

Updated March 23, 2015

OPPORTUNITIES FOR RESEARCH SUPPORTED BY THE SPECIAL STATUTORY FUNDING PROGRAM FOR TYPE 1 DIABETES RESEARCH: A WORKSHOP OF THE DMICC

April 8-9, 2015

6001 Executive Blvd., Room C, Rockville, MD 20852

AGENDA

April 8

8:00-8:10 am

Welcome and Introduction

Griffin Rodgers, M.D., M.A.C.P., Director, NIDDK

8:10-8:30 am

Overview of the *Special Statutory Funding Program for Type 1 Diabetes Research*

Judith Fradkin, M.D., Director, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK

TOPIC 1: DIABETES COMPLICATIONS

Primary Discussants: **Matthew Breyer, M.D.; Nigel Calcutt, Ph.D.; Robert Eckel, M.D.; Mark Espeland, Ph.D.; Thomas Gardner, M.D., M.S.; James Meigs, M.D.; Robert Sherwin, M.D.; Timothy Wysocki, Ph.D.**

8:30-8:40 am

Current Efforts in Diabetes Complications

Judith Fradkin, M.D., Director, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK

8:40-8:55 am

Presentations and Questions/Answers on:

Proposal 1: Cardiovascular Disease in Type 1 Diabetes – Biorepository and Human Tissue Research

Primary discussants: Dr. Breyer, Dr. Calcutt, Dr. Eckel

Proposal 2: Cardiovascular Disease in Type 1 Diabetes – Cohort, Registry, and Database Consortia

Primary discussants: Dr. Eckel, Dr. Espeland, Dr. Meigs

Teresa Jones, M.D., Program Director, Diabetic Complications, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK

8:55-9:15 am

Panel Discussion on Proposals 1 and 2 (~10 minutes of discussion for each proposal)

9:15-9:30 am

Proposal 3: Neurocognitive Effects of Glycemic Dysregulation in Type 1 Diabetes

Luke Stoeckel, Ph.D., Program Director, Cognitive & Clinical Neuroscience of Obesity & Diabetes, Division of Diabetes, Endocrinology, and Metabolic Diseases, NIDDK

Primary discussants: Dr. Calcutt, Dr. Sherwin, Dr. Wysocki

9:30-9:50 am

Proposal 4: Continuation of Diabetic Complications Consortium (DiaComp)

Kristin Abraham, Ph.D., Senior Advisor, Model Systems and Resources, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK

Primary discussants: Dr. Eckel, Dr. Gardner

9:50-10:10 am **Proposal 5: Continuation of Diabetic Retinopathy Clinical Research Network (DRCR.net)**
Frederick Ferris, III, M.D., Clinical Director, Division of Intramural Research, NEI
Primary discussants: Dr. Espeland, Dr. Gardner, Dr. Meigs

10:10-10:30 am **Topic 1 panel discussion led by primary discussants**

10:30-10:40 am **BREAK**

TOPIC 2: ARTIFICIAL PANCREAS

Primary Discussants: **Richard Bergenstal, M.D.; Irl Hirsch, M.D.; Robert Sherwin, M.D.**

10:40-10:50 am **Current Efforts in Artificial Pancreas**
Judith Fradkin, M.D., Director, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK

10:50-11:05 am ***Presentations and Questions/Answers on:***

Proposal 1: Expansion of Clinical, Behavioral, and Physiological Research Testing of Current and Novel Closed-Loop Systems
Primary discussants: Dr. Bergenstal, Dr. Hirsch, Dr. Sherwin,

Proposal 2: Expansion of Research to Develop New or Improved Closed-Loop Automated Technologies for Type 1 Diabetes Therapy and Monitoring
Primary discussants: Dr. Bergenstal, Dr. Hirsch, Dr. Sherwin,

Proposal 3: Advanced Clinical Trials to Test Artificial Pancreas Device Systems in Type 1 Diabetes
Primary discussants: Dr. Bergenstal, Dr. Hirsch, Dr. Sherwin,

Guillermo Arreaza, M.D., Program Director, Clinical Immunology of Diabetes, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK

11:05-11:25 am **Panel Discussion on Proposals 1, 2, and 3 (~7 minutes of discussion for each proposal)**
Led by Drs. Bergenstal, Hirsch, and Sherwin

