision of ongoing palliative care, and educational needs depending on the level of morbidity that each individual experiences.

Emerging treatments

Treatments now being developed include technologies such as gene and stem cell therapy which may offer more effective


"...we have high hopes that progress will be made to resolve rare inherited diseases..."

solutions to the current drug based approaches, and although these too are likely to have a high price, it may be that only one round of treatment is required. The key factor in their effectiveness however, is that they need to be administered prior to the onset of clinical symptoms or, preferably earlier before damage to cells of organs and tissues takes place.

Restoring the central nervous system (CNS) following damage must be one of the greatest challenges that medical science faces and I suspect that if it is possible, then the timescale for clinical trials will be well into the future. Gene therapy however is a very real proposition and significant advances have been made with a new breed of gene delivery viral vectors that are able to reach the CNS. The problem of achieving diagnosis before CNS damage has occurred remains a major obstacle to whatever treatment is available and affordable.

Prevention

One element of a disease prevention strategy is new born screening (NBS). It may not save the first child detected with a disease/potential disease but it will give parents options such as IVF/PGD when considering having other children. All is not lost for the first child diagnosed however in that the therapeutic options either available or in trial can be administered at a very early stage and probably before clinical symptoms become apparent. The therapy would then have a good chance of success and brain damage may be avoided.

The process of designing NBS testing is highly complex technically and from a management perspective. The main problem is scaling up from laboratory samples of a few hundred to the 700,000 or so babies born each year. Errors will affect many individuals in the form of false positives so the applied test must be reliable. A test is in development for each of the Niemann-Pick diseases both in the UK and in the USA, and if the technical difficulties can be overcome, which it seems they will, then the problem will be to get the test adopted as part of the existing NHS NBS procedure.

(Left to right) Prof. Mark Patterson, Prof. Dan Ory, and Bill Owen provided insight into ongoing research into Niemann-Pick disease(s) at the NPUK Annual Family Conference & Interactive Workshop 2017.

This is not a simple task and we are currently looking at ways that we can obtain the information required in order to answer each of the questions contained in the Public Health England criteria for population screening. NBS is not specifically recognised as being different from screening adults. The headings under which information is required are:-

1. The condition – a description of the disease for which a test is proposed including its natural history.

2. The test – details of the test procedure which will need to conform to the current NHS practice of using dried blood spots (DBS), and meet the time and cost constraints.

3. The intervention – a description of the interventions available to treat the person found to be at risk.

4. The screening programme

5. Implementation criteria

6. References

There is too much to go into in this research article. Further information can be found at - https://www.gov.uk/government/publications/uk-nsc-evidence-review-process/uk-nsc-evidence-review-process, and also on the National Screening Committee (NSC) website.

We are currently gathering information on Items 1, 2 and 3 in collaboration with professionals at UCL and Oxford. We are also seeking views and comments from other colleagues in similar charities to our own that have had experience in NBS programmes.

What is interesting to note is that after many years of trying to get the professional organisations, including big charities, interested in addressing the need to research prevention topics, it now seems that it is becoming an accepted item on many agendas. If the Department of Health/Public

Health England can now pick up this unmet research need then perhaps it may be introduced into the UK Rare Diseases Strategy!

Watch this space for future updates...

If you have any questions for Bill, you can contact him in the following ways:

Email - info@npuk.org
Phone - 0191 415 0693

For information about current clinical trials for Niemann-Pick disease:

Visit www.clinicaltrials.gov, a database of privately and publicly funded clinical studies conducted around the world.

MY STORY...

David A Priestman, Ph.D. is a Senior Research Associate at the University of Oxford, and has been connected with NPUK for many years. This is his story.

When did you first hear about Niemann-Pick disease?

The first time I became aware of Niemann-Pick diseases (NPD) was in the early 1980s when I became a PhD student in the lab of Dr Guy Besley (Department of Pathology, Royal Hospital for Sick Children, Edinburgh). This was in the pre-genetics era and very little was understood about rare inherited diseases at that time. In the lab, we were interested in developing new assays for diagnosing the different types of NP disease. In the late 1970s, Guy had developed a new and sensitive enzyme assay to measure the lysosomal enzyme, sphingomyelinase, in cultured skin fibroblasts from patients.

This was very successful for diagnosing NP-A and NP-B where there are mutations in this enzyme. NP-C was a different story and required new diagnostic procedures when there was a clinical suspicion of NP-C. Marie Vanier, a good friend and collaborator of Guy Besley, had shown that cholesterol metabolism was impaired in a different way in NP-C when compared with NP-A and NP-B. For a short time, before I received my PhD for researching another genetic disorder, I did some research with NP-C patient fibroblasts trying to understand the cholesterol problem better and see if we could develop a new diagnostic assay. However, before I made any significant progress, I graduated and moved to Oxford for a research fellowship working on diabetes.

What is your role within the Niemann-Pick community?

When my Professor retired I had to look for a new position and one came up in Fran Platt's

lab. This was in the early days of the development of miglustat (Zavesca) for substrate reduction therapy, the first small molecule therapy for a lysosomal disease... exciting times. That was twenty years ago and it was very satisfying to return back to clinical biochemistry and genetics and a lab that also had a serious interest in NP-C disease in particular.

I didn't work on NP-C personally until relatively recently, but over the years I followed closely the research projects of a series of truly exceptional doctoral students. Each and every one made significant steps forward in NP-C research in Fran's lab. These include Annie, Emyr, Nada, Ian, Paul, Allie, James, Stephanie, Ecem and Gokhan. In recent times, I've become more and more involved in NP-C projects and supervising project students, both from Oxford and abroad.

What, in your opinion, is the outlook for Niemann-Pick disease research and the potential future benefits for patients?

The current research that I am really most interested in is mostly to do with new therapies. It's still early days, but enzyme replacement therapy for NP-B looks very promising indeed. Now, there's miglustat (Zavesca) for substrate reduction, Arimoclomol, Cyclodextrin, Tanganil, Ursodeoxycholic acid and gene therapy which all show promise pre-clinically for future therapeutic development. We really hope that these and other new treatments will have a significant impact on quality of life for patients.

You're a familiar face at our Annual Family Conference & Interactive Workshop – what do you think makes it so special for families and health professionals alike?

Four years ago, I came to my first NP family conference and was completely overwhelmed by the all the lovely people there. The patients and their families, and all the support and

care workers who are all so dedicated. Last year I was very proud that my elder daughter was able to come as a volunteer and help out at the conference.

This year, I'm hoping that my youngest daughter will also join us as a volunteer. The family conference is a truly moving experience and I have made many lifelong friends amongst you.

Along with the rest of the team from Oxford University you've been involved with a lot of fundraising...what motivated you to take part?

You all inspired us so much that we wanted to give something back and to do a bit of fundraising. The first time, a couple of years ago, we decided to walk all the way from Oxford to Wyboston, that was a blistering challenge!

The next adventure, last year, involved canoeing down the Thames in the coldest, wettest and windiest August weather imaginable. This year, we have decided to cycle to Wyboston which no doubt will be equally challenging and eventful. I look forward to arriving at the conference by bike, probably a little saddle-sore, but raring to get back together with the whole of the NPUK "family" who are our inspiration.

Clinical Nurse Specialist: **Laura Bell**

Hi Everyone,

I hope everyone is doing well and looking forward to some warmer and drier months now that Summer is upon us.

Since I last put pen to paper it has been a very busy few months with lots of travelling far and wide across the UK to visit families in clinics, at home and providing support at meetings in schools, local communities and local health care services.

I have also been very fortunate to travel beyond the UK as well. In February, I had the opportunity of a fully funded place to attend the 14th annual WORLD Symposium held in San Diego, USA. This meeting focusses on Lysosomal Storage Disorders (LSDs), including Niemann-Pick, with leading scientists from around the world selected to present their research findings. This was a very informative event and it was fantastic to meet so many professionals working within the LSD and Niemann Pick field.

In March, I attended the 10th Scientific Symposium on NP-C in the Netherlands, which is sponsored by Actelion. This two-day meeting features a renowned faculty that led attendees through recent developments of the science underlying NP-C, the important advances in identifying, diagnosing and managing the disease and the translation of this knowledge from the laboratory into clinical practice. Again, this was a fantastic opportunity to network with expert professionals and to hear from some excellent speakers working in this field.

Behind the scenes, I continue to work closely with Elizabeth and Steve as part of the NPUK Care & Support team. Together we hope that we provide our patients, families and professionals with the support, information and advice that they require to meet their needs. As many of you know we cover the whole of the UK and we are always happy to travel to wherever is needed to provide this support. There is nowhere that we can't get to!

If you need any help, advice or just a chat I am just at the end of the phone and you are always most welcome to contact me at any time.

As you know I am always happy to hear from you, accompany you to any appointments or meetings or to visit you at home. Please don't forget that you will need to let us know your contact preferences – even if we have been working with you for many years – as data protection rules recently changed. Find out more about this and how to let us know on pages 18-19 of this newsletter.

I am very much looking forward to seeing some of you at our NPUK events happening throughout 2018!

With warm regards,

Laura

laura@npuk.org

07791 499 555

"we hope that we provide our patients, and families with the support they need"

Senior Families Advocate:

Elizabeth Davenport

Hello Everyone,

I can't believe that it is summer already! Thankfully that means that the weather is starting to improve and we can finally look forward to some sunny days! I am sure for many families, this means it is time to think about the summer holidays and spending some time in the outdoors. On pages 44-45 of this newsletter, you can find some helpful links and contacts for organisations that could be of assistance in this area, along with tips and ideas to help your summer go smoothly and ensure your family doesn't miss out on the fun! As ever, I would be happy to answer your questions, help with the application process or to contact the organisations on your behalf.

