



NIEMANN-PICK DISEASE GROUP (UK)

FAMILY NEWS BULLETIN

FEBRUARY 2010

Hello and welcome to the first Niemann-Pick Disease Group (UK) Family News Bulletin of 2010.

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Save the Date—NPDG (UK) Family Conference 2010



This year, the NPDG (UK) Annual Family Conference is moving from our usual spot in June to the weekend of 10-12th September. The venue of the Conference will be The Robinson Executive Centre, Wyboston Lakes on the Bedfordshire/Cambridgeshire border. Further details will be sent out with the Spring 2010 Newsletter and will be available to view and download on our website in due course.

NPDG (UK) Clinical Research Nurse – Liz Jacklin

Liz Jacklin, our Clinical Research Nurse, has provided the following update of her work for the Family News Bulletin. If you have any specific research questions that you would like to see addressed in future issues, please do forward them to us at the Central Office by email at nie-mannpick@zetnet.co.uk, or to Liz direct elizabeth.jacklin@cmft.nhs.uk.

Liz writes:

NPC Research Meeting

Following on from the success of the NPC Research Meeting which was held at the Royal Manchester Children's Hospital on 1st December 2009, we would like to hold another meeting of this kind. Thank you to all those who contributed to the meeting, we have received some useful feedback and this will assist us in organising future events. We now need to decide on a suitable time and venue for the next meeting. Please let us know when and where you would prefer the meeting to be held – for instance, would midweek or the weekend be more suitable for you? Is Manchester a convenient location, or would you prefer a different location? Your suggestions will be most welcome and helpful. We are hoping the meeting will take place in May/June.

Zavesca – FDA Advisory Committee Meets in the USA

On January 12th 2010, an FDA Advisory Committee meeting was held in Maryland, Washington, USA. The Committee was convened to discuss the application for Zavesca (Miglustat) to be used for Niemann-Pick type C patients in the USA. You may recall that the drug was approved by the European Medicines Agency, for use in Europe, in January 2009. Actelion Pharmaceuticals, the company who developed Zavesca, asked that I attend the hearing on behalf of the NPDG (UK) as an 'expert' witness. The Committee were required to vote on a number of points throughout the day, the most critical being "In the light of safety and efficacy data presented in this application, does the risk/benefit of Zavesca support its approval for treatment of NP-C?" Following a tense day of presentations and questions, I am delighted to report that the Advisory Committee voted 10 to 3 in support of approval. Whilst this does not guarantee a positive outcome, the FDA will meet again in March this year to make a final decision, the Advisory Committee's recommendation may mean that the application is looked upon more favourably. The success of this meeting was due in no small part to the presentations made by, and on behalf of, NPC families; Barb Vorpahl, Chair of the Niemann-Pick Disease Foundation, Cindy Parseghian, President of the Ara Parseghian Medical Research Foundation and Phil Marella, Trustee of Dana's Angels Research Trust, all gave tremendous presentations. I was given the opportunity to speak on behalf of the NPDG (UK) and our UK families on several occasions. I am delighted that the NPDG (UK) was able to be involved in this process.

2011 London Marathon



As previously reported in this e-bulletin, the NPDG (UK) has secured a place in the 2011 London Marathon. If you, or anyone you know, are interested in competing in the 2011 Marathon on behalf of the Group, please contact either Toni or Sue at the Central Office.

Design Your Own Place Mats and Coasters and raise funds for the NPDG (UK)



If you are looking for a unique gift, or to preserve your child's artwork or favourite photos, then you might be interested in a company called A G and P Digital. They can reproduce most artwork on to mats and coasters, and will donate 10% of any sales to the Group. If you wish to order, quote the Niemann-Pick Disease Group (UK).

For more details go to www.niemannpick.org.uk/donate/images/PlacematsAdvert_000.JPG.

