Newsletter

Winter 1995

The Society for Mucopolysaccharide Diseases

National Registered Charity No. 287034

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The Society for Mucopolysaccharide Diseases

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The MPS Society is a voluntary support group, founded in 1982, which represents over 700 families in the UK with children or adults suffering from Mucopolysaccharide and related diseases. It is a registered charity, entirely supported by voluntary donations and fundraising by members, and run by the members themselves. Its aims are:

- ☐ **To act as a parent support group**
- ☐ **To bring about more public awareness of MPS**
- ☐ **To promote and support research into MPS**

The Society operates a network of Area Families throughout Great Britain and Northern Ireland, who offer support and links to families in their areas. It provides an information service for families and professionals. At the present time it supports two specialist MPS clinics at the Royal Manchester Children's Hospital and at the Hospital for Sick Children, Great Ormond Street, London. The Society also funds three biochemists, one at Manchester Children's Hospital, one at the Christie Hospital, Manchester, and one at the Institute of Child Health, London. It encourages and assists contact and co-operation between parents and professionals and maintains links with sister societies in Europe and throughout the world.

There is at present no cure for MPS diseases, but much can be done to improve the treatment and care of sufferers. The slogan of the Society is:

"Care today, hope tomorrow"

Front Cover: **Caroline Sweeney** from Caerphilly accepts her present from a very shy Father Christmas at the Welsh Christmas Party. Caroline is fourteen and has MPS IV (Morquio).

Contents

Deadline for Spring Newsletter

30th March 1996

Directors Report

As this is the first MPS Newsletter for 1996 we all wish our readers a belated Happy New Year. We would like to express our thanks for the numerous Christmas greetings we received. Your cards certainly brightened up the MPS office in the weeks leading up to Christmas. May **Sheila, Joan, Sue and I in the MPS office in Amersham and Mary and Pam in the Northern office** return these greetings to you and express our thanks for your continued support of our work.

1995 has been exceptionally busy with the families of 45 newly diagnosed children seeking our support and advice over the past year. The demand for help with "know how" on adapting houses, welfare benefits and education continue to be high in priority of the telephone and written requests received in the MPS offices.

From talking to many of the Area Support Families the Christmas parties seem to have been even better attended than previous years and very successful. On behalf of all the MPS members I would like to say a very big thank you to all the families who quietly in their areas are there for other MPS families in the area as well as organising local Family Days.

I would also like to thank our elected Trustees who have the task of deciding MPS policy, seeing that it is implemented and of course managing the finances. In 1994/1995 this has not always been easy due to the considerable drop in income and the need to make ends meet in 1995/96. Later in this newsletter the Trustees explain some of the major decisions taken at the November budget meeting.

I am pleased to say that with your support in encouraging donations and fund-raising I believe that we can look forward to a better year financially in 1996. To help us on our way as many of you will have seen in the National press, the Society was awarded over £64,000 by the National Lottery Charities Board. This money will underpin the Society's commitment to MPS Family Holidays and MPS Teenage Activity Holidays in 1996, 1997 and 1998.

Although the cheque is not in the bank yet we have also with the help of **Edward Hodgkins** (Mary Gooch's brother) secured a donation of £17,000 from the Al Fayed Charitable Trust to meet the cost of the MPS research project 'Gene Therapy for Hurler Disease'.

We recently tried very hard for a major donation from LIFFE (those working in the banking world will know of this). Although we were not successful this time we will try again in November. However without the help of MPS parents, **Jackie Chisling and Neil Pickard** we would have been unable to "pass go" let alone collect the £50,000.

So many of you are helping in your own way and we want to say thank you and anytime the opportunity arises 'Think MPS'.

As some of you know Joan will be leaving the employment of the MPS Society in the New Year to live in Cyprus where her husband has been posted. Replacing Joan in proving difficult and we will miss her very much but wish her well for the future.

Finally I know you would want to endorse our support and understanding for what is a very difficult time for Sheila Duffy, who works in the MPS office, her husband, John and young daughters, Natalie and Michelle. One week before Christmas their home was gutted by fire. Thankfully they escaped unharmed physically but having lost everything they are now trying to pick up the pieces.

May I wish you all a peaceful New Year.

Christine Lavery

Decisions from Trustees Budget Meeting 4th November 1995

Area Family Training

This will take place 1st - 3rd March 1996 at Glenbrook, Hope, Derbyshire. The cost is budgeted at £3000. Two thousand pounds has been achieved in a grant from NCCVO with the understanding that the training will address "Disability and the Children Act".

MPS Family Holiday

Seventeen family chalets and three volunteer chalets have been booked for the week of Saturday, 27th of July - 3rd of August 1996 at Primrose Valley in Filey, North Yorkshire. The cost of the holiday per family is £250.00, this represents less than 50% of the true cost of providing the holiday. A grant from the National Lottery will go towards the subsidy.

MPS Activity Holiday

Fifteen places are available for physically disabled MPS teenagers and young adults. The holiday will take place at the Bendrigg Trust, Kendal, Cumbria from Sunday 7th of April - Saturday 13th April 1996. The contribution towards the cost of the Activity Holiday is £100.00. This includes full board and activities, but not travel to Kendal. The balance is being met by a grant from the National Lottery.

International Collaboration

International Symposium on MPS and Related Diseases, 24-27th of May 1996, Wollongong, Australia. It was accepted by the Trustees that no economical group fare deal offered by BA or Quantas came close to matching the very competitive "bucket shop" prices available and therefore MPS families wishing to travel to this conference would be best advised to make their own arrangements. The Trustees agreed that the number of professionals and MPS families planning to attend this conference warranted both Christine and Mary travelling to Australia. Christine's Jerwood Award prize of £1000 will go towards the cost and a further grant is being sought from Manager International.

Sweden and Holland

Christine has been invited to speak at the first Swedish and Dutch MPS conferences and to share her experiences of MPS. The cost of these two weekend conferences will be shared between respective conferences and money granted by CSL for this purpose.

MPS Family Conference, 20 - 22nd of September 1996

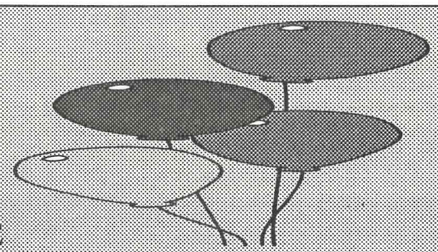
Stakis Country Court Hotel, Northampton.

The MPS conference programme and booking forms are enclosed with this newsletter. If last year is anything to go by, **early booking is advised.** The Trustees considered the conference budget very carefully and reluctantly agreed an increase in the contribution for family delegates in line with a policy of 50% subsidy. This is necessary because there is no financial underpinning for the conference. All the Trustees recognised that for some families, even with a subsidy, the cost of holidays or conference may be prohibitive. If you are concerned about meeting these costs but would like to participate, please do contact the MPS office, preferably in writing and we will consider where help towards these costs can be obtained.

The budget set for 1995/96 stands at over £250,000. This means that unless we can achieve an income of this amount, excluding "Jeans for Genes" money, we will incur a deficit for three years in succession.

On a more cheery note, we have achieved some significant goals already for this financial year, with major grants from the National Lottery Charities Board, the Department of Health (London) the Department of Health (Belfast) and Al Fayed Charitable Trust. With your support over the coming year I am sure we can meet the challenge.

Alf King
Chairman of Trustees



MPS FAMILY CONFERENCE

WE HOPE THERE IS SOMETHING FOR EVERYONE AT THIS YEAR'S CONFERENCE SO PLEASE COMPLETE THE ENCLOSED BOOKING FORM FOR THE 1996 MPS CONFERENCE AND RETURN IT TO US AS SOON AS POSSIBLE.

New Families

Mrs Sharon Birch from Rochester, whose son, **Thomas** born on the 2nd of December 1992 has been diagnosed with Hunter Disease.

Samantha and Richard Griffiths from Swansea, whose son, **Benjamin** has a storage disease with MPS features. Benjamin was born on the 5th May 1993.

Cathy and Andy Flaig from Kent, whose son **Thomas**, born on the 2nd of January 1994 has been diagnosed with Sanfilippo Disease.

Jasmin and Curtis McLaren-Hall whose son **Matthew**, born on the 17th of December 1993 has been diagnosed with Hunter Disease.

Sally and Karl Cooke from Hitchin, Herts., whose son **David**, born 20th of May 1995 has been diagnosed with Hurler disease.

Deaths

David Farrington, husband of Barbara and father of Mark who had Hurler disease and who died following bone marrow transplant in 1882, died in October 1995. The family live in Oldham, Lancs.

Karen and Michael Wheeler's daughter, **Rhianneth** from Surrey, died on the 23rd of November 1995 aged three years. Rhianneth suffered from Mucolipidosis II.

Carol Phee and Andrew Martin's daughter, **Ashley** from Fife, died on the 8th of December 1995, aged 8 years. Ashley suffered from Hurler disease.

Congratulations

To Mark Fitzgerald from Luton on passing his driving test. Well done Mark!

"Jeans for Genes" Day, Friday 1st of March 1996

GO TO WORK (OR SCHOOL) WITHOUT YOUR TROUSERS ON .....

.....but wear your Jeans instead.

MPS is joining forces with three other charities in what promises to be a massive national fund-raising event for pioneering genetic research. The aim is to raise at least **£2 million** and to generate hope that inherited conditions will finally be eliminated.

This exciting venture is called **Jeans for Genes** and it is something that everyone can take part in.

What do you have to do?

Quite simply, wear your jeans on **Friday the 1st of March 1996** - and pay a small amount for the privilege!

Jeans for Genes aims to see jeans worn in offices, banks, factories, supermarkets, playgrounds, schools, universities, streets, churches, Buckingham Palace, the Houses of Parliament - in fact, wherever people happen to be on the **1st March 1996!**

Organisations from the biggest city banks to the smallest shops can encourage their staff to wear jeans on **Jeans for Genes Day**.

We are delighted that Tesco is also supporting **Jeans for Genes** day, and you will see promotional material in all 519 of their supermarkets too. If you live near a Tesco supermarket do go and introduce yourself and explain what **Jeans for Genes** and research for MPS means for your family.

The other charities involved in the project are the CGD research Trust, Primary Immunoeficiency Association and the Great Ormond Street Children's Hospital Research Fund. Each will receive 25% of the proceeds from the appeal. From January MPS Trustees will be inviting grant proposals for genetic research that may help or lead to Gene Therapy and Enzyme Replacement therapy for the Mucopolysaccharide and Related Diseases.

So whilst asking you to zip into your **jeans** and see what you can achieve. May I also remind you that this money can't be used for any of our support activities including conference, holidays, advocacy work in area of education, housing and benefits, family support and MPS clinics. We still need to raise £200,000 for this work.

Ron and Linda Snack

As most of you will know we retired at the end of year as an Area Support Family and our last event was the Christmas Party, jointly arranged with Richard Millward at the Army camp at Kineton, near Banbury.

