BIOMARKERS OF PROGRESSION RFA WEBINAR

15 December 2015

JDRF IMPROVING LIVES. CURING TYPE 1 DIABETES.

JDRF and Janssen's Disease Interception Accelerator
REQUEST APPLICATIONS FOR

BIOMARKERS OF PROGRESSION IN THE AT-RISK SETTING FOR TYPE 1 DIABETES

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Agenda

- Goal/Objectives of RFA
- Background and Rationale
- Examples of investigation topics
- Proposal submission process

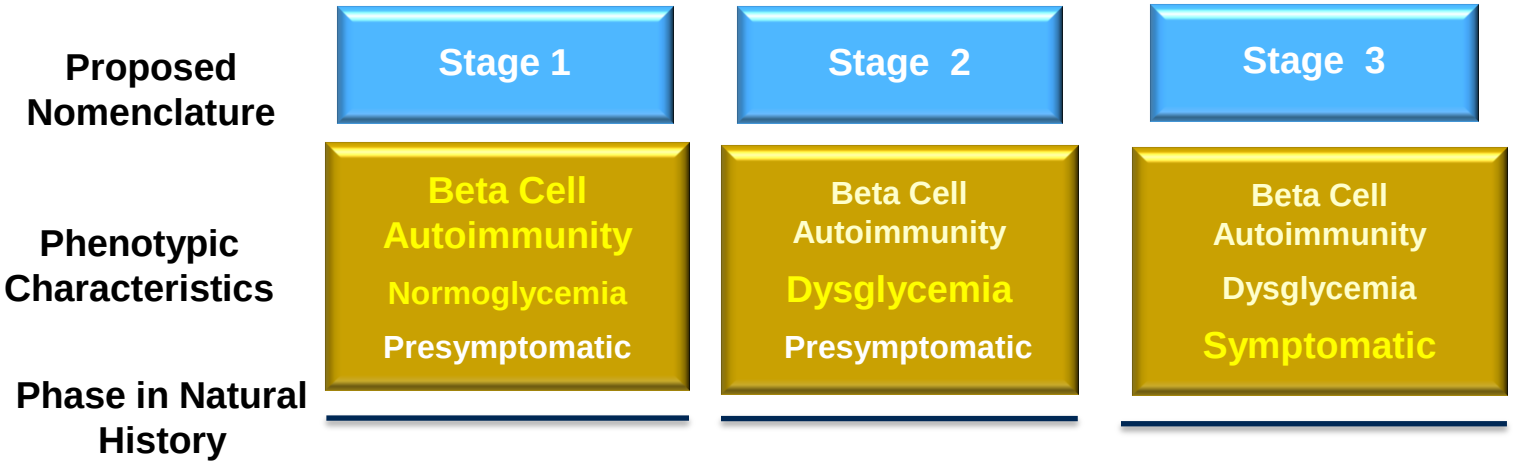
Long-Term Goal

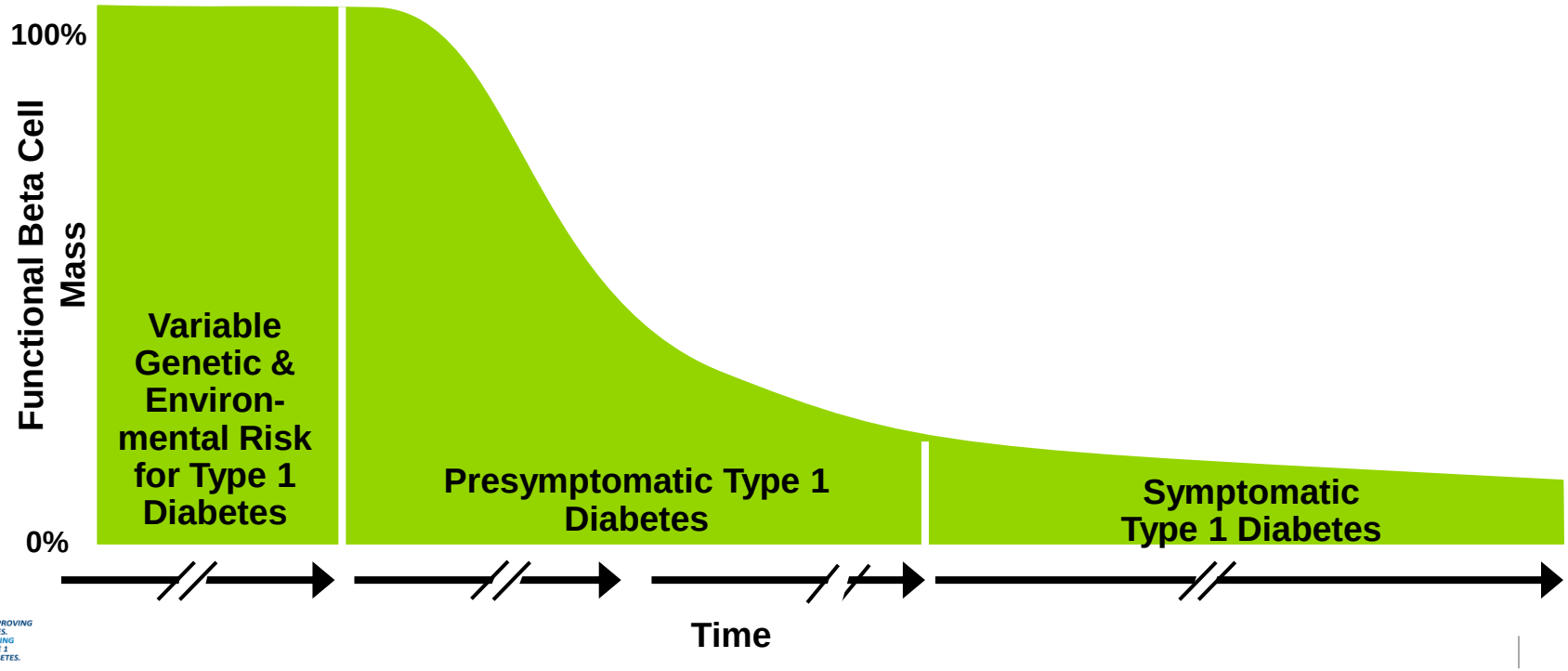
To provide a framework for:

- Clinical trial design,
- Benefit/risk decisions around interventions, and
- The practice of predictive medicine to prevent symptomatic T1D or stage 3 T1D*

** Insel, Dunne et al., (2015) Diabetes Care 38:1964*

Type 1 Diabetes Staging





Objectives

To develop

- Improved prognostic risk scores of progression to optimize design of trials and tailor therapy
- Predictive biomarkers of efficacy of preventive interventions to accelerate clinical development
- New or improved, cost-effective risk screening assays.

- The translational potential of the investigations should be highlighted, and thus, only research plans involving human samples will be considered

Potential biomarkers may include biomarkers (metabolites, proteomics, gene expression patterns, additional autoantibodies, etc.) or non-invasive imaging that detect:

- Beta cell stress, dysfunction, and damage
- Functional beta cell mass
- Islet inflammation
- General status of immunoregulation or inflammation
- Glucose and metabolic control

Examples of pertinent topics include, but are not limited to biomarkers that:

- Refine current staging of type 1 diabetes in the at-risk setting
- Predict risk/rate of progression in autoantibody positive individuals
- Detect early risk in the general population, with an emphasis on the pre-autoantibody stage
- Serve as surrogates of efficacy of preventive/interception interventions
- Allow for subject stratification or refined inclusion/exclusion in clinical trials in the at-risk setting.

This RFA is NOT intended to support:

- Discovery and development of biomarkers in preclinical models of disease
- Discovery and development of biomarkers in other stages of T1D
- Discovery and development of biomarkers for the prediction or prevention of complications of T1D
- Studies without potential for translation or informing future clinical approaches

Collaborations during and after funding

- Collaborative projects, where possible, to interrogate common sample or data sets are encouraged, and higher budgets may be allowed for such projects.
- Depending on progress, JDRF may identify synergistic projects
- JDRF seeks investigators who are willing to discuss their projects to establish collaborations prior to application submission

Funding Mechanism and Eligibility

- Funding Mechanism
  - **Strategic Research Agreements (SRA)** :Up to a maximum of $250,000 USD per year including 10% indirect costs for up to 2 years
  - **Pilot & Feasibility Grants (P&Fs):** Up to $110,000 (including 10% indirect costs) for one year only.
  - Collaborative projects, where possible, to interrogate common sample or data sets are encouraged, and higher budgets may be allowed for such projects.
- Eligibility
  - M.D., D.M.D., D.V.M., Ph.D., or equivalent and have a faculty position or equivalent at a college, university, medical school, or other research facility
  - Applications from for-profit entities or industry collaborations accepted

PROPOSAL SUBMISSION PROCESS

RFA Timeline:

- **RFA Release Date:** December 7, 2015
- **Email of Intent to Submit Application:** January 15, 2016
- **Application Due Date:** February 5, 2016
  - Applicants should register and submit their application in RMS360 (http://jdrf.smartsimple.us).
- **Proof of Access to Human Samples:** March 18, 2016
- **Response to Applicants Date:** May 2016
- **Earliest Anticipated Start Date:** August 2016

Proposal Application

Proposal applications should be submitted via the RMS360 system (jdrf.smartsimple.us) using the research plan template provided and including the following information:

- Background and Significance of this work to Type 1 Diabetes
- Proposed research (What?)
- Rationale for proposed research (Why?)
- Research Design and Methods (How?)
- Advantages over alternative approaches that would address goal.
- Future plans if research is successful.
- Proof of access to biosamples, if not included with the application, should be emailed to the administrative contact listed on this RFA by March 18, 2016

RMS360

- JDRF is using a grants management system to collect online application submissions called RMS360. The RMS360 link is as follows: https://jdrf.smartsimple.us/s_Login.jsp.
  - Please note that if you are new to the system, you must register and log in details will be generated.
- Call details and deadlines can be found in the "Funding Opportunities" tab of RMS360.
- All materials and templates pertaining to the application can be found once you've initiated an application in RMS360.
- It is recommended to use Google Chrome or Firefox when using RMS360, as these browsers are most compatible with the system.

Where should questions be directed?

- Questions on eligibility and suitability of proposals:
  - Jessica Dunne (jdunne@jdrf.org)
- Questions on logistics, deadlines or submission problems:
  - Randy Rowe (rrowe@jdrf.org)
- Non-grant specific inquiries or issues, please contact SmartSimple Support Services via email support@smartsimple.com or phone (866) 239-0991. Support hours are Monday through Friday between 5:00am and 9:00pm US Eastern Standard Time.

THANK YOU

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