We are now counting down the days to our 25th Annual Family Conference, which as always, will provide a fantastic opportunity for those affected by Niemann-Pick disease to come together, along with health professionals and researchers working in the field of NPD, to share their thoughts and experiences and to meet with the NPUK staff team. You can read more about this year's Conference on page 20, however if you would like further details or require some assistance in attending or travelling to this event, I may be able to help - just send me an email elizabeth@npuk.org or call 07896 197 576 and I will do all I can.

Please remember I am here to support you with non-clinical advice and guidance, which can include anything from benefits to housing, education to employment and everything in between.

Due to changes in data protection regulation that came into force on May 25th 2018, in order to ensure we can continue to provide you with any support or information you may need, please don't forget to complete the 'How Do You Want to Hear From Us?' form (which can be found on the NPUK website at: http://www.npuk.org/how-do-you-want-to-hear-from-us-gdpr/).

If you have any questions about this, or the ways in which I could help you, please get in touch at any time.

With warm wishes,

Elizabeth

elizabeth@npuk.org

07896 197 576

"I am here to support you with non-clinical advice and guidance"

NPUK PROJECT FAMILIES OFFICER

STEVE NEAL

Our NPUK Project Families Officer, Steve Neal, continues to develop links with individuals and families within the NPUK community as a part of our Big Lottery funded project "Shaping Our Future Together". Steve writes:

"...I've been very busy going up and down the country visiting our lovely families, focusing primarily on emotional health and wellbeing - this is so important and something which I am dedicated to providing.

I've recently attended the 'Breaking Down Barriers Project' workshop along with NPUK Project Team Leader Louise Metcalfe. The workshop was funded by the Sylvia Adams Charitable Trust and led by Alström Syndrome UK. It served as a fantastic opportunity for me to network with other charities that support those with rare diseases, to both share ideas and discuss best practice on how we can more effectively engage and support the BME community.

I'm looking forward to the upcoming NPUK Annual Family Conference & Interactive Workshop 2018, which will take place on the 21st-23rd September at Wyboston Lakes, Bedfordshire. I have so much planned, with a focus on the 'Lads, Dads & Carers' group and other workshops of interest which will deal with anxiety, stress management, and mental health and wellbeing.

As someone who has been part of the NPUK community for many years now, I know better than most how much of an impact NPUK's Care & Support team can make...please know that I am here for YOU along with the rest of the team, no concern is too little or too large. I look forward to seeing you all soon..."

You can contact Steve by email at steve@npuk.org or by phone on 07787 818 885 should you need advice, guidance, or just a friendly ear!

Lads, Dads and Carers

NPUK's 'Lads, Dads & Carers' Group was created by Project Families Officer Steve Neal, and launched at our Annual Family Conference in September 2017. We are happy to report that it continues to grow...

'Lads, Dads & Carers' is primarily a closed group on Facebook, although face to face sessions will be held regularly, including at NPUK events throughout the year. The Group offers a shared space for the men within our NPUK community, encouraging communication and enabling open and honest discussions in an informal and inclusive atmosphere.

The Group provides a source of regular information for members, including NPUK activities and updates, useful articles and helpful guides from other organisations with the aim of encouraging discussion and interaction, addressing concerns and providing motivation and inspiration. In addition, well-respected counsellors Gaz Anderson and Tony Somers, who are also members of the group, kindly share their expert thoughts and information through feature videos and articles.

We hope that those that are already part of the 'Lads, Dads & Carers' Group are finding value in being in touch with others going through similar experiences and having the opportunity to share their thoughts and feelings.

If you would like to join the Lads, Dads & Carers' Group, please don't hesitate to get in touch with our Project Families Officer, Steve Neal (Email: steve@npuk.org / Tel: 07787 818 885) or our Social Media & Communications Officer, John Lee Taggart (Email: john@npuk.org / Tel: 07984 366 334) who will be pleased to help you to get involved!

NEED ADDITIONAL SUPPORT?

There are a number of dedicated charities and support services that are in place to help men who are experiencing difficulties in their everyday lives, whether this be through stress, anxiety, relationships, and anything in between.

For a list of organisations which may be able to help, please visit: https://www.avoiceformen.com/men/mens-issues/uk-charities-that-help-men/

NPUK Project Team Leader:

Louise Metcalfe

Shaping our Future Together

In February 2016, NPUK were awarded a grant from the Big Lottery Fund for £447, 108. This five-year project, called 'Shaping our Future Together' allows us to help the increasing number of people and their families diagnosed with Niemann-Pick disease each year. As well as our practical and clinical work with families, we aim, with this grant, to give more support to newly diagnosed patients and their families, to more members of our BME community, dads, siblings as well as offering various counselling services.

We are already in Year 3 of this five-year project – how time flies! We have had a busy couple of years with more patients being diagnosed and more patients needing the support and help of our staff team. However, we have made great progress towards our project goals and are looking forward to helping even more people as the project progresses.

Through this project, we want to reach out to as many newly diagnosed patients and their families as possible, so they know we are there to support them through difficult times and that there is a community around them ready to help. Last year we worked with 17 new patients and 46 of their family members – welcoming them into the 'Niemann-Pick' family. Feeling isolated after a diagnosis is something we hear from many of our families – being diagnosed with something so rare as Niemann-Pick can be a very lonely experience.

With events like our Annual Family Conference, Family Days in the summer and our Christmas party, we aim to bring Niemann-Pick families together, so they realise that they are not alone and that there is a whole community of people waiting to help. One person who came to our Annual Family Conference for the first time said it 'Opens a door to a world we didn't know existed and reassures us we don't have to deal with it on our own'. Our social media sites and discussion groups are part of this, recognising that our community lives far and wide and it is not always possible to find someone local to you who has Niemann-Pick disease.

Every year, we have a small number of iPads that can be given to adults living with a Niemann-Pick disease and their family members. To date, we have distributed 21 iPads to help patients and they have told us about the many ways in which they are using them – such as to communicate, connect with the NP community, learn more about Niemann-Pick disease or our charity, play games, watch films, listen to music and share memories. If you would like to learn more about the iPad project, please ring me at the Central Office on 0191 415 0693, and I can tell you all about it.

We hope that through our work, patients and families will be more emotionally resilient and better able to manage the impact of disease progression. Some families need regular support from our staff team, and others just dip in and out of our services when they need to. Our goal is for everyone to have a better knowledge of Niemann-Pick disease and be confident in knowing what they are entitled to when asking for health and statutory services.
When we asked people last year, everyone agreed that they felt more confident in dealing with statutory services because of NPUK's support.

We are always there to help you, especially when things are tough and something unexpected happens. Our Senior Families Advocate, Elizabeth Davenport, worked with 67 patients and family members last year alone, helping them navigate the increasingly complex world of statutory services and benefits. A small survey of those supported indicated that the majority felt more informed about statutory services and entitlements because of our help. As one family member pointed out, 'You don't know what you need until you need it' and we are always there to help you.

We provide regular information on treatments, clinical trials and research in our newsletters, on our website and at our Annual Family Conference as well as updating our publications and videos on the various types of Niemann-Pick diseases to try and improve families' knowledge of the disease. We asked you if the information we provided as a charity helped improve your knowledge about Niemann-Pick disease and we were thrilled that 89% of you agreed that it did!

Our staff team try to attend clinic days to meet new and existing patients and help them understand and manage their condition. For example, Laura Bell, our Clinical Nurse Specialist attended 51 Clinic Days last year, made 59 home visits, attended 77 Multi-Disciplinary Meetings and took part in 19 Niemann-Pick Disease awareness events!

If anyone would like to learn more about any aspect of the 'Shaping our Future Together', please do give contact me at the Central Office or email me at louise@npuk.org.

Louise

louise@npuk.org
0191 4150693

BREAKING DOWN BARRIERS

GDPR • what you • need to know

In May 2018, data protection law changed. The General Data Protection Regulation (GDPR) replaced the Data Protection Act (1998) offering greater protection for individuals' personal data in an increasingly connected world.

How does this affect me?

In recent months, we have been working to update our processes and practice to ensure that we comply with the new regulations.

Going forward, we will only contact you in the ways you prefer, making our communications more effective and ensuring you receive the information you want, in the way you want to receive it.

The new regulation requires us to ask for your consent before we can continue providing our information and services, even if you have supported us for many years,

If you would like to continue to use our services, receive our updates and information about research and clinical trials, our events, details of fundraising activities or other items relevant to our work, please ensure you let us know by updating your contact preferences.

How do I update my contact preferences?

You can choose how you would like to interact with us, use our services or receive information on the subjects important to you. You can do this in the following ways:

• Complete our short online form, visit www.npuk.org/how-do-you-want-to-hear-from-us-gdpr

• We can send you a form by post, simply contact us by email info@npuk.org or call 0191 415 0693 to arrange this.

• Call us on 0191 415 0693 and we can assist you in updating your preferences.

• Write to us at: NPUK, Suite 2 Vermont House, Washington, NE37 2SQ.

What if I change my mind or I do not want to hear from NPUK anymore?

If you change your mind about your choices, or you wish to unsubscribe completely, you can update your preferences at any time by contacting us:

- Call us: 0191 415 0693
- Email us info@npuk.org
- Write to us: NPUK, Suite 2 Vermont House, Washington, NE37 2SQ

Your details are safe with us

We take our obligations under privacy and data protection law very seriously. We will always store your personal details securely. We will only use them to provide the service(s) that you have requested and communicate with you in the way(s) you have agreed to. Your details may also be used for analysis purposes, to help us provide the best possible service. We will not pass on your details to anyone else and we will only share them if required to do so by law.

Your rights with respect to your persona information

You are entitled at any time to ask us for a copy of the personal information we hold about you, this is known as 'a data subject access request'. You are also entitled to ask that any information we hold about you is updated, removed or restricted. Please note that if you ask for your data to be removed or restricted, this may affect the ways in which we can provide services or information to you/and or your family. We will aim to respond to your request within one month.

For further information about your rights, including the circumstances in which they apply, please see the Guidance from the UK Information Commissioner's Office (ICO) on individuals' rights under the General Data Protection Regulation: www.ico.org.uk.