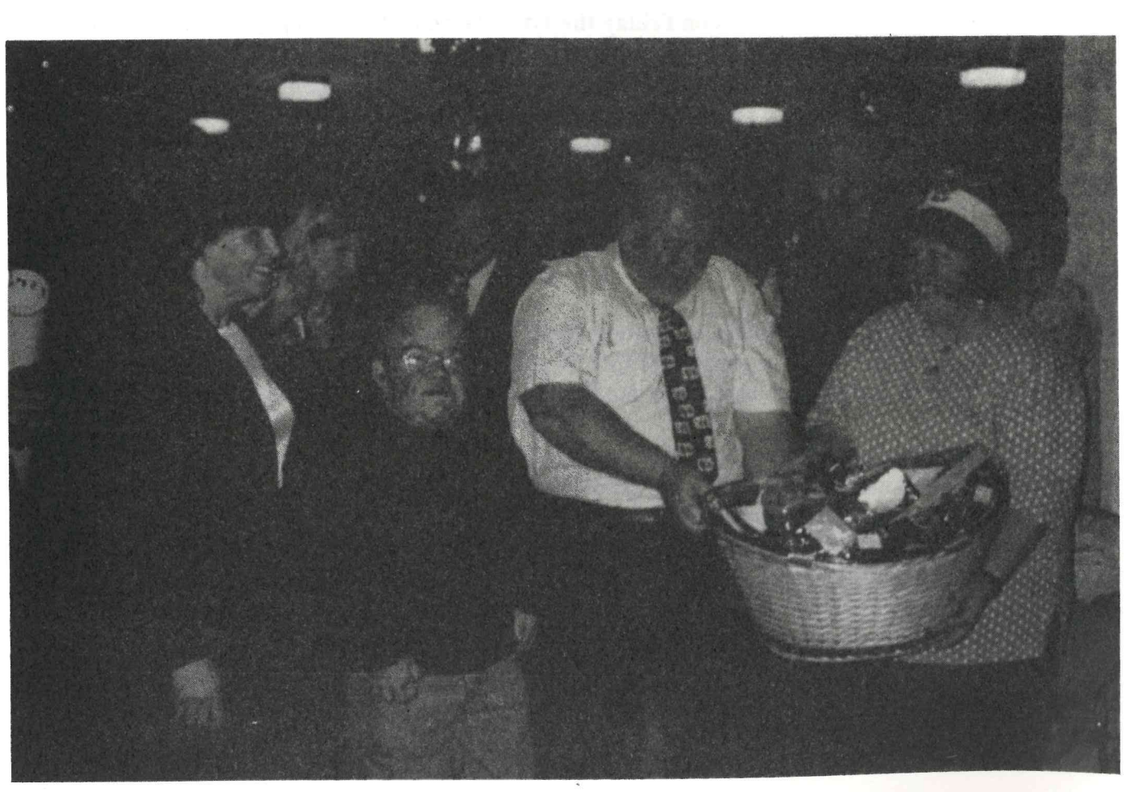
We were delighted that so many families joined in the festivities, from our own area and from **Sue and Geoff Hodgetts's** area. In fact, along with the army families, 80 children sat down to tea.

It was a surprise, and delight, that we were presented with a wonderful hamper of food and drink which included some really luxury items. This ensured that we would have a really good time at our Christmas holiday in Nottinghamshire.

We would like to thank everyone who contributed towards our present and would just like to say that we have enjoyed arranging family days in the past, however many years. We are still at the end of the phone if anyone wants to chat, and we are looking forward to being invited to family days in the future by the new Area Family.

Once again - thank you all for the wonderful present.

Linda & Ron



CHRISTMAS PARTY
17TH DECEMBER 1995
' THE SOUTH EAST AND MIDLANDS JOIN FORCES'

When back in the Autumn **Richard and Jayne Millward** suggested that the MPS Christmas Party could be held at the Army camp at Temple Heredewyke near Stratford upon Avon it was an offer too good to be turned down.

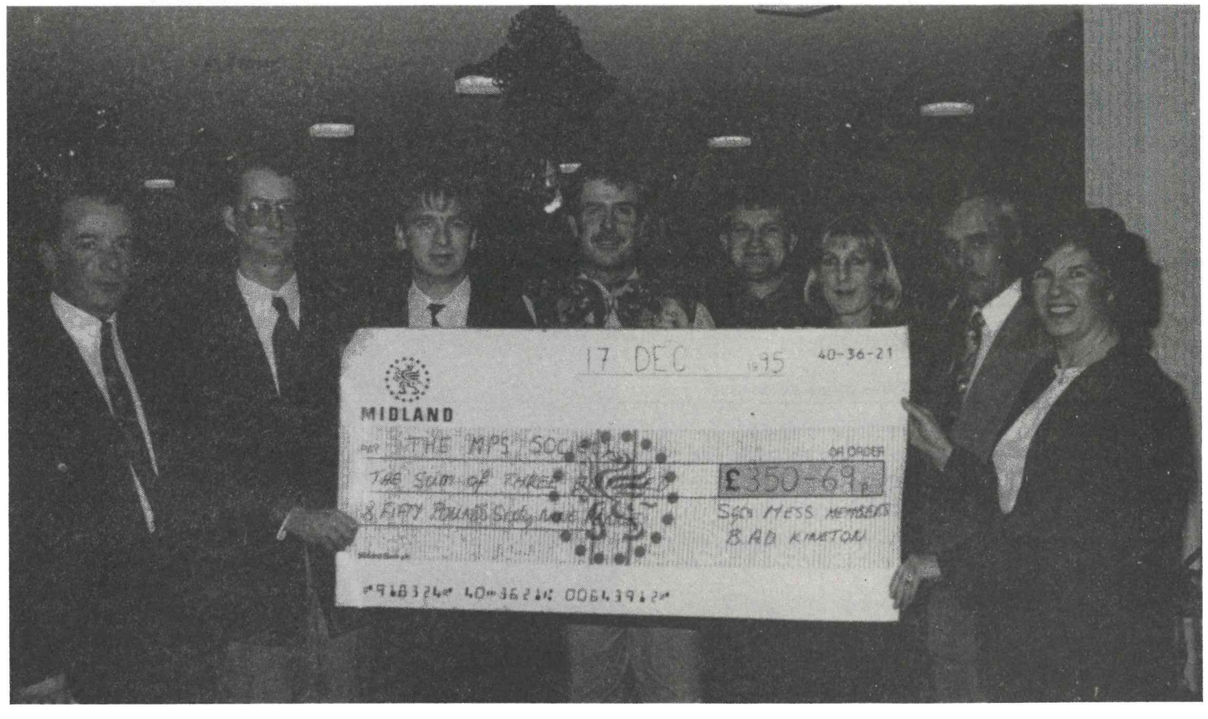
Richard and Jayne's youngest child, **Abigail** died in June 1994, aged 3 years from Mucolipidosis Type II. Richard is a sergeant in the Royal Logistics Corp.

After several months of work by Richard, supported by his RSM, **Alistair Rea** and Ron and Linda Snack, the Christmas Party was upon us. More than 29 MPS families from the South and Midlands came together with army families for a thoroughly enjoyable occasion. A disco was laid on for the children who were then all seated for a Christmas Tea, followed by a visit to Santa's Grotto.

A surprise donation of £350.00 was presented to Christine on behalf of the MPS Society. The money had been raised by the soldiers through a sponsored snooker game and other events.

The Society was then able to spring its own surprise and present Ron and Linda Snack with a Christmas hamper in recognition of many years of dedicated hard work as Area Support Family and Fund-raising Co-ordinator. Unbeknown to many, using a very outdated computer system Ron has mailed thousands of begging letters to companies on behalf of the Society with fantastic results.

Thank you to Ron and Linda for all you have done. You are a credit to Colin's memory.



Magnificent Seven

RSM WOI Ali Rae, Sgt Rick Millward, Sgt Lionel Cooke, WOII Steve Evans, Sgt Dave Berkow, WO Jack Larose (Canadian Armed Forces), Sgt Rangi Cotter (Royal New Zealand Army) prepare to attack the green baize in the snooker tournament at BAD Kineton, where they contributed to raising £350 towards the MPS Society. (Names not in order)

Page 9: **Christine Lavery and Jayne Millward** join the Magnificent Seven (less Sgt Rangi Cotter) as they present the cheque to the Society at the equally magnificent Christmas Party in the Seargeant's Mess at BAD Kineton.

Brave children's party boost

By PAUL HERBERT

A VERY special group of children from across the West held their Christmas party at a hotel near Bristol.

About 17 children suffering from a terminal illness joined in the fun with their brothers and sisters at Stakis Bristol Hotel.

And organiser Shirley Eyre said the party was also a vital boost for the youngsters mums and dads.

She said: "The contact at these parties is very important - it shows parents that they are not alone.

"They can get real help because doctors don't always have the answers that a mother who has gone through it might have."

The families are members of the Society for Mucopolysaccharide Diseases.

The society represents children who suffer from a range of diseases connected with missing enzymes.

The diseases lead to a slow mental and physical deterioration and few, if any, of the children will outlive their teenage years.

Mrs. Eyre, aged 39, and her husband, Tony, 47, have lost a five-month-old baby girl called Hayley and a seven-year-old boy called Sam to the illness.

The couple, who used to live in Yatton, Bristol, have now moved to Malmesbury and are the society's South West support family.

They have a healthy daughter called Carly, who is seven.

Top Left: Christine with Jason and Jamie George.

Above: Julie with Josephine and Francesca Kembrey.

Left: The clown entertains the MPS South West Party at the Stakis Hotel Bristol.

Congratulations to Shirley and Tony Eyre for a successful event.

A tribute to brave Sarah

by JONATHON DAVIES

SADLY one of Newtown High Schools former students Sarah Kilvert, died at the Royal Shrewsbury Hospital, on September 26, 1995, aged 22 years

Sarah had courageously fought the rare genetic disease Morquio Muccopolysaccharide, throughout her short life, but despite all her suffering, she always had a beaming smile on her face whenever you met her.

Sarah was a very determined person. When entering Penygloddfa School at the age of five she made sure she was treated as the 'norm' and one of the gang. She joined in almost every aspect of school life and, against all odds, learnt to swim.

Upon leaving Penygloddfa, Sarah continued education in Newtown High School, moving there with all her friends. By this time she had bought an electric wheelchair, which was a great asset to her, enabling her to enjoy more independence to move around the school, except when it came to the curse of all schools - stairways! This is when she sought the help of all her old friends, who were always there to lend a hand.

Mrs Esther Yates, Deputy Head at Newtown High School, remembers the day Sarah arrived in school with the new chair, likening her to, "A Grand Prix Driver, racing through the the corridors between lessons".

Make a Wish

In 1991 after recovering from major surgery, Sarah recalled her great dream to travel on the *QE2*. In 1992 this dream became a reality, thanks mostly to Audrey Thomas from Coleg Powys, who nominated her to the children's charity "Make A Wish". As a result, Sarah and her loving parents, Anne and Mike, sailed from Southampton, calling in at Tenerife and Madeira, before heading back to Southampton, having enjoyed a holiday of sheer luxury.

During her time in school, Sarah's hearing also deteriorated, but she did not readily admit to this, in fact she became adept at "lip-reading," which was typical of her fighting spirit.

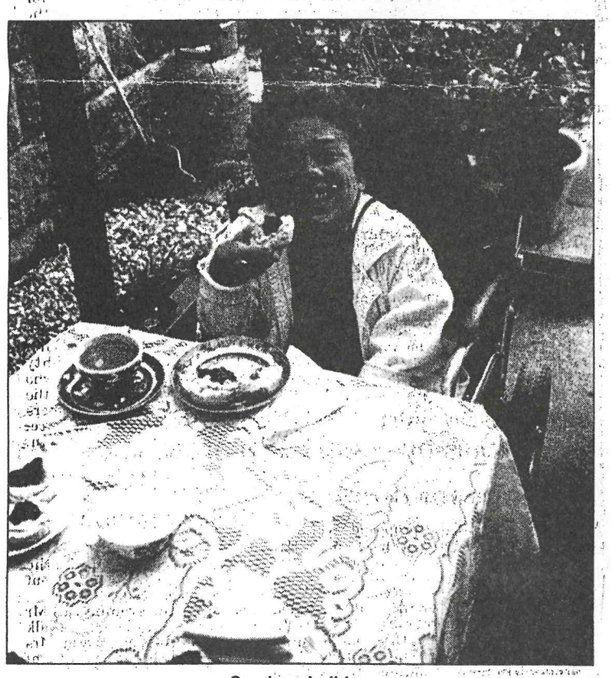
Whilst at Coleg Powys, Sarah became Secretary of The National Deaf Society. She also contributed greatly to the MPS Society. It was through this society that Sarah enjoyed her first holiday away from her parents. This was a very worrying time for Anne and Mike as it was the first time they had been apart for longer than 24 hours. Typically, Sarah enjoyed every minute of this experience.

Sarah with parents Anne and Mike, boarding QE2.

Sarah was a very brave, fun-loving person who lived her life to the full, but finally succumbed to her illness in September.

Farewell Sarah. You were and still are an inspiration to all who had the privilege to know you, and a credit to your family – in particular your very special parents Anne and Mike.

● The money for Newtown High School's non-uniform day will be going to the MPS charity.



Sarah on holiday

Welsh Christmas Party

A wonderful time was had at our Welsh Christmas party last Sunday 10th December, entertainment food and lots of friendliness was shared by all. We were so pleased to welcome two new families, **Chris Jones and Benjamin Griffiths**. Father Christmas arrived with presents for all, and we held a wonderful draw and festive bric-a-brac stall for all ages. I am enclosing a tribute to Sarah, which was featured in our local paper last week, written by a pupil at Newtown High School where Sarah went, we were really pleased with it. Also a cheque for two thousand pounds given in donations in memory of Sarah.