Calling all Carers – Survey to Assess the Emotional Impact of Caring for a Loved One Affected by NPC

As we reported in the previous e-bulletin, Actelion Pharmaceuticals have commissioned Insight Research Group to carry out a survey to look at the emotional impact of caring for a loved one affected by NPC. **Insight have already interviewed three carers, and are looking to interview two more.** The interviews are conducted face to face, with a view to distributing the outcomes to all professionals supporting affected families at a later date. At this point there may also be the possibility of media involvement, to assist in raising awareness of NPC, although this is not a requirement and your permission would be sought prior to any media involvement. If you would like to take part in the survey, or would like more information, please contact Toni or Sue at NPDG (UK) Central Office. They will be happy to send you a full information sheet and contact details for the representative at Insight, either by post or email.

The NPDG (UK) Welcomes Cash Boost to Improve Childcare for Disabled Children

Families with disabled children in England are set to benefit from investment to improve childcare provision for disabled children. The Government has announced an extra £12.5 million investment to be given to local authorities to improve childcare for disabled children using a range of practices. It is part of the Disabled Children's Access to Childcare (DCATCH) project and follows successful pilots in 10 local authority areas. Now the remaining 142 local authorities in England will each receive at least £59,000 to adopt some of the successful practices to improve childcare developed by the 10 pilots. These include creating



'Parent Champions' who are parents in the local area with experience of using childcare for their own disabled children and who help other parents understand the services available to them; specialist training for childminders and staff so that they have the skills they need to work with disabled children; better information for parents of disabled children so they are

more aware of the options available to them. For more information visit www.dcsf.gov.uk/everychildmatters/healthandwellbeing/ahdc/childcare/childcare

Lamb Inquiry recommendations—A Step in Right Direction to Improve the Special Educational Needs (SEN) system

The NPDG (UK) welcomes the publication of the final report of the Lamb Inquiry, a government commissioned review of the SEN system. The Lamb Inquiry's recommendations included ensuring parents are better informed and are at the heart of the decision making process about their disabled child's educational needs. To view the full report visit www.dcsf.gov.uk/lambinquiry/downloads/8553-lamb-inquiry.pdf

Parents' Experiences of Disabled Children's Services 2009-10

The Department for Children, Schools and Families (DCSF) has published the main findings from the second national sample survey of parents of disabled children in England. The survey was conducted between July and November 2009. This survey also provided, for the first time, figures for 145 local authorities and 150 Primary Care Trusts. To see how they performed in your area visit www.dcsf.gov.uk/rsgateway/DB/STR/d000902/index.shtml

Stay Warm and Healthy Over Winter



Keep Warm Keep Well, a national government campaign targeting the reduction of cold-related illnesses and deaths during winter, offers tips and advice on keeping you and your family warm and well this winter. The campaign offers a variety of practical help and tips such as how to keep your house effectively insulated to reduce heat loss and how to stay healthy and fit during cold spells. You can also find information on financial assistance, such as grants and benefits, that are available to you to help make your home warmer or simply to assist you in meeting the financial demands of your heating bills. Keep Warm Keep Well produces informative leaflets which can be found in your local supermarket, GP's surgery, local pharmacy or Council offices. For more information visit <http://keepwarmkeepwell.direct.gov.uk/index.html>

Rays of Sunshine Need Your Wishes



You can turn wishes into happy memories by contacting Rays of Sunshine, a Children's Charity formed to help children who are living with serious or life-limiting illnesses between the ages of 3-18, by granting their wishes, however impossible the wish may seem. You can refer a child by completing a wish application by visiting www.raysofsunshine.org.uk/wishes/index.php. For further details of the work of Rays of Sunshine visit www.raysofsunshine.org.uk/about

-US.

For a list of other charities helping to make children's wishes come true visit www.niemannpick.org.uk/care_and_support/holidaysspecialtreats.html.

Living Made Easy for Children



Living Made Easy for Children, is a website set up by the Disabled Living Foundation which offers information and advice on wheelchairs and other items for children with disabilities such as clothing, switches and toys (including disability awareness toys). For more information visit www.livingmadeeasy.org.uk/children

The Pleasure of Audio Books

The Calibre Audio Library is a free postal library of audio books for people with disabilities which prevent them from reading ordinary print. There are two sections, a general library and a special collection for children and young people up to 16 years old. Calibre offers a nationwide service of books recorded cover to cover. The special Young Calibre collection has 1400 titles for under-16s and members can also choose from the 6000 books in the general library. The collection includes the latest bestsellers, the most popular authors and classic fiction. There is nothing to pay—no subscription, no postage costs, no fines and no charge for lost or damaged books, although there is a small charge for print and CD-R catalogues for adults) For more information visit their general website www.calibre.org.uk or the section for young calibre members at www.youngcalibre.org.uk. You can also call **01296 432339** for more information.