To view our policies and procedures relating to data privacy, please visit the NPUK website at www.npuk.org

NPUK ANNUAL FAMILY CONFERENCE & INTERACTIVE WORKSHOP ON NIEMANN-PICK DISEASES

Our Annual Family Conference and Interactive Workshop on Niemann-Pick Diseases bring together affected individuals, families, health and research professionals under one roof.

This year we will return to Wyboston Lakes, Bedfordshire, over the weekend of 21st-23rd September 2018, to celebrate our 25th Conference! Wyboston Lakes is one of our favourite locations, with many memories made over the years, and we look forward to welcoming friends old and new, those newly diagnosed, living with Niemann-Pick or those that have experienced bereavement.

The Conference will once again offer the opportunity to hear the latest developments regarding therapies and clinical trials for ASMD Niemann-Pick disease types A and B, and Niemann-Pick disease type C, plus related care issues, breakout sessions and workshops.

The Interactive Workshop on Niemann-Pick Diseases, an event for professional delegates only, will take place on Friday 21st September.

Hosted by Professor Fran Platt, University of Oxford, the meeting will feature clinical and scientific presentations and workshops on relevant topics, including clinical management, research and potential therapies. This special weekend is about sharing information, meeting new people and getting together with friends.

The weekend offers a unique opportunity for individuals and families affected by Niemann-Pick disease from across the UK and further afield, to share their thoughts and experiences and meet professionals working in the field. There will be many opportunities for discussion and questions with those attending, including family and professional delegates and speakers,

during the presentation sessions and through lunch, coffee, and social time. Suddenly this rare disease does not seem so rare, and the isolation so often experienced by those living with Niemann-Pick disease is lessened.

Our Children and Young Person's Activity Programme, which runs alongside the main conference programme, offers a jam-packed agenda for the younger members of our community, allowing the adults to listen to presentations, take part in discussions and catch up with friends. This year our younger delegates will enjoy a fun-packed trip to Wicksteed Park www.wicksteedpark.org which has 30 exciting rides and activities for all ages and abilities, as well as on-site activities and entertainment, including a star performance by Party Band Makapaka,!

Booking forms are now available - as ever, places for the NPUK Annual Family Conference & Interactive Workshop 2018 are limited and tend to fill up quickly! For further information on this event, or to book, please visit our website at www.npuk.org.

If you are thinking of joining us, perhaps for the first time, or you have any questions at all, please get in touch with a member of our staff team (details on page 51) or contact our Central Office by email info@npuk.org or call 0191 415 06963.

Limited funds are available to support those who may need assistance to attend this event. For further information, please contact a member of the NPUK Staff Team; all applications will be treated in confidence.

Everyone at NPUK looks forward to seeing you there!

"...the event is a big hug to parents and family. My family and I feel totally welcome..."

"...the Conference is fantastic - far better than I could have ever imagined..."

"...attending the Annual Conference made an incredible difference...."

OUR 25th ANNUAL FAMILY CONFERENCE

#ForYou #ForTheFuture

This September marks our 25th Annual Family Conference, a milestone worthy of celebration!

NPUK has come a long way since its formation back in 1991 and the first family meeting in 1993 – a satellite seminar hosted by the dedicated Professor Marie Vanier, whose involvement continues and is appreciated, to this day.

This event helped to underpin relationships between affected families and those working to progress clinical care, research and therapies for Niemann-Pick diseases. Over the years the event has gone from strength to strength, helping to create the sense of 'community' and 'family' we have today. The atmosphere is informal, the subject matter at times difficult to understand or listen to, but everyone in attendance has a shared goal – to make a difference for those affected. This may be through research, by providing care and support or simply by being there as a friend.

Many families and professionals have been involved since those early days and continue to join us year after year. We are grateful for the on-going support of Professor Marc Patterson, who has only missed one of our events in 25 years! Along with Professor's Patterson and Vanier, Professor Frances Platt has also played a key role in our development and continues her invaluable support today. Sadly, we have suffered many losses within our community over this time, but it is heart-warming to see families joining us year after year, providing inspiration and support to others on this journey.

As we look back on 25 years of our Annual Family Conference, we remember those that are no longer with us, who will forever remain in our hearts, we consider the positive steps made in this time and the progress yet to be made.

Now we are looking to the future, and to the achievements and challenges yet to come. We have great hope for the next 25 years, in the knowledge that we are in this together, and that we are stronger, together.

In the 27 years since the charity formed, there have been many pivotal moments that have contributed to the development of NPUK, including:

- The creation of the 'RTMDC Niemann-Pick Disease Group' in 1991 – formed by parents Jim and Susan Green, who at the time were told they were the only family in the country living with this rare disease.
- Our first family event in 1993.
- The creation of a Clinical Centre of Excellence, led by Dr. Ed Wraith, in 1995.
- Being granted charitable status as an independent charity in 1997.
- Employing our first dedicated Clinical Nurse Specialist, Jackie Imrie, in 1999.
- Our first awareness campaign in 2000, which reached out to hospital trusts across the UK.
- Establishing the International Niemann-Pick Disease Alliance (INPDA) in 2009, a global

(Left to right) NPUK Trustee Joella Melville, NPUK Clinical Nurse Specialist Laura Bell, and The Hollie Foundation founder and NPUK Trustee Helen Carter help support the launch of our Instagram account at 2017's Conference.

network of non-profit patient support groups. in partnership with the US based group, NNPDF.

- Organising the first Interactive Work-shop for Niemann-Pick Diseases, hosted by Professor Frances Platt in 2010.
- Creating a base for our activities in 2011 – The NPUK Central Office.
- Employing our first Families Officer, Elizabeth Davenport, in 2013.
- Embarking on an EU funded project in 2014, to create the International Niemann-Pick Disease Registry (INPDR) with 7 partners and 14 associate partners worldwide.
- Revealing a new look in 2016, to mark our 25th birthday and strengthening our ability to communicate and share information through our website, social media and wider networks.

For further information on NPUK's history, please visit: www.npuk.org.

Fundraising FOCUS

Our fundraisers are central to everything that we do here at NPUK, as without voluntary donations and grants we would not be able to continue to support individuals and families affected by Niemann-Pick disease.

Apex Pro Wrestling Event

To say that our fundraisers are bigger and better than most has never been more true than at the Apex Pro Wrestling event (pictured) held in February 2018. The event was held in honour of Alfie Burns (NP-B) and Joshua Cullip (NP-C), (pictured). Joshua's mum, Jodie, commented:

"Two very inspirational boys sharing a very rare incurable disease, Alfie (left) has Niemann-Pick type B that affects his respiratory system with an enlarged spleen - Alfie kicks type B's butt but does get tired easily. Joshua (right) has Niemann-Pick type C that affects his brain.

These two young boys share a similar genetic condition and share a passion for wrestling, [so to have Apex Pro Wrestling help] us make their day means a million times more than the amount of money raised. We cannot thank you enough and cannot thank all the supporters who kindly donated to our popcorn, sweets , raffle and tombola. This picture speaks a 1, 000 words on what it meant to the boys. Huge thanks from myself and The Burns (Alfie's family)."

Memorial Charity Concert

A charity concert was held in memory of Juliet Corbett (22/10/70-26/08/17, NP-C) at St. George's Church in Brighton earlier this year, in support of NPUK. The evening featured internationally acclaimed award winning musicians, Guy Johnston (Cello) and Tom Poster (Piano), playing music by Rachmaninov, Smyth, and Grieg.

The fantastic duo have a long history with NPUK; Guy has supported our charity since 2000 as a patron, and Tom has kindly given his time and talent performing in many concerts at St. George's Church in support of NPUK. The event was organised by Juliet's father, former NPUK Trustee Tony Jellings...upon hearing of the death of Tony's daughter, Juliet (22/10/70 - 26/8/17), NP-C), both Guy and

BECOME AN NPUK VOLUNTEER

Want to become part of the amazing NPUK volunteer team? As part of the dedicated group you could:

- Make a difference to those affected by Niemann-Pick
- Help raise vital funds and awareness
- Make new friends
- Gain new skills
- Have fun!

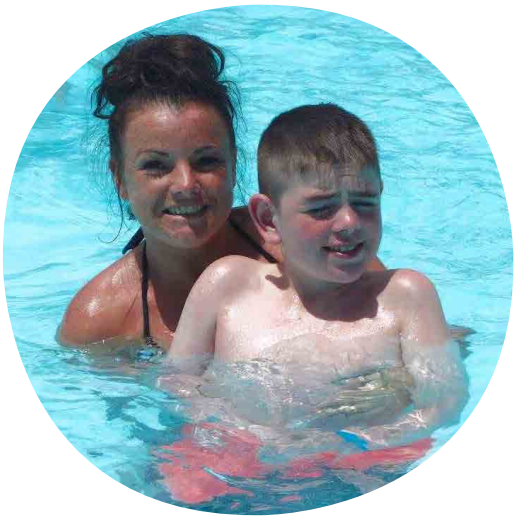
Make 2018 your year by getting involved: email us at info@npuk.org for further details

Alfie Burns (NP-B) and Joshua Cullip (NP-C) having fun with the APEX Wrestling superstars at their fundraiser in February

Tom immediately offered to take part in this memorial concert.

Following this successful event Tony commented: "I am very fortunate in the support I receive from my wife, family and many good friends. Juliet was a very special person and much loved by all who knew her".

We thank all of those who were in attendance, for joining together in memory of Juliet and supporting Niemann-Pick UK.



A smashing donation!

Many thanks to David Darsey, President of The Institute of Demolition Engineers (IDE), along with their members, for the hugely generous donation of £10,000, resulting from fundraising activities the Institute has undertaken in support of NPUK.

