



*NIEMANN-PICK DISEASE  
GROUP (UK)  
FAMILY NEWS BULLETIN  
February 2011*

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

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## Save the Date –Conference 2011



Our Annual Family Conference 2011 will once again be held at the Robinson Executive Centre, Wyboston Lakes, in Cambridgeshire, a venue which proved very popular with last year's delegates. The Conference will be held over the weekend of 23rd-25th September. Full details will be available on our website soon.

## Niemann-Pick Christmas Cards



We would like to thank all those who purchased NPDG (UK) Christmas Cards in 2010, raising just under £550.

## The NP-C Natural History Study.



The NP-C Natural History Study at the National Institutes of Health (NIH), Bethesda, Maryland, USA, was launched in July 2006. This successful study, headed by Dr. Forbes "Denny" Porter, relies on the continuing participation of affected families from the USA and the UK in order to gather much needed data regarding NP-C. Families that have taken part are very pleased with their experience and are complimentary of Dr. Porter and Study Nurse Nicole Yanjanin, as well as the many specialists who conduct the numerous tests over the course of their 3-5 day stay. The study is still recruiting patients from both the USA and the UK. For NP-C families/patients from outside of the United States, the NIH will cover travel expenses from a port city in the US to Washington DC, make arrangements for ground transportation to and from the airport to the NIH, and will also accommodate the family in the Children's Inn on the NIH campus or another close-by facility free of charge. The family would be required to pay for their own round-trip flights from their homes in the UK to the port city in the USA. To help with this, the **Hope for Hollie Campaign** has established a **Travel Fund** to assist families with this expense. Helen Carter, co founder of the Hope for Hollie Campaign explains: *"As a family, we are only too aware, having taken part in the NIH study, how much of a financial burden it can be on families not only to cover the cost of flights but to cover spending costs (however minimal) and if necessary unpaid leave from work. We also understand how vital it is that all families are given the opportunity of having access to these research programmes. We are therefore delighted that in collaboration with the NPDG (UK) we have been able to set up this fund. We hope it will not only help families but also help research going forward."* If you are interested in finding out more about the NIH study or using the travel fund please contact Toni Mathieson at the Central Office by telephone 0191 415 0693 or email [niemann-pick@zetnet.co.uk](mailto:niemann-pick@zetnet.co.uk). Alternatively, you can email Nicole Yanjanin, Study Nurse at the NIH [nyanjanin@mail.nih.gov](mailto:nyanjanin@mail.nih.gov)

## NPDG (UK) Family Directory 2011

Just a reminder, we are in the final stages of producing the 2011 Family Directory so we would be grateful if you could please check your entry in the 2010 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 **28th February 2011**.

### **Cerebra Grant Scheme**



Cerebra has increased their budget this year to give more grants to help children who have disabilities because of a brain related condition or injury. The condition may be of a physical nature or a learning difficulty or both. The equipment or resource requested must improve the child's quality of life and not be available from statutory agencies like social services or the NHS. The child also must be aged 16 or under. Some examples of grants made are touch screen computers, holidays, approved therapies, power wheelchairs, and sensory toys.

Parents and carers can request an application form by calling **01267 244200**, download one from their website at [www.cerebra.org.uk](http://www.cerebra.org.uk) or by e-mail: [debbieg@cerebra.org.uk](mailto:debbieg@cerebra.org.uk) Applications are accepted throughout the year but allow 4-6 weeks for a decision.

### **The Saga Respite for Carers Trust**



The Saga Respite for Carers Trust aims to give carers a break from their caring responsibilities by providing free holidays for carers and a companion and ensuring their loved ones receive care whilst they are away. If you would like to be considered for a break you can nominate yourself. Nominations opened in January 2011. Nominees who were unsuccessful in 2010 will be able to re-apply for 2011. To qualify as a nominee, you must be over 50, have been caring for someone for more than a year, not taken a holiday away from your caring responsibilities within the last year and not be a professional carer. You can apply yourself or nominate someone you think deserves it online at [www.saga.co.uk](http://www.saga.co.uk) or if you have no access to the internet you can obtain a nomination form by sending a stamped, self-addressed envelope to: The Trust Manager, Saga Respite for Carers Trust, The Saga Building, Enbrook Park, Folkestone, Kent CT20 3SE.

### **Job Centre Plus Support for Carers**



If you are caring for someone and want to combine paid work with your caring responsibilities, there is a variety of help available from your local Jobcentre Plus. This can include replacement care and benefits advice as well as help and advice for you to make a successful move into work. Even if you don't feel ready to work now but might like to in the future, you can still take advantage of Work Focused Support for Carers (WFSC). It is a voluntary service which you can choose to take part in at any time. You can also stop taking part at any time.

