

Pennsylvania Department of Health Final Performance Summary Report Formula Grants

Overview of the Health Research Project Performance Review Process and Criteria

An applicant that receives a health research grant under Tobacco Settlement Act / Act 77 of 2001, Chapter 9, is subject to a performance review by the Department of Health upon completion of the research project. The performance review is based on requirements specified by Act 77 and criteria developed by the Department in consultation with the Health Research Advisory Committee.

As part of the performance review process, each research project contained in a grant is reviewed by at least three experts who are physicians, scientists or researchers. Reviewers are from the same or similar discipline as the research grant/project under review and are not from Pennsylvania. Reviewers use the applicant's proposed research plan (strategic plan), the annual progress report and final progress reports to conduct the review. A grant that receives an unfavorable performance review by the Department may be subject to a reduction in funding or become ineligible for health research funding in the future. The overall grant evaluation rating is based on the ratings for the individual research projects contained in the grant.

This performance review report contains the outcome of the review for the grant as a whole (outstanding, favorable, or unfavorable), strengths and weaknesses of each research project, as well as recommendations for future improvement.

The following criteria were applied to information submitted by research grant recipients:

- **Criterion 1 - How well did the project meet its stated objectives? If objectives were not completely met, was reasonable progress made?**
 - Did the project meet the stated objectives?
 - Were the research design and methods adequate in light of the project objectives?
 - Consider these questions about data and empirical results: Were the data developed sufficiently to answer the research questions posed? Were the data developed in line with the original research protocol?
 - If changes were made to the research protocol, was an explanation given, and, if so, is it reasonable?
 - Consider (only for clinical research projects) the extent of laboratory and clinical activities initiated and completed and the number of subjects relative to the target goal.
 - Were sufficient data and information provided to indicate or support the fact that the project met its objectives or made acceptable progress?
 - Were the data and information provided applicable to the project objectives listed in the strategic research plan?

- **Criterion 2 - What is the likely beneficial impact of this project? If the likely beneficial impact is small, is it judged reasonable in light of the dollars budgeted?**
 - What is the significance of this project for improving health?
 - Consider the value of the research completed towards eventual improvement in health outcomes.
 - Consider any changes in risk factors, services provided, incidence of disease, death from disease, stage of disease at time of diagnosis, or other relevant measures of impact and effectiveness of the research being conducted.
 - Consider any major discoveries, new drugs and new approaches for prevention, diagnosis and treatment, which are attributable to the completed research project.
 - What are the future plans for this research project?

- **Criterion 3 - Did the project leverage additional funds or were any additional grant applications submitted as a result of this project?**
 - If leveraging of funds were expected, did these materialize?
 - Are the researchers planning to apply for additional funding in the future to continue or expand the research?

- **Criterion 4 - Did the project result in any peer-reviewed publications, licenses, patents, or commercial development opportunities? Were any of these submitted/filed?**
 - If any of the above listed were expected, did these materialize?
 - Are the researchers planning to submit articles to peer-reviewed publications, file for any licenses, or patents or begin any commercial development opportunities in the future?
 - Consider the number/quality of each.

- **Criterion 5 - Did the project enhance the quality and capacity for research at the grantee's institution?**
 - Were there improvements made to infrastructure?
 - Were any new investigators added or were any researchers brought into the institution to help carry out this research?
 - Were funds used to pay for research performed by pre- or post-doctoral students?

- **Criterion 6 - Did the project lead to collaboration with research partners outside the institution, or new involvement with the community?**
 - Are the researchers planning to begin any collaborations as a result of the research?
 - For clinical research only: consider the number of hospitals and health care professionals involved and the extent of penetration of the studies throughout the region or the Commonwealth.

Overall Evaluation Rating

An overall evaluation rating is assigned to each research project. The rating reflects the overall progress the project attained in meeting the stated goals and objectives. The rating is based on a scale of 1–3, with 1 being the highest. An average rating is obtained from all the reviews (minimum of 3) of each project and is the basis for the determination of the final overall rating for each project as follows:

1.00 – 1.33 = *Outstanding*

1.34 – 2.66 = *Favorable*

2.67 – 3.00 = *Unfavorable*

The grant level rating is an average rating from all projects as above. The numerical rating appears in parentheses for the grant and each project in the ***Overall Grant Performance Review Rating*** section of the report.

Overall Grant Performance Review Rating

Grant Rating: Favorable (2.33)

Project Rating:

Project	National Disease research Interchange	Average Score
0864201	Genetic Susceptibility for Microvascular Complications in Patients with Type 1 Diabetes	Favorable (2.33)

Project Number: 0864201
Project Title: Genetic Susceptibility for Microvascular Complications in Patients
with Type 1 Diabetes
Investigator: Lonsdale, John T.

Section A. Project Evaluation Criteria

Criterion 1 - How well did the project meet its stated objectives? If objectives were not completely met, was reasonable progress made?