11:25-11:35 am **Topic 2 panel discussion led by primary discussants**

11:35 am-12:10 pm **LUNCH**

TOPIC 3: CLINICAL MANAGEMENT

Primary Discussants: **Richard Bergenstal, M.D.; Irl Hirsch, M.D.; Georgeanna Klingensmith, M.D.; Timothy Wysocki, Ph.D.**

12:10-12:20 pm **Current Efforts in Clinical Management**
Judith Fradkin, M.D., Director, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK

- 12:20-12:40 pm** *Presentations and Questions/Answers on:*
- Proposal 1: Improving Adherence in Young Children with Type 1 Diabetes**
Primary discussants: Dr. Klingensmith, Dr. Wysocki
- Proposal 2: Improving Adherence in Pre-teens, Adolescents and Young Adults with Type 1 Diabetes**
Primary discussants: Dr. Klingensmith, Dr. Wysocki
- Proposal 3: Understanding Barriers and Facilitators to Type 1 Diabetes Management in Adults**
Primary discussants: Dr. Hirsch, Dr. Wysocki
- Christine Hunter, Ph.D., ABPP, Director of Behavioral Research, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK
- 12:40-1:00 pm** **Panel Discussion on Proposals 1, 2, and 3 (~7 minutes of discussion for each proposal)**
Led by Drs. Hirsch, Klingensmith, and Wysocki
- 1:00-1:15 pm** **Proposal 4: Impact of the Use of Glucose Monitoring and Control Technologies on Health Outcomes and Quality of Life in Older Adults with Type 1 Diabetes**
Guillermo Arreaza, M.D., Program Director, Clinical Immunology of Diabetes, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK
Primary discussants: Dr. Bergenstal, Dr. Hirsch, Dr. Wysocki
- 1:15-1:25 pm** **Topic 3 panel discussion led by primary discussants**
- TOPIC 4: RESOURCES**
Primary Discussants: **Michael German, M.D.; Ronald Gill, Ph.D.; James Meigs, M.D.; Elizabeth Selvin, Ph.D., M.P.H.; Robert Sherwin, M.D.; Stanislaw Stepkowski, D.V.M., Ph.D., D.Sc.**
- 1:25-1:35 pm** **Current Efforts in Resources**
Judith Fradkin, M.D., Director, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK
- 1:35-1:50 pm** **Proposal 1: Human Pancreas Procurement and Analysis Program (HPPAP)**
Olivier Blondel, Ph.D., Director, Endocrine Systems Biology Program, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK
Primary discussants: Dr. German, Dr. Gill
- 1:50-2:10 pm** **Proposal 2: Continuation of the Integrated Islet Distribution Program (IIDP)**
Kristin Abraham, Ph.D., Senior Advisor, Model Systems and Resources, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK
Primary discussants: Dr. German, Dr. Stepkowski
- 2:10-2:25 pm** **Proposal 3: Continuation of Programs to Standardize C-peptide and HbA1c Assays**
Beena Akolkar, Ph.D., Senior Advisor, Immunopathogenesis and Genetics of Type 1 Diabetes, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK
Primary discussants: Dr. Selvin, Dr. Sherwin

2:25-2:45 pm **Proposal 4: Continuation of the Collaborative Islet Transplantation Registry (CITR)**
Thomas Eggerman, M.D., Ph.D., Program Director, Clinical Islet Transplantation Trials, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK
Primary discussants: Dr. Gill, Dr. Meigs

2:45-3:00 pm **Topic 4 panel discussion led by primary discussants**

3:00-3:15 pm **BREAK**

TOPIC 5: ATTRACTING NEW TALENT TO TYPE 1 DIABETES RESEARCH

Primary Discussants: **Rudolph Leibel, M.D.; James Meigs, M.D.; Maïke Sander, M.D.; Robert Sherwin, M.D.**

3:15-3:25 pm **Current Efforts in Attracting New Talent to Type 1 Diabetes Research**
Judith Fradkin, M.D., Director, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK

3:25-3:40 pm **Proposal 1: Type 1 Diabetes Pathfinder Award**
Carol Renfrew Haft, Ph.D., Program Director, Senior Advisor for Cell Biology and Associate Director for Grants Administration, Division of Diabetes, Endocrinology, and Metabolic Diseases, NIDDK
Primary discussants: Dr. Sander, Dr. Sherwin

3:40-3:55 pm **Proposal 2: Career Development Programs in Diabetes Research for Pediatric Endocrinologists**
Carol Renfrew Haft, Ph.D., Program Director, Senior Advisor for Cell Biology and Associate Director for Grants Administration, Division of Diabetes, Endocrinology, and Metabolic Diseases, NIDDK
Primary discussants: Dr. Leibel, Dr. Meigs

3:55-4:05 pm **Topic 5 panel discussion led by primary discussants**

TOPIC 6: SMALL BUSINESS INNOVATION RESEARCH (SBIR) AND SMALL BUSINESS TECHNOLOGY TRANSFER (STTR)

Primary discussants: **Richard Bergenstal, M.D.; Betty Diamond, M.D.; Ronald Gill, Ph.D.; Irl Hirsch, M.D.; Jane Salmon, M.D.; Maïke Sander, M.D.**

4:05-4:15 pm **Current Efforts in SBIR/STTR Research**
Judith Fradkin, M.D., Director, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK

4:15-4:30 pm ***Presentations and Questions/Answers on:***
Proposal 1: Towards a Bio-artificial Pancreas: Development of New Technologies for the Advancement of Cell Replacement Research for Type 1 Diabetes
Primary discussants: Dr. Gill, Dr. Sander

Proposal 2: Towards an Artificial Pancreas: Expansion of Support for Research to Develop New Therapeutics and Monitoring Closed-Loop Automated Technologies for Type 1 Diabetes
Primary discussants: Dr. Bergenstal, Dr. Hirsch

Guillermo Arreaza, M.D., Program Director, Clinical Immunology of Diabetes, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK

- 4:30-4:45 pm** **Panel Discussion on Proposals 1, 2 (~7 minutes of discussion for each proposal)**
Led by Drs. Bergenstal, Gill, Hirsch, and Sander
- 4:45-5:00 pm** **Proposal 3: New Methods and Technologies for Assessment of Risk and for Early Diagnosis and Prognosis of Type 1 Diabetes**
Guillermo Arreaza, M.D., Program Director, Clinical Immunology of Diabetes, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK
Primary discussants: Dr. Diamond, Dr. Salmon
- 5:00-5:15 pm** **Topic 6 panel discussion led by primary discussants**
- 5:15-5:45 pm** **Day 1 closing discussion led by Dr. Judith Fradkin, NIDDK**

April 9

TOPIC 7: AUTOIMMUNE ETIOLOGY, CLINICAL TRIALS, AND EPIDEMIOLOGY

Primary Discussants: **Jeffrey Bluestone, Ph.D.; Nigel Calcutt, Ph.D.; Betty Diamond, M.D.; Robert Eckel, M.D.; Mark Espeland, Ph.D.; Michael German, M.D.; Peter Gregersen, M.D.; Irl Hirsch, M.D.; Georgeanna Klingensmith, M.D.; Rudolph Leibel, M.D.; James Meigs, M.D.; Jane Salmon, M.D.; Elizabeth Selvin, Ph.D., M.P.H.; Robert Sherwin, M.D.**

- 8:00-8:10 am** **Current Efforts in Autoimmune Etiology, Clinical Trials, and Epidemiology**
Judith Fradkin, M.D., Director, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK
- 8:10-8:25 am** **Proposal 1: Mechanisms Underlying the Contribution of Type 1 Diabetes Risk-associated Variants**
Beena Akolkar, Ph.D., Senior Advisor, Immunopathogenesis and Genetics of Type 1 Diabetes, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK
Primary discussants: Dr. Gregersen, Dr. Leibel
- 8:25-8:40 am** **Proposal 2: Elucidating Mechanisms Underlying Clinical Heterogeneity in Type 1 Diabetes**
Andrew Bremer, M.D., Ph.D., Program Director, Clinical Research on Type 2 Diabetes Prevention and Treatment in Adults, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK
Primary discussants: Dr. Klingensmith, Dr. Sherwin
- 8:40-8:55 am** **Proposal 4: SEARCH for Diabetes in Youth**
Sharon Saydah, Ph.D., CDR USPHS, Senior Scientist, Division of Diabetes Translation, Centers for Disease Control and Prevention
Primary discussants: Dr. Meigs, Dr. Selvin