NPUK Chief Executive, Toni Mathieson, (pictured, left, alongside David Darsey) was invited to receive the cheque at the IDE Annual Meeting, held at the Royal Armouries, Leeds, where she had the opportunity to thank attendees in person. The funds raised will go a long way to support our work with affected families and our efforts to facilitate much-needed research – huge thanks .for helping us to continue making a difference for those affected by Niemann-Pick disease, and their families.

Sober for October

Paula O'Hara, Aunt of Taylor Smith (13, NP-C) (pictured) and her friend Joanne Darby went 'Sober for October' in a bid to raise funds for Niemann-Pick UK...in the process they managed to raise an absolutely staggering £920!

Thank you to the kind people of Barrow-in-Furness for your donations, and of course thanks to the amazing fundraising duo - you're both absolute inspirations.

With thanks to Morrison Construction

After being inspired by Shona Beveridge's participation in BBC The One Show's Rickshaw Challenge for BBC Children In Need, the wonderful people at Morrison Construction donated more than £1,000 in support of Niemann-Pick UK.

Morrison Construction Environmental Sustainability Manager and mum to Shona, Helen Beveridge, commented: "I am proud of my daughter Shona, of her achievements and of the inspiration she generates with her positivity for life in spite of her disability. The people at Morrison Construction have been incredibly generous and my family are grateful for their support and fundraising for Niemann-Pick UK, a charity which helps us in many ways to cope with this rare and fatal disease."

It was last November that 17-year-old Shona (NP-C) took part in the BBC Children in Need Ride to the Clyde Rickshaw Challenge from London to Glasgow, raising more than £5 million for the charity, which has generously supported NPUK's work, via grant funding, since 1999.

Go Lynne!

Lynne Valentine completed her first ever 5K back in March, and managed to reach her impressive target of a lofty £500...truly amazing!

Lynne was inspired to take part in this event due to her deep connection to Niemann-Pick UK, which starts with her family. When speaking of her reasons for running, Lynne commented: "NPUK is a charity that is close to my heart. They not only help and support Matthew, but are always there when the family need help and support".

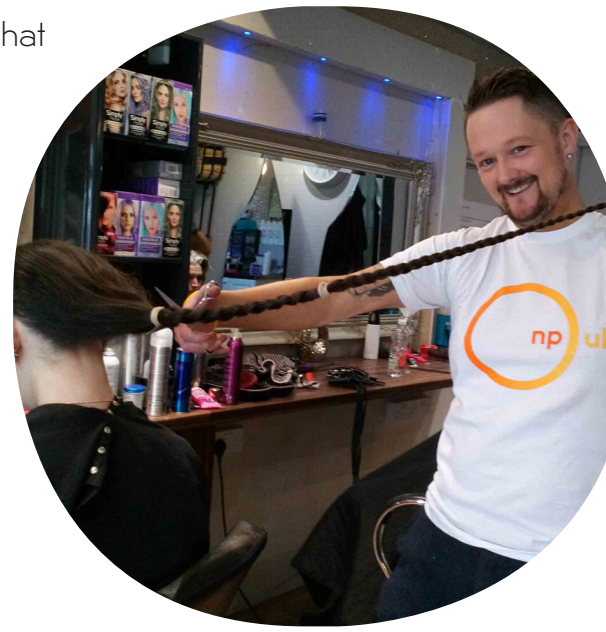
Lynne and her family also raised funds this year by giving up sweets during the Lent period up until Easter, and donated the amount that would have been spent directly to NPUK. What a fabulous challenge!

Charlie Don't Surf

Huge thank you to the fantastic Charlie Don't Surf, whose fundraising benefit gig (organised by long-standing NPUK volunteer Graham North) raised over £500 for Niemann-Pick UK!

We are also happy to announce that on Friday 14th September there will be another gig at the Lightning Club Warton. For further updates follow us on Facebook @NiemannPickUK.

The Big Chop



The hugely dedicated Caitlin Reid, whose hair once rivalled that of Rapunzel herself took part in "The BIG Chop" earlier this year, cutting off around 30 inches of her locks in support of two charities that are close to her heart - one of which, was Niemann-Pick UK (NPUK).

Speaking on her connection with NPUK in the build up to the event, Caitlin commented:

"...I'm taking part as, unfortunately, my cousin was diagnosed with Niemann-Pick a few years ago. Since his diagnosis our family has received so much support from the charity, and I'd love to give back so you're able to continue helping my cousin and other people like him; it's such an unknown disease and your work deserves so much support..."

The event was a HUGE success, with the grand total currently at a staggering £1,227.25 (including Gift Aid), this goes to show that NPUK fundraisers are some of the best in the world as they always go above and beyond!

Sisters Sponsored Haircut

Super sisters Gemma and Tanya Dickens also took part in a sponsored haircut in support of Niemann-Pick UK. In the build up to the event, Tanya detailed why they had been inspired to take part in this fundraiser:

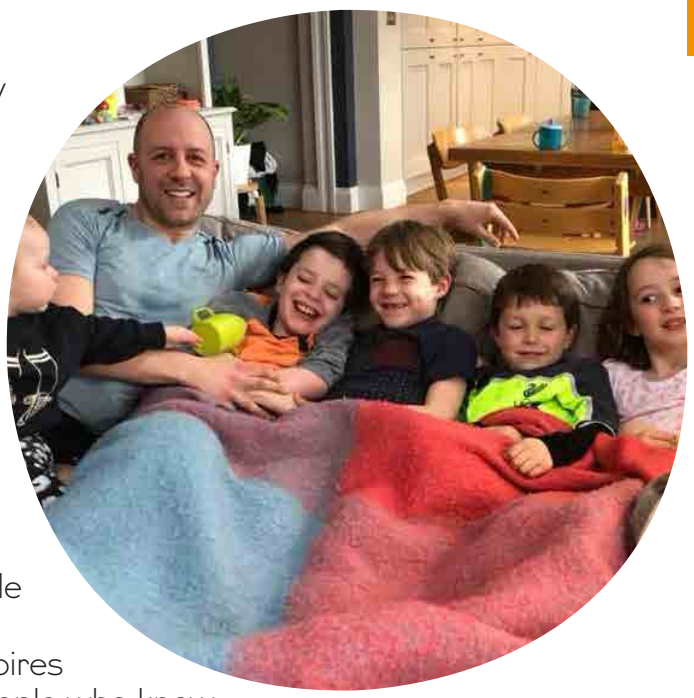
"...My sister and I will both be having a minimum of 7 inches (17cms) cut from our hair so we can donate this to 'The Little Princess Trust'. All donations will go to a charity close to our hearts - Niemann-Pick UK. It's a charity not known by many and it affects a very small amount of children worldwide but our friend's son is living with this cruel terminal disease that's described as childhood Alzheimer's. We know that every penny they receive in donations counts in the hope that one day they find a cure..."

The girls well and truly smashed their original fundraising target of £300, raising £500 and counting. Well done girls, you are such an inspiration!

Harvey and the Brave Little Soldiers

This is a huge shoutout to Nick Vakis-Lowe, who plans to run six marathons in six months in support of NPUK...two down, four to go! Nick is part of the fundraising super team 'Harvey and the Brave Little Soldiers', who are incredibly active in the community raising funds and awareness as they go! Whether it is tackling Snowdon, dancing the night away, car boot sales, or even a ukelele orchestra...you name it, they've done it! We have been bowled over by their motivation and dedication. Thank you to all involved, keep up the fantastic work!

Marcos White

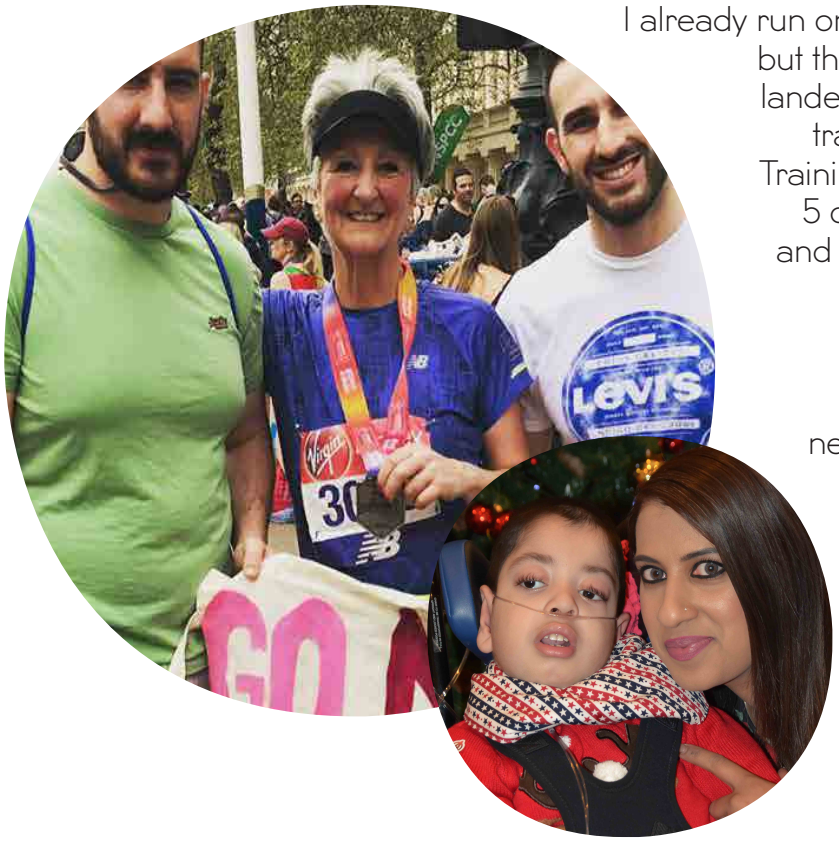


"...I ran the London Marathon support of a little boy called Sam with a very rare condition called Niemann-Pick disease. His dad (Will Evans) is a GP and helps run NPUK, a charity who are looking for a cure for the condition. Sam's mum (Miriam Evans) was going to run the marathon but broke her ankle in training. I was honoured to have the opportunity to run in her place. I won't ever forget my first London marathon...frighteningly hot, but I felt compelled to give it everything I had from my first step to the last.

