NEWSLETTER SPRING 1996

THE SOCIETY FOR MUCOPOLYSACCHARIDE DISEASES

National Registered Charity No.297034

MANAGEMENT COMMITTEE

MPS OFFICE: 55 Hill Avenue, Amersham, Bucks. HP6 5BX

NORTHERN MPS OFFICE: 168 Hesketh Lane, Tarleton, Lancs PR4 6AT

The Society for Mucopolysaccharide Diseases

55 Hill Avenue, Amersham, Buckinghamshire HP6 5BX
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The MPS Society is a voluntary support group, founded in 1982, which represents over 800 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 fund-raising by members, and run by the members themselves. Its aims are as follows:-

1. **To act as a parent support group**

2. **To bring about more public awareness of MPS**

3. **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 a biochemist at the Christie Hospital, Manchester. 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 disease, 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 : ***Lauren Cawthorne from Hull aged 2 years who suffers from Hurler Disease.***

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Deadline for the Summer Newsletter
26th of June 1996

CHAIRMAN'S REPORT

Charity Award

The Annual Guardian Jerwood Award is designed to acknowledge and reward individual excellence in small to medium charities. To our absolute delight our Director, Christine Lavery was one of the three to receive a prize from the Duchess of Kent.

The Duchess said in presenting the award, "Christine had suffered the death of a son from an MPS disease, but had gone on to devote her life to helping other families in the same position. The award is quite an exceptional means of paying tribute to such dedication."

Christine's parents nominated her for the award and the prize money is being used to send Christine to the International Symposium on Mucopolysaccharide Diseases, Wollongong, Australia in May 1996.

Christine with her Guardian Jerwood Award presented by the Duchess of Kent.

DUNBLANE

I am sure that all families and members of the MPS Society would wish to express our deep sadness to the families of the children of Dunblane.

CHAIRMAN'S REPORT

Thank you Charles

The Society, or to be more precise Christine, is very alert to finding grants for specific things. We have been able to up-date much of our office equipment and move some way into the field of high technology through obtaining certain grants (these monies have to be spent on specific items and cannot for example be used for the general running of the Society or family support).

With this new technology the Committee decided that the Quarterly Newsletter could best be produced "in house" at the MPS Office and this is the first example of the work.

Charles O'Toole as you all know has done a splendid job as Editor for very many years and I am sure everyone joins with me in thanking him for all of his efforts. The job of Editor is far from easy particularly as you are often reliant on information being sent to you regularly by your readers. Some quarters Charles has been inundated with letters, articles etc. but some quarters there was almost nothing. We all feel that the Newsletter is a most important part of the Society so please do send in articles, pictures, letters etc., not now to Charles but to the MPS Office. With our new technology, photographs can be returned almost immediately and they will never ever have to be cut in any way. If you have any suggestions or thoughts on what you would like to see in the Newsletter do write to the office, or to me if you like, setting out your views. The Newsletter is your Newsletter but it does need your continued contributions and ideas to make it flourish.

Thank you again Charles - let's hope that the standard you set does not diminish.

Jeans for Genes

At the time of writing this I have no idea as to the amount which has been collected but what has come over loud and clear to me is that thousands of people who went to work in their jeans on the 1 March, enjoyed very much dressing in this way and contributing their money for what to all of us is a very worthy cause. Very many members of the Society took part in various ways in publicising the event, 'phoning and contacting local and distant firms and encouraging them all to join in. Several members appeared on television and on the radio and countless were featured in local and national newspapers. I am not a believer in self praise but on this occasion I do think all the members of the Society should congratulate themselves on doing a marvellous job. There will no doubt be very many funny stories and pictures - what may amuse you all is that Christine had to borrow her husband Robin's jeans for her appearances on 1 March as she had nothing suitable to wear!

A. G. King
Chairman

DIRECTOR'S REPORT

The following pages in their own way illustrate the energy and enthusiasm being devoted to awareness of Mucopolysaccharide Diseases, by professionals, families, extended families and friends alike.

Jeans for Genes has given us a unique platform from which to inform a huge number of the population about MPS diseases and how they affect our children. In the lead up to the 1st March, the 'Jeans for Genes Day', MPS Society staff and families featured in more than fifty radio, television and newspaper articles. This has generated three enquiries from families who are concerned that MPS may account for their child's problems. We have given these families advice on where to turn for help in ruling out MPS. We hope their children don't have MPS but will be here for them if necessary.

MPS would not exist without its families and could not provide the help and support it gives families or fund research without the fund-raising efforts often inspired by you.

With the help of the Department of Health and Buckinghamshire County Council we have been able to up-date our computer system and are the proud owners of a flat bed scanner. In the next newsletter we will be giving you our Email address and telling you where we can be found on the Web.

I too would wish to offer my sincere thanks and appreciation to Charles O'Toole for all the time he has committed over many years to compiling the Newsletter. There is a feeling of both excitement and trepidation at taking on what undoubtabley is the Society's most important 'vehicle' of communication with all the families.

We need your help with news, information, ideas, photographs and constructive criticism.

We need lots of personal stories.
How you felt about diagnosis?
How you cope?
Practical hints.
Advice on how others have solved or dealt with a problem.

Don't forget also to tell us about the successes.

We look forward to hearing from you in the MPS office. Don't forget the next Newsletter deadline is the **26th of June 1996**.

Christine Lavery

MILESTONES

Births

Congratulations to Evelyn Kennedy from Stockport on the birth of her daughter, Emma Rachel who was born on the 13th of February 1996.

Congratulations to Mandy Owst and Chris Johansson whose baby son, Jack Daniel was born on the 21st of March 1996.

New Families

Jackie and Barry Perfect from London, whose son, Benjamin, born on the 13th of August 1993, and daughter, Emma, born on the 25th of January 1995 have been diagnosed with Maroteaux/Lamy.

Lynn and Mike Halsall from Manchester, whose son, Nathan, born on the 13th of December 1992 was diagnosed as suffering from Hunter Disease.

Sajjad Hussain, from Birmingham who is 22 years old has recently been diagnosed with Morquio Disease.

Vicky Moore from Warwickshire, whose daughter, Samantha who was born on the 23rd of April 1991 was diagnosed as suffering from Scheie Disease.

Keith and Jacqueline Jones from South Glamorgan, whose daughter, Melanie, was born on the 9th of February 1991 has been diagnosed with Sanfillipo Disease.

Julie and Neil Pope from Mid Glamorgan, whose son, Craig, born on the 22nd of July 1990. Craig has recently been diagnosed as suffering from Sanfilippo Disease.

Michelle and Colin Lowe from London, whose daughter, Sophie, born on the 19th of August 1994 has been diagnosed with Hurler Disease.

Ceri and Andrew Coleman from Cardiff, whose son Joseph born on the 13th of December 1989 was diagnosed on the 8th of March 1996 as suffering from Aspartylglycosaminuria.

