

Collaborative working to advance standards of care and the well being of lysosomal disorder patients and families in the UK



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WORKING TOGETHER SHAPING SERVICES FOR PATIENTS

Gauchers Association, Gloucestershire, UK ¹

The Society for Mucopolysaccharide Diseases (MPS Society), Buckinghamshire, UK ²

Niemann-Pick UK (NPUK), Tyne and Wear, UK³

Krabbe UK, Leeds, UK 4

AGSD-UK, (Pompe), Southampton, UK ⁵

Batten Disease Family Association, Farnborough, UK ⁶

The CATS Foundation, (Tay Sachs), London, UK ⁷

In 2005 following the designation by the Department of Health of central funding for enzyme replacement therapy (ERT) for patients with lysosomal storage disorders (LSDs) in England, seven registered patient associations representing Batten, Gaucher, Fabry & MPS, Krabbe, Pompe, Niemann-Pick, and Tay-sachs agreed to work together to improve standards of care for those affected by LSDs.

The LSD Collaborative has a Facebook page and website. Members of the Collaborative are the Chief Executives of the associations and each took on a responsibility in representing the patient voice on national stakeholder groups for commissioning, homecare, drug framework development and the Expert Advisory Group (EAG) meetings with NHS England and the eight LSD Centres of Excellence responsible for the clinical management of all LSD patients in England.



The Society for

Mucopolysaccharide

Diseases (MPS)

1275 Patients



National Centres

8 clinical centres in England where

of Excellence

expert multi-disciplinary teams

are based that clinically manage

all patients with LSD's, prescribe

treatment and run clinical trials

Annual meetings between the

Collaborative and each centre once

a year for updates and discuss

challenges and opportunities

Outcomes:

for join working i.e. patient

New patient information card

for newly diagnosed patients

satisfaction survey

Operate multi-disciplinary

clinics for some diseases

Patient advocacy

representatives attend

clinic in the centres to support patients and

family members/carers

Description:



Future Proposals

Description: The Collaborative have an annual business plan which is a live document and is updated each time it meets, that sets out our annual plans.

Outcomes:

economics.

 To develop a set of disease models for LSDs that will assist in health

> To promote the use of telephone clinics to support LSD families whose children have moved to palliative care.

> > **Future Proposals**

Expert Advisory Group Description:

A bi-annual meeting of doctors from the 8 national centres of excellence with NHS England and representatives of the patient organisations.

Outcomes:

 A maximum BMI of 27 set for dosing ERT Standard Operating Procedures for all

Diseases A national drugs tender to achieve cost

efficiencies

for Home Therapy i.e. Type 3

Gaucher infusions

 Collaborative research studies Standardisation of Hospital to Home

Expert

Advisory

Group

• Improved patient outcomes

Improved process time in the patient journey

transition in their lives.

Improved clinical outcomes

Transistion Pathway

to look at the transition processes in

moves from paediatric to adult health

to improve and develop this service in

the best interests of the young people

who are approaching this period of

services and to make recommendations

place when a young person with an LSD

An NHS England funded piece of research

Description:

 Patient focused booklet explaining transitioning for those affected by LSDs

Outcomes:

Transistion Pathway



Medics4RareDiseases

Targeting medical professionals to increase awareness of rare diseases especially around diagnostics. M4RD will supersede students4rarediseases which was an initiative of medical students from Barts in London, UK, in 2011, to educate medical students about rare diseases.

Description:

S4RD was supported by the Collaborative to access funding to further the aims of this project and to transition into Medics 4 Rare Diseases which is now its own organisation

Outcomes:

 Interest from medical students at 33 medical schools

6 medical schools holding rare disease

events in England, Wales and Ireland • An iconic brand, a zebra with a

stethoscope

Employment of an educational co-ordinator

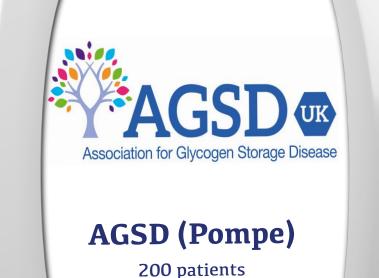
• Face, twitter and website sites

Annual symposium and evening seminars

on rare diseases with parent/patient involvement

Interest in this model from medical

students in Prague, Beirut and the University of Western Australia



Save Babies

Through Screening

Foundation

Krabbe UK

13 Patients

Clinical Reference Group

National Centres of Excellence

Collaborative

Diagnostic Workshop

Medics4Rare

Diseases

Diagnostic Workshop Description:

A one-day workshop initiated by the LSD Collaborative and developed in conjunction with lead scientists to discuss the current challenges in diagnostics i.e. access to materials, succession planning for staff and to work jointly to resolve the issues to benefit both labs and patients.

Outcomes:

 Representation from the key UK laboratories

Agreement for an annual meeting

 A paper to outline the challenges and some recommendations to present to NHS England for future service development



Glasgow Royal Hospital for Children Glasgow Royal Infirmary Belfast Belfast City Hospital

Cardiff

University Hospital of Wales

National Centres of Excellence

Clinical Reference Group

A multi-stakeholder group led by

NHS England Commissioning

Description:

to review and recommend

new services, treatments,

and guidelines

Outcomes:

A member of the

on the CRG for 4 years

Collaborative has had a seat

Manchester Royal Manchester Children's Hospital

Birmingham Birmingham Children's Hospital New Queen Elizabeth Hospital Cambridge
Addenbrooke's Hospital

> **London Great Ormond Street Hospital National Hospital**

> > Royal Free Hospital

Mental Health Workshop Description:

Mental Health

Workshop

Setting up and running a one-day workshop to raise the issue of mental health challenges in patients and parents with LSDs, followed by a survey of

patients and parents to identify the challenges they face and their experience of access to services Outcomes:

All National Centres were

represented at the workshop

 Recognition of the need to include 'think mental health' in clinic with patients

• The sharing of best practice across the centres

LSD Patient Survey

LSD Patient Survey Description:

An independent on line and paper survey sent to all LSD patients and parents/carers to understand their access to information, services and the quality of their experience when using the national centres of excellence and homecare services

Outcomes: • A total of 264 respondents across the diseases

- Feedback broken down for each centre
- Feedback broken down for each homecare company
- Feedback broken down for each disease/patient organisations
- This will now be an annual survey

Homecare

Framework

Homecare Framework Description:

A national framework for homecare services to all LSD patients across England, Scotland and Wales, overseen by NHS England and developed by representatives from the 8 National centres and a representative of the LSD Collaborative

Outcomes:

Standardised services and care for patients

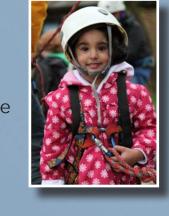
A drive to increase cost efficiency and care

• An increase in patient independence through

training to become self-infusers

Different levels of nursing care to support individual

• The majority of LSD patients receive their treatment at home





Batten Disease

Family Association

235 Patients