You will receive help and support from a Jobcentre Plus personal adviser. This might include: help preparing for work; help to find suitable training; support in looking for and applying for a job. You will also receive advice on finding replacement care while you attend appointments with the personal adviser, take part in a training course or attend job interviews.

You do not need to be participating in WFSC to access funding for replacement care. WFSC is available to carers who: do not work or work less than 16 hours a week; are aged 18 or over or are not able to get help from any of the other Jobcentre Plus employment support programmes such as New Deal or Pathways to Work. To find out more about Work Focused Support for Carers, contact your local Jobcentre Plus office.

## Cold Weather Payment



Cold Weather Payments (not to be confused with winter fuel payments for over 60s that are automatically paid every year) are cash boosts (for paying energy bills) for those on certain benefits when the weather gets especially cold. You get £25 per week if the temperature drops to below zero Celsius (or is predicted to) for seven consecutive days between 1 November and 31 March.

Depending on your circumstances you may be eligible for a Cold Weather Payment for each week of very cold weather in your area if you are getting a disability premium, enhanced disability premium or severe disability premium; a disabled child premium; Child Tax Credit that includes a disability or severe disability element or a child who is under five in the family. Cold Weather Payments will not affect any other benefits you may be getting and you don't need to apply; you'll get a Cold Weather Payment automatically as long as you qualify. You can find out if your area is due to receive a Cold Weather Payment by doing a postcode search on the Directgov website <http://www.direct.gov.uk/en/index.htm>. If you think you should have received a Cold Weather Payment but you haven't had one you should get in touch with Jobcentre Plus. For more information on Disability Premiums please visit [www.niemannpick.org.uk/care\\_and\\_support/benefits.html](http://www.niemannpick.org.uk/care_and_support/benefits.html).

## The Family Care and Interactive Support Project



**LOTTERY FUNDED**

In our spring edition of Niemann-Pick News we told you about the Family Care and Interactive Support Project, supported by a grant from the Big Lottery Reaching Communities Programme, which has given the NPDG (UK) an exciting opportunity to provide an improved and more efficient support and advice service to those affected by the Niemann-Pick diseases. The overall aim of the project is to improve outcomes for families with Niemann-Pick diseases, through the use of interactive technology that will enhance access to support and information services. Through the use of video cameras, webcams and media links, families will have increased access to expert care and advice provided by NPDG (UK)'s Clinical Nurse Specialist for Niemann-Pick Disease, Jackie Imrie, and also the ability to communicate with other families within the Niemann-Pick community.

The pilot scheme for this project is now underway with six families enrolled at present. If you would like to know more about this project, or if you would be interested in participating, please contact Toni Mathieson at the NPDG (UK) Central Office by telephone 0191 415 0693 or email [niemann-pick@tiscali.co.uk](mailto:niemann-pick@tiscali.co.uk)

## Genzyme ASMD Clinical Trial



We have received the following statement from Genzyme regarding the ASMD Clinical Trials:

*“Genzyme is continuing efforts to develop recombinant human acid sphingomyelinase (rhASM) for the potential treatment of the non-neurological manifestations of acid sphingomyelinase deficiency (ASMD, also known as Niemann-Pick disease Types A and B). After completing the Phase 1 clinical trial in 2009, we engaged regulatory authorities in discussion about plans for a Phase 2 clinical trial and conducted additional preclinical research in 2010. This regulatory dialogue is ongoing. We remain committed to the development of a therapy for ASMD and will keep the community informed once our regulatory discussions are complete and we can confirm a start date for the Phase 2 clinical trial for Niemann-Pick B patients.*

*We are pleased to note that an abstract from the Phase 1 clinical trial was selected for a podium presentation by Dr. Margaret McGovern at the 7th annual lysosomal disease network's WORLD conference, to be held in Las Vegas, February 16-18, 2011.”*

## Have you joined the ‘Hope for Hollie’ Forum?



The ‘Hope for Hollie’ Campaign e-forum enables families affected by NPD to share their thoughts and experiences of living with Niemann-Pick disease. All aspects of the disease can be discussed, with contributions from families of people affected, carers, experts, and also discussion about fundraising activities. New discussion topics have recently been added—so why not sign up and join the debate about research, aids and adaptations, fundraising and much more. If you would like to add any topics, please let us know. To join, visit

[www.hopeforhollie.co.uk](http://www.hopeforhollie.co.uk) or follow the link on the NPDG (UK) Website – [www.niemannpick.org.uk](http://www.niemannpick.org.uk) .