Michaela Proctor and Warren Slinger from Slough, whose daughter Charlotte, was born on the 23rd of January 1994 and has been diagnosed with Hurler Disease.

Debbie and Colin Mills from Windsor, whose son Jordon, born on 1st of March 1995 has been diagnosed with Hurler Disease.

Our apologies go to Mary and Andy Wragg from Hereford for omitting to include their son, Jacob in the newsletter last year. Jacob was born on the 23rd of November 1993 and suffers from Hunter Disease.

Marriages

Congratulations to the parents of Jade Robinson, Dawn and Mark, who were married on the 13th of January 1996. Jade is 5 years old and suffers from Hurler Disease.

Deaths

Amanda Corcoran from West Ealing died on the 30th December 1995 aged 3 years. Amanda suffered from Hurler Disease and is remembered along with her brother, William and sister, Marion, who also died from Hurler Disease.

Matthew Whitehouse from Shrewsbury died on the 7th of January 1996, aged 15 years. Matthew suffered from Sanfilippo Disease.

Alistair Reid from Dunoon died on the 29th of January 1996, aged 8 years. Alistair suffered from Hurler Disease.

Sadly Lauren Murray from Belfast died on the 20th of January 1996 aged 4 years. Lauren suffered from Hurler Disease.

Linda and Alan Fuzzard's son Thomas from Birkenhead died on the 20th of March 1996. Thomas who was 11 years old suffered from Hunter Disease.

Abigail Pullin from Colerne died on the 21st of March 1996. Abigail who was 13 years old suffered from Sanfilippo Disease.

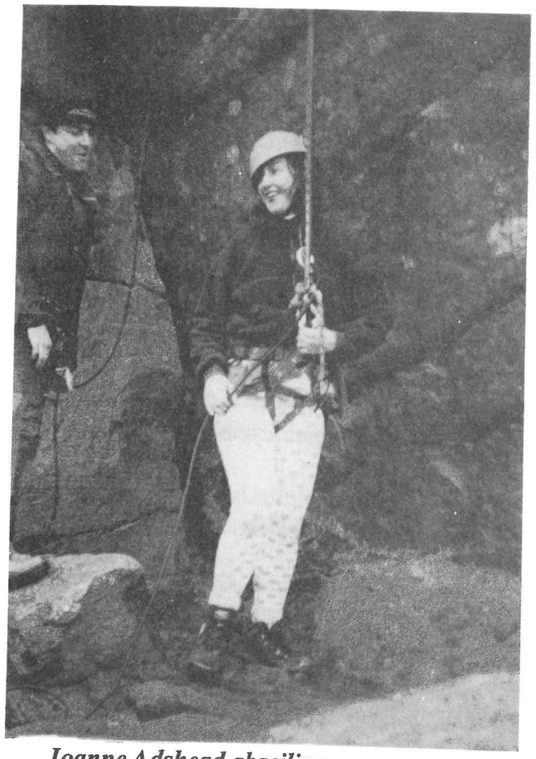
Diane Bulloch from Bishop Aukland died on the 23rd of March 1996. Diane was 48 years old and suffered from Morquio Disease.

CONGRATULATIONS

We would like to extend our best wishes to Ron Snack on his .....birthday at the end of March. Happy Birthday Ron from all your Friends!

MP told us you were **50** Ron but we don't believe it!

AREA FAMILY TRAINING



Joanne Adshead abseiling

GLENBROOK GIRL GUIDES CENTRE HOPE, DERBYSHIRE

1st - 3rd of March 1996

The Society's Area Support families travelled from all parts of the United Kingdom to receive training in a number of areas including:

Advocating the needs of individual MPS families

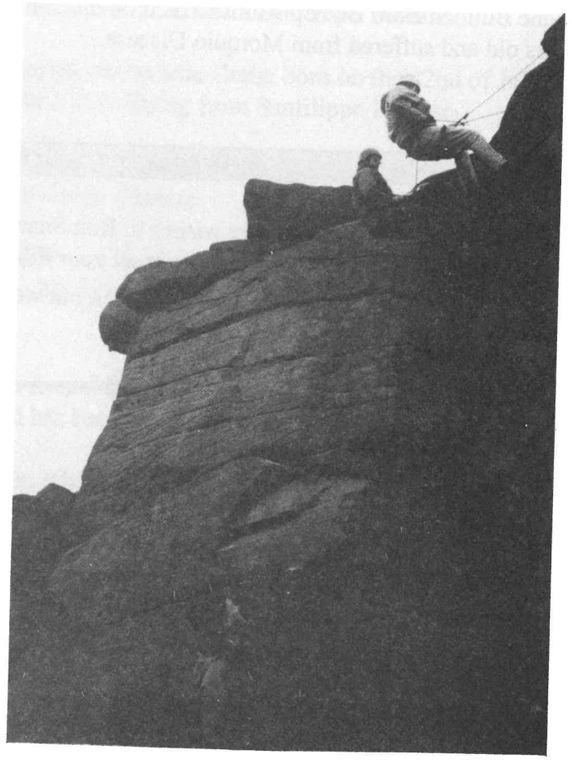
What more can the Area Family do?

Recognising the needs of fami lies from Ethnic Minorities.

Working within the Children's Act.

Making the MPS Society work for families.

A very important quality of our Area Support Family is being able to cope and function when faced with a new or challenging problem. As seen from the photographs these Area Families participating in the session on group dynamics and leadership skills did very well.



Tony Eyre abseiling with the help of Rob Paget

AREA FAMILY TRAINING

People

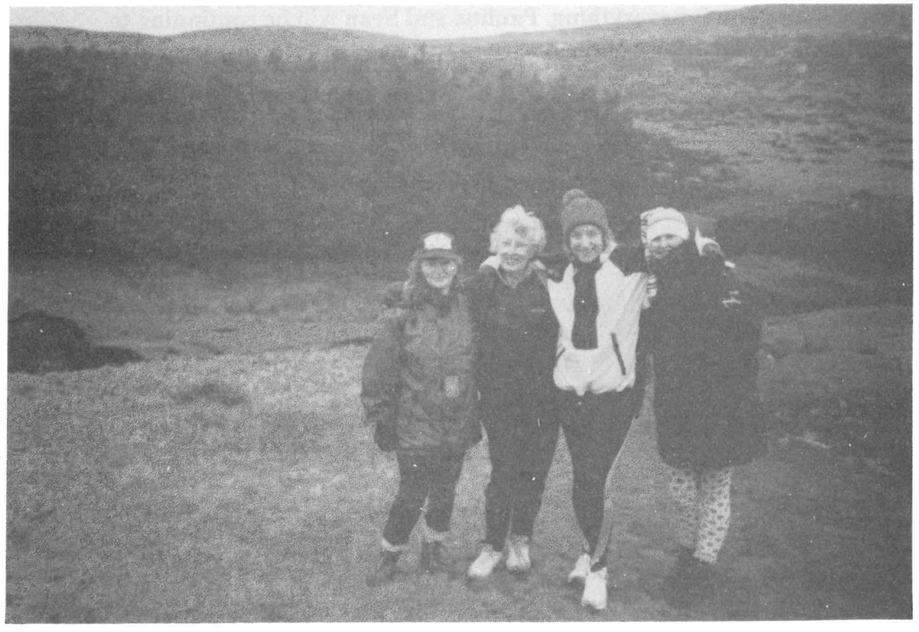
What do we know about people?

