



**NIEMANN-PICK DISEASE  
GROUP (UK)**

**FAMILY NEWS BULLETIN**

**DECEMBER 2009**



Hello and welcome to the latest Niemann-Pick Disease Group (UK)'s Family News Bulletin. In this month's edition:

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

As we are all aware, life can be very challenging, as well as very rewarding, when caring for a child or loved one with NPC. With this in mind Actelion Pharmaceuticals have commissioned a survey to look at the emotional impact of this caring role. Insight Research Group, a leading UK based independent Healthcare Marketing Research Agency, will be carrying out the survey on behalf of Actelion. Insight would like to interview five carers, in face to face interviews, 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.

## Niemann-Pick Christmas Cards

We would like to thank all those who have purchased NPDG (UK) Christmas Cards this year, so far, a total of almost £800.00 has been raised.

## NPDG (UK) Family Directory 2010

Just a reminder, we will shortly be in the process of updating the Family Directory so could you please check your entry in the 2009 Directory and let us know if there are any changes or additions, such as email address, that need to be made. If you are not currently in the Family Directory and would like to be included, or you wish your entry to be removed, please let us know. Details of any changes should be emailed to [sue.npdg@tiscali.co.uk](mailto:sue.npdg@tiscali.co.uk) or posted to Sue at The Central Office address, (which can be found at the end of this bulletin) by 7th January 2010.

## NPDG (UK) on “Who Wants To Be A Millionaire?”



Coleen and Maureen Nolan recently recorded a special celebrity edition of “Who Wants to be a Millionaire?”. The Niemann-Pick Disease Group (UK) was one of three charities they chose to represent. To find out how much money they raised tune in to ITV 1 at 7pm on **Sunday, 20th December**.

## Online Shopping



If like millions of others you plan to do your last minute Christmas shopping online, you can help raise money for The Niemann-Pick Disease Group (UK) at the same time. By visiting [www.easyfundraising.org/uk/niemannpickdiseasegroupuk](http://www.easyfundraising.org/uk/niemannpickdiseasegroupuk)

## **NPDG (UK) Clinical Research Nurse – Liz Jacklin**

Liz Jacklin, our Clinical Research Nurse, has kindly agreed to provide a regular 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 [niemann-pick@zetnet.co.uk](mailto:niemann-pick@zetnet.co.uk), or to Liz direct [elizabeth.jacklin@cmft.nhs.uk](mailto:elizabeth.jacklin@cmft.nhs.uk).

*Liz writes: "On 1st December we hosted the first Research Meeting for families affected with Niemann-Pick Type C Disease. This was held at the new Royal Manchester Children's Hospital and was very well attended. We have so far received some very positive comments about the event; if you have not yet told us your thoughts about the meeting please do get in touch, we would be grateful for your feedback. We are aware that some of those who expressed an interest in attending were not able to do so, and again we would appreciate it if you could tell us your preferred day/venue for this type of meeting – perhaps a different location, or a different day/time in the week would be better for you – this will help us greatly in planning future meetings. All those who attended the meeting or who expressed an interest will shortly receive a feedback form and a copy of the presentations. These will also be available to all on our website – [www.niemannpick.org.uk](http://www.niemannpick.org.uk). The NPDG (UK) Central Office will also be distributing a questionnaire regarding children with NPC taking Curcumin, a subject that was discussed at the meeting. We are aware that a number of children are already taking Curcumin, and as no formal study has been undertaken into the effects of this, it would be of great benefit if we could collect some information from families who are using it. We would like to collate this information and to share it with members of the medical profession who have an interest in NPD and other Lysosomal storage disorders.*

*I would also like to reiterate that the NPDG (UK), and the Medical Professionals who participated in the meeting, cannot give their approval to you giving any substance to your child without prescription, such as Curcumin. We would urge you to ensure that the product has been manufactured for pharmaceutical use and to be fully aware of any potential side effects before you start to use it. Giving any substance to your child without prescription, and correct medical guidance, is potentially very dangerous, and the full implications of this should be understood before you decide on this course of action. If you have any questions or concerns in this area, please do contact us, as we are here to support you and your family in the care of your loved one.*

