

Quarterly Reports



RESEARCH PROJECT TITLE: Pre-clinical Screening Program to Establish New Indications in Autoimmunity for Existing Generic Drugs
RESEARCHER NAME: Denise L. Faustman, MD, PhD
RESEARCHER INSTITUTION: Massachusetts General Hospital
FUNDING AGENCY: The Friends United for Juvenile Diabetes Research, Partnership for Cures and other funders
FUNDING PERIOD: July 2008 – June 2009

TOTAL OPERATING BUDGET: \$203,500

PROJECT CODE #: DF PSP

SPECIFIC AIMS AND OBJECTIVES OF THE RESEARCH PLANS

I. PRE-CLINICAL SCREENING PROGRAM TO ESTABLISH NEW INDICATIONS IN AUTOIMMUNITY FOR EXISTING GENERIC DRUGS

Fourth Quarter: April 1, 2009-June 30, 2009

Research Objective 4: Continue blood draws from patients and their matched controls at intervals of Monday, Tuesday and Thursday.

Please provide the following information:

1. Description of research work completed, including any unreported work from previous objectives: We have a very brisk flow of patients who want to participate in our studies. The patients that volunteer their time and resources are from all over the US and all over the world. They learn of this program from the Internet and almost always show up for their appointments without reminders and hours prior to their appointment time. We are using these human blood samples after the T cells are separated to screen for generic drugs that might kill a subset of autoreactive T cells.
2. What problems were encountered? Because of the enthusiastic participation of humans, we have been able to invite back the subjects at 3-6 month intervals to rescreen their T cells for sensitivity to the compounds (generic drugs) of interest.

3. What discoveries were made? One of our first objectives is reproducibility of the drug killing on the same patient at many different months between blood samples. In the past, researchers blamed “patients” for why the T cell assays would fluctuate but with our standardizations of blood separation methods we are with this Generic Drug Screening Program seeing reproducibly of the generic drug killing on the same patient separated by many months of time.

4. How did results of work on this objective create any need to alter the plan going forward? No alternations

5. Please check all outcomes related to this project that took place during this quarter.

Scientific Publications:

Submitted article[s] for publication [list journal(s)]

Article[s] accepted for publication [list journal(s)]

Article published [list citation]

(Just so all know, a typical paper from our lab usually comprises about 5 years of data so it is unlikely to have papers within short time periods. This is in large part due to the fact that we work on human samples, not mouse studies which have totally different time lines of development but on the other hand yield answers directly relevant to disease).

Publicity: If checked, list citations

Featured/mentioned in institutional publication [eg: newsletters, alumni magazine]

Featured/mentioned in public media [eg: newspaper, magazine, TV]

We were featured this month in US News and World Report for one of the top 14 medical research pioneers in the world.

Funding:

Applied for grant(s) [list where]

Grant(s) approved [list funding source]

(We continuously write grants to many different sources)

Intellectual Property: If checked, please explain

Patent(s) applied for

Patent(s) approved

License(s) under negotiation

License(s) approved

6. Confidentiality

When we share information about your research with donors or the public, is there anything proprietary that we should not disclose?

No

7. Impact of Research

In terms of increasing understanding of diseases processes and/or developing treatments, what primary and secondary diseases is your work likely to have an impact on? We think most academic centers should be pursuing similar projects on how to develop cheap drugs for the public by re-cycling existing drugs with known safety. Only philanthropic support will allow us to move these cheap drugs forward to the public since most late stage clinical testing is supported by the pharmaceutical industry that does not have a model for development of drugs with high human testing costs and with an outcome of the potentially inexpensive products at the approval time. We think the compounds/drugs we identify in type 1 diabetes may have broader applications to other autoimmune diseases such as lupus, Sjogren's Syndrome, multiple sclerosis, etc.

8. Lay Summary

Please briefly sum up your progress during this quarter for a lay audience.

Our goal is to identify old drugs that may help to kill one subpopulation of bad T cells in type 1 diabetes. We do this by designing fresh human T cell assays from people with type 1 diabetes compared to non-diabetic controls. With the compounds we now have proof our screening assays for this project that has reproducibility over months and we are identifying lead compounds or compound families that appear to have activity. As we move forward we will now center our research on our favorite generic compounds and start testing their ability to kill bad T cells in larger populations of type 1 diabetics.

9. Additional Comments

What else you would like to share with those supporting your research?

Thank you