We are people. We come in all different shapes and sizes, tall ones, short ones and large ones. We all have different personalities, temperaments, points of view, aims and outlooks in life. Whatever sort of person, we are together a group of people. We are able to make ourselves noticed, heard and able to make people aware of the MPS Society. We aim to give support to other people who find themselves in the same situations as we are experiencing, or have experienced in the past.

Regardless of religion, beliefs or race we will be there for the people if they need us.

Barbara Rollinson

Editor's Note
Those attending the Area Family Training Weekend were asked as part of their public speaking session to talk on a specific subject for 2 minutes. Barbara was given the subject 'People'. We thought what she had to say was worth sharing with you.



Barbara Arrowsmith and Joanne Adshead enjoying the healthy weather along with two of the volunteer cooks, Jane and Pam at the Area Family Training at Hope in Derbyshire.

AREA FAMILY SUPPORT

Diary of Events

Suffolk Wildlife Park - Caroline and Bob Fisher	**14th of April 1996**
Garden Party - Edward Nowell	**29th June 1996**
BBQ - Anne and Mike Kilvert	**21st July 1996**
Scottish Clinic - Alan and Fiona Byrne	**October 1996**
Childhood Wood	**25th October 1996**
Christmas Party - Anne and Mike Kilvert	**8th December 1996**

Retiring Area Families and New Area Families

After many years as Area Family, Pauline and Sean Mahon are retiring. They have given generously of their time in supporting families in Yorkshire and Humberside as well as organising many family days. From our hearts we say an enormous thank you for everything. Pauline and Sean will be continuing to manage the financial affairs of the Society and I know would always be pleased to hear from 'their families'.

Monica and David Briggs and Trevor and Barbara Rollinson both of whom attended the recent training have kindly agreed to take over the role of Area Support Families for Yorkshire and Humberside from Pauline and Sean.

Sadly we have seen a significant growth in the number of families receiving an MPS diagnosis. This in turn puts considerable strain on the personal resources of the Area Family who are usually caring for one or more MPS children and emotionally supporting MPS families in their area.

The Society is therefore adopting a strategy whereby two or three families work together to provide support in a given area.

Zerina and Sajjid Shah have joined Sue and Jeff Hodgetts in the West Midlands to concentrate support to our Asian families. Zerina speaks Urdu, Punjabi and Kafhmiri.

Joanne and Gary Adshead from West Houghton are stepping in as Area Family along with John and Martine Brennan for the North West.

AREA FAMILY SUPPORT

SCOTTISH CHILDREN'S HOSPICE OPENS

On March 12, the first ever Scottish Children's Hospice (CHAS), opened its doors. Five years ago, in 1991, Lorraine Dickson, whose son Marc had HUNTER syndrome, and Nancy McCalman, mother of another terminally ill boy called Daniel, began their quest for a Scottish Hospice.
Lorraine, like many other MPS families, had used Martin House in York, but wanted to see a hospice for Scottish children. Their first cheque was for £50, with a target of £2 MILLION.

Two years later, in March 1993, the Scottish Daily Record set up an appeal to raise funds, and its readers have donated £4 MILLION of the £10 MILLION raised to date. Lorraine knew that Marc did not have many years to live when she and Nancy started their appeal, and sadly he died in the same month that the 'Record Appeal' began. Lorraine said " it was almost as though, on the day the Record took up the campaign, Marc let go of life, for his part was now over".

The Hospice is a beautiful place, set peacefully in the little town of Kinross, Perthshire, overlooking Loch Leven. The Scottish MPS families had a pre-opening visit on the Sunday before the Hospice opened, and we were all most impressed. Two MPS families were booked in for the first weekend, with another two following on within a few weeks - it looks as though Rachael House, as it is to be called, will be well used by our families.

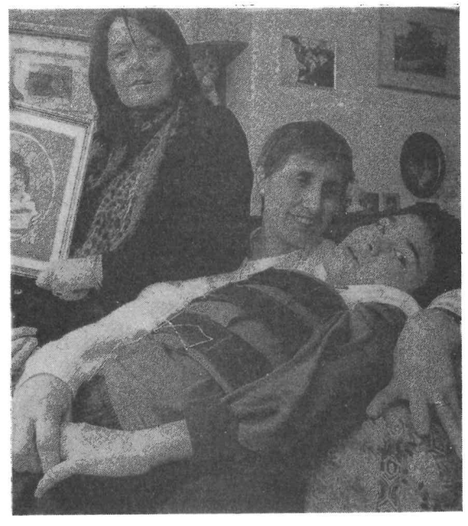
A thank you must be given to the staff at Martin House for all their support to Scottish MPS families in the past.May I pay special mention to Lorraine,who, along with Nancy, must feel very proud to have been the founders of the first Scottish Children's Hospice.
No MPS child will ever be forgotten, but Marc certainly did not loose his life without helping children like him in the future.

The photograph shows Nancy and Daniel, along with Lorraine holding a picture of Marc, at the new Hospice.

Alan Byrne

ALAN AND FIONA BYRNE

SCOTTISH CONTACT FAMILY



AREA FAMILY SUPPORT

Dates for Your Diary 1996

14th of April 1996

Sunday the 14th of April is the date of the Family day being organised by Caroline and Bob Fisher from Essex.
Families should meet at the Park entrance at 11am.

The Park contains 100 acres of beatuiful countryside where you can follow the trail around the Park on foot or you can take the Safari Road Train.

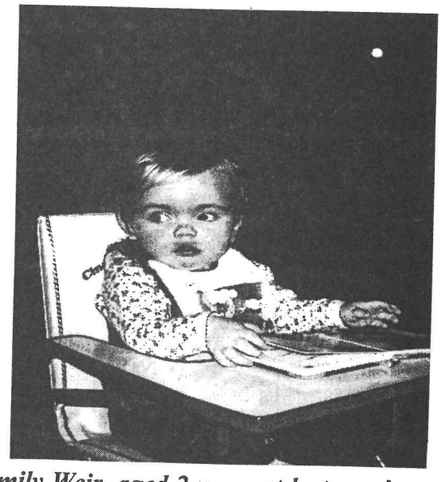
There is access to the whole park for wheelchairs

We hope everyone will have a great day and that the sun shines.