- 8:55-9:10 am** **Proposal 5: Surveillance for Type 1 Diabetes among Young Adults**
 Sharon Saydah, Ph.D., CDR USPHS, Senior Scientist, Division of Diabetes Translation, Centers for Disease Control and Prevention
Primary discussants: Dr. Meigs, Dr. Selvin
- 9:10-9:25 am** **Proposal 3: Validation of Self-report Measures of Type 1 Diabetes in National Surveys**
 Sharon Saydah, Ph.D., CDR USPHS, Senior Scientist, Division of Diabetes Translation, Centers for Disease Control and Prevention
Primary discussants: Dr. Meigs, Dr. Selvin
- 9:25-9:45 am** **Proposal 6: Research Using Biosamples and Subjects from Selected Type 1 Diabetes Clinical Studies**
 Lisa Spain, Ph.D., Program Director, Immunobiology of Type 1 Diabetes and Autoimmune Endocrine Diseases, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK
Primary discussants: Dr. Hirsch, Dr. Salmon, Dr. Selvin
- 9:45-10:00 am** **Proposal 7: Clinical Trials in People with Established Type 1 Diabetes and Endogenous Insulin Production**
 Lisa Spain, Ph.D., Program Director, Immunobiology of Type 1 Diabetes and Autoimmune Endocrine Diseases, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK
Primary discussants: Dr. Bluestone, Dr. Hirsch, Dr. Sherwin
- 10:00-10:15 am** **BREAK**
- 10:15-10:45 am** **Proposal 8: Continuation of Type 1 Diabetes TrialNet**
 Lisa Spain, Ph.D., Program Director, Immunobiology of Type 1 Diabetes and Autoimmune Endocrine Diseases, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK
Primary discussants: Dr. Diamond, Dr. Eckel, Dr. Salmon
- 10:45-11:00 am** *Presentations and Questions/Answers on:*
- Proposal 9: Immune Tolerance Network (ITN)**
- Proposal 9a: Preserving Beta Cell Function with Immune Modulators in New-Onset Type 1 Diabetes**
Primary discussants: Dr. Eckel, Dr. Gregersen, Dr. Salmon
- Proposal 9b: A Robust Technology Platform for Assessing Islet-specific Autoimmunity in Type 1 Diabetes Clinical Trials**
Primary discussants: Dr. Gregersen, Dr. Salmon
- Proposal 9c: ITN NOD Preclinical Consortium**
Primary discussants: Dr. Calcutt, Dr. German
- James McNamara, M.D., Acting Associate Director, Clinical Research Operations Program, Division of Allergy, Immunology, and Transplantation, NIAID
- 11:00-11:15 am** **Panel Discussion on Proposals 9a, 9b, and 9c (~7 minutes of discussion for each proposal)**