I hope you all have a wonderful Christmas and a Happy New Year to all your family and Office Staff.

Many thanks,

Love from **Anne and Mike Kilvert**.

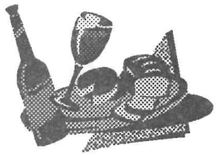
FUN DAY AT GREAT WARFORD

On a very pleasant Sunday in September, Sylvia and I went to a Fun Day at The Stag Inn at Great Warford, it was organised by Kevin and Angela Lawley, who's daughter Vicky has Sanfillipo disease. With a great deal of help from their family and friends the day turned out to be very rewarding. I have never seen so many friendly people enjoying themselve. The weather was very kind and the drinks cool, we had a barbecue, music, fun bus, troupe dancers and many more games. Many people (old and young) joined in the fun by having their faces painted or their hair sprayed. Later in the afternoon Kevin and some of his 'brave' pals had their heads shaved and what a laugh that was!!. Some people will do anything to raise funds for MPS. I was very impresed how helpful the Landlord and his wife and staff were and thought what a good idea it was to have a fun day at a pub. Some photographs were taken and you can see what a success the day was - two other MPS families came along and really enjoyed themselves.

In October Sylvia and I and Mary and Alex went along to the Stag Inn again - this time for a Karaoke Evening and also to collect a cheque from the Landlord for the amount raised at the Fun Day and the Sponsored Head Shaves - it was for a grand total of £2,500 - a superb amount. Mary Paget and Vicky Lawley accepted it and Rebecca Byrom was also there to give support!!!

Bill Blackburn

"Kojak" Kevin Lawley and "Chrome Dome" friends after their fund-raising head shave.



FUNDRAISING

CHARITY COOKBOOK FOR YOUNG ELEANOR

A six year old Cambridge girl who has a rare illness was the inspiration behind a new charity cookbook. 'Food for Friends' was written by Nicola Longdon, of Huntingdon Road, Girton to raise funds for the MPS Society.

The Society helps people like little Eleanor Gee from Cottenham, whose parents are friends of the author and her husband Richard.

Eleanor suffers from Sanfilippo Disease which has robbed her of speech and much of her understanding. She is not expected to live to reach maturity.

Mrs Longdon says she is an enthusiastic cook and over the years has devised a number of recipes.

"Friends often ask me for a note of how to make something I've cooked for them. But I found it difficult because I'm an instinctive cook of the 'pinch of this, handful of that' variety and don't always write my ideas down".

" I eventually decided I would have to make an effort and then had the idea of turning the recipes into a book which could help both to raise funds and increase awareness of this awful disease", she said.
With sponsorship and assistance from six local companies, Falcon Print Services, Scotthall, BMW,Cambridge Book Production Consultants, MoDo Merchants Ltd, Simpers Ltd and Kernow Plusfile, she has produced 1,000 copies of the paperback book, which has 26 original recipes for everything from starters to main courses, desserts, cakes and chocolates.

See enclosed order form for information on how to order a copy of 'Food for Friends'.

Eleanor Gee, Summer 1995 on holiday in Vassaliki, Greece. (Sanfilippo Disease -MPS III)

Lauren Cawthorne and Family

Hello! My name is Dawn Cawthorne. My husband is Graham, we have five children , Steve, twelve, Rachel ten, David James eight, David Graham eight and Lauren aged two. It has been one year now since Lauren was diagnosed with Hurler Syndrome and we made contact with the MPS Society. I would like to say thank you to everyone for making us feel welcome and part of a huge family and also for giving us the opportunity to enjoy ourselves with days out and the family holiday. Also for giving us the chance to learn as much as possible about Hurler disease at the conference.
But most of all thanks for the chance to make new friends. We could not have got throughout the year without you.

Lauren is doing very well and is popular with her little friends. She has a wicked sense of humour and loves watching Jeremy Beadle's "You've been framed." Unfortunately she is not keen on grown ups but is learning to accommodate them.

Lauren is having a heart operation in January and I am sure you all know how we are feeling. It is comforting to know that whatever happens now or in the future our new friends will be there with the support we need.

Best wishes,

Dawn Cawthorne
50 Drapers Lane
Hedon, Near Hull, HU1 8BG

Our prayers and best wishes to Lauren for her heart operation this month.

From Sidney and Betty Shiff

Enclosed a cheque for £160-00 as a donation to the society £100 collected amongst friends and families collection boxes. £60 collected by holding a raffle for a tapestry picture made by a senior citizen who wanted it raffled on behalf of a children's charity, enclosed a copy of sample. I hope the society has asked for help from the lottery profits, everyone but the right ones seem to be getting hand outs. We find it difficult now raising cash like other organisations. Also acknowledge receipt of donation, and if possible ask if the picture of the tapestry can be placed in the journal.

With our best wishes Sidney and Betty Shiff.
87 Laxton Road, Hunts Cross, Liverpool.
0151 280 3213

Holly Thompson and Family

After getting over the shock and coming to terms with Holly's condition, our family felt that we needed to do something to help children with MPS disorders. After discussion and reading about how funds need to be raised this year, our parents suggested we have a charity night in aid of MPS.

A hall was arranged at Our Lady's social club and the entertainment was booked. A quiz night was held before the charity night to raise money to pay for the entertainment - by some very good friends who have been really good and rallied round to give their support.

My mother in law Anne wrote to a number of companies asking for gifts to be raffled on the night. This proved very successful. We decided to keep some of the gifts and hold a second charity night at the Great Western Social Club.

The family and friends all got together and donated food to be made up for refreshments for sale on both nights. This also proved successful. We even had a few games of bingo. A good time was had by us all on both occasions. Therefore it gives us great pleasure to send you this cheque which was presented to us by Anne Thompson for a grand total of £1,444.50. Anne, who was the main fundraiser, lives at 24 Plumber Street, Birkenhead, Merseyside.

Angela Thompson
35 Fenderside Road
Beechwood
Birkenhead, Merseyside

Joanne gets Star of Merit

I thought you might like to include the enclosed photo in the next newsletter. It shows Joanne receiving her Star of Merit from **Mrs. Isobel Grey**, the County Commissioner of the Guide Association in Renfrewshire.

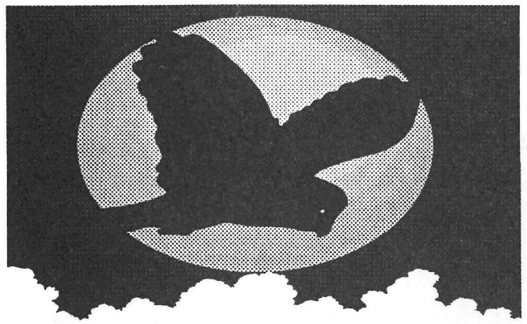
It was a great honour for Joanne to receive this award (although, I think she thoroughly deserved it!). It is a very prestigious award and is rarely given; Joanne was nominated by Brown Owl in her Brownie Pack, and the nomination was supported by her GP, teachers at school, her ballet teacher, the choir mistress, the minister of St.Machar's Church and various other people who know her and admire her courage and determination to lead a full life. It is awarded for meritorious conduct - in Joanne's case, for the way she copes with her disability, her cheerfulness and the inspiration she gives to everyone else.

Graham and I knew nothing about it until we received the invitation to the award ceremony which was held during a special family service at our church, attended by all the Brownies and their parents, her teachers and numerous friends. Afterwards, everyone joined Joanne in the church hall for coffee and a special cake which was decorated to show Joanne in her Brownie uniform and her Star of Merit.

Needless to say we are very proud of her.

With best wishes, **Judy Evans.**

Joanne is nearly ten and comes from Bridge of Weir, Renfrewshire. She suffers from Morquio disease (MPS IV)



FIRST CHALFONT AND CHENIES BROWNIE PACK

Like many MPS siblings Lucy is a keen fund-raiser for the MPS Society. As, sadly, the Headteacher of the school that Lucy attends and where both Andrew and Ben have been pupils, has never had it in her heart to support MPS, Lucy was overjoyed when her Brownie Pack decided to raise funds for the MPS Society.

One evening I gave a short child orientated talk to the Brownies and showed lots of positive slides of our MPS children on the Family and Activity Holidays and at the Conference. Each of the Brownies went home with a fund-raising leaflet and sponsor form.

One Sunday, a few weeks later, Brown Owl, Brownies and some parents met to start their 4 mile walk. On route they had to collect or identify objects related to nature for each letter in the words, 'MUCOPOLYSACCHARIDE DISEASES'. It was as challenging to the parents and helpers as to the Brownies and everyone had a great time.

Thank you Brown Owl for making Lucy's day and raising £136.00 for the MPS Society.

Christine Lavery

Brown Owl with her Brownies waiting to start the walk.

Children of Courage

Sam Wheeler aged 6 years from Reading in Berkshire was one of eight children to receive a 'Children of Courage Award' from the Duchess of Kent. Sam suffers from Morquio Disease and his parents, **Rachel and Mark** hope that the award will help to raise awareness of the MPS Diseases and encourage research into MPS.

Well done from all of us, Sam.

Christine Lavery.

Children of Courage: Four-year-old Suzanne Shepherd (left), from Ayrshire, who has had two kidney transplants, and Sam Wheeler, five, from Reading, who suffers from the rare Morquio disease, after receiving their awards from the Duchess of Kent, at Westminster Abbey, yesterday Photograph: Edward Webb

Rhianneth's Dad vows to fight on.

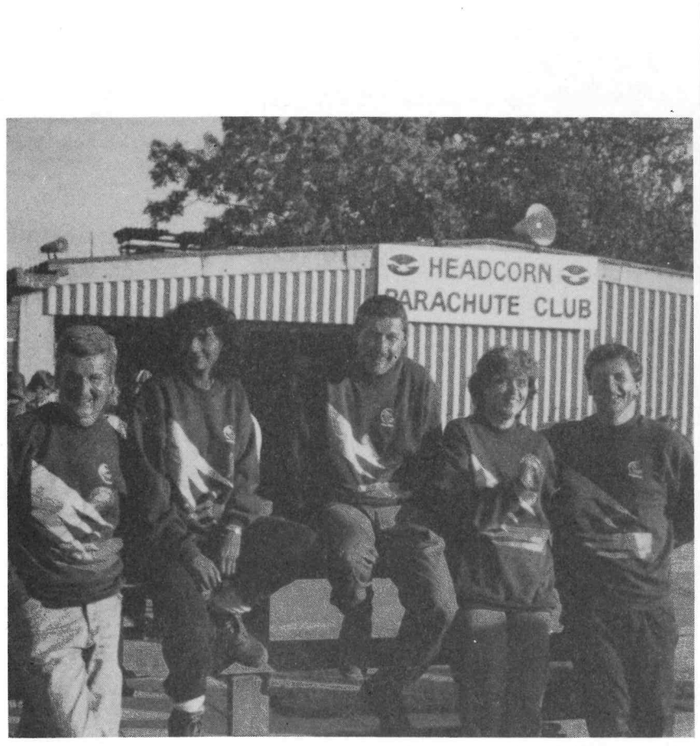
Michael and Karen Wheeler's daughter Rhianneth sadly died on the 7th of November 1995. Rhianneth was diagnosed as suffering from Mucolipidosis II when she was ten months old. Michael and Karen were first alerted to the condition when health visitors noticed her lack of weight and height. Shortly before her death, age three years, Rhianneth weighed eighteen pounds four ounces and her healthy brother Christopher, aged seven months at the time, weighed one pound four ounces more than his sister. Rhianneth was treated by doctors at St Peter's Hospital Chertsey and St George's Hospital Tooting throughout her illness, which she bore, said her father, with cheerfulness and good nature.

Michael and four friends, including Mark Banks from Walton and Mark Dover of Esher, recently took part in a sponsored jump to raise money for research into MPS diseases. They have raised more than £1,500 to date. Karen and Michael live near Esher in Surrey.

Michael is determined to carry on helping to raise funds towards research into MPS diseases.

Nicola and Richard Longdon from Girton, Cambridge.

Nicola has written a cookbook called "**Food for Friends**", to raise funds for MPS, inspired by her friends who are parents of Eleanor Gee. (See page 14)



Members of the Headcorn parachute club. Micheal Wheeler is on the right.