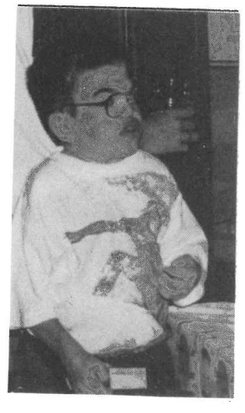
October 1996 (date not yet decided)

Scottish Family Clinic

The clinic will be held in a Glasgow Health Centre and each family will have a 30 minute appointment with Dr Wraith and Dr Vellodi. You will also have the opportunity to discuss with Christine or Mary any MPS related issues.



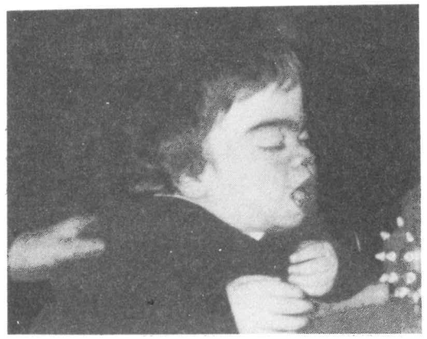
Emily Weir, aged 2 years at last year's clinic. Emily suffers from Hurler Disease.



Edward Nowell's Garden Party

Edward Nowell's parents, Holly and Edward are organising a garden party to be held on the 29th of June 1996. Shirley and Tony Eyre will be organising this day as an area family day and families who wish to attend should bring a picnic lunch to have before the garden party.

FAMILY SUPPORT



Sadly Alistair Reid aged 8 years who suffered from Hurler Disease died on the 29th of January 1996.

Daniel Singh and Mickey Mouse with Mum and Dad at 'Disney World'.

Sandra and Rash Singh had a great time in 'Disney World' with their 4 year old son Daniel who suffers from Hunter Disease.

It was due to the generosity of their friends at the Tesco Store in Radford, Coventry. Through their numerous sponsored events, raffles, donations and a disco night they were able to raise enough money to help Sandra, Rash and Daniel have a holiday of lifetime.

A PROMISE TO ALISTAIR

MY PRIDE AND JOY

There is an emptiness inside
That is so hard just to describe.
There's no one else who knows the fear
Of losing one who is so dear.
He was a special child you know
That's why it's so hard to let go.
But now I've learned he's lost his pain
I know that I will see him again.

He's up in Heaven high above
And fills the whole world with his love.
Although that people cannot see
I know he's watching over me.
I love and cerish all the time
I had with him for he was mine.
This boy, his name was SUPER ALLY
Who's love and warmth I'11 always carry.

He was so soft and did no harm
Now God will take him in his arm.
He warmed your heart with his great smile
And that would always last a while.
I know he was a special boy
That's why he's called my pride and joy.
Well now it's time to say goodbye
I know I will always wonder why.

Love and remember you always.

Daddy
This poem is dedicated to Alistair

FAMILY SUPPORT

Problem Page

We would like to start a question and answer page where you send in a problem and we print it and invite solutions from readers. Following are two examples of recent problems which have been put to Christine and the office staff.

Question?
How do you prevent a child putting everything in his mouth? (Hunter)

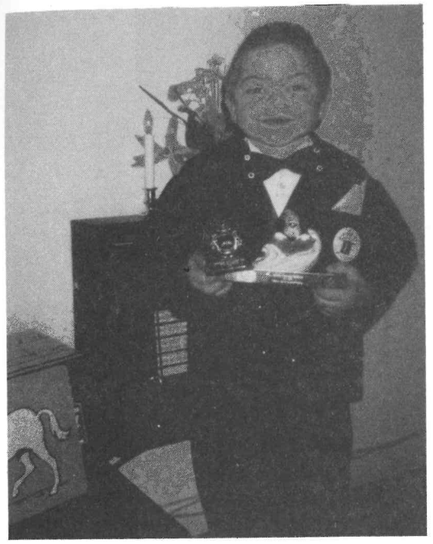
Answer:
Try giving your child an appropriate teething toy or ring and hopefully this may distract the child from putting less suitale things in his mouth.

Question:
I would like some information on how other parents deal with puberty and menstruation in a teenage girl suffering from Sanfilippo Disease. Also what experience have parents had in using contraception to control periods and pre-menstrual tension?

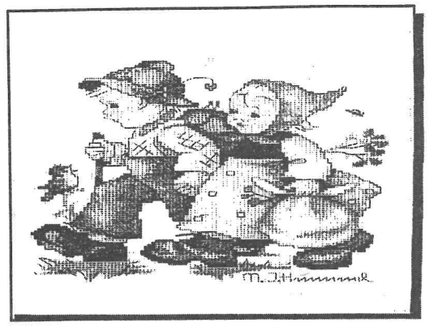
Answer:
We would be grateful if parents and professionals who have experience of these problems would send their replies to the MPS office in Amersham so that we can pass on this information.

Don't be shy. Send those questions in now to the MPS office Amersham. We will of course treat your correspondence with anonymity.

FAMILY SUPPORT



This is a copy of the tapestry that David's Grandparents, Mr and Mrs Shiff raffled for MPS.



The little boy who is Magic.

Extract from the Daily Post, 23rd of February 1996.

Eight year old David Oulton has a passion for magic. His family are constantly on the alert for his next trick, but he cannot wave his wand and transform the faulty gene which has made him different from other children. Next Friday we will be alerted to an Appeal to fund research into 5,000 genetic disorders which affect David, as well as the more commonly known cystic fibrosis. Four charities have joined forces to hold the Jeans for Genes Appeal day (1st of March) encouraging us all to don jeans for the day and donate just £1 to raise £2m to set up a national Gene Therapy Centre which will advance knowledge and may lead to a more effective treatment.
For Selma and Geoff Outlon of Old Swan, Liverpool, the discovery that their son had a Mucopolysaccharide Disease, named after the Canadian Professor, Charles Hunter, who discovered it in 1917, was a complete surprise. There had been no previous sufferers in the family or indication that anyone might be a carrier. Children with this disease, who usually look alike, are missing enzymes in the genetic structure which deal with the disposal of building tissue, so that instead of being eliminated it is stored in cells and causes damage.
It is a very rare disease affecting only boys, although girls may be carriers. David's sister, aged 12 years will be able to have tests when she is planning a family of her own, to see whether she is a carrier. Selma Outlon still remembers the shock of the discovery when she was told by a paediatrician, during a routine hospital visit for chest problems that her 18 month old son was dying. "I was devastated. I can look back now and see that there were problems from when he was born but at the time I didn't have a clue about the disease. I had to take the bus home to tell my husband that the doctor thought David was dying. My Mother was with me and we just sat on the bus wondering how we could hide it from my Dad who was ill and who was devoted to David. It was so strange it was like we were talking about the common cold."
As it turned out David's disease wasn't the severest kind and now aged eight he attends a special school in Woolton, Liverpool, where his Mum is a voluntary helper. He is also an enthusiastic member of Merseyside Junior magic Club and his local Beavers' Pack.