What can a Disabled Facilities Grant do for you?

The Disabled Facilities Grant (DFG) is the local council grant intended to cover the cost of adaptations which is not means tested for disabled children. In England, it can be up to £30,000, in Wales, £36,000 and Northern Ireland, £25,000. To qualify for the grant, there has to be an agreement that the adaptations are necessary and appropriate and that the works are reasonable and practicable. Contacting social services or the occupational therapist (OT) with a request for a DFG assessment is not the same as making a formal application. To minimise delays, it is recommended that you fill in and submit the formal grant application form, available from your local authority, before the assessment is carried out. A local authority then has a duty to make a decision within six months of receiving your application. For more information visit www.direct.gov.uk/en/DisabledPeople/HomeAndHousingOptions/YourHome/DG_4000642

Family Fund Survey



The Family Fund are conducting a survey to find out whether it would be feasible to set up a new banking facility that would provide financial products and services designed specifically to meet the needs of families with

disabled children. They would welcome any views or ideas. To view, or complete the survey, visit <http://tinyurl.com/ygo42sy>

Fundraising



Since our last e-bulletin in December the NPDG (UK) has received just under £32,000 from donations, collection boxes and fundraising events organised by families and friends and we would like to thank all those involved.

As reported in the last e-bulletin Coleen and Maureen Nolan appeared in a special celebrity edition of Who Wants to Be a Millionaire and chose the Niemann-Pick Disease Group (UK) as one of their four nominated charities. For those of you who were unable to watch the programme, we are delighted to report that between them the sisters raised a total of £50,000 with £12,500 being donated to the Group. The Nolan Sisters also performed at a Charity Christmas Carol Service at a branch of Marks and Spencer in Blackpool—the donations were shared equally between the NPDG (UK) and the Alzheimer's Society. We are very grateful to the Nolan Sisters who have decided to support the Group after Denise Nolan, now one of our Patrons, met Calum Burdon, NPC aged 5, and his family at a fundraising evening organised by Campaign for Calum in Blackpool.

Recent fundraising events were organised by Maisy Dyer (Hope for Hollie) Barge Inn, Denton West End Primary School, Coral and Andy McLean, Lynne Valentine, Church of the Blessed Virgin Mary, Montgomery Primary School, Stowupland High School Year 10 pupils, Tony Jellings, Gwen Clark, Uplands Bowls Club and Abingdon School.

General donations were received from Janet Tungate, Warners of Wingate, The Lodge of Harmony No. 372 (Free and Accepted Masons), Mr Thornton-James, Mrs Lunt, Mrs Sumner, Nissan Employee Christmas Banking Fund, Mr and Mrs Walker, Mr and Mrs Peacock, Streatham Common Group Practice, Ian Newham, Mr and Mrs Elliott, Amanda Ball and Gloucester Minis.

We also received generous donations in loving memory of Ellie Sixsmith and Linda Mary Webb.

ANP Stores, Janice Brooks, Bill and Jane Owen and The Three Horse Shoes Pub donated the contents of their collection boxes.

We received £1,900 in 2009 Christmas Card sales and we would like to thank all who placed orders for cards last year.

Information about holding a fundraising event on behalf of the NPDG (UK) can be found on our website www.niemannpick.org.uk/get_involved/get_involved.html. You can also contact either Sue or Toni at the Central Office for more details or a fundraising pack.

We hope you have enjoyed reading this edition of the Family News Bulletin. If you have any articles, suggestions or comments for future editions of the Bulletin, or have received this e-bulletin by post and would now like it by email, please contact **Sue Lowe, 11 Greenwood Close, Fatfield, Washington, Tyne and Wear NE38 8LR**, or email at sue.npdg@tiscali.co.uk.

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