Archives of Disease in Childhood 1995; 73: 77–81

CLINICAL PRACTICE

Behaviour in mucopolysaccharide disorders

Martin C O Bax, Gillian A Colville

Abstract

This paper reports a study of the nature and prevalence of behaviour problems in 258 children with mucopolysaccharide disorders. Questionnaire data obtained through the post was supplemented by home visits to 42 families in the sample and by regular discussions with families at meetings of the Society for Mucopolysaccharide Diseases. High rates of behaviour problems were found, particularly in children with Sanfilippo's and Hunter's disease aged 5 to 9 years. These included destructiveness, restlessness, and aggressiveness. Sleep problems were common across subtypes with an overall prevalence of 66%. Parents reported that they received little or no support in the management of these difficult behaviours. It is concluded that behaviour problems are a primary feature of the mucopolysaccharide disorders and place a major strain on families. Services to help families cope with these problems are urgently needed.
(*Arch Dis Child* 1995; 73: 77–81)

Keywords: mucopolysaccharide disorders, behaviour.

While many genetic disorders are rare, cumulatively they make up a significant proportion of the disabled population of children in our society.[1] Behaviour problems in such children are common, often in association with severe developmental delay. Also there are certain behaviours in genetic disorders that are specific to the particular condition, for example the self mutilating behaviour of children with Lesch-Nyhan syndrome[2] and overeating in Prader-Willi syndrome.[3]

Identifying behaviours which are phenotypically determined in individuals with particular conditions may throw light on some of the behaviours we see in 'normal' children. Furthermore, an understanding of the natural history of these behaviours may help us develop measures of the progress of the condition and thereby provide another dimension along which to judge the efficacy of potential treatments.

In this paper we describe the behaviour problems of children with mucopolysaccharide disorders. While there are reports in the literature of high rates of behaviour problems in one subtype – Sanfilippo's syndrome[4–6] – relatively little is known about the behavioural characteristics of the other three main subtypes – Hunter's, Hurler's, and Morquio's syndromes. Elsewhere we have described the features of the early development of children with mucopolysaccharide disorders that first led parents to suspect that all was not well.[7]

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Table 1 Details of the 258 children studied

Syndrome	M/F	Age (years) Mean (SD)	Range	Total
Hurler's/Scheie's (MPS I)	39/24	6·5 (7·0)	0–41	63
Hunter's (MPS II)	54/–	8·6 (5·9)	0–30	54
Sanfilippo's (MPS III)	50/56	8·4 (4·2)	2–24	106
Morquio's (MPS IV)	15/20	10·3 (7·0)	0–28	35

Survey

The families of a total of 258 subjects completed a postal questionnaire on the development and behaviour of their affected children. One hundred and forty of these were contacted via the Society for Mucopolysaccharide Diseases (which was founded in the UK in 1982), with a further 28 being recruited mainly through the Family Fund.* In all 90 non-UK cases were recruited, mainly through families attending meetings of the Society for Mucopolysaccharide Diseases in the UK. A breakdown of the sample by sex and subtype is given in table 1. In succeeding sections we have analysed data for the 218 children aged 14 or under.

The confidential questionnaire elicited basic demographic and medical information concerning time of diagnosis and current health problems on each child. There followed a section on developmental abilities in a number of different areas based on the American Association on Mental Deficiency adaptive behaviour scale[8] and behaviour difficulties were screened using Rutter's parent checklist.[9] Additional information was sought on eating and sleeping behaviour and there was also space made available for comments on behaviour and general worries.

After administration of the questionnaire we visited 42 of these children, usually at home but occasionally in hospital or at school. This direct observation of the children provided us with an opportunity to check the reliability of the questionnaire with parents and to establish

*The Family Fund is an organisation funded by central government which provides financial help for families who have additional material needs as a result of having a child with a moderate or severe disability.

Table 2 Hurler's syndrome

	Age group (years)*		
	0–4 (n=25)	5–9 (n=24)	0–9 (%) (n=49)
Abilities			
Able to walk	16	12	28 (57)
Able to speak in sentences	7	9	16 (33)
Toilet trained	3	5	8 (16)
Behaviour			
Sleep problem	16	13	29 (59)
Destructive	1	3	4 (8)
Cannot settle	12	8	20 (41)
Fearful	18	14	32 (65)
Faddy	1	4	5 (10)

*There were only two children aged 10–14 years.

its validity. Recently O'Brien has shown that a parental questionnaire, which we helped develop and which contained many similar items to our own questionnaire, was consistently and reliably answered by parents of another group of disabled children.[10]

HURLER'S SYNDROME (MPS I; TABLE 2)

The vast majority of children with Hurler's syndrome in this study were aged under 10 years, reflecting the severity of this particular subtype, in which survival beyond the first decade is unusual. Their mobility was compromised from early on, as compared with the other main subtypes and they were often sickly in early childhood. As a group their language abilities were quite poor although there were notable exceptions, presumably those with the milder Hurler/Scheie phenotype, who were fluent speakers, keeping up with their peers at school. Only one in eight was continent under the age of 5 years and this only rose to one in five in the 5 to 9 age group.

From a behavioural point of view, the children were often described as anxious and fearful, particularly when they were below school age, and although a proportion were somewhat restless at times, they were rarely aggressive or destructive (in contrast to the children with Hunter's and Sanfilippo's syndromes). Sleep problems were common but anecdotally the nature of the sleep disturbance exhibited was more obviously related to medical symptoms such as cough and sleep apnoea than the difficult behaviour seen in Sanfilippo's syndrome. Lastly, although many children had difficulty feeding themselves because of coordination and swallowing difficulties (obstruction from large tongue), they were not described as particularly fussy about what they ate.

Table 3 Hunter's syndrome

	Age group (years)			
	0–4 (n=13)	5–9 (n=28)	10–14 (n=7)	0–14 (%) (n=48)
Abilities				
Able to walk	12	27	5	44 (92)
Able to speak in sentences	4	12	5	21 (44)
Toilet trained	0	2	4	6 (13)
Behaviour				
Sleep problem	10	14	6	30 (63)
Destructive	9	10	1	20 (42)
Cannot settle	10	21	2	33 (69)
Fearful	12	18	5	35 (73)
Faddy	3	3	2	8 (17)

HUNTER'S SYNDROME (MPS II; TABLE 3)

The boys with Hunter's syndrome, which is the only X linked subtype of mucopolysaccharidosis, were generally more physically able than children with Hurler's disease, but this mobility, when combined with more exuberant behaviour, had implications for management as their disease progressed. Their language was slow to develop and toilet training was a problem in those under 10, although 57% of the boys aged between 10 and 14 were continent. (It should be borne in mind, however, that this older group may be less severely affected, surviving as they have done into their teens.[11])

As in all four main subtypes, a diagnosis of Hunter's syndrome was associated with rates of sleeping problems much higher than the rate of 14% given for a community sample of 3 year olds[12] and rates of 32% and 33%[13] reported elsewhere for children with brain damage and mental handicap.

Overactivity was common up to the age of 10 years and aggressive/destructive behaviour seen frequently in those under 5. In several homes all breakable objects were kept out of reach and glass removed from doors, and in one case chairs were routinely lain on the floor so they could not be knocked over. Fearfulness was also characteristic of this group and most particularly of the younger boys.

SANFILIPPO'S SYNDROME (MPS III; TABLE 4)

In marked contrast with the accounts given by the other parents, those caring for children with Sanfilippo's syndrome frequently stressed the apparent normality of their early development. Coarse hair and facies were often not a feature until the child was of school age and there were several cases of strikingly attractive, fine haired children who had very severe behaviour problems. In many the children's language had developed normally initially, only to be lost over the next year or two. Thus by their early teens, fewer than one in 10 could speak fluently. As with the boys with Hunter's disease, children with Sanfilippo's syndrome were often physically quite strong with good mobility under the age of 10, which made their difficult behaviour hard to manage, particularly in public. Their incontinence was also a serious management problem.

The prevalence of behaviour problems was highest in this group. Specifically parents

Table 4 Sanfilippo's syndrome

	Age group (years)			
	0–4 (n=19)	5–9 (n=54)	10–14 (n=23)	0–14 (%) (n=96)
Abilities				
Able to walk	19	48	12	79 (79)
Able to speak in sentences	5	17	2	24 (25)
Toilet trained	0	2	4	6 (6)
Behaviour				
Sleep problem	18	46	19	83 (86)
Destructive	13	37	5	55 (57)
Cannot settle	15	38	13	66 (69)
Fearful	16	33	4	53 (55)
Faddy	1	5	1	7 (7)

described unpredictable, aggressive, and destructive behaviour and in practice this had a devastating effect on the lives of their parents. Family trips were curtailed for fear of attacks on members of the public and visits from friends reduced. Often there did not appear to be any malice intended, children would lunge at people and strike them for no apparent reason. We hypothesise that this may represent an abnormal reaction to invasion of the child's social space, leading to disturbed 'greeting' behaviour. One boy picked up rapidly at a bus stop, struck his unfortunate mother in the face and broke her nose. However, observed in his special school we noted that he caused no trouble because the other children avoided the space around him or approached him slowly. We did this and were then allowed to participate in his play.

Restlessness was common until the disease progressed to a point where mobility was limited. Children would wander round the house, upstairs and downstairs and be constantly on the move. Even when they were sitting on a sofa with someone they were likely to shout and pull the person's hair and clothes. It was difficult to take them out because they would wander off and in some cases reins were used to restrain them far beyond the age usually considered appropriate.

Also, mouthing and biting clothing and other objects was often described and seen on home visits. Some parents provided their child with a large 'teething' ring to try and reduce some of their biting behaviour. Seventy one per cent of the children aged between 5 and 14 sucked their thumb regularly.

But perhaps the most difficult area was that of sleep disturbance. Further information obtained during home visits to a number of children with Sanfilippo's syndrome, and by means of a further questionnaire completed by parents of 80 children,[14] indicated that these children displayed many unusual night time behaviours such as staying up all night (45%), wandering around the house (38%), and in some cases singing and laughing in the small hours (15%). Many school age children with this syndrome were found to be sharing a room with their parents, mainly in order that they could be supervised closely, while others slept in bedrooms from which all furniture has been removed and were effectively locked in.

MORQUIO'S SYNDROME (MPS IV; TABLE 5)

Mobility in the sample with Morquio's syndrome was generally good, although the progressive nature of their skeletal deformities would be expected to lead to increasing numbers requiring artificial aids/wheelchairs in adulthood. There were problems with toilet training in the under 10 age group but four out of five of the older children were fully continent, and all except one of the 25 cases under 14 were fluent speakers.

Behaviour problems over the age of 5 were few, although four out of the 10 children aged between 10 and 14 were described as fearful. Sleep problems were present but to a lesser degree than in the other subtypes. The only other finding of interest was the significant proportion of the children with Morquio's disease (40%) who were felt by their parents to be particularly faddy. (This compares with a rate of 13% for normal 3 year old children.[15]) In discussions, parents, several of whom had older children (over 10 years) with Morquio's disease reported persistent faddiness, but obviously it would be necessary to gather more information on this, and ideally make comparisons with a group of similarly disabled children, matched for age, before any claims could be made for a definitive link with the syndrome.

SUPPORT

The overwhelming majority of families in our survey had no regular help with the day to day care of their child (72%). To some extent this reflected the failure of services to meet their needs but many parents had reservations about leaving their child with someone else, either because of their child's difficult behaviour or because of their fear that they might not be there when their child died.

Parents were asked to give information on their contact with professionals in the previous year. Around 76% of families had seen a paediatrician or a general practitioner, but far fewer had seen a physiotherapist (4%), health visitor (32%) or speech therapist (31%), and less than a third had had any contact with a social worker, psychologist, education worker, or psychiatrist. Paediatric contact consisted of an annual appointment at a specialist centre, not uncommonly some distance away from home. Where contact had been made with a psychiatric unit the service offered (for example, family therapy) had sometimes seemed inappropriate when what parents wanted was advice about behaviour management. Many commented that the Society for Mucopolysaccharide Diseases, with its regular newsletters, annual conferences and support network of parents, was their main source both of support and information.

