

Introduction

The Children's Liver Disease Foundation (CLDF) is a national registered charity founded in 1980. Its mission is to advance knowledge of childhood liver disease through:

- ✚ funding pioneering medical research;
- ✚ providing effective education;
- ✚ giving a professional and caring support service to families and young people with liver disease.

CLDF funds medical (basic and translational), nursing and social research connected with addressing the basic mechanisms, causes, prevention, diagnosis, cure and treatment of diseases of the liver and biliary system in children.

The CLDF research strategy will apply for the period 2006 – 2009 but will be subject to annual review.

Development of research strategy

In the development of a research strategy the CLDF trustees have canvassed views from a wide range of stakeholders sharing the common goal of advancing knowledge in all aspects of paediatric hepatology. The process adopted is reported in appendix 1.

Why does CLDF fund research?

Children's Liver Disease Foundation is the only national registered children's charity working to advance knowledge of all childhood liver diseases. As such it has a pivotal role in supporting the paediatric hepatology research base in the UK.

In May 2006, UKCRC launched its analysis of UK health research (available at: http://www.ukcrc.org/PDF/UKCRC_Health_Research_Analysis_Report.pdf). The top four areas for spend in the UK (in decreasing order) were cancer (27%), neurological disease (16%), infection (13%) and cardiovascular disease (9%). Liver disease was included in 'Oral and Gastrointestinal Health' and total spend on that appeared to be about 2.5%. The total spend on liver function and liver disease is likely to be a fraction of that and the amount on paediatric even less.

Childhood liver disease is little known to the public despite the fact that it encompasses a diverse range of diseases; its epidemiology is little understood in the UK and research is poorly funded.

There are a number of reasons why CLDF should fund research:

- Paediatric hepatology is not a priority area for the government (or other funding bodies) to fund. Thus, CLDF support of research is important as is its role in lobbying other bodies to make it a higher priority.
- The evidence base for current practice in paediatric hepatology is not strong.
- Research to improve outcomes lies at the heart of supporting families with a child with a liver disease. It provides hope for the future. Thus, research is linked inextricably to care.
- Parents are very positive in stating that they want answers to key questions; Why has my child got this disease? Can you treat it? Can you cure it? Is it preventable? Is it genetic i.e. will my other children get it?
- Success in current research activities may assist in the leverage of funds from other funding bodies and thus gather momentum for wider support for a particular topic.
- Most of the children and young people with liver disease have a chronic condition, thus research which can help improve their quality of life may have a direct impact on their life and their family.
- The development of three supra-regional centres for the care of children with paediatric liver diseases provides the UK with a unique opportunity to address a wide range of research questions, particularly clinical issues and thus provide a sounder evidence base for current practice.
- To pump-prime research into childhood liver disease which could then provide research teams with the evidence to apply for larger grants from other bodies e.g. MRC, Wellcome.

- Knowledge of basic mechanisms at the cellular level and liver development is limited. Improved knowledge of such mechanisms in the normal liver could lead to greater insight into the mechanisms of damage and repair, for instance, and in the long term, could lead to new therapies.
- Recent research amongst CLDF supporters has corroborated CLDF's anecdotal view that research is of high importance to them and is regarded as an important area for CLDF to support and influence.

What are key research/scientific area that CLDF will support?

CLDF wishes to give clear guidance to research applicants and potential funders as to what it hopes to achieve by its research activities. **Given the diverse nature of paediatric liver diseases which engages within a very dynamic research and clinical environment, CLDF wishes to ensure that it allows sufficient flexibility within the strategy to fund blue sky research, support the "good idea" and be responsive to opportunities.** Therefore, if a project does not fit within the priority areas given below prospective applicants are encouraged to speak to CLDF's chief executive and seek guidance as to whether an application would be advisable.

Areas of research which are identified by CLDF as a priority:

- The burden of paediatric liver disease e.g. epidemiology, health economics, prevalence
- Clinical / translational paediatric hepatology research
- Advancing fundamental knowledge within hepatology (with particular reference to paediatric hepatology, where relevant) such as the mechanisms involved in the progression of liver disease, cellular activity, failure to thrive, molecular biology
- Specific paediatric hepatology diseases – biliary atresia, alpha1 antitrypsin deficiency, autoimmune liver disease, non alcoholic fatty liver disease (NAFLD), inherited liver diseases
- Medical management of the symptoms and signs disease of paediatric liver disease e.g. pruritus, portal hypertension, nutrition
- Assessment of treatment modalities in paediatric hepatology e.g. immunosuppression post liver transplant
- Psychosocial issues in paediatric liver disease e.g. quality of life, transition to adulthood, psychological functioning, symptom management, socialisation in early years, family adaptation

How will CLDF support its research activity?

The main determinant of how CLDF will support its research activity will be the amount of funds available for research. CLDF trustees identify on an annual basis the amount of money that CLDF will commit to research in the next financial year. Once the amount is agreed they will agree as to the best way that the funds can be deployed during that year. This will be publicised on the CLDF website.