STRENGTHS AND WEAKNESSES

Reviewer 1:

By far the greatest strength of this effort is the unique database and sample collection from families with type 1 diabetes. Although not unique (there is a very high quality registry at the Joslin Diabetes Center), this is an important contribution to efforts to understand the origins of diabetes associated complications. Follow-up and continued survey of the individuals and cases are of paramount importance for the database to have continued value. Apparently the HBDI is a participant in a grant proposal to combine many datasets for analysis of complications; this is an important and very appropriate use of the data.

The downside of this effort is that tangible progress in achieving goals has not been great for publications or gaining extramural funding. Especially disappointing is the cursory and not very helpful summary of the continued follow up of the HBDI populations. It is acknowledged that the funds requested and received are relatively modest, but even with this, the progress report is not encouraging.

Reviewer 2:

For the most part, this project met its stated objectives. Overall, the specific aims were achieved. If you look at the details, it appears that the researchers did not perform family-based association analysis or linkage analysis. Clearly, they performed the SNP association analysis and had some interesting findings. For Aim 2, they did do the follow-up questionnaires. Their success rate was adequate in terms of response. For Aim 3, they did select the SNPs for saturation genotyping, although that data has not yet been analyzed.

Reviewer 3:

Overall this project had a number of strengths including: 1) continued data collection on individuals from an already comprehensive and rich database, 2) general achievement of ambitious objectives in light of the duration and amount of funding, 3) clear goals for expanding on the current research, 4) extensive efforts to obtain additional funding, and 5) the establishment of strong collaborative ties with researchers at Columbia University. Some weaknesses were identified, but none appear to be major weaknesses and the strengths generally outweigh the identified weaknesses.

The project met its stated objectives and used appropriate design and data analytic methods to address the specific aims. A major strength of the project was continued data collection on individuals from an already comprehensive and rich database. An additional strength is the fact that the investigators were able to achieve their ambitious objectives in light of the duration and amount of funding. This is particularly true for Aims 1 and 3, for which the data and analyses were sufficient to identify significant SNPs associated with the three diabetic complications of retinopathy, nephropathy, and neuropathy, and to conduct an SNP saturation study. However, the response rate for the follow-up survey (Aim 2) was only around 11% by the end of the project, which is considerably less than the stated goal of 25%.

Criterion 2 - What is the likely beneficial impact of this project? If the likely beneficial impact is small, is it judged reasonable in light of the dollars budgeted?

STRENGTHS AND WEAKNESSES

Reviewer 1:

The HBDI is widely recognized as an important repository for information and materials for research into type 1 diabetes and its complications. Its prominence in this field adds to the perception of Philadelphia/Pennsylvania being an important and highly visible participant in health related research. With this, more tangible benefits from this research effort have not been obvious. Again, there are limits as what can be done with approximately \$55,000, but more could have been done.

Reviewer 2:

There is likely impact for this project in the research of type I diabetes. Fortunately, they are building a very nice database of cases, controls and families including those with type I diabetes complications (i.e., retinopathy, nephropathy, etc). All of the genotyping already done on these samples will be available for future research. Additionally, the samples and clinical data collected can be used for follow-up studies.

Reviewer 3:

The project is significant in that its results add to knowledge about genetic factors related to susceptibility to diabetic complications. The project met its stated research goals and is likely to contribute to better understanding, and possibly improved treatment, of diabetic complications. A major strength of the project was continued data collection on individuals from an already comprehensive and rich database. However, the low response rate at the time the project ended is somewhat of a concern as it could impact statistical power and generalizability. In addition, the investigators did not provide a focused statement on how the findings specifically contributed to the changes in outcome, impact, and effectiveness of research in this area. A more detailed explanation would have been helpful for evaluating the true impact of the study. The researchers have plans to extend this research to include autoimmune diseases and genetics of glycemic control. To conduct this expansion, they have submitted two grant applications, one unfunded and one currently under review, and intend to submit an R21 application.

Criterion 3 - Did the project leverage additional funds or were any additional grant applications submitted as a result of this project?

STRENGTHS AND WEAKNESSES

Reviewer 1:

Some attempt has been made to leverage the funds spent on research. One grant did not get funded and a second grant, in which the HBDI is a member of what is likely a large consortium, is under review. This latter effort probably has a reasonable chance of being funded. Realistically these types of genetic studies require very large numbers and the only way to achieve those numbers is through extensive collaboration.

Reviewer 2:

Proposals for additional grant support were:

An NIDDK R21 grant application submitted in October 2008 - not funded.

An NIH grant application submitted in February 2009 - not funded.

A nonfederal grant from Italy is pending.

An R21 grant application was submitted.

Reviewer 3:

The researchers have plans to extend this research to include autoimmune diseases and genetics of glycemic control. To conduct this expansion, they have submitted two grant applications, one unfunded and one currently under review, and intend to submit an R21 application. The current project provides a strong basis for future research, and the significant effort to obtain additional funding is a strength.