PARENTAL CONCERNS

Parents had four main areas of concern. Firstly there were many who were still very concerned about their child's prognosis which in most cases was one of deterioration leading to death. The second most common worry was about their behaviour and associated with this was

Table 5 Morquio's syndrome

	Age group (years)			
	0–4 (n=8)	5–9 (n=7)	10–14 (n=10)	0–14 (%) (n=25)
Abilities				
Able to walk	7	6	8	21 (84)
Able to speak in sentences	7	7	10	24 (96)
Toilet trained	1	2	8	11 (44)
Behaviour				
Sleep problem	3	5	3	11 (44)
Destructive	1	0	0	1 (4)
Cannot settle	2	0	0	2 (8)
Fearful	5	0	4	9 (36)
Faddy	3	1	6	10 (40)

the great distress they felt about not being able to communicate with their child in many instances. Thirdly, many children had health problems such as epilepsy, apnoea, diarrhoea, and chest infection and fourthly parents were concerned as to whether they could have further children and were desperately anxious for the latest information about antenatal screening. For all these problems parents found it difficult to get good help and advice at a local level because of the rarity of the disorder and many reported that they got most support from the parent group.

Discussion

The results of this survey strongly suggest that children with mucopolysaccharidoses display high rates of behaviour problems. These were not only higher than for the general population and for disabled groups, but also higher than for those other genetic conditions where behaviour has been studied.[16 17] Given that these conditions are currently incurable, degenerative, and disfiguring and also that it is not uncommon for more than one child in a family to be affected, the strain on the family is clearly enormous.

We acknowledge a number of methodological flaws, particularly concerning the reliability and validity of the questionnaire. The behaviour labels, taken from the Rutter questionnaire, may have been interpreted differently in different countries and its validity for use with children under the age range for which it was designed could be brought into question. Nevertheless, the patterns of behaviour which our results suggested were subsequently confirmed to us in discussions with parents. Also, although we could have asked parents to fill out questionnaires a second time in order to gather information on reliability, we decided not to on ethical grounds as many parents found the exercise very painful, highlighting as it did the difficulties they were facing.

The fact that the study was cross sectional, as opposed to longitudinal, and that there was no control for developmental level or severity of disease, meant that while we were able to make preliminary observations on which behaviours were most prevalent in the different age groups, these data did not allow us to map out the natural history of these conditions.

Lastly, the way in which the sample was recruited may well have introduced biases. Such information as was available suggested that professional and other non-manual groups were over-represented and it is possible that parents with poor local support and/or more problems would be more likely to be members of a parent group.

Notwithstanding the above limitations, we would hope that, given the extreme rarity of these conditions which very few doctors will encounter in their careers, this study constitutes a useful addition to the literature, both on mucopolysaccharidosis and, more generally on behavioural phenotypes.

Traditionally the disorganised behaviour of children with mucopolysaccharide disorders is ascribed to the effects of abnormal metabolites being deposited within the central nervous system. Of course the behaviours may be related to the resultant low cognitive levels of the children, but an alternative explanation is that some of these abnormal behaviours reflect more direct effects of abnormal genetic make-up. Understanding the basis of these behaviours in these unusual children may give us clues to similar behaviours seen in the normal population where it may be that many behaviours have as yet undiscovered genetic elements.

It is further hoped that this work will stimulate further research into the natural history of these distressing conditions and investigation of the ways in which they can be managed behaviourally. There is now a large body of expertise in the management of behaviour in disabled children and our experience of working clinically with a small group of children with Sanfilippo's disease with sleep problems, suggests that even a limited intervention can produce significant improvement.[14]

Lastly it is hoped that by disseminating information about the association between mucopolysaccharidosis and behaviour problems the chances of doctors in general practice identifying currently undiagnosed children may be increased. A significant proportion of families mentioned that difficult behaviour was the first abnormality they noticed, although sometimes it was many years before the child's condition was diagnosed. Thus some children with Sanfilippo's disease who had presented originally as overactive retarded children, were diagnosed quite late in life, by which time, sadly, they had acquired one or more similarly affected siblings. Until such time as a viable treatment is found, great emphasis needs to be placed on the need for earlier diagnosis. This would reduce the likelihood of families being faced with the awful knowledge that they have not only one, but several children with an incurable degenerative disease.

Firstly, we would like to thank parents for cooperating with our research. Their detailed observations provided the data for this paper and discussions with them about the ways they have coped with their children's behaviours have enabled us to think of strategies that we believe can help other children.

We also thank the Society for Mucopolysaccharide Disease for funding and Christine Lavery, Director, in particular, for her support. Lastly, at the Community Paediatric Research Unit, Andrew Thomas, Hazel Plastow, Irene Sclare and Joy Allsop provided useful advice, technical and secretarial help.

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FOOD SUPPLEMENTS

You got me again Charles, conference time and that familiar phrase "That will be of interest to other families can you do me a bit for the Newsletter." It's not a problem until you come to that first sentence and then you get stuck and think, 'Where do I start?'. So in an attempt to find a starting point I will go back to somewhere near the beginning of when the problems started.

For the last three months of '94 Kristina's eating was very erratic, eating like a pig one day and eating nothing the next. During our visit to the American MPS Conference Kristina was eating, but was very slow. She was also sleeping a great deal so we put it all down to jet lag. Kristina enjoyed Florida, Disney World and then Christmas when we arrived home.

1995 started quietly, well as quiet as a house with an MPS child can be! Kristina was not eating to well, coughing and choking on drinks and we were having to chop her food up smaller as chewing became difficult. At first you don't notice the weight loss. It wasn't until Kristina had one of those times when she would suddenly grow taller, then you remember that the baggy jeans she is wearing were the one you were going to get rid of because they were to tight and that the easier lifting was not due to us developing more muscle, that it became obvious that Kristina was loosing weight. During this period meal times had slowly lengthened until it took an hour then 1½ hours just to get one weetabix into her at breakfast. It seemed as if one mealtime finished just in time to start the next. She still managed to eat her chocolate with no difficulty? and we purchased expensive supplement drinks from a health shop and plenty of chocolate puddings which Kristina liked. WE were getting worried as eating itself was tiring her. Probably we should have realised earlier the problem was developing but it is so easy to explain away the small problems as just another phase of MPS, until they all fit together like a jigsaw and you say to yourself, "The brains going, should of seen that weeks ago."

When we were visiting Martin House for a few days they suggested Maxijule as they had had some success with it. Maxijule is a calorie supplement in the form of a powder. It can be mixed into milk, soups, stews, mashed potatoes and liquidised food or any food that has some form of moisture. It has no taste but does have a slight sweetening effect. This allows you to keep up the calorie intake without large amounts of food.

On visiting our GP for the prescription for the Maxijule he also prescribed Fortisip which is a drink, a bit like a milkshake, which has a high calorie content and most of the vitamins, iron and trace elements needed in a diet. It comes

in about 6 flavours, most of which are nice, although a couple taste awful and Kristina would just spit them back at me.

Luckily about this time Kristina's appointment to see Ed Wraith came through and off we went to pick his brain. I have to admit a feeling of guilt when he told us that if Kristina lost any more weight he would want to do a gastrostomy before she became to weak for the operation. A naso-gastric tube was not an option as not only would Kristina keep pulling it out but as she had broken her nose some years ago passing a tube would be very difficult. It wasn't until I was talking to another MPS family who's child had tube fed and told them that I felt that the possibility of Kristina having to have gastrostomy was in some way showing I had failed her, that things were put into perspective. Their point was that we all do the best we can for our children, it doesn't always work out, so we have to choose an alternative that may appear a poor second to US. Irrespective of being best or second best if it improves their lives we do it. Anyway, Monica explained to Dr. Ed what we were doing with Kristina's diet. Kristina had no objection when Dr. Ed said she could have as much chocolate as she wanted. Kristina likes Dr. Ed!

We had decided to keep track of her weight and check it every two weeks. We tried at home and would Kristina keep still so we could weigh her - NO WAY. In the end we asked school to try it - No Problems, Kristina cooperated! Kristina slowly put weight on, then suddenly lost a couple of pounds. We were having some problems with keeping track of how much Kristina was eating as the choking got worse. A bad meal time could see a large proportion of her dinner coughed all over your shirt and trousers. Then the hospital O. T. suggested this stuff called Thick & Easy. Basically it is a powder that thickens up fluids, depending on how much you add it can turn a glass of water or juice into a consistency that varies from milkshake to wallpaper paste. It has no taste at all and can also be added to stews, soups etc. The O. T. felt that due to the deposits of MPS that little flappy thing that should close off the lungs when you swallow was not providing a proper seal and fluids were leaking through and causing her to cough. The thickener would reduce this to a minimum. Well we though 'It's free, we will give it a try.' It worked and had a few laughs trying to get used to how it worked. We also found that using it with Maxijule affects it and you have to use more. Kristina still has the occasional cough, but now we were able to keep a better track of what she was consuming. If she had a bad eating day we would concoct a mixture Maxijule, Fortisip and Thick & Easy with icecream, chocolate and banana or strawberries. The idea being, get as much of her daily nutrients needs in as smaller volume as possible. As I told a friend, "you could put a couple of pounds on just looking at this concoction". Anyway Kristina liked it and steadily put weight on. We feel that she has now reached an ideal weight and we are trying to balance her diet to keep her weight

steady. Kristina is now more alert and is happier and enjoying life more now. The only downside is that she now has a sweet tooth and we still have to add some maxijule to her dinner to sweeten it a touch or she will refuse to eat it.

The 'threat'? of a gastrostomy has receded, for the time being. If Kristina becomes unable to cope with eating, then for her there is only one option, a gastrostomy. I must admit to still being a little uneasy about the prospect, but it will be done for Kristinas' benefit so I will have to overcome these feelings.

Well Charles, that's an abridged version and hopefully not to boring. Also I hope it will be of interest to others and hopefully helpful to some. I did forget to mention that Maxijule, Fortisip, Thick & Easy and some other similar products are all available on prescription from friendly GP's. It's helpful to know that because buying such products is very expensive. Oh! and if I seem a little reluctant to write for the Newsletter it's usually because it takes me three weeks to think of the first line and a couple of hours to write and another week to re-arrange it to make some sort of sense (I hope!)

The Scottish MPS Clinic

With 17 families (19 MPS children) booked for the MPS clinic at Rutherglen Health Centre, Glasgow, both **Dr Ed Wraith and Dr Ashok Vellodi** were on hand to see the children.

Behind the scenes Mary and I spent the day meeting with the families and providing help and advice on all aspects of caring for children with MPS as well as issues around welfare benefits, house adaptations and education.

It was also lovely to hear the success stories of last year's clinic. **Laura and Kerry Graham** from Annan now have their house extension to provide 2 bedrooms and en suite bathroom with double whirlpool bath for the girls who have Sanfilippo Disease.

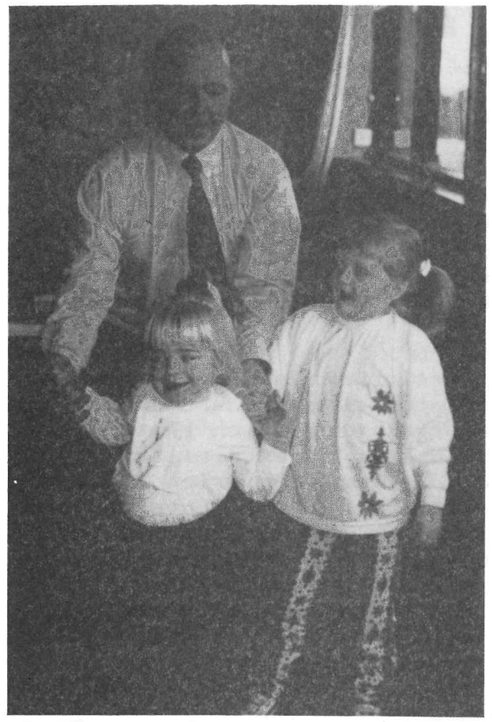
Donna McLellan with her little daughter, **Emma** who suffers from Sanfilippo Disease, have recently been rehoused and are beginning to feel their feet.

A special thank you must go to **Alan and Fiona Byrne** who despite caring round the clock for Louise found the time and energy to set up and run the clinic with such warmth and efficiency. Again the local hotel looked after us so well over lunch.

Finally thank you to the staff and practitioners at Rutherglen Health Centre for letting us invade them for one day each year.

