Key Considerations For Selecting Research Proposals

The Lowe Syndrome Association (LSA) currently seeks proposals for investigator-initiated, proof-of-principle clinical trials evaluating therapeutic approaches for renal tubular dysfunction in individuals with Lowe syndrome. The LSA will also consider proposals that address other major complications of Lowe syndrome such as neurologic and behavioral problems, corneal keloid, degenerative bone and joint disorders.

Key considerations include:

- A scientifically justified clinical trial design with clinically meaningful renal, metabolic, and safety outcome measures.
- The potential to generate data that can inform future multicenter studies, regulatory discussions, and broader therapeutic development.
- Priority will be given to proposals that:
- Evaluate FDA-approved repurposed pharmacological therapies that have established safety and toxicity data but have not yet been evaluated for clinical efficacy in individuals with Lowe syndrome.
- Are supported by compelling preclinical evidence demonstrating potential benefit for renal tubular dysfunction in relevant *in vitro* cell models and *in vivo* mammalian animal models.
- Evaluate non-pharmacological interventions, such as dietary or metabolic therapies, when supported by relevant preclinical evidence and sufficient human safety data.
- Include a scientifically justified clinical trial design with clinically meaningful renal, metabolic, and safety outcome measures.
- Have the potential to generate data that can inform future multicenter studies, regulatory discussions, and broader therapeutic development.
- Collaborative and multicenter proposals are encouraged when appropriate.
- Has the researcher identified and/or requested additional sources of funding to share the research cost or is willing to seek additional sources of funding?
- Does the proposal include a detailed budget, timelines and anticipated outcomes?

- Does the proposal include provisions for a summary report midway through the end of the funding period and the start of the project?

Policies Governing Research Grants

A. Background

The Lowe Syndrome Association, Inc., (LSA) is an international, voluntary, non-profit organization made up of parents, professionals, and others who are interested in Lowe syndrome. It was founded in 1982 and incorporated in 1986.

The primary goals of the LSA are to: foster communication among families, promote a better understanding of Lowe syndrome and the potentials of individuals with this condition, provide information, and encourage and support medical research relating to Lowe syndrome. The LSA publishes a digital newsletter, On the Beam, conducts informational sessions for the Lowe syndrome community, communicates via email, Facebook and other social media outlets and sponsors international conferences. The official slogan of the LSA is “Circle of Care…Vision for a Cure.”

The LSA is governed by a Board of Directors. In scientific and medical issues, the Board solicits the advice of the LSA Medical and Scientific Advisory Board, which is made up of physicians and scientists representing various backgrounds.

B. Areas of Research Interest

- Developing a treatment of the major complications of Lowe syndrome including but not limited to renal disease, neurologic and behavioral problems, corneal keloid, degenerative bone and joint disorders or other complications.

C. Size and Time Period of Grant

The LSA provides funding, typically annually, to support appropriate research. Funding amounts vary. The researcher may re-apply for multiple years of funding, if offered.

D. Funding Eligibility Requirements

The LSA invites applications from researchers of all types, including but not limited to universities, hospitals, and other non-profit organizations with interest in research in the previously specified areas.

E. Grant Funding Restrictions

The LSA grant money may not be used as salary for the principal investigator, for honoraria for consultants, for the purchase of standard, non-consumable laboratory equipment, to purchase services from other research laboratories, or for overhead or indirect costs. Also, grant funds must be held in interest-bearing accounts payable to the grant.

F. Reporting Requirements

The researcher must submit a progress report at the end of six months for annual grants. This report must include a financial statement showing the expenditure of grant funds. A discussion midway through the project is also requested. Consideration for re-application for a subsequent grant will depend on satisfactory progress demonstrated in the progress report. A final report must be submitted after twelve months and all unexpended funds must be returned to the LSA.

G. Safeguarding the Rights and Welfare of Human Subjects

If any human subjects are involved in research supported by the LSA, the researcher must obtain the informed consent of each subject, or the informed consent of a parent or guardian for each minor subject, prior to the participation of human subjects in any study or procedure.

No research involving human subjects shall be made unless the research is given initial and continuing review and approval by an appropriate committee of the researcher's institution. The institution must provide written assurance that the research project complies with the regulations of the United States Department of Health and Human Services (see IV.A.10 below).

Guidelines for Proposals

A. Application

The total application must be no more than 10 pages (8″ x 11″) long, not including curriculum vitae and bibliography. Each application must contain the following items:

- The name of the applicant, the title of the research project, the name and address of the institution, the name and approval signature of the institution's financial officer, the name and address of the chairman of the department in which the research will be done, and the name and address of the supervisor of the research.
- A brief (about one paragraph) summary of the purpose, nature, and potential significance of the proposed project, written as much as possible in lay language for a non-scientific reader.

- A scientific summary (no more than 1 page long) of proposed research, including a statement of what the researcher proposes to learn, how the researcher proposes to conduct the research, and the expected duration of the study.
- A summary of the qualifications of the researcher, including his or her qualifications to carry out the study and including studies he or she has done previously that make him or her competent to do the study.
- A description of the facilities available to carry out this study.
- A detailed budget, including identification and explanation of all proposed expenses.
- A statement listing all sources, amounts, and purposes of governmental or philanthropic support the researcher is receiving for this study.
- A detailed research plan, including all information necessary for reviewers to fully understand the proposed research. The plan should provide a clear statement of the research question and the theoretical base for the project. The plan should summarize similar and relevant work by the applicant and others as reported in the scientific literature or known to be in progress. The plan should also state the specific aims and hypotheses of the research. The plan should describe the strategies to be employed, including the nature and sources of data to be collected.
- Curricula Vitae of investigators to be involved in the study.
- A statement by the institution's review board assuring that the proposed study has been reviewed and approved.
- A letter from the applicant's supervisor of research (or from the applicant's department chairperson if the applicant is supervising his or her own research), approving the research, confirming the availability of equipment and facilities for the project, and describing the capacity of the investigator to carry out the proposed study.
- A statement by the researcher agreeing to provide a 6-month progress report and a final 12-month report, to return unexpended funds to the LSA after twelve months, and to acknowledge the support of the LSA in all publications resulting from this grant.
- A statement by the researcher agreeing to make available to the scientific community any reagents and/or data generated using the support of the LSA, once they are published.

No research involving human subjects shall be made unless the research is given initial and continuing review and approval by an appropriate committee of the researcher's institution. The institution must provide written assurance that the research project complies with the regulations of the United States Department of Health and Human Services (see IV.A.10 below).

B. Submission of Application

Proposals can be submitted via the submission form above or email before the deadline to: Jeri Kubicki, President, Lowe Syndrome Association at president@lowesyndrome.org