*There continues to be much work going on in the field of research into Niemann-Pick Diseases, with developments regarding Cyclodextrin, chaperone therapy, NAC and the biomarker study for NPC, and we also hope to hear more news about the phase 2 study in NPB in the near future. You can view the papers from the Annual Ara-Parseighan Medical Research Foundation Conference, which was held earlier this year and looks specifically at NPC, on our website. We share your frustration at the length of time that research studies take - but we want to assure you that we are involved at the very heart of everything that is going on and our aim is to share this information with you through this bulletin, our newsletter and website.*

*As always, if you have any questions about the projects mentioned above, or any other aspect of research, please do not hesitate to get in touch.*

*Wishing you all a very happy and peaceful Christmas, and a bright New Year."*

Liz, NPDG (UK) Clinical Research Nurse

## NPDG (UK) Supports Adapted Trolley Campaign



Julie, a mum from Devon has successfully persuaded her local Sainsbury's to purchase a specially adapted trolley seat for disabled toddlers and she is now calling for every supermarket to stock the adapted trolley. Julie had struggled to push a trolley and her 3 year old daughter Rose in her wheelchair every time she went shopping so she approached her local Sainsbury's and explained the difficulties she was experiencing—the Customer Services Manager, Katie Southworth agreed to order a specially adapted trolley. Julie has now set up a Facebook petition group **Every Supermarket Needs this New Disabled Trolley** to support her campaign. You can sign up to this campaign by visiting [www.facebook.com:80/group.php?gid=180123000906&ref=mf](http://www.facebook.com:80/group.php?gid=180123000906&ref=mf).

## Our Family, Our Future

Families with disabled children have the same hopes and dreams as other families but often face bureaucracy and prejudice trying to achieve them. These are the findings of a new Contact a Family report "Our family, Our Future" which features the stories of 30 UK families whose children are affected by a range of disabilities and rare conditions. All families show strength, determination and creativity in overcoming the challenges and pressures they face and there is a real sense of overwhelming love and pride for their children. Contact a Family would like to see families with disabled children given the same opportunities as others and is calling for the Government to invest in a campaign to raise awareness of the needs of families with disabled children with a stronger focus on disability awareness taught in early years and schools and people working in a public facing role to be given disability and equality training - so that they can recognise the unique talents that children bring - because of their disability. The "Our family, Our Future" report is accompanied by an exhibition of photographs which will be touring the UK. To view the full report please go to [www.cafamily.org.uk/pdfs/ourfamilyyourfuture.pdf](http://www.cafamily.org.uk/pdfs/ourfamilyyourfuture.pdf)

## Taking Control



In Control is a social enterprise that was set up to transform the current social care system into a system of Self-Directed Support. Taking Control; part of In Control, is piloting this support to children, young people, their families and services. For the past two years a growing number of children's services have taken up the challenge of developing individual budgets for children and young people. With over 1,000 live individual budgets across different groups this work is beginning to move from the pilot to roll out. If you are interested in finding out more and want to join with 35 other services (including health services) in developing individual budgets; or if you are a family member who wants to hear from other families about the difference it makes to them, then email [nic.crosby@in-control.org.uk](mailto:nic.crosby@in-control.org.uk) or visit [www.in-control.org.uk:80/site/INCO/Templates/GeneralChild.aspx?pageid=440&cc=GB](http://www.in-control.org.uk:80/site/INCO/Templates/GeneralChild.aspx?pageid=440&cc=GB).

## **New Communication Champion for Children with Communication Needs**

Jean Gross has been announced as England's first Communication Champion. This appointment was a recommendation of the 2008 Bercow Report on services for children with speech, language and communication needs, and forms part of the government's Better Communication Action Plan developed in response to this review. The Communication Champion will be independent of Government and will play a key role in promoting the importance of communication skills to children and in helping make a success of the action plan commitments.