Christine Lavery
Director

Next Page
Photos taken at the Scottish Clinic



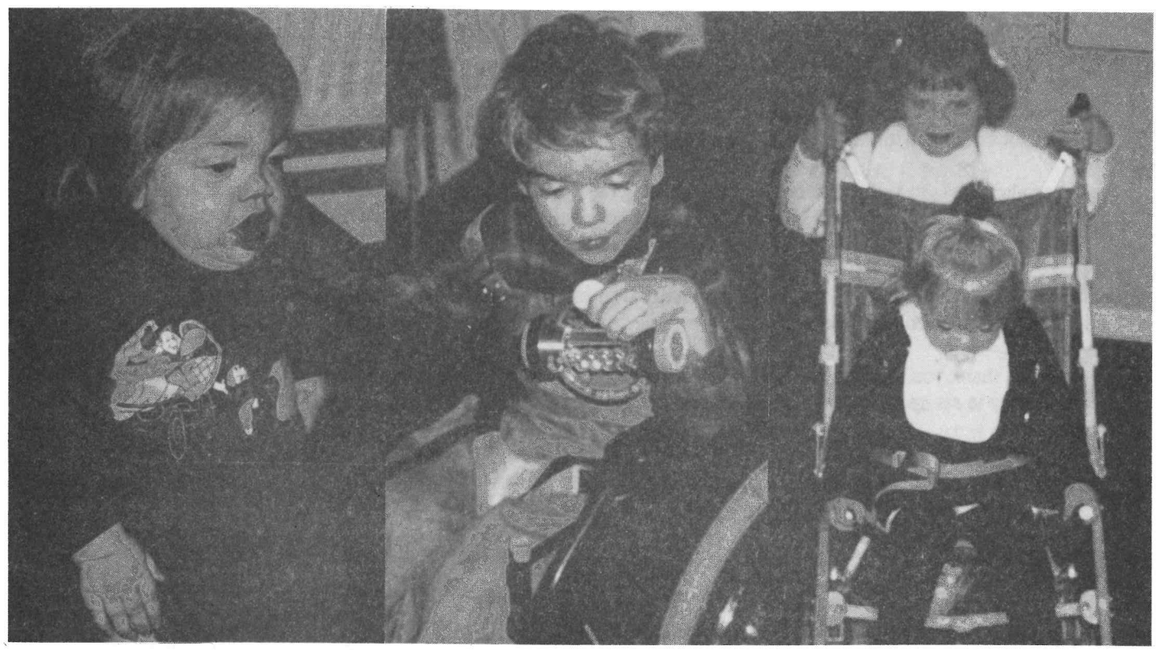
Alan Byrne with Emma Mc Clellan and Zara Watson

Emily Weir with her mother Gail

Claire Dempsey age 7
MPS I (Hurler disease)

Martin Stevenson age 7
MPS III (Sanfilippo)

Zara Watson age 6 pushing
Emma Mc Clellan age 4



Living with MPS : An account from Australia

My name is Colin Thompson and I am a 26 year old solicitor.

I have a mild form of Hunter Syndrome otherwise known as Hunter disease (MPS II). I don't regard the term "disease" as an accurate description as it has connotations os something one acquires rather than a condition you are born with. Anyway this argument is a bit semantic as the condition is fairly debilitating in its overall effect.

The mild degree of inconvenience and ill health caused by my condition has enabled me to complete a Bachelor of Laws Degree as well as a Bachelor of Arts Degree with Honours in Criminology at Melbourne University. I completed my Articles of Clerkship with the Victorian Government Solicitor's Office in 1994 which enables me to practice as a solicitor. Unfortunately since that time I have been unemployed due to the poor state of the economy. (At least that's the excuse people use, not the fact that my appearance and small size makes me stand out like a black South African bathing on a Whites' Only beach.)

Currently while technically unemployed, I have branched out into a different field of interest. I have used my research and literary skills in the endeavour of freelance writing/journalism. I have written a number of travel articles, of which, only one has thus far been published in a major daily newspaper. However for that one published piece I have at least a dozen on my computer that have either been rejected or as yet remain unpublished. I eventually hope to use my creative writing talents to write a crime fiction novel. (Although its a slow process.)

My first memory of the Children's' Hospital was having a large needle when I had an operation on my belly button when I was about 3 years old but it was another 5 years before it became more familiar. I was told that my parents received an adverse diagnosis about my future.

We all moved to Hervey Bay in Queensland when I was in the third year of schooling. The family, mum, dad and my sister Sally and I settled into life in a beautiful seaside town.

Coming back to Melbourne for a holiday I went back to the Children's' Hospital. The minute the doctor walked in and saw me sitting on a chair in the corner he knew the original diagnosis was wrong. According to the earlier diagnosis I should have been dead or at least severely deteriorated. As a result of a series of blood and other tests my parents were told that I had a genetic condition called Hunter.

At the time very little was known about the genetic condition amongst the medical profession and even less amongst the general community. My parents were effectively shown the hospital door and were expected to make the best of life.

In hindsight our move to Hervey Bay was best for everyone. Growing up in a small coastal community was very rewarding, an ideal environment for anyone to develop regardless of their physical condition. The town itself was fairly small and in its infancy so to speak.

I developed many friends. Some friendships have survived my moving back to Melbourne to undertake Tertiary studies and have lasted even to the present day.

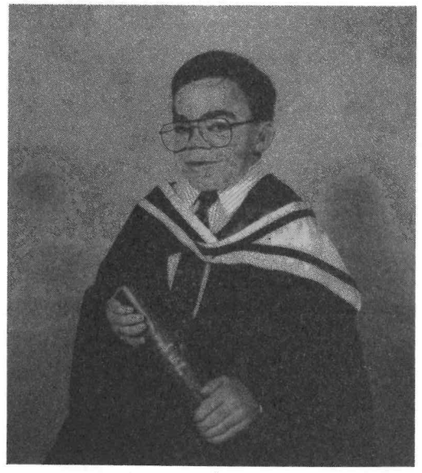
I don't wish to describe the world through rose coloured glasses for there were many trials and tribulations associated with growing up and adolescents not to mention problems associated with my disability.

Even to this day life has been a challenge and learning experience. Despite appearances to the contrary, I try not to regard myself as being disabled. I have to learn to live within my physical limitations and it often gets me down because I can't do what most people my age can do. I don't want to sound over optimistic or a resident of utopia but my life has been quite fulfilling. I recently travelled to Europe and the UK with my parents. It was such an eye opening experience and I can't wait for the next trip.

It is easy to get over zealous about my abilities and life in general but all my achievements, however insignificant or otherwise, have been possible because of the mild nature of the Hunter Syndrome. I am always guided by the doctors and genetic specialists but, at the end of the day what you do with your life is up to you and you alone.

With Regards

COLIN THOMPSON



HELPING TERMINALLY ILL CHILDREN
react

React

Over a period of little more than five years, **React**, a charity committed to Research Education and Aid for Children with potentially Terminal Illness has provided about 1,000 individual grants.

React's support takes many forms, but underpinning its work is the promise of a quick response to specific needs.

Grants have been arranged from the relatively small, but still important, to the somewhat larger - £50 - £100 for travel expenses, £62 for a telephone, £185 for a suction machine, £279 for a washing machine, £900 for a special mattress, £2,917 for an electric wheelchair.

React, which has been adopted by the London Evening Standard newspaper as its Charity of the Year for 1995/96, also offers holidays in its own mobile holiday homes, funding towards home and nursing care or child care and help with funeral expenses.

It is currently funding the work of a community nurse on the Isle of Wight.

For more information about grants contact:
React, 73 Whitehall Park Road, London W4 3NB Tel: 0181 995 8188 Fax: 0181 742 1867.

CSL INTERNATIONAL

Dear Christine,

As John Marshall has already explained to you, CSL regretfully decided it cannot proceed alone to develop therapeutic, recombinant MPS enzymes. This was a difficult decision based entirely on internal considerations and priorities. CSL was concerned that it might not be able to ensure, on a timely basis, the availability or level of personnel and physical resources needed to assure a successful project outcome. Therefore a decision has been made to seek alternative means of pursuing the development of therapeutic recombinant MPS enzymes and their commercialisation. As part of this decision, we are winding down CSL's research and development activities on MPS, and withdrawing from making any further commitments, internally or externally. CSL's future involvement in this field largely depend upon the terms of the licensing arrangements we will enter into.

I regret that CSL will not be able to make any further financial contributions to the database project which is going so well. I think you have done a fantastic job on it. CSL is proud to have been able to support the creation of such a valuable asset like this for the MPS Societies, and sincerely hopes the Societies will be able to find a way to carry it forward. Good luck in your European Biomed 2 Grant Application, and thanks for the help you have given John Marshall in getting an extension on the grant submission date and in putting together a broader MPS project proposal for the grant application.

CSL firmly believes that in the medium term, the development of enzyme replacement therapy offers good prospects for therapeutic treatment of MPS Disease. During the past three years CSL has made progress towards this end and a strategy for MPS product development now exists. In partnership with colleagues at the Adelaide Woman's and Children's Hospital, we have developed many of the required tools and technologies for process development and future manufacturing. We have also supported external research efforts and are grateful for the information and expertise shared with us by the many contacts made in the field. We are now seeking other commercial organisations interested in continuing the development of therapeutic MPS enzymes, either on a sole basis or in collaboration with CSL.

We highly appreciate and value the co-operation and assistance MPS Societies and collaborators have give CSL. We would also welcome referrals to potential commercial partners that Society members, friends and collaborators may wish to make to CSL and remain hopeful that a way to carry the work forward will be found. Please let your members and the other Societies with which you interact know of these developments at CSL. Feel free to extract from this letter if you wish.

Please contact me if you need to discuss anything at all. I have enjoyed working with you and I am personally disappointed that CSL cannot carry the project forward with the same momentum as before but I am also convinced that our involvement in the field has helped raise awareness and contributed towards progress to a therapeutic solution.

Sincerely

Attilio Demicheli
Commercial Development Manager - CSL International
10th April 1995

Although the decision by CSL not to proceed in developing enzyme replacement therapy for MPS Diseases and the failure to secure BIOMED 2 funds from Europe are set backs there had been some significant benefits from the relationship with CSL.

Firstly the funding which has allowed us to develop the European MPS database and the improved networking of the embryonic and established European MPS Support Groups.

Over the last 8 months whilst aware of CSL's decision we have been working together to achieve a commercial partner. Regrettably to date this has not been possible hence CSL's final decision taken in December 1995 to withdraw completely.

However all is not lost and there may be light at the end of the tunnel. John Marshall introduced us to Dr Otto Doblhoff-Dier of the University of Vienna, when we were putting together the BIOMED 2 application last March. In turn in May of this year Mary and I were able to introduce Marian Kraft and Dr Suzanne Fang-Kircher to Otto at the MPS European Working Party Meeting in Essen, Germany.

Marian and Suzanne have worked tirelessly to raise money for Enzyme Replacement. A breakthrough came in November when the Austrian Government offered to fund a major part of the work that is needed to bring about Clinical Trials for MPS Disease.
Otto, who has the skills to develop enzyme replacement at the University of Vienna is now working with the Austrian Society to bring about clinical trials.

Through the newsletter I will keep you informed of developments.

Christine Lavery

Christopher Rees and Christopher Jones

at the Welsh Christmas Party.

(See page 12)

The MPS group in Norway
Hunter/Hurler-gruppa
Aspehaugvegen 4B
0376 Oslo

Tel 47 22490316
Fax 47 22854381
Email oddrun.gronvik@inl.uio.no

Dear Christine,

You asked me to put down a few words about food etc., which may account for the healthiness of Norwegian MPS children. I'll just put down what we do, I don't want to make a case that we know how to do it better in Norway, especially as there are no scientific surveys to support any general conclusions.

Known factors affected by diet:

The importance of diet is highlighted if we list just some of the known effects it can have: Food affect growth, bodily functions and mood.

Problems with diet can contribute to difficulties in the following areas:

hyperactivity
teeth
weight
sleep problems
diarrhoea

At a later stage in the child's life as the illness progresses, the following areas can also be affected by the child's diet.

swallowing problems
teeth
failing circulation
physical passivity
coldness
constipation

Family considerations:

Preparing food for the handicapped child shouldn't be a nuisance (double work or unwanted diet for the others). What is desirable for the MPS child not essentially different from what is recommended for everyone else. But the wrong food may have bigger consequences (more pain/discomfort etc.)

Norwegian meals

Most Norwegian households still cook from raw materials although processed foods are on the increase. Pregnant mothers and young families are encouraged to make their own baby food rather than buy Nestlé jars, and you get a little recipe book to help you get started when you take your baby

to controls. Cooking (from raw materials) is taught at school in the 6th and 7th year (to everyone, both boys and girls).