CLDF plans to support research in any of the following ways:

- CLDF PhD Student Fellowships; fellowship holders will be required to hold a first class or upper second degree
- Project grants – funding salary and consumables to a maximum annual amount, excluding equipment, and for a maximum of three years

- Pump-priming support
- Clinical fellowships
- Under certain circumstances CLDF may invite applications for a particular topic
- Small grants programme offering amounts up to £5K which will be available on a quarterly basis

CLDF will welcome collaborative applications from the three UK supra-regional centres.

All CLDF applications considered by the CLDF Medical Advisory Committee (MAC) will be subject to peer review. CLDF is a member of the Association of Medical Research Charities (AMRC) which has clear policy on peer review (www.amrc.org.uk) and CLDF abides by all the requirements identified by the AMRC.

The CLDF application and assessment process will be clearly outlined on the CLDF website.

Who will CLDF support?

CLDF will support applications from scientists, clinicians, surgeons, nursing staff and other allied healthcare professionals. The track record of the research team will be important in the assessment process as will any previous experience as a CLDF grant holder. Those who are unable to show a track record in the field will not be penalised but will need to demonstrate their commitment to the project, their probity and ability.

Where will CLDF be conducted?

CLDF research support is not confined to any one centre. It will fund work at centres throughout the UK on the basis of the scientific quality of the application, its relevance to the CLDF research strategy priorities and its relevance to advancing knowledge within the general field of paediatric hepatology. CLDF may fund work outside of the UK but it is emphasised that this is envisaged as being an exceptional situation with a strong case being made as to why the project cannot be conducted in the UK. Research awards funded by CLDF and carried out outside of the UK will need to demonstrate that UK paediatric hepatology patients will benefit.

Dissemination of the results of CLDF research

CLDF regards the dissemination of research results as an integral part of the research process and includes publishing in peer-review journals and oral and poster presentations at scientific meetings. CLDF recognises that despite the best endeavours of the research team, not every award will result in outcomes which can be published.

During the course of a grant CLDF will post updates on the CLDF website, as appropriate, as well as publishing the final project report on its website. Each piece of work sponsored will be reviewed by the CLDF team to look at its potential to be communicated in different ways to supporters and discussion will take place with the research team about opportunities to disseminate findings. This will include lay articles in CLDF publications such as Delivery.

CLDF funding of charitable research in universities

CLDF abides by the AMRC statement on university funding (www.amrc.org.uk). A relevant part of the statement for this strategy is given below:

“AMRC’s position statement, revised in October 2004, is clear that with regard to university funding its members will not fund research on a percentage basis. AMRC member charities will continue to pay

for the directly incurred and identifiable costs of research but charities do not consider it appropriate for them to fund university indirect costs or to be required to cover some of the other costs, such as investigators' salaries or estate costs, identified under a new accounting methodology, Transparent Approach to Costing (TRAC)."

Appendix 1

Canvassing views from supporters. The following process was adopted:

- 1 Open meeting on June 29th 2005 at the Novartis Foundation, London which was externally facilitated by Dr Wendy Ewart. This was advertised widely and self-nominations invited.
- 2 The open meeting had 20 attendees and included:
 - Clinicians (adult and paediatric)
 - Surgeons (hepatobiliary)
 - Research Nurse
 - Pathologist
 - Immunologist
 - Scientist
 - Parents
 - Trustees
- 3 In addition to the main meeting CLDF arranged for the Chairman of the CLDF Medical Advisory Committee (MAC) and the Chief Executive to hold meetings at the three supra regional paediatric liver centres to gather topics and ideas which were presented to the main meeting and brought into the main discussion.
- 4 The trustees agreed to accept written responses to a set of key questions (none were received)
- 5 The trustees also canvassed the opinion of supporters.
- 6 Following the meeting the Chairman of MAC, the Honorary Medical Director and the CEO put together a paper for consideration by the MAC which was subsequently taken to the board of trustees for their approval.
- 7 The following views were sought at the meetings:
 - The scope / range of topics within paediatric hepatology research
 - The topics identified as priority
 - The topics in which CLDF can make a difference by funding
 - The topics which CLDF should not be funding
 - The topics which CLDF should be funding
 - The future areas of paediatric hepatology research
 - The areas future research CLDF should be looking to fund
 - What is the balance of clinical and laboratory research which should be supported
 - The ways in which CLDF should fund research - E.g. PhD student fellowships, projects etc?
 - Who and what are the bodies CLDF should seek to influence and why
 - How can CLDF support the endeavours of the medical research community more fully
 - How should CLDF employ its money – pump prime or pursue a longer term role within a topic
 - How can / should CLDF respond to exciting ideas or provide a rapid response mechanism?
- 8 CLDF supporters were asked about their perceptions of paediatric liver disease research via postal questionnaire in September 2005.