## UK charity ACT launches Family Companion Resource For Parents Caring for a Life-Limited Child

ACT, the UK children’s palliative care charity has updated its popular booklet, A Family Companion to the ACT Care Pathway, which is now available free to family members of children with life-limiting and life-threatening conditions, and children’s palliative care professionals. To make this resource as accessible and as widely available as possible, ACT has produced the Family Companion in a range of different formats. For more information visit the website [www.act.org.uk/familycompanion](http://www.act.org.uk/familycompanion)

## Family Fund



To make sure they are helping families who are on the lowest of incomes and in greatest need, the Family Fund will be making a change to its income criteria and will now be guided by a family’s entitlement to government financial support in the form of benefit and tax credits.

This means that they will now consider a grant application from any family caring for a disabled child or young person aged 17 and under in England, Northern Ireland, Scotland and Wales, where they are eligible for, and can send evidence of entitlement to, one the following: Child Tax Credit, Working Tax Credit, Income based Job Seekers Allowance, Income Support, Incapacity Benefit, Employment Support Allowance, Hous-

ing Benefit and Pension Credit. Families must still however meet all other eligibility criteria in order to be eligible. For more information visit website [www.familyfund.org.uk](http://www.familyfund.org.uk) or call **0845 130 4542**

### **Rare Disease Day—February 28th—Would you Like to Share Your Story?**



To mark Rare Disease Day, EURORDIS is organising an event in Brussels on February 28th. The "Rare Diseases and Health Inequalities in Europe" Symposium will bring together key people involved in the field of rare diseases in order to raise the issue of rare diseases and health inequalities. Those attending will include patients and patient representatives, European federations, health professionals, social researchers and academics working in the field of rare diseases, members of the EU Committee of Experts on Rare Diseases, industry representatives as well as high-level officials from the European Medicines Agency and the European Commission's Health and Social Affairs General Directorates. The programme will be devoted to presenting the rare disease landscape in Europe and showing existing inequalities through patient testimonies, case studies and surveys. If you have a story that illustrates health inequalities for rare disease patients, please send an email to: [rarediseaseday-stories2011@eurordis.org](mailto:rarediseaseday-stories2011@eurordis.org)

### **Rare Disease Patients are Getting Lost in the System**



Over the summer of 2010, Rare Diseases UK (RDUK) carried out one of the largest surveys of patients and families affected by rare diseases in the UK. The conclusions of this study are alarming and will be used to launch a report outlining RDUK's recommendations on a national strategy for rare diseases in the UK on Rare Disease Day 2011. For more information on this report go to on <http://www.eurordis.org/content/new-survey-rare-diseases-uk>.

### **Have Your Say—Take Part in the Annual Carers' Survey**

Every carer in the UK is invited to take part in the annual carers survey, just launched, and the results of which will form the core of Carers Week 2011, being held from 13-19 June 2011. You can take part in the survey by going to [www.surveymonkey.com/s/carersweek2011](http://www.surveymonkey.com/s/carersweek2011)

### **Disabled Children at the Heart of Newlife**



Newlife Foundation for Disabled Children is a national charity, founded in 1991, that specialises in making life better and brighter for disabled children and their families. Newlife achieves this through its services and funding of 4 key areas of action: **Nurse Services** a free phone National Helpline, **0800 902 0095**, staffed by a qualified team of Nurses who listen, advise and offer help and support to all who contact them. **Equipment Grant Services** where Newlife Nurses become the access point for families applying for a grant for equipment relevant to their child's disability. One free phone call can result in completion of a simple application form for one of Newlife's Equipment Grants or signposting to another funder.

Newlife Equipment Grants are non-means tested and often a decision on equipment can be made in days. A new equipment loan scheme '**Just Can't Wait**' started in Jan 2011, and it is targeted to specifically help children with terminal/life-limiting conditions, where the need for equipment at home is urgent. **Medical Research:** Newlife has spent £11 million on research in the last 19 years; **Campaign and Awareness:** Newlife Foundation's campaign and awareness activities help to give families a voice on important issues.

All this help starts with a phone call to the Newlife Nurse Helpline **0800 902 0095** which is available Monday - Friday 9.30am-5pm and Wednesdays until 7pm. Live Nurse Chat is available during office hours at [www.newlifecharity.co.uk](http://www.newlifecharity.co.uk). Newlife ask for no subscriptions or memberships and all services are free.

### Family Action's Welfare Grants



Family Action's Welfare Grants are available for clothing and general household needs such as beds and cookers but they can also help with more varied needs such as communication aids and medical adaptations. While there are no set limits, in general grants are made for £100 to £300. You can apply for a Welfare Grant between the following dates each year: 1-31 March; 1-30 June; 1-30 September and 1-31 December. The grants are available for adults and children but we would suggest you visit <http://www.family-action.org.uk/section.aspx?id=8304> to check your eligibility.

### Fundraising

For details of our fundraisers and forthcoming fundraising events look out for our new Fundraising Bulletin coming soon.

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](mailto: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.**