Most households also have food processors/mixmasters/liquidizers. My favourite tool is the grater. I have two, one small French food mill and one of the square box type.

Breakfast:

Many families have oatmeal porridge (from oatmeal, not pre-cooked porridge mixes) for breakfast, with sugar and milk to drink (sweet or soured) (both adults and children). Others prefer open sandwiches (with cheese, sliced sausage, jam ..). Brown bread. Many also have orange juice for all, coffee or tea for adults. We are recommended to take cod liver oil or a multivitamin pill (or mix) in addition, especially in the winter months.

Formiddagsmat: (Lunch)

(About 11.00am.-12.00) Everyone brings open sandwiches (1-3 pieces, most often with cheese, sliced sausage, liver pate etc.). Children get milk at school, Adults normally can get milk and coffee or tea at work. The school dentist will check on children's packed lunches, and if they contain white bread/sweet spreads you're quite likely to get spoken to. It is still quite usual to bake bread at home, as we do.

Middag: (Tea)

(About 5.00pm - 6.00pm) Our only regular hot meal. Norwegians are still potato eaters, more than rice, pasta etc. Favourite vegetables, especially in winter, are carrots, swedes, onion and cabbage, although salad vegetables are becoming more important. On weekdays you'll probably find more mince and sausage than proper meat. Fish is popular because it is quick to cook (poaching or pan frying most common, we don't use batter much).

Many Norwegians use the deep freeze to stock up in autumn when supplies are cheap, and many (myself included) make up enormous portions of meat balls, spaghetti sauce, casseroles etc., not to mention stock for soup in that connection, to simplify cooking through the winter. So I suppose we use our own pre-cooked foods even if we don't buy them. Most Norwegians have coffee and something sweet after middag, sometimes with a break in between - around seven or a bit later.

Kveldsmat (Supper):

At bedtime, for those who are up after eight: normally only an open sandwich, porridge or cereal. Milk. Scrambled eggs, soup etc. occasionally as a treat.

Food and MPS children

I've talked to a couple of the mothers and none of us have had major problem in getting our children to eat - more often than not the children enjoy their food and have healthy appetites. Einar could probably have done with less. We also all seem to follow the rule of adjusting ordinary meals, rather than cooking special meals.

Basically I adjust the ordinary meals to fit Einar. When he was little, he had loose stools and was hyperactive, so he got more white bread and less brown, more soured milk products and less fresh, and as little sugar as possible (sugar goes straight into the blood stream and certainly doesn't lessen hyperactivity). For instance, I gave him plain yoghurt or soured milk with home made apple pure, sweetened with artificial sweetener, rather than the shop product, sugar free squash etc. He got ice cream, chocolate and cakes as a treat but not often. Instead I gave him fresh fruit, peeled and chopped, grated or whatever, which he still loves. I've never given him shop sweets, sweet biscuits etc., but of course if he's been offered them he's been allowed to have them, provided that he can eat them.

I've stuck to the low sugar policy since, to spare his teeth.

Now that he is less active and has a degree of constipation, he always has brown bread (home made, with us) and a spoonful of oatmeal in his yoghurt (I soak it in a little boiling water to soften it, before mixing it in). We also routinely add oatmeal bran to his porridge and cereal.

He normally has porridge for breakfast, with milk and sometimes orange juice. We always heat his drinks enough to take the chill off them, as his circulation is failing, and we've found it useful to have a small aluminium saucepan at hand for that, and for any one-portion meal he needs to have cooked especially.

He brings an open sandwich to school, and they cut it small and feed it to him in bits. Home made bread is crumblier than shop bought, so it dissolves more easily in his mouth. He has milk with his sandwich. He has a drink of sugar free squash when he arrives and another at three, with some (peeled and grated/mashed/stewed) fruit.

At lunch he normally gets what we get. Fish that has been frozen gets too dry, so we always have fresh fish just cooked through. Root vegetables I often grate coarsely and steam with a little water and a pat of butter and serve in their cooking liquid. The whole family like this, but it is quick to do for one only as well. Meat now has to be extremely tender and chopped very small indeed. Lamb and chicken goes down more easily than beef and simmered stews better than roasts - I don't even attempt steaks on their own.

But anything he cannot handle goes straight into the liquidizer before we give it to him, so it isn't really a problem. Most things seem to go down if camouflaged in a little mashed potato. Rice, minced meat and macaroni used to be favourite foods, but nowadays they tend to set him coughing, so I avoid them.

He can manage salad type meals with raw vegetables if all vegetables are peeled and the stiff ones (apples, cucumbers, carrots) are grated rather than chopped (carrots very finely). Hard-boiled eggs, tuna and finely chopped ham are popular ingredients. Cheese I grate. Quite often I take Einar's helping aside before serving all and chop it extra fine on the chopping board. It may not look too exciting afterwards but he enjoys it and it is less trouble than cooking him special meals.

Like most children he is very fond of every variety of pancake, which is used here quite a lot anyway. In winter we have a vegetable soup and pancake dinner most weeks (like you, I've tried to teach my children to cook, and this is Helga's meal). Other vegetarian options are vegetable bakes and Spanish omelette, both of which the children like very much.

Einar's diet when he was little:

Einar had a low-sugar diet, as sugar can make hyperactivity worse (its "reviving" effect well known to us all) sugar-free squash, no sweets except chocolate and ice cream, and not much of that (Saturday afternoon was the established sweet time). We used all the old-fashioned remedies against diarrhoea, for example,

cooked blueberries
apple purée
soured milk products (diet yoghurt, skimmed soured milk)

Two recipes

Fig and prune mix.

After this letter was sent we got a recipe for a fig-and-prune mix which is used in a lot of nursing homes for the elderly, with good results. We've tried it for three months now and it works. The ingredients are:

250 g (About 1/2 pound) stoned prunes
250 g (" " ") dried figs

Soak over night in just enough water to keep all covered. Chop finely or mince with the soaking liquid, store in a clean jar in the fridge. This mixture will keep for a month. One big tablespoon a day. Einar gets his in his evening meal, which he has every day (He produces a stool every two days):

Oddrun special mix

1-2 tablespoons of unprocessed oat meal soaked in a little boiling water (which gets absorbed in the oatmeal)
1 topped teaspoon of oat bran
2-3 tablespoons of plain yoghurt
1 tablespoon fig and prune-mix (see recipe above)
a teaspoon sweetener (sugar or artificial)

MPS Children and physical exercise

Einar started in nursery school at eighteen months, before that he was at home with me half the week and at a day nanny's house with another one-year-old the other half. Since he was in such poor shape physically we encouraged him to get moving, and from eighteen months he was running around. Norwegian nursery schools have to have fairly well equipped outdoor play ground areas, and most schools have the children outside for at least two hours a day, more in spring and summer. They also

have to have an indoor room for jumping, running, climbing etc. and most parents are concerned that their children should be physically active. Norwegian MPS children probably benefit from this, both because they get stronger and because their hyperactivity is less of a nuisance out of doors.

We don't have school uniforms of the English type, but children have to have warm clothes and play suits and boots for outdoor playing in all weathers. Otherwise your child may loose his place in nursery school. Ordinary schools can't turn your child out, but they won't let you get away with poor outdoor equipment (a bit of a shock to many immigrant parents!).

Anyway, the parents I have spoken to, agree that the opportunity for physical activity is important for their children, both in the early years and because they are in better shape when the decline starts. It also helps to get them really hungry before meals, and really (physically) tired before bedtime.

Helping Einar sleep

I've thought a lot about the sleeplessness problem. I realise that San Filippo children are different class from Hunter/Hurler, so I'm not saying what follows would hold good for all. But both Knut and I feel sure that Einar's periods of broken sleep tie up with periods when his school tried to keep him sitting still too much, and he didn't understand or enjoy the school activities. The best thing we could do, then, was to take him to the playground in the evening before bedtime, have him walk there and back, if possible, take him out sleigh riding etc. Even now, when he is almost totally wheelchair bound, we always try exercise and fresh air if we feel that he is sleeping less than he should at night - the chief indication being a tendency to fall asleep in the day time! I suppose this has contributed to keeping us in shape as well, though it is more of a chore than a pleasure at times.

Exercises at home

Einar also has a regular set of exercises which we put him through every day, and which have been adjusted over the years in co-operation with his physiotherapist.

I don't know whether this is any help to English families - probably too detailed. However, use it according to need!

Oddrun Grønvik Bachke

~~INFORMATION~~

SATFA

Support Around Termination for Abnormality

A new handbook for parents who discover that their unborn baby is abnormal has just been published by SATFA. This book has been specially developed to help parents at the time of a diagnosis of abnormality in their unborn baby. Rapid developments in ante-natal screening technologies mean that many more parents are now facing the decision of how to manage an affected pregnancy. The SATFA Parent's Handbook is designed to help parents through this painful and difficult decision making process.

If you would like any more information on SATFA Please contact them on
0171 631 0280

GENERAL INFORMATION

New Rights for Parent Carers

The Carers (Recognition and Services) Act

Francine Bates, Assistant Director – Public Affairs and Training Services, of the Carers National Association writes about the Carers Act due to come into force next year

"This year in England and Wales, parents caring for children with disabilities got an extra bonus with the passing of the Carers (Recognition and Services) Act. The Carers Act, which is designed to help all carers, started life as a Private Member's Bill proposed by Labour MP, Malcolm Wicks.

"Originally drafted by Carers National Association (CNA), the Act places a duty on Local Authorities to carry out a separate assessment of the ability of a carer to provide, and to continue to provide care, and a duty to take this assessment into account when deciding what services to provide to the person in need of care. The Carers Act is not due to come into force until 1st April 1996 – so how exactly will it benefit parent carers?

"The Act links the principles of the NHS and Community Care Act with the Children Act which makes clear that the views and preferences of family and relatives should be taken into account when assessing the needs of somebody who may be in need of either adult community care services or services aimed at children with disabilities.

"Parents or people with parental responsibility, who provide or intend to provide, a substantial amount of care on a regular basis for children with disabilities, are covered by the Act. The right of a carer to an assessment is triggered when a child is being assessed for services by the Social Services Department under Part III of the Children Act and/or Section 2 of the Chronically Sick and Disabled Persons Act. Note that this right does not apply if a child is having an educational assessment, although good practice dictates that a Local Education Authority should liaise closely with Social Services when carrying out such assessments.

"Published guidance to the Children Act (Volume 6) already addresses the importance of providing early information to parents, assessing the needs of the child and the family, leading to a plan which is regularly reviewed. However, the Carers Act will help to ensure that parents are definitely included within the assessment process and give them an opportunity to express their own needs. If it is clear to Social Services that a parent needs help as a result of the assessment, it must take this into account when deciding what services to offer, such as provision of short-term respite care. Carers will be entitled to talk to a professional worker, usually from Social Services, and have their views recorded and written down. A copy of the written assessment and subsequent plan should be sent to the carer.

Role of Health Professionals

"Local Social Services Authorities may, in certain situations, arrange for the Health Authority or local GPs to undertake an assessment of their behalf. If this is the case, health staff will also be expected to assess carers. In any event, health workers and primary care teams will be required to tell carers about their right to an assessment and refer them to Social Services.

Siblings and Young Carers

"The Act gives young people under the age of eighteen years the right to ask for an assessment in their own right. This will greatly assist the thousands of young people who are obliged to take on considerable caring responsibility for a member of their family, usually a parent with a disability. However, in some cases a sibling may also be entitled to have an assessment under this Act, helping to qualify them for services under the Children Act, such as organised outings, counselling, and so on.

GENERAL INFORMATION

Does the Act Apply to Other Parts of the UK?

"The Act applies to Scotland in respect of carers caring for adults. Unfortunately, parents will have to wait until the end of 1996 when the Children (Scotland) Act comes into force before being able to exercise their right to request an assessment. Prior to April 1996, the Northern Ireland Executive will be issuing Directions to Health and Social Services Boards and Trusts which will require them to offer assessments to all carers in line with the principles outlined in the Carers Act.

What Now?

"The Carers Act has been warmly welcomed by carers and professional workers alike. For the first time ever, carers have a clear legal right to have their voices heard and influence important decisions about future service provision. The Act is not going to change the world overnight for most carers and CNA recognises that it is only a first step towards helping carers solve the many different problems they face. CNA is working closely with the DoH to ensure that the Act is fully implemented by local authorities. The Department's Social Care Group have very recently issued draft policy and practice guidance on the Act. CNA is anxious to alert as many carers as possible to the new legislation which becomes law on 1st April 1996, and will be producing information materials targeted at carers prior to implementation. "CNA has also developed a training package for Local Authorities, Health Authorities and other organisations which work with carers about the full implications of the new Carers (Recognition and Services) Act."