There were many times I chose to recall the people and the reason I was running. A happy, brave boy called Sammy Evans. A supportive family who inspires EVERYONE who knows them. A charity built of people who know that every penny and every minute spent seeking a cure is worthwhile. So when my technique fell apart, and the miles still lay ahead I just kept moving forward. I am honoured to have been of help. And thank everyone who donated..."

Sarah Bullimore

"...I could not begin to imagine how my friend Nadia and her family felt after losing their son Zayn - caring and fighting for 4 years 24/7. There was nothing I could do to make this any easier. I saw the Virgin London Marathon was promoting the ballot entries for 2018 so I thought - the challenge just does not compare but it will be my challenge because I have that choice and I can raise much needed money for a charity of Nadia's choice.



I already run once or twice a week, maximum 6/7 miles but then on October 2nd the 'acceptance card' landed on the doorstep. That night I looked at training plans and started on October 3rd! Training to start with was OK I could get out at 5 o'clock in the morning and it was still light and the runs were quite short. Then the days got shorter, the runs got longer and the weather became wetter and colder.

Marathon day arrived and to say I was nervous was an understatement - the heat and an IT band injury added to my nervousness but I decided I would focus on getting to the end which was what I trained for not the finishing time. The crowd and seeing friends on the way carried me to the end. I am so proud to have been able to run for Niemann-Pick UK and The Neuro Foundation with the gift aid raising £3000+..."

THE MALEY BOYS TYNE TREK 2018

The Maley Boys (Benjamin, 11, Jonathan and Matthew, 9) are a young fundraising team, who chose to take on the huge challenge of the "Tyne Trek" to raise awareness of Niemann-Pick disease, as well as funds for Niemann-Pick UK (NPUK)...this is their story.

They were moved to support NPUK after watching the BBC Children in Need 'Pudsey Rocks the 80s Concert' which aired in 2017 and featured the inspiring yet heart-breaking story of the Hitchens' family, explained by mum Fiona and dad Carl. Their story and the wonderful footage of their beloved children Harry (NP-C 07/09/03-22/09/10) and twin sisters Grace (NP-C 27/02/05 -15/03/18) and Emily touched many hearts and brought home the true impact of NP-C.

Having twins in their own family, the boys particularly identified with the Hitchens' family and so, led by big brother Benjamin, the boys decided to take steps to help NPUK raise awareness and much-needed funds to continue their work. In addition, the boys' parents, Sean and Carla, are friends of Stewart and Toni Mathieson (parents of Lucy, NP-C, 27/05/03-29/09/07) and NPUK Project Team Leader Louise Metcalfe.

The boys chose to take on the 'Tyne Trek', a route of 93 miles established by Peter Donaghy, author

of 'River Tyne Trail: Sources to Sea'. The book helped the boys to plan each stage of the challenge, which took five months to complete and saw them walk from the north source of the River Tyne to Tynemouth. Upon hearing of the boys venture, Peter was so impressed he joined the boys on many stages of the trek - including the final stage.

Upon completing the Tyne Trek, Benjamin commented: "We have finished our Trek and want to say a thank you from the deepest part of our hearts to everyone who donated and followed us on the blog. With events held at our school and people learning more about this devastating disease, the families affected by Niemann-Pick will have support and help since we raised over £4,000. Finally we dearly hope we've inspired others to do something for this incredible charity."

To date the magnificent Maley Boys have raised almost £4,000 – it is not too late to support the boys, to find out how visit www.maleyboys.simplesite.com.

HOW TO PLAN YOUR NPUK FUNDRAISER...

Much of the work we do is funded entirely through fundraising activities and successful grant applications - that's why we value our fundraisers so much! By using these five steps, you will be well on your way to planning a successful event:

1 **Choose something fun:** The only limit to fundraisers is your imagination. There are so many events to choose from...check out our fundraising pages (page 24-29) for inspiration!

2 **Set a date and a target:** Setting a date is the first step towards making your fundraising goals happen - so set a date and target, and stick to it!

3 **Get in touch with NPUK:** Let us know what you have planned, so that we can share news of your event and provide information and materials to support you.

4 **Spread the word:** People need to know about your amazing event, so make sure you utilise social media to get the word out to everyone!

5 **Recognise that you are an absolute hero:** Your dedicated efforts keep NPUK going, and we appreciate this more than words can say! So never doubt - whether your event is large or small, raises a little or a lot - you are making a difference!

REMEMBER: We are here to support you! You can download our fundraising guide at www.npuk.org or if you would like a copy sent to you by post, simply get in touch with our team by email info@npuk.org or telephone 0191 415 0693

For further information on fundraisers including the NPUK Fundraising Guide please visit our website at: www.npuk.org

DEMENTIA STRIKES CHILDREN TOO

Dementia can be a challenging symptom of Niemann-Pick disease type C (NP-C), affecting children as well as adults. Outside of our community, few people understand what it means to have childhood dementia, or to care for a child living with it.

To help raise awareness, advance understanding and improve care, NPUK has joined the 'Dementia Strikes Children Too' Campaign, which launched in April 2018.

This community-driven campaign has been co-developed by families, and three patient support organisations, Niemann-Pick UK (NPUK), the Batten's Disease Family Association (BDFA) and the Society for Mucopolysaccharide Diseases (MPS Society), with the support of biopharmaceutical company BioMarin, in order to share the stories of real children living with dementia.

Childhood dementia exists – but people all too often haven't heard of it, as it is normally associated with adults. Several different groups of neurological diseases cause dementia in children, including NP-C. There is little research into the prevalence of the different forms of childhood dementia in the UK, so the total number of children affected remains an estimate.

Parents and families affected by childhood dementia experience many challenges in everyday life with their children, including distressing symptoms and an often, rapid, clinical decline.

In a recent survey by YouGov, only 4% of MPs said they were familiar with childhood dementia as a disease. This Campaign aims to reach out and raise awareness, especially with key decision makers and those who have a hand in the development of government policies.

As well as raising awareness, The Dementia Strikes Children Too campaign aims to achieve real change, by lobbying for better clinical education, earlier diagnosis and access to care and treatment for all those affected.

We would like to say a huge thank you to Joanne and Lee Coombes and their son Lleyton (age 11, NP-C) and to Jodie O'Grady and her son Joshua (age 11, NP-C) for sharing their stories, alongside families from the BDFA and the MPS Society, all of whom greatly helped to make this campaign a success!

RARE DISEASE DAY 2018
28 FEBRUARY

In the UK 1 in 17 people will be affected by a rare disease at some point in their lives – that's over 3.5 million people! Although there are more than 6000 rare diseases, having one isn't rare.

People with rare diseases like Niemann-Pick, often have to wait years for a diagnosis, have difficulty accessing the treatment and care they need and can feel isolated or alone because they don't know anyone else affected by the same condition as them.

Rare Disease Day, which takes place at the end of February each year, is a patient-led campaign that brings together millions of patients, families, carers, medical professionals, policy makers and members of the public in solidarity - everyone can get involved! The campaign targets primarily the general public and also seeks to raise awareness amongst policy makers, public authorities, industry representatives, researchers, health professionals and anyone who has a genuine interest in rare diseases.

2018 marked the tenth annual Rare Disease Day, with the theme of Research. For rare conditions such as ours, research contributes to diagnosis and to the development of effective treatments and therapies, as well as improved health and social care outcomes for patients and their families.

We were very grateful to each of you who participated this year. Whether you hosted an event, shared your story, or showed that you #careaboutrare , your involvement has helped to increase awareness of the challenges faced by those living with Niemann-Pick and other rare diseases, ensuring that, no one is left behind because they have a rare disease.

Here at NPUK, we joined forces with patient groups in over 90 countries, who hosted over 500 events and activities. Toni Mathieson, NPUK Chief Executive participated in a #ShareOnRare Tweet chat hosted by Actelion Pharmaceuticals Ltd, involving patient groups and professionals from across Europe. In addition, Toni travelled to Copenhagen to address employees of Orphazyme ALS and share her thoughts and experiences of living and working with Niemann-Pick.

In addition, our members undertook events of their own, including the inspiring Shona Beveridge (age 17 NP-C), who worked with friends to host a Dress Down Day raising awareness and funds to support and mark this important day.

Want to get involved? #RDD2019 takes place on 28th February 2019, learn more at www.rarediseaseday.org

WEAR JEANS CHANGE LIVES.

JEANS FOR GENES DAY

Make sure you get Friday September 21st in your diary - as that marks this year's Jeans for Genes Day, a day that aims to put genetic disorders on the map, to raise funds and awareness encourage greater support for those affected.

Life-altering genetic disorders, such as Niemann-Pick disease, affect around half a million children in the UK in total. Jeans for Genes Day helps to put genetic disorders in the spotlight and to bring attention to the issues faced by those affected.

On Jeans for Genes day, participating schools and workplaces allow students and staff to wear jeans and casual wear to school and work instead of their usual uniforms and formal workwear. Participants raise funds in return for wearing casual wear and by holding events throughout the day.

The money raised on Jeans for Genes Day funds a range of initiatives that improve the quality of life of children and families affected by genetic disorders.

As in previous year's, NPUK is proudly partnering with Genetic Disorders UK on #jeansforgenesday2018! We hope this initiative will raise funds and awareness for NPUK whilst making a difference for all those affected by a genetic condition.

We need your help to spread the word amongst schools, colleges, shops, businesses, libraries - and anyone else you can think of – by reaching out and asking them to join in and support us. All those willing to help can register their interest on the Jeans for Genes website: https://www.jeansforgenesday.org/ and select NPUK as their chosen charity.

All participants will receive a free fund-raising pack. We will also be informed and can offer our support by providing customised posters, letters of support for schools and businesses and much more!