## **New Blue Badge Facilities Online**

Directgov's online map of parking facilities for Blue Badge holders now includes a rail map feature, with accessibility information for more than 2,000 train stations. This will enable people who have a Blue Badge to plan journeys in the UK more easily and book direct assistance in advance. The map has been expanded to feature accessibility information for more than 160 major football stadiums. This should help Blue Badge holders, their friends and family have a better experience at the game, whether home or away! The mapping service has been developed by Directgov's Disabled People and Caring for Someone franchise, part of the Office for Disability's Communications Team. Go to [www.direct.gov.uk/en/DisabledPeople/MotoringAndTransport/Bluebadgescheme/DG\\_10038295](http://www.direct.gov.uk/en/DisabledPeople/MotoringAndTransport/Bluebadgescheme/DG_10038295)



## **Autoadapt**

Autoadapt is a company that offer award-winning, safety tested solutions for adapting your vehicle, to increase your independence and regain your freedom. Their premium products Turny, Turnout and Carony are innovative solutions for getting in and out of your vehicle. For further information see their website [www.autoadapt.com](http://www.autoadapt.com).



## **An Introduction to Stem Cells—New Booklet**

Stem cells hold much promise for the development of novel treatments for many serious medical conditions. This booklet, written by Jess Buxton and published by the Galton Institute in association with Progress Educational Trust, provides an introduction to the science, ethics, regulation and future applications of stem cell research. If you would like a free copy of this booklet, or the previous booklet in the series 'A Guide to Pre-implantation Genetic Diagnosis', please send a self-addressed envelope with a 'large-letter' stamp to: **Progress Educational Trust, 140 Gray's Inn Road, London, WC1X 8AX.**

## **Saving Scheme for Parents of Disabled Children**

The government has included families with disabled children in their plans for a savings scheme called the 'Saving Gateway'. From 2010 the savings scheme will be open to people of working age on low incomes and in receipt of specified benefits, including



Carer's Allowance. For every pound saved the government will give a contribution of 50p. Savers will be able to pay up to £25 a month into their account for two years, which means a potential contribution from the government of £300 after the two year period. For more information visit [www.direct.gov.uk/en/MoneyTaxAndBenefits/ManagingMoney/SavingsAndInvestments/DG\\_10010450](http://www.direct.gov.uk/en/MoneyTaxAndBenefits/ManagingMoney/SavingsAndInvestments/DG_10010450).

### **Contribute to a New Special Needs Handbook**

If you are a parent of a child with special needs and have a tip you would like to share with other parents and professionals you can contribute to a new Special Needs Handbook which is being compiled. The Special Needs Handbook will be available freely over the internet and will contain useful tips, ideas and solutions for day-to-day problems encountered by people living with, or supporting a child or adult with a disability or special needs. To send your tips or for more information about this project e-mail: [mail@specialneedshandbook.com](mailto:mail@specialneedshandbook.com).

### **Direct Payments for Children's Healthcare**

The Government is consulting on proposals to pilot direct payments for health care. The Health Bill would provide power to allow direct payments, where the individual would be given the money to buy their own health care as agreed in a care-plan, much like direct payments in social care. The main points for disabled children and their families are; proposals would apply to all conditions where direct payments are used, including children; those with parental responsibility could hold a direct payment on children's behalf, with parental/guardian consent and PCTs would be advised on how to tailor support to individual or group needs: such as learning disabilities, communication difficulties, or those in transition between children's and adult services. To view the consultation document visit [www.dh.gov.uk/prod\\_consum\\_dh/groups/dh\\_digitalassets/documents/digitalasset/dh\\_107451.pdf](http://www.dh.gov.uk/prod_consum_dh/groups/dh_digitalassets/documents/digitalasset/dh_107451.pdf). If you wish to give your opinion on these proposals you can download a form by visiting <http://www.cafamily.org.uk/pdfs/92B1DEFB.pdf>. All comments need to be submitted by 8th January 2010.

### **Custom-Made Equipment**



MERU is an organisation that produces custom-made equipment for children and young people with disabilities, when nothing else exists to meet their needs. They have recently started an outreach project linking volunteers with people who need small uncomplicated items and fixing broken equipment. Contact MERU [www.meru.org.uk](http://www.meru.org.uk) for more details. There is a charge for their larger projects which they expect to be met by the NHS or Local Authorities.