FAMILY NEWS

This lovely postcard was sent to the MPS office from Singapore by Sarah Long.

Sarah is on holiday travelling to a few destinations throughout Asia. Sarah is 25 years old and although she suffers from Morquio Disease Sarah does not let anything get her down.

We hope Sarah had a great time.

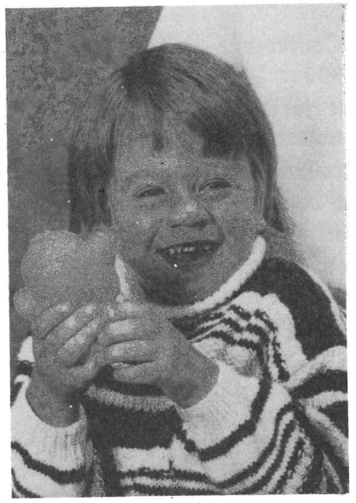
Sarah's Dream Ski Holiday

Sarah Burgess from Luton was lucky enough to be chosen to go on a ten day ski holiday.

Fourteen year old Sarah who suffers from MLIII and cannot walk more than a few steps will use specially adapted equipment to allow her to take part in the winter sports on the holiday.

The holiday was organised by Harpenden Across Trust and sponsored by Happy Days, a Luton based organisation which raises thousands of pounds each year to help children with disabilities enjoy an active holiday.

OVERSEAS NEWS



News from South Africa

This is TJ whose Mum, Lynn is developing communications to as many families as she can in South Africa..

She is trying to set up a Society in South Africa as well as caring for her six year son, TJ who suffers from Hunter Disease.

Lynne was successful in having an MPS story broadcast on South African TV.

We would like to wish TJ and Lynne lots of luck with their endevours in trying to raise the awareness of MPS in South Africa.

Dear Ms. Lavery,

I am a second year Mathematics student at Oxford University. For the past two summers I have been an au pair for Liz and Richard Volk in Sinzig near Bonn, Germany. They have twelve year old twins with an MPS Disease , Sanfilippo Disorder, I believe.

With the Volks I generally looked after their other two healthy eight and two year old children as well as cleaning etc. Understandably, with special children the household tasks sometimes just don't happen.

I was wondering if you knew of any families in the area who would like a hand during the vacations when I am at home I am happy to visit them on a voluntary basis and either look after the children for a couple of hours to give their parents a break or do whatever chores are needed.

If you would like a reference, I am certain that the Volks would be more than happy to oblige.

Yours sincerely

Anthea Rowe.

Anthea Rowe has asked that we print this information in the Newsletter and we hope that her kind offer will be taken up. If you would like to contact Anthea please write to:-

Anthea Rowe, 23 Portsmouth Ave.
Thames Ditton,
Surrey KT7 ORU Tel: 0181 398 2700

FAMILY WEEKEND CONFERENCE

The Society for Mucopolysaccharide Diseases

Fourteenth Annual Conference
20 - 22 September 1996
Stakis Country Court Hotel Northampton

Any families who would like to attend the Conference but feel that they can't afford the subsidised cost could possibly apply to their local Social Services Department for financial help. Please let us know at the Amersham office if you do apply so that we are aware of the application and can add our support to your request for funding.

Anyone applying to Social Services for help with the cost of attending the National Conference may wish to use the following letter as a guide:

Dear Social Services (or named Social Worker)

Our family/son/daughter suffers from a rare Mucopolysaccharide Disease.

We as a family belong to the Society for Mucopolysaccharide Diseases, which gives us practical help, understanding and support. This year the Society for Mucopolysaccharide Diseases is holding its 14th National Family Conference on the 20th to 22nd of September 1996 at the Stakis Hotel, Northampton. The weekend will consist of Medical seminars and a variety of Workshops, which will provide information and advice for families and individuals suffering from these life limiting conditions. There will be opportunities to share experience with other families who are in the same situation as ourselves.

(Affected person's name) .....aged ..... suffers from .....which is a Mucopolysaccharide Disease.

The Society of Mucopolysaccharide subsidises 50% of the cost for the Conference for families like ours. However we still can't afford the cost but know how valuable it would be for us to be there and learn more about our child's problems.

To enable us to attend this weekend, it will cost our family £.....

We are able to pay £.....

We would appreciate it if the Social Services could help us to attend this conference by providing a grant of £.....

Yours sincerely

.....

Please try and book the conference as soon as possible.

INFORMATION

Schools, Special Schools & Statements (*extract from Straight Talk on Disability*)

John Wright runs IPSEA the Independent Panel on Special Educational Advice. IPSEA can give parents expert second opinions on the special educational needs of their children.

Children have special needs if they have a learning difficulty or a disability which causes them to need additional help at school. Such children might have moderate or severe learning difficulties, be physically disabled, emotionally disturbed or sensory impaired.

The Government have produced a Code of Practice which sets out a school's duties towards children with special educational needs:

- how they should be assessed
- how their progress should be monitored
- how parents should be involved in the decisions made about their needs.

If a child's special educational needs cannot be met by the resources available in an ordinary school, then the Local Education Authority must assess the child's needs and decide what is needed. You can ask for this assessment and you can appeal to a Tribunal if the LEA refuse. The LEA will collect reports from an educational psychologist, a teacher, a medical officer, a social worker and the parents.

If an assessment shows that a child's needs cannot be met by the resources available to an ordinary school, the LEA will issue a Statement of Special Educational Needs. This sets out all of the child's needs and how the LEA should meet them. The LEA might decide that a child should attend a special school. If an LEA cannot cater for a child in its own area, they must arrange for the child to attend either a school in another LEA or a private school.

Before a Statement is finalised, parents have the right to see a draft copy, to see all of the professional reports collected by the LEA and to have meetings with any of the professionals who wrote the reports. At this stage, parents can say which school they want their child to attend.

If a parent is unhappy with the way their child's needs are described in a Statement, or with the provision the LEA says is needed, or with the school named, they can appeal to the Special Educational Needs Tribunal. If they win their appeal, the LEA will be ordered to change its Statement.

Once a Statement is issued, an LEA has a legal duty to arrange that the special education provision

INFORMATION

set out in it is made. They must also arrange for a Statement to be reviewed at least once a year, to make sure that it is still meeting the child's needs.

Parents can ask for the school named on their child's Statement to be changed, or for a fresh assessment to be made. If the LEA refuses, parents can appeal to the Tribunal. They can also appeal if the LEA amends the Statement in a way which they do not agree with.

"*Special Educational Needs a guide for parents*" and the "*Code of Practice*" are available to parents free of charge from the Department of Education. Tel.No. 01787 880946. The guide contains a list of voluntary organisations which offer parents independent advice and support.