Your event can be held at any time in the month of September – the aim is to encourage as many people as possible to join in, wear their jeans and donate £1 (or more!) towards the cause.

Every penny that your school, business, etc. raises will be split 50:50 with Jeans for Genes. For example, if one small school with 200 staff members and pupils gave £1 each, that would mean £100 to NPUK. Expand that to a high school with 2000 that's another £1000.

For more information please contact NPUK Trustee Jackie Imrie on 07414 529 392 or email jackie@npuk.org.

PROGRESS TOGETHER

Inpda

International Niemann-Pick Disease Alliance

Who is the International Niemann-Pick Disease Alliance (INPDA)?

The International Niemann-Pick Disease Alliance (INPDA) is a global network of non-profit organisations working in the field of Niemann-Pick disease (NPD). Founded in 2009, through the collaborative efforts of Niemann-Pick UK and the National Niemann-Pick Disease Foundation (USA), the INPDA now has 18 member groups in 15 countries; Argentina, Australia, Brazil, Canada, China, France, Germany, Italy, The Netherlands, Norway, Spain, Switzerland, Taiwan, United Kingdom, USA.INPDA members work together, overcoming language and cultural differences to facilitate progress in research and improve health and social care outcomes for all those affected by Niemann-Pick diseases.

What does the INPDA do?

All of the activities undertaken by the INPDA fall within its aim of facilitating progress in the field of Niemann-Pick disease.

By providing a forum for mutual support, the INPDA aims to establish links between all non-profit Niemann-Pick support groups and to raise awareness of the nature and operations of these, sometimes very different, organisations.

Support for those wishing to establish a new group is also available.

INPDA representatives attend and present at key conferences around the world, including patient group events and wider rare disease events.

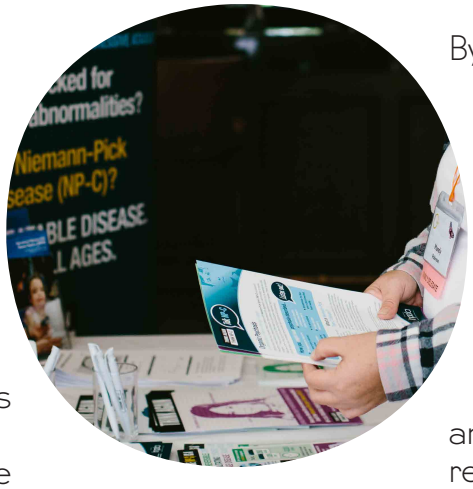
The INPDA is a member of EURORDIS and has representation on Rare Disease International.

As a result of its work, INPDA members are increasingly invited to participate in high-level meetings with statutory bodies, authorities and regulators.

INPDA Key Projects:

The INPDA is currently engaged in delivering four key projects. Each aim to make a difference in the areas of scientific, clinical and therapeutic research, patient care, access to treatments and time to diagnosis.

The International Niemann-Pick Disease Registry (INPDR) has the power to increase knowledge and understanding of NPD through the collection of much needed patient data on a global scale. More information on page 40.



The Think Again. Think NP-C Campaign aims to improve diagnosis of NP-C by targeting specialist health care professionals who are currently unfamiliar with the condition and giving them the tools they need to recognise and act upon the symptoms of NP-C. Learn more on pages 38-39 and at the dedicated website: www.think-npc.com

The Loire Valley Meeting which takes place in France every two years, creates networks and opportunities within the research and clinical community that otherwise would not exist. The meeting is planned for 19th-21st October 2018

The INPDA Information Portal was developed with the support of a Sanofi-Genzyme PAL grant. This open access Portal, hosted on the INPDA website, brings together a wide range of helpful information for patients, families, and professionals working in the NPD field, in a range of different languages and formats. The Portal reflects the collaborative spirit of the INPDA and continues to grow along with our respective member groups, who provide articles and documents, and translations where necessary, further boosting the ongoing global effort. Visit: www.inpda.org/inpda-information-portal.

Benefits of the INPDA:

The INPDA has a global reach and therefore it is ideally placed to effectively communicate information to the NPD community, offering equity of access and ensuring consistency and accuracy of the information provided.

By sharing information and maximising communication, we can help each organisation to use their time and resources efficiently and effectively.

The INPDA aims to ensure that all member organisations are informed of, and if appropriate, able to act upon, the most recent scientific and therapeutic developments.

Wherever possible, the INPDA will help to encourage research by supporting the formation of networks, providing seed funding and working in partnership to secure grant funding.

By working together, members have a stronger voice with which to influence change and bring improvements for NPD patients everywhere.

For further information and recent updates on the International Niemann-Pick Disease Alliance please visit: www.inpda.org.

THINK AGAIN
THINK NP-C

The 'Think Again. Think NP-C' Campaign Continues...

Niemann-Pick type C disease (NP-C) takes, on average, five years to diagnose, however in our experience, it often takes much longer, leaving patients without treatment or access to expert care and support.

Think Again. Think NP-C is a campaign led by the International Niemann-Pick Disease Alliance (INPDA), an alliance of non-profit Niemann-Pick disease patient support organisations across the world.

The campaign aims to reduce the time to diagnosis by supporting healthcare professionals who are unfamiliar with NP-C to recognise the key signs and symptoms of the disease. The main goal is to help patients affected by NP-C by speeding up diagnosis and enabling access treatment and support in a more timely manner, therefore improving their quality of life.

The Think NP-C. Talk NP-C symptom cards have been developed to explain how key symptoms of Niemann-Pick type C disease may be described by a patient or carer. The cards aim to help healthcare professionals understand and identify the key symptoms of NP-C through the provision of real-world patient and carer insights.

Patients and healthcare professionals can share the symptom cards at meetings and events, in a clinic or a hospital department or distribute through their family or professional networks. All campaign materials, including our symptom cards, are available for you to download at: www.think-npc.com/think-np-c-campaign-materials

It can be difficult to diagnose patients with NP-C as symptoms can be varied and non-specific to the disease, working together with the Think Again. Think NP-C Campaign, we hope to raise greater awareness and ensure NP-C is recognised – that professionals 'think NP-C'.

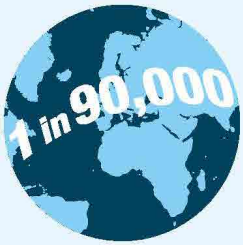
As members of the NP-C community, you have been making a fantastic effort to describe the symptoms of NP-C - the development of the campaign, the creation of the symptom cards and the ongoing support that Think Again. Think NP-C receives from individuals and organisations is all thanks to the NP-C community, and is instrumental in our on-going success.

For further information on the Think Again. Think NP-C campaign, please visit: http://think-npc.com/ you can also follow the campaign on Facebook at @Think AgainThink NPC1.

NIEMANN-PICK TYPE C DISEASE (NP-C) IS A

RARE PROGRESSIVE IRREVERSIBLE CHRONICALLY DEBILITATING LYSOSOMAL STORAGE DISEASE[1–3]

NP-C affects all ages[1]



Incidence of NP-C is 1 in 90,000 live births[4]

Likely an underestimate due to lack of clinical awareness[1]

NP-C takes on average 5 YEARS to diagnose[5]

That's...

43,824 HOURS

...waiting for an answer, watching a loved one get worse

THINK AGAIN. THINK NP-C aims to support healthcare professionals unfamiliar with NP-C to recognise the key signs and symptoms of NP-C and reduce the time to diagnosis

Individual symptoms are non-specific to the disease[1,3]

If you are a:

Paediatrician

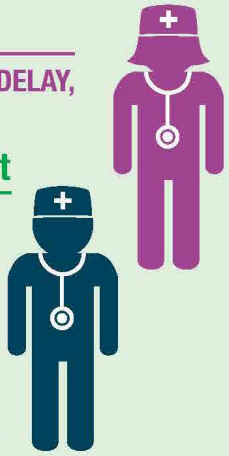
LOOK FOR ATAXIA, DEVELOPMENTAL DELAY, HEPATOSPLENOMEGALY

Paediatric hepatologist/neonatologist

LOOK FOR HEPATO/SPLENOMEGALY, NEONATAL CHOLESTATIC JAUNDICE, NEONATAL LIVER DYSFUNCTION

Adult neurologist/psychiatrist

LOOK FOR COGNITIVE DECLINE, ORGANIC PSYCHOSIS, PROGRESSIVE ATAXIA



Have you checked for eye movement abnormalities?

Vertical supranuclear gaze palsy (VSGP) is present in virtually all patients[1,3]

References

1. Patterson M, Hendriksz, Walterfang M, *et al.* on behalf of the NP-C Guidelines Working Group. Recommendations for the diagnosis and management of Niemann-Pick disease type C: an update. *Mol Genet Metab* 2012; 106(3): 330–344.
2. Vanier, M. Niemann-Pick disease type C. *Orphanet J Rare Dis* 2010; 5: 16.
3. Wijburg FA, Sedel F, Pineda M, *et al.* Development of a suspicion index to aid diagnosis of Niemann-Pick disease type C. *Neurology* 2012;78(20):1560–1567.
4. Wassif C, Cross J, Iben J *et al.* High incidence of unrecognized visceral/neurological late-onset Niemann-Pick disease, type C1, predicted by analysis of massively parallel sequencing data sets. *Genet Med* 2016; 18(1): 41–48
5. Mengel E, Kl nemann H, Lourenço C, *et al.* Niemann-Pick disease type C symptomatology: an expert-based clinical description. *Orphanet J Rare Dis* 2013; 8: 166.

To help reduce the time to diagnosis visit www.think-npc.com today

This is a project co-ordinated by the International Niemann-Pick Disease Alliance with the collaboration and financial support of Actelion Pharmaceuticals Ltd

The International Niemann-Pick Disease Registry (INPDR)

The International Niemann-Pick Disease Registry (INPDR) is a not for profit, registered company based in the UK. Developed as a subsidiary of the INPDA, this initiative encourages global collaboration between patient groups, clinicians, scientists and researchers, all of whom wish to improve care and treatment options for NPD patients everywhere.