### **Opportunity for Young Disabled People to Have Their Say**



Whizz-Kidz is a charity that is all about giving disabled children the chance to lead a more independent life. Their service meets individual mobility needs and ensures the right mobility equipment, advice and training is provided at the right time. They are carrying out a questionnaire asking young

disabled people about their school life, including: school trips, teaching assistants, PE and work experience. To take part in the questionnaire [www.whizz-kidz.org.uk/getinvolved/nationalschoolsconsultation](http://www.whizz-kidz.org.uk/getinvolved/nationalschoolsconsultation)— the closing date is Friday 15 January 2010.

## Disabled Children's Manifesto

Disabled children and young people, in partnership with Every Disabled Child Matters and the Council for Disabled Children, have produced a manifesto called "Disabled Children's Manifesto for Change". The manifesto challenges political parties to set out their policies on increasing respect, inclusion and participation for disabled children. To view the report and a film which accompanies the manifesto visit the website [www.edcm.org.uk/manifesto](http://www.edcm.org.uk/manifesto).

## Back By Popular Demand



We now have supplies of the yellow and white Niemann-Pick wristbands in the office. These are available for a suggested donation of £1.00 each. If you need any supplies, please contact either Toni or Sue at the central office.

## Fundraising



The Niemann-Pick Disease Group (UK) would like to thank all our families and friends who have worked so hard to raise money for the Group throughout 2009. Remember, it doesn't matter how small or large the donation, every penny helps the NPDG (UK) to make a positive difference to the lives of those affected by Niemann-Pick Disease.

Since our last e-bulletin in September, the NPDG (UK) received just under £26,000 from donations, collection boxes and fundraising events organised by families and friends and we would like to thank all those involved.

Recent fundraising events were organised by Paul Bayliss and Pear Tree Inn Golf Society (Hope for Hannah). Freckleton Sports and Social Club (Campaign for Calum). Three Horseshoes, Jamie Perkins, Andy Munden, Denise and Roy Lodger, Mrs G Clark, Helen Catterall and Jeremy Tipper and 1st Dane Bank Beaver Colony. Full details of these fundraising events will be featured in the next edition of Niemann-Pick News which will be distributed by the end of March 2010 and can also be viewed on our website.

General donations were received from Mr and Mrs Smith (Hope for Hollie), West End Football Club (Campaign for Calum); Denis Burn and Mr Kemp (Hope for Hannah); Mr and Mrs Akooji, Mrs Sumner, Mr and Mrs Holland, Mrs A Valentine, Melanie and Karl Evans, Mr and Mrs Grainge, Sadie Ridley, John and Sheila Finch, Shirley and Val Ridley, The Reverend John Godfrey, John Fairhurst and Co, Mr and Mrs Dalglish, Jackie Colquhoun, Ena Malcolm, Kim Gomes

and Bhrat Getalal.

Billy, The Barge Inn and John and Blaikie all donated the contents of their NPDG (UK) collection boxes.

We have also received a generous donations "in loving memory" of Roger Franklin, Ellie Sixsmith, Myra Scott, Ethel Edwards, Sky Davidson and Jean Raven.

If you would like information about holding a fundraising event, please contact Sue or Toni at the Central Office on **0191 4150693** or email **niemann-pick@zetnet.co.uk**

**And Finally .....**



*.....from the Trustees and Staff of the Niemann-Pick Disease Group (UK), we would like to send you our warmest wishes for a very Happy Christmas and a good New Year.*



**PLEASE NOTE: The NPDG (UK) Office will be closed from Monday 21st December to Monday 4th January 2010. If you need to speak to one of us during this time, please do leave a message on the answerphone and we will call you back as soon as possible. If your enquiry is urgent, please call Toni Mathieson on 07816 398591.**

We hope you enjoyed reading this edition of the Family News Bulletin. If you have any articles, suggestions, announcements or comments for future issues of the Family News Bulletin which you would like included, please send them to **Sue Lowe, 11 Greenwood Close, The Pastures, Fatfield, Washington NE38 8PD** or by email to **sue.npdg@tiscali.co.uk**.