Questions to ask:

- How many children are in the Unit/School and what are their ages?
- Are there sufficient children in the Unit/School to provide a broad and balanced curriculum?
- What are the opportunities for integrating children in mainstream school?
- What are the standards as measured by the SATs scores and external examinations?
- What is the reputation and standards of the school?

(DFE/OFSTED Inspection information)

- How many of the teachers are fully qualified teachers of the deaf?
- Is the National Curriculum followed?
- What are the SAT/ GCSE results?
- What happens to the children after they leave school?
- How many speech therapy, physiotherapy and audiology sessions are offered each week?
- What is the method of communication and what are the reasons for this.
- How do they manage behavioural problems?

The method of communication is a crucial area in the determination of appropriate placement. Children who can absorb language and are able to listen, lip read and speak well should be able to manage in an oral environment. Other children learn better when supported by sign language.

Parents know their own child better than anyone. An objective assessment of needs is imperative and the choice of placement should meet the needs in the Statement. The best thing parents can do is visit the schools themselves, ask all the necessary questions, speak to other parents and then decide where your child will be most happy.

The MPS Society is very willing to help and advise parents on educational matters or you can contact IPSEA:-

Independant Panel on Special Educational Advice, 22 Warren Hill Road, Woodbridge, Suffolk, IP12 4DU - Tel: 01621 779781

RESEARCH

Research into Sanfilippo Disease

As most of you are aware, treatment of Sanfilippo Disease is still very difficult. This is partly because we still do not understand what is responsible for the abnormal behaviour. We do not know, for example, why there is virtually no clinical benefit from bone marrow transplantation. A team of clinicians and scientists at Great Ormond Street Hospital, the Institute of Child Health, and the Institute of Neurology propose to carry out some clinical and laboratory studies to address this. This research has been approved by the hospital research ethics committee.
Anyone interested in knowing more about this should contact Dr.Vellodi in the first instance.

Dr Vellodi - Institute of Child Health, Great Ormond Street, 30 Guilford Street, London WC1N 1EW

Gene Therapy for Hurler Syndrome

I thought it would be useful to write to you to give you some clarification of the position with regard to this project. In addition it gives me the opportunity through you, to thank the trustees of the MPS Society for all their help in the funding of this project without which it would have been impossible to have reached this stage.

As you know we attended the GTAC meeting on 13 December to present our proposal. We have subsequently heard from them that they have given approval to our trial subject to certain undertakings and conditions none of which fundamentally alter the trial, but do involve us making some alterations to our protocol. As a result of which I am not in a position to provide you with the final document as yet. I certainly will make available to the MPS Society as well as to Dr Ashok Vellodi a copy of the finalised protocol as soon as this has been approved by GTAC.

In addition we require clearance from the 'MEDICINE CONTROL AGENCY' (MCA) under the provision of the Medicines Act for clearance to put forward this as a new therapy. I am told that this merely involves filling in a number of forms and getting this rubber stamped. I know that Linda Lashford is in the process of going through this at the moment.

Once we have attended to all of these minor details we should be in a position to start the clinical trial proper.

The final paragraph of the letter from GTAC does say that the proposers i.e. us, should wait for confirmation of approval before commencing the research trial. We would all very much hope to be in a position to have satisfied all of the minor points they have raised by 1st of April.

I hope that this provides some clarification of the status of the trial so that all of the members of the society can be brought up-to-date.

Dr. J. E. Wraith

RESEARCH

Early Presentation in the Mucopolysaccharide Disorders

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Community Paediatric Research Unit, Department of Child Health, Chelsea & Westminster Hospital, London, UK

Accepted for publication 24 July 1995

Summary

The findings of an international questionnaire study of 258 children, affected by the four main subtypes of mucopolysaccharidosis, are presented. Questionnaires were completed by a parent or main carer and all subjects were alive at the time of contact and suffering from Hurler, Hunter, Sanfilippo or Morquio syndrome. A significant proportion of parents of Hurler children (24%) were unaware that anything was wrong with their baby before diagnosis but a larger number (45%) had felt concerned about their child's appearance. Similarly, in the case of the Morquio children, in 75% of cases, parents had been worried about some aspect of their child's physical appearance. In contrast, it was frequently delayed or regressing language which alerted parents of Sanfilippo (56%) and Hunter (32%) children, and this was associated with behaviour problems in 43% of Sanfilippo cases. There were many cases of delayed diagnosis, often occurring more than 2 years after concerns were first raised.

Keywords: Mucopolysaccharidosis, Hurler syndrome, Hunter syndrome, Sanfilippo syndrome, Morquio syndrome.

Introduction

The mucopolysaccharidoses are a group of inherited lysosomal storage disorders with distinctive phenotypes and a progressive course. There are seven recognised subtypes of which the Hurler, Hunter, Sanfilippo and Morquio syndromes are the most well known (McKusick & Neufeld 1983). These conditions are rare, the incidence of Hurler's syndrome has been estimated at 1 per 100,000 (Spranger 1972; Lowry & Re1 icks 1978) although there is reason to suppose that they are often under-diagnosed.

This rarity has meant that reports on natural history and the range of individual variation have usually only been based an very small numbers of cases. However, there have been suggestions that subtypes of syndromes can be distinguished from each other an the basis of severity of course or other associated clinical features (Van Der Kamp et al. 198I; Young et al. I982) and that behaviour problems are associated particularly with the Sanfilippo syndrome (Nidifer& Kelly 1983).
As part of a wider investigation of the natural history of these disorders, systematic information was collected, by means of a parental survey, on initial presenting features and an the time taken to arrive at the eventual diagnosis.

Method

The families of 293 live subjects were contacted for the most part via the Society for Mucopolysaccharide Diseases (founded in the UK in 1982), and of these a total of 258 (89%) responded. One hundred and forty of these cases were resident in the UK and

RESEARCH

90 were recruited from abroad. A further 28 cases were recruited via the Family Fund.
A breakdown of the distribution of sex and subtype is given in Table I.
A confidential postal questionnaire was completed by the parent or carer. This elicited basic demographic information on the index child together with information on the presenting signs and timing of diagnosis. (The findings relating to the other sections of the questionnaire concerning behaviour problems, development and day to day health worries are reported on elsewhere (Bax & Colvilie 1995).

Specifically parents were asked the following questions:

1. At what age did you suspect your child was not progressing normally?
2 What was it about your child that alerted you?
3 At what age was your child's condition diagnosed?

Results

First signs

Parents were asked to explain in their own words what it was that first alerted them to the fact that their child was not progressing normally. The answers were then categorised under nine main headings (see Table 2).
The 21 'family history' cases, in which the index child was tested because a previous sibling already had a diagnosis of mucopolysaccharidosis, were excluded from the main analysis and in I7 cases no information was given.
Consequently, the data for a total of 220 cases are presented.