Criterion 4 - Did the project result in any peer-reviewed publications, licenses, patents, or commercial development opportunities? Were any of these submitted / filed?

STRENGTHS AND WEAKNESSES

Reviewer 1:

The project has not been productive in the areas of publications, licenses, patents, etc., and leads one to question whether the sample is adequate to support effective levels of research.

Reviewer 2:

No peer-reviewed publications have been submitted thus far, but there are plans to submit papers on this work. There are no licenses, patents, or commercial developments.

Reviewer 3:

The researchers indicated that their progress would be marked by submissions to top-tier peer-reviewed journals. There were no publications at the time the final report was generated, although there are plans to write an article about the findings on the roles of specific SNPs in susceptibility to diabetic complications. The lack of publications is not unreasonable given the length of the project, but it is a slight weakness because it makes it difficult to ascertain the full impact of the project. There is no way to know whether this article will be submitted and/or accepted for publication.

Criterion 5 - Did the project enhance the quality and capacity for research at the grantee's institution?

STRENGTHS AND WEAKNESSES

Reviewer 1:

The enhancement of research, if any, is very modest. If one presumes that the genetic analysis of the modest number of samples alone will have much impact, it would be hoped that the continued follow-up of the cohort for complications would continue to be a valuable effort. The report is disappointing and provides little confidence that information of value has been gained; it is simply a listing of the number of questionnaires returned.

Reviewer 2:

No additional improvements were made to the infrastructure at the grantee's institution.

Reviewer 3:

This project did not appear to enhance the quality and capacity for research at the grantee's institution. This is a slight weakness because the extent to which there was potential to use project funds to hire and train new investigators is unclear.

Criterion 6 - Did the project lead to collaboration with research partners outside of the institution or new involvement with the community?

STRENGTHS AND WEAKNESSES

Reviewer 1:

The project has led to participation in a pending grant for a network of studies. This is an appropriate and very relevant direction for the study to go.

Reviewer 2:

The project continued collaboration between the grantee and Drs. Monti and Greenberg at the University of Columbia.

Reviewer 3:

This project has led to continued and successful collaboration with researchers from Columbia University, resulting in the use of rigorous statistical methods. This a strength of the project.

Section B. Recommendations

SPECIFIC WEAKNESSES AND RECOMMENDATIONS

Reviewer 1:

1. It is unclear whether much has been done other than sending out letters to study participants. At least a summary of the results of those letters could be presented.
2. Results of Aim 1 met only the most liberal criteria for acceptance, as being genetically associated with the diabetes complications.
3. The sample sizes for the subjects with complications are small. Thus, the dataset will likely only be useful in concert with other samples from other studies. Some effort is being made (i.e., the consortium grant), but this has not yet materialized.

Reviewer 2:

It would be nice to see the results of Specific Aim 3.

Reviewer 3:

1. The response rate for the follow-up survey (Aim 2) was significantly lower than the stated goal of 25%, which could impact statistical power and generalizability. In the final report, the researchers did not discuss their actual efforts to increase response rates and whether or not they have used any new methods to do so. It is recommended that the researchers indicate what, if any, methods they are (or will be) using to increase response rates. Some possible methods might include additional follow-up contacts, incentives, and mailings to other additional family members. Other possible methods might include alternative data collection methods such as making phone calls or using the internet. Although some of these methods may not be ideal for the kind of data being collected, there should be more detail regarding efforts to maximize the response rate and more discussion of the potential impact of a low response rate.
2. The investigators did not provide a focused statement on how the findings specifically contributed the changes in outcome, impact and effectiveness of research in this area. Instead they provided a brief and general discussion of the potential impact very much like the one provided in the strategic research plan prior to conducting the research. A more detailed explanation of the impact of the actual results would have been helpful for evaluating the true impact of the study.
3. There were no publications at the time the final report was generated. The lack of publications is not unreasonable given the length of the project, but it is a slight weakness because it makes it difficult to ascertain the full impact of the project. There is no way to know whether this article will be submitted and/or accepted for publication. It is recommended that the investigators discuss any progress that has been made in writing articles for submission to journals.
4. This project did not appear to enhance the quality and capacity for research at the grantee's institution. This is a slight weakness because the extent to which there was potential to use

project funds to hire and train new investigators is unclear. The investigators should provide comments on why aims under this criterion were not achieved.

Additional Comments

Reviewer 1:

Even given the limited funding for the project, the progress made in achieving the specific aims and the more global aims of the program are modest. No data or analysis results are presented, nor is a summary of data from returns of questionnaires given. No manuscripts have been published. Only a single pending grant has a possibility of being funded.

Reviewer 2:

Future research efforts should include the analysis of data for Specific Aim 3. Additionally, they should continue to maximize follow-up data for Specific Aim 2.

Generic Recommendations for the Institute

Reviewer 1:

This is an underfunded project that has made slim accomplishments. The real value comes from the follow-up of the participants in the study. This does not seem to be a focus of the project.