INPDR Project Co-ordinator Tony Wilson gives an update on the INPDR's recent activities since the last issue of NPUK News:

Our main aim for 2018 is to increase the number of patient records held on the Registry. We currently have 236 and we are looking to increase this to around 1, 000 by the end of the year...

How will we do that? We do recognise that there are some challenges we need to tackle. Firstly, we need to understand how best to manage the time commitment of the clinicians who enter the patient data and secondly, we need to explore and explain, how to encourage patients and their families to get involved. NPUK Trustee, Jackie Imrie, is leading on this by initially focusing on the UK centres and then meeting with our main contacts in our European centres.

Further afield, at the WORLD Symposium, we met a number of encouraging discussions about the potential for expanding the Registry beyond UK and Europe. This resulted in agreement to run a pilot exercise in the USA, at the Mayo Clinic as a first step towards achieving expandion. We are working closely with our colleagues at the NNPDF to make this happen!

We are currently close to completion of an all-new INPDR website, which we trust will make the INPDR more accessible for all. If you haven't already, please 'Like' and follow us on Facebook @INPDR to ensure you don't miss any and all of our updates. *We thank you all for your ongoing support.*

The INPDR is a single, disease specific registry created for the global Niemann-Pick community:

- Created by professionals and patient groups for worldwide use

- Uses the power of data to improve the understanding of NPD

- Supports and facilitates research and therapy development

- Encourages earlier diagnosis and equitable access to diagnostic testing

- A unifying initiative, much-needed by patients and professionals alike

For more information visit: www.inpdr.org

THE hollie FOUNDATION

The Hollie Foundation Continues to Support NPUK

The Hollie Foundation provide invaluable support to NPUK via their grant programme, which provides much-needed grants in support of activities and individuals within the Niemann-Pick community.

This is the fifth consecutive year that the Foundation has generously supported the post of NPUK's Senior Families Advocate, held by the dedicated Elizabeth Davenport. Without funding such as this, Elizabeth would not be able to provide the high level of individualised non-clinical support currently available to those affected by all types of Niemann-Pick disease and their families.

In addition, the Foundation also kindly grants much-needed funding to cover the cost of our Children and Young Persons' Activity Programme, held at our Annual Family Conference in September each year. The Activity Programme provides various activities for younger conference attendees, which include affected children, their siblings and children of those affected by Niemann-Pick disease. It allows family members to participate in the main conference programme with the knowledge that their loved ones are being properly cared for and appropriately entertained by our team of dedicated volunteers.

Our community has also benefitted from travel grants that have enabled families to attend our social and networking events across the UK, including our Annual Christmas Party! Furthermore, it is important not to forget the Foundation's much-needed grant programme for individuals and families affected by Niemann-Pick disease, which has made such a difference to the lives of so many.

This year, the Hollie Foundation marked their tenth anniversary with the highly successful 'Hollie's Secret Garden Ball' held at Flaxbourne Gardens in Aspley Guise. The event was a complete sell out and highlighted the amazing support the Charity receives from their local community, which enables them to provide their much-needed grant programme and to raise vital awarenessfor the Niemann-Pick community.

Thank you to the Hollie Foundation for their ongoing support and amazing achievements over the last ten years.
We hope that the Foundation's many friends and supporters continue to provide such a tremendous response over the next ten years, making an even bigger difference on behalf of all those affected by Niemann-Pick disease!

#DreamingofaCure #DreamingofaFuture

For more information on the Hollie Foundation or their grant programme, visit: www.theholliefoundation.com

np uk

Supporting those affected by Niemann-Pick

Education and Learning

Some children and young people affected by conditions, such as Niemann-Pick, need more support than others to achieve their full learning potential.

They may need extra help, for example, because they have difficulty with reading, understanding, talking, managing their emotions or behaviour, or developing physical skills. A child who needs a lot of extra help in any of these areas has special educational needs (SEN).

Educational settings (schools, nurseries and colleges) have a legal duty to support children and young people with additional needs and disabilities and to treat them fairly.

Support in the early years

Early years settings can include childminders, day nurseries, pre-schools, holiday playschemes and childcare in your own home. The law refers to these as "early years settings" or "providers."

Some providers specialise in support for disabled children and children with special needs. If your child has complex SEN they may be offered a place in a specialist nursery. Local authorities have a duty to make sure there are enough early years options for all families in the area who need it and must help to find suitable arrangements.

Is a mainstream or special school best for my child?

The type of school your child goes to will depend on their needs, your preference and the schools in your area.

Your child may learn well in a mainstream school with extra help from staff within or outside the school. If your child has complex needs, a special school with specially trained teachers, therapists and equipment may suit them best.

It's a good idea to visit different schools in your area to get an idea of what kind of school would be right for your child. Although most children with special educational needs (SEN) go to a mainstream school.

The law says that schools must do everything they can to make sure children with SEN get the extra support they need to achieve as well as they can. Mainstream schools do this through a system called SEN support, and are also required to have a policy setting out how it supports disabled pupils to be included in school activities.

Every mainstream school has a special educational needs coordinator (SENCO) who is responsible for organising extra help for pupils with SEN. The SENCO works with the class teachers and subject teachers to plan the help each child needs.

The school must tell you if they are giving your child any extra help. It should also work with you and your child to plan their support and show how your child is progressing with their schooling.

Who to speak to if you are worried about your child's education...

If you're concerned that your child has special educational needs, talk to a professional who knows your child. This could be a health visitor, someone at their early years setting (nursery, play group, pre-school) or their teacher.

If you live in England, Wales or Northern Ireland, you can also talk to the SENCO at your child's school. People who live in Scotland should explain their concerns to a teacher, giving examples, and ask what support is available. Scotland follows an approach called 'Getting it right for every child', so ask about this.

For further information on this, and any other issue, please get in touch with Contact on their free helpline at: 0808 808 3555.

SOURCE:

contact *For families with disabled children*

HELP FOR THE HOLS!

As the sun finally starts to make an appearance, it's always nice to have something to look forward to...after all everyone needs a break from time to time. If you, or a family member, have additional needs organising a holiday can seem like an uphill climb - but it doesn't need to.

We recognise there are difficulties such as finding out what facilities are available, making appropriate travel arrangements, setting up insurance, and then the small matter of how to come up with the funds to make the dream a reality - but help with financing your holiday is available, provided you meet the required criteria of the funding body or know where to find them in the first place!

Don't worry, our Senior Families Advocate Elizabeth Davenport is always on hand to point you in the right direction, whether this be by speaking with your local social services department in the first instance (as most funding bodies require a referral from either a GP or social worker) and following the process until a grant, or other positive arrangement is made. You can contact Elizabeth by email at: elizabeth@npuk.org or by phone on: 07896 197 576.

On the following two pages we have put together a comprehensive list of organisations who may be able to help. Please note: to avoid disappointment, please make sure you apply well in advance of your intended departure date, as generally speaking these organisations are inundated with requests and grants are offered on a case by case basis.

Family Fund: The Family Fund works to promote an inclusive society where families with severely disabled children have access to choices and the opportunity to enjoy ordinary life.

The organisation provides grants to families with severely disabled children (aged 15 and below) based on their views and needs.

Please call 01904 550055 for any general enquiries. For further information on The Family Fund please visit: www.thefamily fund.org.uk.

Happy Days Charity: This charity will pay for a special needs youngster and their family to go on holiday, consequently giving the whole family a fun and relaxing break so they can focus on what is important: quality time together as a family in an environment which can cater for their child's disabilities. They also fund and organise days out, such as theatre trips throughout the UK for disadvantaged young people (aged 3-17) with learning difficulties, physical or mental disabilities and with acute, chronic or life limiting illnesses. For further information please visit: www.happydayscharity.org.

Family Holiday Association Fund: By providing grants for families in urgent need of a break, Family Holiday Association are an invaluable resource for so many! They offer funding for up to a week away at a location of the family's choice - however, each respective family must be referred to the Association by social services, a health professional or local voluntary organisation. For further details, please visit: www.fhaonline.org.uk.

KidsOut: With KidsOut the focus is always on fun, their 'Funds-4-Fun' grants help with the provision of new toys and special play equipment, as well as fun days out, holidays and activities for individuals and families. Visit their website at: www.kidsout.org.uk for further information.

FAMILY HOLIDAY ASSOCIATION
The charity that gives families a break

happy days
helping children experience what life has to offer

KidsOut
THE FUN & HAPPINESS CHARITY

Family Fund
Helping disabled children

TRAVEL INSURANCE

Please note: NPUK cannot endorse any particular insurance company, however the following have provided insurance cover for NPUK families in the past and may be of interest:

Post Office Travel Insurance
Telephone: 0800 294 2292
Website: www.postoffice.co.uk

Freespirit
Telephone: 0845 230 5000
Website: www.free-spirit.com

Travelbility
Telephone: 0845 338 1638
Website: www.travelbility.co.uk

Allclear Travel Insurance Services
Telephone: 08712 088 579
Website: www.allcleartravel.co.uk

Marks and Spencer Travel Insurance
Telephone: 0800 051 6670
Website: www.bank.mark-sandspencer.com/insurance/travel-insurance/

Please contact NPUK Senior Families Advocate, Elizabeth Davenport for further details and/or assistance regarding arranging travel insurance by email at: elizabeth@npuk.org or phone on: 07896 197 576.

The Lonely People

by Tony Somers

The Beatles sang, "All the lonely people where do they all come from? All the lonely people where do they all belong."

The lonely people come from another dimension where ironically they are never alone. Love is every where, every body loves everybody else, all decisions are made from a place of love.

There is nothing but love, it's impossible to be alone when you are bathed in unconditional love.

Love for yourself and everyone else and everything, you breathe in love and you exhale love.

