



2015 Helmsley Breakthrough Initiative Funding Opportunity

Call for Expressions of Interest: The Leona M. and Harry B. Helmsley Charitable Trust seeks to support studies of unique clinical cases of type 1 diabetes.

Overview

The goal of the Helmsley Breakthrough Initiative is to generate new hypotheses about type 1 diabetes (T1D) pathogenesis and new therapeutic strategies. In 2015, the Helmsley Breakthrough Initiative will support early-stage studies of unique clinical cases of type 1 diabetes. These studies of rare patients and clinical outliers may lead to unexpected discoveries that could transform our understanding of the disease. Applicants will submit expressions of interest by August 3, 2015.

Rationale

T1D is a chronic autoimmune disease characterized by the loss of beta cells, which produce insulin in response to glucose. Because the cause(s) of T1D are unknown and its development in humans is not fully understood, prevention and treatment strategies have been difficult to devise. In other fields, coordinated studies of rare patients and clinical outliers have generated new hypotheses and better understandings of disease. For example, identification and characterization of elite controllers of HIV and exceptional responders of cancer therapies promise breakthroughs in their respective fields. Furthermore, because the original observations are made with human samples, and not model systems, discoveries have a higher potential to translate into human therapies.

To capitalize on this potential, the 2015 Helmsley Breakthrough Initiative will support early-stage studies of unique clinical cases of T1D. These cases, which include rare patients and clinical outliers, represent “exceptions to the rule” that if understood may generate surprising discoveries that advance our understanding of T1D and reveal new therapeutic strategies to help those affected by the disease.

Call for Expressions of Interest

Applicants may submit Expressions of Interest (EOIs) and propose early-stage projects to identify individuals or cohorts, collect biosamples and/or analyze these materials and data. Principal

investigators will interact within a scientific working group to share expertise, coordinate analyses and pool data when appropriate.

Study topics include, but are not limited to:

- Individuals or rare patients with perplexing clinical presentations, comorbidities, pedigrees or inexplicable phenotypes;
- ‘Elite’ nonprogressors;
- Rapid progressors; or
- Unexpected responders to interventions (*e.g.* exceptional/super responders).

Example projects might include:

- The identification of, or collection of samples from, a cohort of clinical outliers;
- The analysis of samples from existing cohorts (*e.g.* immune, genetic, pedigree, biochemical, proteomic or microbiomic analysis); or
- Case studies of a single or small group of individuals

Proposed research does not necessarily require preliminary data or testable hypotheses; a goal of the Initiative is to help transform early-stage ideas into sustainable research programs.

Studies must aim to advance the scientific understanding of T1D. Topics that fall outside of the scope of this EOI and will not be considered for funding include: type 2 diabetes, complications associated with diabetes and known cases of maturity-onset of diabetes of the young (MODY).

Funding

The Helmsley Breakthrough Initiative will launch with up to 10 principal investigators and will be supported for 12 months with up to a total of \$1,000,000 USD for direct research costs. Depending on the number of samples and analyses required, each applicant will propose a budget of \$50,000 to \$150,000. An additional 10 percent can be requested to support institutional indirect costs. Awards will be disbursed at the commencement of the grant period.

Grant application process

The 2015 Helmsley Breakthrough Initiative has three phases: submitting an Expression of Interest, the coordination of a scientific working group, and submitting full grant proposals:

Phase I: Express interest

EOIs must include:

- Applicant’s contact information;
- 500-word scientific proposal that describes samples and experiments;
- Proposed budget (see “Funding” section above); and
- Relevant biographies (*e.g.* new or old format NIH biosketches).

A scientific review of submitted EOIs will identify potential working group participants based on the following criteria:

- Novelty and value of samples and analyses;

- Potential that the early-stage studies will develop into research projects that inform etiology, pathogenesis or new therapeutic avenues;
- Alignment with the EOI's stated goals; and
- Synergy and complementation within the working group.

Phase II: Coordinate a scientific working group

Selected applicants will be invited to convene for a teleconference on October 8, 2015, to plan a working group. Common analyses, such as genomics and immunophenotyping, will be identified and coordinated. After establishing these research plans, each participant will be invited to submit a full grant proposal for funding consideration. **EOI applicants are encouraged to reserve October 8, 2015 on their calendars.**

Phase III: Submit full grant proposals

Invited applicants will submit full grant proposals. Full grant proposals will include:

- Applicant's contact information;
- 500-word lay summary of project;
- 1500-word scientific proposal that describes samples and experiments;
- Information related to receiving all patient, safety and ethical approvals, including appropriate consent and approval from an Institutional Review Board (IRB);
- Appropriate plan to share and distribute data to the research community;
- Expected outcome and impact; and
- Full budget and justification.

Eligibility

Applications are welcome from any U.S., Canadian, Australian, or European nonprofit research institution. Physicians unaffiliated with a nonprofit institution should seek collaborators at academic or research centers. Grants will not be made to individuals.

Timeline

Helmsley Breakthrough Initiative announcement	June 8, 2015
Expression of Interest submission deadline	August 3, 2015
Teleconference to coordinate working group	October 8, 2015
Full grant proposal deadline	November 5, 2015
Notification of awards	December 31, 2015
Earliest anticipated grant start date	February 1, 2016

Submitting an application

Applicants should visit the [Trust's website](#) and follow the instructions to submit an Expression of Interest. Note: the portal is not HIPAA compliant; protected health information (PHI) must not be included.

Progress reporting

Grantees will be expected to participate in working group teleconferences and submit nine-month and final written reports.

Frequently Asked Questions (FAQs)

Please check the [Trust's website](#) for a list of FAQs before emailing questions. Program- and science-related questions not addressed in the FAQs may be directed to T1Dresearch@helmsleytrust.org and will be answered in a timely manner. Please ensure that the subject line clearly states "Helmsley Breakthrough Initiative". For assistance with the online application, please e-mail grants@helmsleytrust.org.

About the Helmsley Charitable Trust

The Leona M. and Harry B. Helmsley Charitable Trust aspires to improve lives by supporting exceptional nonprofits and other mission-aligned organizations in health, selected place-based initiatives, and education and human services. Since 2008, when the Trust began its active grantmaking, it has committed more than \$1 billion. The Helmsley Type 1 Diabetes Program is the largest private foundation funder of T1D in the United States focused on understanding the disease, developing better treatments and improving care and access. For more information, visit helmsleytrust.org.