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

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 LSD Collaborative is based in Birmingham Children's Hospital, Cardiff, the CATS Foundation (Tays, London), AGSD-UK, Pompe, Southampton, Krabbe Disease Family Association, Barnsley, and Addenbrooke’s Hospital. Birmingham Children’s Hospital, Cardiff, the CATS Foundation (Tays, London), AGSD-UK, Pompe, Southampton, Krabbe Disease Family Association, Barnsley, and Addenbrooke’s Hospital.

The Collaborative has an annual business plan which is a live document and is updated each time it meets, so set out our annual plans.

Future Proposals

Expert Advisory Group

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

Outcomes:
- Agreed on list of 27 centres for dosing ERT
- Agreed on standard operating procedures for all diseases
- A national drug tender to achieve cost efficiencies
- Collaboration on research studies
- Standardisation of Hospital to Home for Home Therapy, e.g., Type 1 Gaucher infections
- Improved clinical outcomes
- Improved transition process in the patient journey
- Patient focused booklet explaining transitioning for those affected by LSDs

Clinical Reference Group

A multi-stakeholder group led by NHS England and the LSD Collaborative to review and recommend new services, treatments, and guidelines.

Outcomes:
- Identification of new services on the CRG for 4 years
- Feedback broken down for each centre
- Feedback broken down for each homecare company
- Standardised services and care for patients and parents to identify their experience when using the national framework

Future Proposals

Medics4Rare Diseases

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

Outcomes:
- Improved transition process in the patient journey
- Patient focused booklet explaining transitioning for those affected by LSDs

National Centres of Excellence

Description:
- A clinical centre is assigned where expert multi-disciplinary teams are based that clinically manage all patients with LSDs, produce treatment and care plans.

Outcomes:
- Annual meeting between the Collaborative and each centre over a year for updates and discussion, for joint working in patient clinical follow-up survey
- New patient information card for newly diagnosed patients
- Establishment of multi-disciplinary teams for some diseases
- Patient advocacy representatives for some centres

Diagnosis & Treatment

Description:
- Diagnostic workshop initiated by the LSD Collaborative and developed in partnership with NHS England.

Outcomes:
- Improved process time in the transition from paediatric to adult health services
- Improved patient outcomes
- Pathway development for LSDs that will assist in health economics.

Future Proposals

Medics4Rare Diseases

Description:
- A one-day workshop initiated by the LSD Collaborative and developed in partnership with NHS England.

Outcomes:
- Improved clinical outcomes
- Improved transition process in the patient journey
- Patient focused booklet explaining transitioning for those affected by LSDs

LSD Collaborative

Description:
- An NHS England funded piece of research to look at the transition processes in place when a young person with an LSD moves from paediatric to adult health services and to make recommendations to improve and develop this service to ensure the best interests of the young people who are approaching this period of transition in their lives.

Outcomes:
- Improved clinical outcomes
- Improved patient outcomes
- Improved transition process in the patient journey
- Patient focused booklet explaining transitioning for those affected by LSDs

LSD Patient Survey

Description:
- An independent on-line and paper survey sent to all LSD patients and parents in England, Wales, and is updated each time it meets.

Outcomes:
- A total of 204 respondents across the diseases
- Feedback broken down for each centre
- Feedback broken down for each disease/patient organisation
- This will now be an annual survey

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Reference

LSD Collaborative