Many of the parents of Sanfilippo children cited a combination of behaviour problems and developmental delay, in frequent association with language problems. The latter were often very specifically described in terms of loss of previous function; anecdotally families described having had difficulty in persuading professionals that their child had been able to say more words in the past, when younger.

In the case of the Morquio children, it was most often something about the children's physical appearance and particularly the shape of their ribs, back and legs that first gave rise for concern.
The appearance of the babies with Hurler syndrome was also mentioned frequently although here parents were struck particularly by facial appearance

Table I Breakdown of sex and diagnosis

		Male	Female	Total
Hurler/Scheie	(MPSI)	39	14	63
Hunter	(MPS 11)	54	-------	54
Sanfilippo	(MPS 111)	50	56	106
Morquio	(MPS IV)	15	20	35
Total				258

Table 2 Parental report of 'first signs' that child was not progressing normally

	Hurler n	%	Hunter n	%	Sanfilippo n	%	Morquio n	%
Language	0	-	15	32	53	56	0	-
Development delayed	8	16	15	32	50	53	1	4
Behaviour	3	6	5	11	40	43	0	-
Appearance	23	45	15	32	8	9	21	75
Hearing	7	14	13	28	19	20	1	4
Infections	10	20	8	17	17	18	1	4
Other including clumsy, stiff, hernias	11	22	10	21	7	7	5	18
Intuition	4	8	0	-	3	3	6	21
No idea	12	24	21	4	1	1	2	7
Total	51	100	47	100	94	100	28	100

and hirsutism, as well as occasionally by the child's distended abdomen. One mother described her anguish as she watched her son's 'little face changing'.
Proportionally, hearing problems were the most common presenting feature in the Hunter group, with associated language difficulties and general delay, and with the exception of the Morquio children, a sizeable proportion of each group was noted to suffer frequent infections with many families referring to a characteristic constant' runny nose'.

In a small number of cases a parent or other member of the family had felt intuitively that there was something wrong from birth, but there were several instances of babies with Hurler syndrome in particular, being diagnosed often as a result of investigations following observation of unusually large head circumference or kyphosis, before parents had noticed anything was amiss.

Time of diagnosis

In the vast majority of the Hurler cases a problem was suspected by the time the child reached its first birthday. Final diagnosis was reached on average at the age of 2 years and 6 months. In contrast, often nothing unusual was noticed about the Sanfilippo children in this sample until they had passed the age of 2 years and in some cases a problem was not suspected until the child was 4 or over.

The average age at diagnosis in this group, and for the children with Morquio Syndrome, was 4 years 8 months, and for those with Hunter syndrome, 4 years 3 months. Worryingly there were examples in all four main categories of diagnosis being reached much later, in some cases over the age of 20 years.

In summary, the average time interval between the age at which the problem was first suspected by parents and the time at which the final diagnosis was made was 1 year 5 months (Hurler), 3 years 1 month (Morquio) 2 years 6 months (Hunter) and 2 years 5 months (Sanfilippo) (see Table 3).

Table 3 Age at diagnosis

	Average age suspected	Average age diagnosed		Range
Hurler Scheie	1 year 1 months	2 year 6 months	-	0-30 years
Hunter	1 year 9 months	4 years 3 months	-	0-9 years
Sanfilippo	2 years 3 months	4 years 8 months	-	0-23 years
Morquio	1 year 7 months	4 years 8 months	-	0-35 years

Discussion

In the single largest attempt to study the natural history of the Mucopolysaccharidoses, a comprehensive questionnaire covering details of initial presentation, timing of diagnosis, development, health and behaviour problems was sent out to the families of 258 affected children, world-wide.

In particular, the findings on initial presentation highlight the need for an improvement in diagnostic services, both from the perspective of optimising the child's medical management and in order that genetic counselling and prenatal testing can be made available for subsequent pregnancies.

Furthermore, it should be noted that in the case of Sanfilippo syndrome, the latest to be diagnosed in our sample, it was frequently a combination of the children's behaviour problems and language delay (or in many cases actual regression) that first gave rise to the suspicion that all was not well. Difficult behaviour was also reported to be a major concern for families across the whole sample, as the disease progressed, particularly in Hunter and Sanfilippo syndrome (Bax & Colville 1995).

By drawing the attention of community paediatricians and general practitioners to parents' observations and, to the link with behaviour problems in these conditions it is hoped that this research will lead to earlier diagnosis in the future.

Acknowledgements

The authors thank all participating families for their co-operation and gratefully acknowledge the encouragement and financial support for this study by the Society for Mucopolysaccharide Disease.

FUNDRAISING

A Chance Remark

Not very long ago after being at the 1995 Conference I was telling my Mum how the Society had been hit by the Lottery in terms of fund-raising and donations. She was at work some weeks later when a colleague asked her about Steven and they began talking about the Society. A short while after this the colleague asked Mum if they could do some fund-raising and that she had approached some friends who would support her. Mum of course said yes. Mum and I thought they were going to do a sponsored walk in the usual manner however what they did do was a sponsored walk up Whernside the highest peak in Yorkshire.

Mrs Susan Henshaw organised the walk and her children also did a sponsored swim for MPS. Whernside is one of the "Three Peaks" and is some 2419ft high found in the Yorkshire Dales near Ingeborough. Susan was sponsored by her colleagues and relatives. Mr R I Howe, the employer of one of the walkers, Steve Morley gave a cheque for £100.00 to MPS and in total they raised £567.50 for the Society.

I would like to thank all those involved in the walk and in particular Susan and Mr Howe for their efforts. It just goes to show how a chance remark can lead to other people becoming involved in fund-raising.

Lynne Grandidge.

Jeans for Genes

I would like to thank everyone who supported Jeans for Genes Day on the 1st of March 1996. Many of you helped by telling your 'story' on television, radio or in the newspapers. This really helped spread the word as well as bring about awareness of MPS. But what you all want to know is after costs what have we raised?

Well so far, as of the 21st of March, between the four charities we have raised **£250,000**.

Christine Lavery

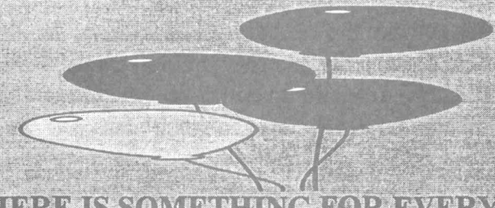
Please keep sending us your newspaper cuttings and if possible would you send the original photographs as newspaper photographs are sometimes a bit grainy.