Led by Drs. Calcutt, Eckel, German, Gregersen, and Salmon

- 11:15-11:35 am** **Proposal 10: Continuation of Cooperative Study Group for Autoimmune Disease Prevention (CSGADP)**
Thomas Esch, Ph.D., Section Chief, Autoimmune and Primary Immunodeficiency Diseases Section, Autoimmunity and Primary Immunodeficiency Diseases Branch, Division of Allergy, Immunology, and Transplantation, NIAID
Primary discussants: Dr. Bluestone, Dr. Diamond, Dr. Salmon
- 11:35-12:10 pm** **LUNCH**
- 12:10-12:25 pm** *Presentations and Questions/Answers on:*
- Proposal 11: The Environmental Determinants of Diabetes in the Young (TEDDY)**
- Proposal 11a: Continued Follow-up of TEDDY Subjects**
Primary discussants: Dr. Espeland, Dr. Gregersen
- Proposal 11b: Immunological Assessments of TEDDY Subjects**
Primary discussants: Dr. Diamond, Dr. Gregersen
- Proposal 11c: Epigenetic Modifications in TEDDY Subjects**
Primary discussants: Dr. Gregersen, Dr. Leibel
- Beena Akolkar, Ph.D., Senior Advisor, Immunopathogenesis and Genetics of Type 1 Diabetes, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK
- 12:25-12:45 pm** **Panel Discussion on Proposals 11a, 11b, and 11c (~7 minutes of discussion for each proposal)**
Led by Drs. Diamond, Espeland, Gregersen, and Leibel
- 12:45-1:00 pm** *Presentations and Questions/Answers on:*
- Proposal 12: Trial to Reduce IDDM in the Genetically at Risk (TRIGR)**
- Proposal 12a: Completion of Follow-up of TRIGR Participants**
Primary discussants: Dr. Diamond, Dr. Espeland
- Proposal 12b: A Multiomics Approach to the Pathogenesis of Type 1 Diabetes: Validation of the Omics Findings in TEDDY and Other Prospective Studies**
Primary discussants: Dr. Diamond, Dr. German
- Gilman Grave, M.D., Chief, Pediatric Growth and Nutrition Branch, NICHD
- 1:00-1:15 pm** **Panel Discussion on Proposals 12a and 12b (~7 minutes of discussion for each proposal)**
Led by Drs. Diamond, Espeland, and German
- 1:15-1:45 pm** **Topic 7 panel discussion led by primary discussants**

TOPIC 8: BETA CELL: ASSESSMENT AND THERAPIES

Primary Discussants: **Richard Bergenstal, M.D.; Nigel Calcutt, Ph.D.; Michael German, M.D.; Ronald Gill, Ph.D.; Rudolph Leibel, M.D.; Maïke Sander, M.D.; Robert Sherwin, M.D.; Stanislaw Stepkowski, D.V.M., Ph.D., D.Sc.**

- 1:45-1:55 pm** **Current Efforts in Beta Cell Research**
Judith Fradkin, M.D., Director, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK
- 1:55-2:10 pm** **Proposal 1: Development of Tools and Techniques to Enhance the Study of Type 1 Diabetes and its Complications in Zebrafish**
Kristin Abraham, Ph.D., Senior Advisor, Model Systems and Resources, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK
Primary discussants: Dr. Calcutt, Dr. German, Dr. Sander
- 2:10-2:25 pm** **Proposal 2: Clinical Validation of Biomarkers for Pancreatic Beta Cell Mass and Inflammation**
Maren Laughlin, Ph.D., Senior Advisor, Integrative Metabolism, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK
Primary discussants: Dr. German, Dr. Leibel
- 2:25-2:40 pm** **Proposal 3: Cellular Therapies for Type 1 Diabetes Consortium**
Nancy Bridges, M.D., Chief, Transplantation Branch, Division of Allergy, Immunology, and Transplantation, NIAID
Primary discussants: Dr. Gill, Dr. Sander, Dr. Sherwin
- 2:40-2:50 pm** **BREAK**
- 2:50-3:00 pm** **Proposal 4: Development of New Technologies for the Advancement of Cell Replacement Research for Type 1 Diabetes**
Guillermo Arreaza, M.D., Program Director, Clinical Immunology of Diabetes, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK
Primary discussants: Dr. Bergenstal, Dr. Gill
- 3:00-3:15 pm** **Proposal 5: *In Vivo* Cell Engineering to Protect or Replenish Functional Human Beta Cell Mass**
Olivier Blondel, Ph.D., Director, Endocrine Systems Biology Program, Division of Diabetes, Endocrinology, & Metabolic Diseases, NIDDK
Primary discussants: Dr. German, Dr. Leibel
- 3:15-3:35 pm** **Proposal 6: Continuation of the Nonhuman Primate Transplantation Tolerance Cooperative Study Group (NHPCSG)**
Kristy Kraemer, Ph.D., Section Chief, Transplantation Basic Sciences Section, Transplantation Branch, Division of Allergy, Immunology, and Transplantation, NIAID
Primary discussants: Dr. Gill, Dr. Stepkowski
- 3:35-3:50 pm** **Topic 8 panel discussion led by primary discussants**
- 3:50-4:00 pm** **Patient perspective – Ms. Ellen Leake**
- 4:00-4:45 pm** **Closing discussion led by Dr. Judith Fradkin, NIDDK**