In their quest and passion to spread this amazing sensation of love they ask to be sent to earth so that they can spread the love.
To get here they have to go through a process of forgetting, they forget that they are love...

They are born into an earthly environment and most are loved, at least at first and they do what they are programmed to do, which is to reciprocate that love.

They feel special and this feeling is vaguely familiar, it's as though they have experienced it before (of course they have).

They soon learn that on this earthly plane not everything comes from a place of love.

They feel special but are told that there is nothing special about them.

They experience the opposite to love which is fear, rejection, they become scared to show love.

They become scared to be who they really are, the people who have arrived on this planet before them have forgotten all about love.

They are full of fear, they feel threatened by these new arrivals so from a place of fear, they condition them to conform, to know their place, to not threaten or get above their station.

The light of love dwindles, it flickers, it turns into a dying ember but it never quite goes out...

It waits to be re ignited and sometimes it flares into a flame but often the overwhelming fear of its predecessors dampens it back down once again.

It waits for someone to reignite it, not realising that it has the capacity to reignite itself and spread light, warmth and love throughout the darkness.

Scientists tell us that energy cannot be destroyed, it can be changed but not destroyed. Water can change to ice and vapour and back to liquid but it is still water. You are energy which means you cannot be destroyed. Conditioning can change you but it cannot destroy you. You are and always will be a spiritual being...

Hang onto that thought for just a moment...YOU ARE A SPIRITUAL BEING!

How good does it feel to know that you are connected to everyone and everything in the universe, in fact you are the universe.

You have just lost your faith and your belief but you can get them back. Look how far you have come, look how far we have come as a species.

It wasn't so long ago that we lived in caves, without fire for light or warmth.

It wasn't so long ago that major diseases killed millions of us and poverty was rife.

Gladiators fought to the death for entertainment, a couple of hundred years ago slavery was still legal in western society.

Look how far you have come and you have survived this long and not only survived but prospered and yet we crave more, always more, it's never good enough, we are never good enough!

That is the biggest lie of all because you are good enough, you are the whole universe, you are more than good enough, you have to be, you are perfect energy.

So who are the lonely people?

They are you and me, our brothers, sisters, fathers, mothers, sons and daughters, the people who have forgotten.

Where do they all belong? In a place of love.

We are special because we are all the same, we come from the same place but fear tells us to be different to be better than others and don't let them think that they are any better than you.

And that's what makes us lonely, fear, love will set you free and it starts with self love.

Is there anything that you want?

Is there anything I can do?

Just call on me and I will send it along with love from me to you because you are me and I am you.

Tony Somers is a professional counsellor, life coach, and author. He uses his skills and experiences to make a difference to other people's lives. For further information, please visit: www.tonysomers.com.

NPUK: TEXT DONATE

Whatever network you're on, donating to NPUK is as easy as sending a text

If you live in the UK you can donate to NPUK easily with **JustTextGiving** from your mobile phone – simply text: NPUK02 followed by the amount you'd like to donate to 70070.

You can also donate through **VirginMoneyGiving** (and JustGiving) online. Simply search 'Niemann-Pick UK' on both sites respectively and select your donation amount. Please leave your name so that we can give you a Facebook shout out!

Remember that NPUK relies entirely on grants and donations to continue the work we do in supporting those affected by Niemann-Pick.

In Loving Memory...

TJ Colwell: 27/2/2005 - 15/3/2018

Well TJ what can we say about you?
In the early years it was clear that music would be a big part of your life.

JLS was your ultimate favourite band you knew all the words and dance moves! You would spend hours dancing (naked) to your reflection in front of the fridge, the other time was spent INSIDE the fridge looking for ham or cheesestrings!

Watching TV was your other pastime although a game of cars monopoly would be a daily occurance.

When your baby brother Mason came along you took a liking to one of his teddies, he has been by your side ever since!

You took great interest in history and facts whether it was castles, dinosaurs, planets or the titanic...these were just a few that became great conversations.

Outside wasn't your favourite place to be, but at the beach you would spend hours throwing stones into the water.

You were also a bit of a thrill seeker, you wouldn't be scared of any ride or rollercoaster!

You enjoyed school it gave you the structure and routine that you loved...though you'd be looking forward to Saturday so you can have tea and biscuits for breakfast!

When you moved to your new school, days were full of surprises. Just small clues of what you've been doing dragged home in your chair or other areas lol? You had such a kind and gentle nature that people were drawn to. At times you would love us to be loud and crazy, but also enjoyed quiet times to watch one of many movies from your collection!

These are just a few of the many memories I will hold so close, I will never forget your bright blue eyes that lit up your face..I consider myself lucky to have had you as my son.

It has been an absolute pleasure and a privilege to have been your mum.

In loving memory of Jacinto Gomes:

Jacinto's son Carlos, writes: "Sadly my father Mr Jacinto Gomes passed away at the end of January. Jacinto's eldest grandson Daniel Gomes (agd 27) was diagnosed with Niemann-Pick disease type C when he was aged 14 - it was my father's wishes for donations be made to NPUK. A fundraising page has been set up and over £700 raised."

We thank Carlos, Kim, and Daniel Gomes, as well as the rest of the friends and family who contributed to this touching tribute.

In loving memory of Thomas Hague:

It was the wish of Thomas Hague, that in lieu of flowers at his funeral, donations would be made in the name of NPUK. Thomas and his wife Margaret are close personal friends of the Valentine family, and as a result have been touched by Matthew Valentine (age 9 NP-C) and his positive attitude.

We thank the Hague family and friends for this generosity - may Thomas rest in peace.

If you would like to pay tribute to a loved one in the next issue of NPUK News, then please email John at: john@npuk.org.

In Memoriam
by Victoria Bruce

For a second you were flying

Like you always wanted to

Now you'll fly forever

In skies of azure blue

We'll see your smile in every ray

Of sunshine after rain

And hear the echo of your laughter

Over all the pain

The world's a little quieter now

The colours have lost their hue

The birds are singing softly

And our hearts are missing you

Each time we see a little cloud

Or a rainbow soaring high

We'll think of you and gently

Wipe a tear from our eye

REFLECTIONS

Reflections is a group created by Niemann-Pick UK Senior Families Advocate Elizabeth Davenport, which seeks to provide care and support to those in the NPUK community who have lost a loved one.

The group will meet next at this year's Annual Family Conference & Interactive Workshop, however if you prefer to meet and talk with Elizabeth individually, you can contact her throughout the year by email at: elizabeth@npuk.org or phone on: 07896 197 576.

Volunteer with NPUK

If this issue of NPUK News has inspired you to get involved and help us to make a difference to those affected by Niemann-Pick disease(s), then there are many ways in which you can do so...

It is thanks to the unwavering support of our dedicated volunteers, that we are able to continue to offer our essential service to those individuals and families affected by Niemann-Pick disease. For many of our events across the year, including our Annual Family Conference & Interactive Workshop and Christmas party, we rely heavily on volunteers who lend a hand and make sure the much-loved events run as smoothly as possible.

As well as lending a hand as part of the Children's and Young Persons' Programme at Conference or helping out at other NPUK supported events, there is also ample opportunity for you to get involved with fundraising events. For more comprehensive advice and tips on how to get involved as a fundraiser on behalf of us, check out the NPUK Fundraising Guide, available as a physical copy or online at the NPUK website.

Whatever you choose to do, and however you choose to get involved, you can be fully assured that you are making a true difference.

For further details on volunteering for NPUK, get in touch with us by email at: info@npuk.org or by phone on: 0191 415 0693.

With grateful thanks to our grant providers:

LOTTERY FUNDED

BBC Children in Need

THE hollie FOUNDATION

NPUK

WHO WE ARE...

NPUK, Central Office
Suite 2, Vermont House
Concord, Washington
Tyne & Wear
NE37 2SQ

Website: www.npuk.org
0191 415 0693
Email: info@npuk.org

Chair:
Dr. William Evans

Board of Trustees:
David Roberts, Janice Brooks, Richard Rogerson, Bill Owen, Helen Carter, Jackie Imrie, David Holton, Joella Melville

Co-opted Members of the Board:
Professor Frances Platt,
Stefanie Rymsza

Medical Advisor: Dr Simon Jones
Consultant in Paediatric Inborn Errors of Metabolism at Manchester University NHS Foundation Trust

Toni Mathieson, Chief Executive
Mobile: 07816 398 591
Email: toni@npuk.org

Christine Jopling, Finance & Administration Officer
Email: christine@npuk.org

Louise Metcalfe, Project Team Leader
Email: louise@npuk.org

John Taggart, Social Media & Communications Officer
Mobile: 07984 366 334
Email: john@npuk.org

Elizabeth Davenport, Senior Families Advocate
Mobile: 07896 197 576
Email: elizabeth@npuk.org

Steve Neal, Project Families Officer
Mobile: 07787 818 885
Email: steve@npuk.org

Laura Bell, Clinical Nurse Specialist
Salford Royal Foundation NHS Trust
Mobile: 07791 499 555
Email: laura@npuk.org

Note from the Editor...

I would like to thank all of those who have contributed to this issue of NPUK News. Please continue to submit articles, stories, poems, advice, children's contributions, fund-raising ideas, and anything else you feel may be of interest...especially photographs!

Send your contributions for the next edition of NPUK News to John, our Social Media and Communications Officer, by email at john@npuk.org, or by post at: Niemann-Pick UK, Suite 2, Vermont House, Concord, Washington, Tyne and Wear, NE37 2SQ.

Disclaimer: Information which appears in this newsletter is for the express purpose of raising awareness and does not necessarily reflect the views of NPUK. All medical information should be reviewed with your doctor before being acted upon.

np uk

Supporting those affected by Niemann-Pick

Follow: Niemann-Pick UK

Registered Charity England and Wales (1144406) and Scotland (SCO45407)

Registered as a company limited by guarantee in England and Wales: 07775835