FUNDRAISING

1995 National Draw Winners List

Prize	Winners
1 5 Day P&O ferry crossing for car and 2 passengers plus £500.00 cash	Mrs Vassell from Woking
2 1 Week self catering with Haven Holidays	Mr Varley from Harrogate
3 Admission to Alton Towers for 4	Kim Shillington from Southport
4 Waterman sterling silver pen	Mrs J Pickles from Huddersfield
5 Black and Decker strimmer	Mrs T Savin from High Wycombe
6 Admission to Blackpool Tower 2 Adults and 2 Children	Jean Deeming from Nuneaton
7 Admission to Camelot for 2	Steadman from Dunstable
8 Morphy Richards 10 cup coffee maker	Tony Rogers from Windsor
9 £20.00 gift voucher for Marks & Spencer	M Jenkins from Powys
10 £10.00 gift voucher for Marks & Spencer	Mike Grant from York
11 £10.00 gift voucher for Sainsbury's Homebase	J Rodgers from Hoddesdon
12 Ladybird Book and Audio Cassette	Annette Stanley from Hants
13 Barbie Doll and Accessories	Gail Edwards from Warminster
14 " " "	Des Williams donated the prize to an MPS child
15 L'Oreal skin care and hair products	Chris Pave
16 " " "	Mick from Luton
17 Golf Umbrella	P Hancocks from Nuneaton
18 " "	Angie Blythe from West Sussex
19 Aladdin Play Set	Michelle Stanley from Basingstoke
20 1lb Box of Black Magic	R Bateman from Rhondda
21 4 Claire Attridge Cook Books (small)	C Comber from Surrey

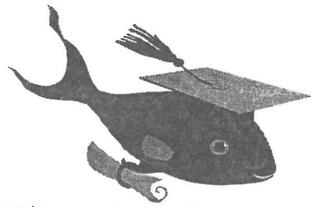
MPS FAMILY CONFERENCE
REMEMBER TO BOOK FOR THE 1996 CONFERENCE



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

FUNDRAISING

Canoe Endurance Race



Mr Howard Turner from Atherstone sent us a cheque for £750.00 which was raised as the result of prize money donated to the winning team in a Bristol to Windsor canoe endurance race which he and seven friends won in September 1995.

The race is the longest of its kind in the country (127 miles) and the winners set a course record. The event raises many thousands of pounds for different charities and is held every year in September. Mr Turner is looking for ideas on raising even more money this year so if anyone has any ideas please let us know.

Mr Turner works for Total Oil GB Ltd., who sponsored this race. Howard and Marilyn Turner also had a local collection.

Auction and Raffle

We would like to thank Deborah Anderson whose 3 year old nephew, James Edwards, suffers from Hunter Disease. She sent us a cheque for £2,0067.00 which was raised from holding an auction and raffle.

Deborah was very impressed with the kind donations she received for the auction from members of the local community and she would like a particular mention to go to, Cardiff City Football Club, Arsenal Football Club, Manchester United Football Club, Neath Rugby Club and the Rottweiler Club of Wales.

The Society would like to express our thanks for all the continued support we receive from members and their friends and families. We really need your help with fundraising!

FUNDRAISING

Jumping for Jacob and Gran went too..

Jacob's Mum, Mary who made the jump joined by more than 50 other jumpers.

More than 50 people took part in a charity splash to raise money for research into one of the rarest diseases in Britain.

Club members picked the charity after hearing about two year old, Jacob Wragg, from Worthing who has the disease which affects one child in every 166,000 born in the UK.

Jacob's uncle, Terry Kelly is a member of Arun Divers, which stages the leap for a different charity every year. Jacob's mother, Mary and his grandmother, Anne also took part in the jump from the 20ft footbridge into the River Arun at Littlehampton. Mr Kelly said, "Jacob's grandmother was not due to take part but she suddenly decided she wanted to give it a go and asked for a wet suit."

Several jumpers, watched by 150 supporters, chose fancy dress for the leap, including an entrant in a tuxedo. The club hopes to have raised around £2,000 for research into the disease, which has left Jacob deaf and suffering mobility problems.

This is an extract of the "Evening Argus" of the 8th of January 1996.

FUNDRAISING

DONATIONS

The Society is grateful to the following who made donations.

Harting Larks
Ann Fraser
Glasgow Boys' Brigade
Mrs Wright
Kuoni Travel Ltd
David Colclough
Rose Warne
Blair Foundation
Mr Stewart
Mr McGirr
Mr Nash
Mr Lamb
Jenny Hardy
Ms Short
Ms Davis
Ms Roberts
Mr Watts
Letchworth Round Table
Ms Walker
Mrs Pack
Hendersons, London
Monica Curry - Estate
Spiffing Stationary
The Fitton Trust
Andreas Charalambous
Hitchin Round Table
Coley Charity Trust
Bay Networks Ltd
Tregowen School
Mr Evans
Mr Egan
Baltic Charitable Fund
Elizabeth Jenkins
Angela Marriott
Mr Meaker
Mr and Mrs Maver
Mrs Hatt
Beatrix Whitlock - Estate
Pam Ballard
Crossfield School
Rodborough Tabernacle
Portland Windows
Lodge St Ninians No66
Lodge St James No123
Mrs McTiffin
Mrs Gelister
Mr P Mathews
Lloyds Bank, London
Mr and Mrs Wheeler
Mars Ltd
Mrs Duckett
Mr and Mrs Arrowsmith
Mr and Mrs Bullock
Saga Holidays
9 Supply Regiment RLC
Mrs Izzard
Mr and Mrs Colbourne
Helping Club, Broad Oak
Wilma Robins
Hillsdown Holdings PLC
Prospect Contract Furnishing
Mr Mills
Mr Morley
Brighton County Court
Mr and Mrs Dixon
Mr Turner

New Zealand Lions Club
London & Scandinavian Co Ltd
South Holderness School
Axa Equity and Law Society
Lowndes Lambert Group Ltd.
Al Fayed Charitable Foundation
Rochester Girls Grammer School
The Independent Order of Forresters, Rutherglen
Royal British Legion, Brechin
Oddballs Golfing Association

STAMPS

Rachel Todd
Miss Rickett
Mr and Mrs Shiff
Kathie Lawrie

FUNDRAISING

DONATIONS IN MEMORY

The Society is grateful to the friends and relatives of:

Gethin Robins
Jack Bradshaw (Grandfather of Sam)
Tom Flanigan (Grandfather of Chris Isaac)
Mrs Nellie Thompson
Sarah Kilvert
Rhianneth Wheeler

SPONSORED EVENTS AND APPEALS

The Society wishes to thank all those who supported:

The Jeans for Genes Appeal

CHARITY BOXES

Karen and Andrew Weedall
Midland Bank
Mrs Ingham
Emma Andrews
Mrs Jordan
Sidney Shiff
Lambs Lane School
Mrs Kirkpatrick
Mary O'Toole
Dalseter Rise Post Office
Wilma Robins
Mrs Weedall
Dawn and Graham Cawthorne

SPECIAL OCCASIONS

The Society would like to thank the following people who gave donations from these special occasions.

Mr and Mrs Wallace on their Ruby Anniversary
Thelma Barton from her 50th Birthday
Mr and Mrs Fraser on their 30th Wedding Anniversary