For further information please contact CNA's Training Unit, 20/25 Glasshouse Yard, London EC1A 4JS Tel. (0171) 490 8818.

This article was printed by courtesy of 'Contact a Family', 170 Tottenham Court Road London W1P 0HA Tel: 0171 383 3555 Fax: 0171 383 0259

Francine Bates is Assistant Director - Public Affairs and Training Services - with the Carers National Association. She has worked for the Association for almost five years. She coordinated the Association's campaign to promote the Carers Recognition and Services Bill which became an Act of Parliament in 1995.

She has kindly agreed to speak at the MPS Conference in Northampton in 1996.

MEDICAL ARTICLES

UNIVERSITY OF MINNESOTA IS FIRST U.S. CENTER APPROVED TO DO GENE THERAPY FOR HUNTER SYNDROME

The University of Minnesota is the first U.S. medical center to receive Food and Drug Administration (FDA) approval to begin an experimental clinical trial of gene therapy on adults with Hunter syndrome, a rare and inherited disorder caused by the lack of an enzyme necessary to recycle complex carbohydrates from cells.

This project also is the university's first FDA-approved gene therapy endeavour.

The University's researchers led by Chester Whitley, Associate Professor of Pediatrics and the Institute of Human Genetics, have approval to begin therapy on two patients. Staff from the university's blood bank will remove white blood cells from the patients' blood and then insert the gene for the needed enzyme through a harmless, genetically-modified virus or "therapeutic vector" produced by the University's Gene Therapy Program. Once the vector has been exposed to the white blood cells, the cells will be injected intravenously back into the patients, much like a blood transfusion. Since white blood cells have a short lifespan, the transfusions will be repeated monthly during the study's one-year period.

Hunter syndrome, also known as mucopolysaccharidosis type II, occurs about once in every 100,000 births. Some 30 people are diagnosed with the syndrome annually in the U.S. The syndrome's more severe form is present in children and causes dementia and mental retardation, along with respiratory and heart disease. Most patients who have this form die before age 15. Adults are affected with the syndrome's milder form, which does not cause mental retardation, but can cause severe heart disease, extreme joint stiffness, and breathing difficulties that require tracheostomies. Adults with this form of the syndrome typically live to about age 40.

The only therapy that has been proven somewhat effective in getting the needed enzyme to the cells is bone marrow transplantation, using marrow from donors who have the enzyme. However, bone marrow transplantation for Hunter syndrome patients has resulted in high morbidity and mortality. As well, the lack of suitable marrow donors and the potentially high cost of treatment makes bone marrow transplantation unsuitable for many of these patients.

"The goal of this trial is to demonstrate that this form of experimental gene therapy is feasible, as well as to learn about adverse effects," said Whitley. "Whether or not this treatment will work is completely unknown, but if the initial treatments appear to be safe, future studies would be required to assess how well they really work."

Whitley said potential volunteers need to seriously consider unknown complications that may result from the treatment, as well as the fact that no benefit may result. The university's trial will be limited to two adults and researchers hope to be able to begin work on the two patients within six months.

Only six U.S. universities have received FDA approval to use gene therapy to treat genetic diseases.

Acknowledgements and Greetings to the Canadian MPS Society.

FUNDRAISING EVENTS

The Society is Grateful to the Following who held Fundraisng Events

Trudy Deacon - Cambridge - Sale of Goods
Jane Heritage - Cambridge - Sold Homemade Marmalade
Mrs Mary Maudling - Isle of Wight - Talk to Woman's Institute
Donna Lowther - Gateshead - Car Boot Sale
Mrs Wilks - Leeds - Alcan Tins
Pam and Ken Ballard - Harrow - Sale of Goods
Lesley and Paul Harvey - Oxford - Sale of Stairlift
Mrs James - Coalville - Flower Party
Alison Oliver - Penryn - Jumble Sale
Sheila and Ken Benbow - Wirral - Raffle and Open House
Sue and Vic Lowry - Harpenden - Fundraising Evening
Jenny and Andy Hardy - Alesbury - Coffee and Doughnut Morning
Shift and Oulton Families - Liverpool - Kareoke Night
Dr Atterton - Somerset - Sale of Antique Spoon

DONATIONS IN MEMORY

The Society would like to thank the friends and relatives of:

James Gooch
Charlotte Pollard
Sarah Kilvert
Rianneth Wheeler
P.J. North
Dorothy Mansfield (Gemma Rollinson's great grandma)

CHARITY BOXES

Ann Thopson - Darlington
Kathy Lawrie - Hull
Edward hurdel - South Heighton

DONATIONS

The Society is grateful to the following who made donations.

Nisa Today's
The Sun
Seaton Health Care
Rebecca Collins
Natalie Pizzo
Towersly Morris Men
Ushers of Trowbridge
Anne Taylor
Vauxhall Motors Ltd.
Dr and Mrs Roberts
Joseph Strong Frazer Trust
Cathkin High School
British Association for Early Childhood Learning
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Mrs R Brown
The Society of Lloyd's of London
Soroptimist International of Aylesbury District
Jill Franklin Trust
John Taggert
Val Merry
The Clover Trust
D Hale
T P Randall
Eddie Lister
The Cotton Trust
Timothy Pidden
Mrs Marriott
Giba-Geigy
J Davis
Mr and Mrs Blundell
Mr and Mrs Strutt

HELP FOR 19 YEAR OLDS

For a disabled person, reaching the age of 19 is rather like falling off a cliff: the statutory provision disappears from beneath your feet. Far from being supported, people find themselves having to negotiate, lobby and campaign for good quality services.
Now **Action 19 Plus**, a consortium of voluntary organisations including Scope, formerly the Spastics Society, Mencap, Contact a Family, Barnardos and SENSE, has published a new guide to enable people to find their way around the system and get the services they need. It is an invaluable tool which I recommend to all disabled people, their families and carers.
The guide shows how to prepare for a local authority assessment, how to use the complaints procedure, get legal advice, run a local campaign and use the media and Members of Parliament.
Brian Lamb
Head of Campaigns, Scope

19 Plus is available to individuals for £3.95 and to organisations for £6.00. Cheques, made payable to Scope's Campaigns Department should be sent to Scope, 12 Park Crescent, London W1N 4EQ

DONATIONS TO MPS

"Jeans for Genes" from schools:

Penryn Junior School Cornwall	£75.00
Christ Church CE Primary School Lancaster	£74.28
South Holdenes School at Preston Yorkshire near Hull held a fund raiser in the summer and raised £300.00. they decided on MPS as Lauren Cawthorne's brother attends the school and suggested the Society as beneficiaries.	£300.00
Angela and Kevin Lawley arranged a fund raiser in August at The Stags Head Hotel ,near Alderley Edge, run by Bill and Lin.	£2,500
Barbara and Trevor Rollinson fund raiser in June	£190.00
Paul Brookshaw ran The BUPA Great Welsh Run in July	£52.12
Rosemary and Lisa Nurse did a car boot in August	£100.00
Sylvia and Bill Blackburn held Olde English Garden Party in June	£987.81
IBM Manchester via the Byrom family donated in June	£100.00
Mr Nelson and sister in-law Mrs Nelson great uncle and aunt of Bradley Evans.	£35.00
People have sold raffle tickets and sent in extra donations	
Mr and Mrs Haigh; Huddersfield	£50.00
Mr and Mrs MacLean; Inverness	£5.00
Dr and Mrs Mansfield; Lowestoft	£45.00
Mr and Mrs Ridley; Leicestershire	£5.00
Mr and Mrs Matthews; Staffordshire	£20.00
Donna McLellan;Glasgow	£5.00
New Families have made donations:	
Jan and David Donegani Loughborough	£10.00
Holly Thompson's(Merseyside) family	£25.00
Mrs Anne Taylor Grandma to Kyle and Dale Taylor, Batley	£10.00
Mr and Mrs Kaye, Sutton Coldfield	£5.00

INCOME FOR FAMILY CONFERENCE 1995

Donations from local charities to help with the cost of the Conference.

The Sir Knott Trust 1990 (North East)
Oxford Round Table
The Farthing Trust (Cambridgeshire)
Henry Smith Charity (Wiltshire)
John Beane's Charity (Surrey)
Wirral CVS
Relief in Need (Chester)
Leeds Family Holiday Fund
Loughborough Welfare Trust
Loughborough Lions Club
The Finchley Charities (London)
The United Charities (Liverpool)
PSS Liverpool Personnel Services
Lions Club, Cranleigh
Annan Round Table
Newcastle Lions Club
Leicester Charity Organization Society
Chronicle Cinderella Fund (Preston)
Finchamptstead Relief in Sickness
Monmouth Charities
The Shelroy Trust (Norwich)
Army Benevolent Fund (London)
The Redcliffe Parish Charity
Lowestoft Round Table
The Francis C Scott Charitable Trust
The Bungay Charity (Suffolk)

Abbot Memorial Trust (Gateshead)
The Lord Mayor's Fund (Norwich)
Reading Dispensary Trust
Winchester Charity Holiday Fund
John Beane's Charity (Surrey)
Chippenham Round Table
Lancaster Round Table
St Laurence Church Charities (Reading
Bassetlaw District Council
Pontypridd Round Table
Scunthorpe Round Table
Lowestoft Lions Club
Wolverhampton Lions Club
Petersfield Round Table
Darlington Bongate Round Table
Gibsons Charity Suffolk
Barnet Round Table
Letchworth Round Table
York Round Table
Hallamshire Round Table
Hall Green Lions Club
Spalding Round Table
Bristol Round Table 9
Herts Convalescent Trust
Frome Round Table
Rotary Club Wolverhampton

SPONSORED EVENTS AND APPEALS

The Society would like to thank those who supported
Michael Wheeler - Sponsored Parachute Jump
Mr B Sara - Great North Run
John and Barbara Arrowsmith - Great North Run
P Loveday - Sponsored Cycle Event
Paul Butler - Sponsored Slim
Penryn Pre-school - Sponsored Bike Ride
Mr Hateley - Great North Cycle Ride
Scott and Stuart McNee - Scottish Half Marathon

Area Support Families

Martine and John Brennan.....Tel: 01524 382164
105 Barley Cop Lane, Lancaster, Lancs, LA1 2PP

Robert and Caroline Fisher.....Tel: 01799 586631
The Horrells, Great Samford, Affron Walden, Essex, CB10 2 RL

Suzanne and Jeffrey Hodgetts .....Tel: 01827 56363
6, Godolphin, Tamworth, Staffs. B79 7UF

John and Barbara Arrowsmith .....Tel: 0191 2921234
11 Penfold Close, Fairways Est. Benton, Newcastle on Tyne NE7 7UQ

Sean and Pauline Mahon .....Tel: 01142 304069
41 Stumperlowe Crescent Rd, Sheffield, South Yorkshire S10 3PR

Mary and Robin Gooch .....Tel: 01435 883329
Highbank House, Swifehill, Broadoak, Nr healthfield, East Sussex. TW21 8XG

David and Monica Briggs .....Tel: 01777 700046
7 Hunber St. Retford, Notts. DN22 6LZ

Bill and Sylvia Blackburn .....Tel: 01270 626809
11 Beatty Rd, Nantwich,Cheshire, CW5 5JP

Tony and Shirley Eyre .....Tel: 01666 825215
7 Elmer Close, Malmesbury, Wilts. SN16 9UE

Ann and Michael Kilvert .....Tel: 01686 624387
Windy Waye, Nantoer, Newtown, Powys, SY16 1HH

Contact for Scottish Families

Alan and Fiona Byrne Tel:0141 6430034
3 Jedburgh Avenue, Rutherglen, Glasgow G7 3EN

Cath and Jim McLean .....Tel: 01463 224615
5 Tern Avenue, Inverness, Highland IV2 3YN

Northern Ireland Co-ordinating Commiee

Kieran Houston (Chairman) .....Tel: 01504 884168
15 Barrack St, Strabane, Co. Tyrone, BT82 8HB

Margaret Kearney (Secretary) .....Tel: 0126 5762073
12 Coleraine Rd. Ballycastle, Co. Antrim BT54 6DU

N.B. John and Barbara Arrowsmith's telephone number has been changed.