The Joslin Diabetes Center is pleased to welcome you to the inaugural Joslin Symposium, which is entitled “Challenges and Opportunities in Type 1 Diabetes Research.” Type 1 diabetes is a potentially devastating disease that can be treated with insulin administration, but robust curative strategies have remained elusive. To overcome this disease will require development of means to block autoimmune destruction of insulin-producing pancreatic beta cells, along with strategies for replacing beta cell function. Each of these areas is addressed in today’s meeting. We are very pleased to have an outstanding group of speakers here to present their work and ideas, and we would like to encourage lively scientific exchange after each talk and during the breaks. It is our hope that by bringing this stellar group of speakers together with a cross section of the scientific community from the Longwood area, greater Boston, and beyond, we will stimulate interactions that will lead us closer to understanding and ultimately conquering this disease.

The Joslin Diabetes Center thanks our co-sponsor the Leona M. and Harry B. Helmsley Charitable Trust and the Juvenile Diabetes Research Foundation for their support.

ORGANIZING COMMITTEE:
T. Keith Blackwell, M.D., Ph.D., Chair
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Stephan Kissler, Ph.D.
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Aldo Rossini, M.D.
Tom Serwold, Ph.D.
Gordon Weir, M.D.
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<td>8:30 – 9:00</td>
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| 9:15 – 9:55 | Matthias von Herrath  
**New insights from human histopathology**  
for treatment and prevention strategies for  
Type 1 diabetes |
| 9:55 – 10:35 | Susan Bonner-Weir  
**Can an endogenous source be used for beta cell**  
replacement therapy for diabetes? |
| 10:35 – 10:55 | BREAK — Refreshments                                                 |
| 10:55 – 11:35 | Diane Mathis  
**Control of T1D progression by an epigenetic modulator** |
| 11:35 – 12:15 | Andrew Stewart  
**Molecular control of human pancreatic Beta cell replication** |
| 12:15 – 1:30 | Lunch                                                                |
| 1:30 – 2:10 | Edward Damiano  
**This is the dawning of the age of the bionic endocrine pancreas** |
| 2:10 – 2:50 | Jay Skyler  
**Human immunotherapy trials**                                      |
| 2:50 – 3:10 | BREAK — Refreshments                                                |
| 3:10 – 3:50 | Emil Unanue  
**Initiation of autoimmune diabetes: From peptides to genes**       |
| 3:50 – 4:30 | Doug Melton  
**How to make Beta cells?**                                         |
| 4:30 – 5:15 | Public Reception                                                     |
Matthias von Herrath, M.D.
Professor at the La Jolla Institute for Allergy and Immunology
Vice President and Head Novo Nordisk Research and Development Center Seattle

Matthias von Herrath earned his M.D. and completed his doctoral thesis *summa cum laude* in 1988 at Freiburg Medical School in Freiburg Germany. He began his research career at the Scripps Institute, where he was a postdoctoral fellow and later a faculty member. In 2001 he moved to the La Jolla Institute for Allergy and Immunology, where he is currently a Professor and head of the Diabetes Research Center. In 2011 he became Vice President and Head of the Diabetes Research and Development Center for Novo Nordisk, Inc., located in Seattle WA. He has received numerous awards for his work, including the American Diabetes Association’s Outstanding Scientific Achievement Award in 2008.

New insights from human histopathology for treatment and prevention strategies for Type 1 diabetes
Can an endogenous source be used for beta cell replacement therapy for diabetes?
Dr. Diane Mathis obtained a Ph.D. from the University of Rochester, and performed postdoctoral studies at the Laboratoire de Génétique Moléculaire des Eucaryotes (LGME) in Strasbourg, France and at Stanford University Medical Center. She returned to France at the end of 1983, establishing a laboratory at the LGME [later the Institut de Genetique et de Biologie Moleculaire et Cellulaire (IGBMC)] in Strasbourg, in conjunction with Dr. Christophe Benoist. The lab moved to the Joslin Diabetes Center, Boston, MA at the end of 1999. Through 2008, Dr. Mathis was a Professor of Medicine at Brigham and Women’s Hospital and Harvard Medical School, and an Associate Research Director and Head of the Section on Immunology and Immunogenetics at Joslin, where she held the William T. Young Chair in Diabetes Research. Dr. Mathis is currently Professor and Head of the Division of Immunology in the Department of Microbiology and Immunobiology at HMS, and holder of a Morton Grove-Rasmussen Chair in Immunohaematology. She is also a Principal Faculty Member at the Harvard Stem Cell Institute and an Associate Faculty Member of the Broad Institute. She presently serves on Scientific Advisory Boards of the Howard Hughes Medical Institute, Genentech, Fidelity Biosciences, and MedImmune, as well as of several research institutes worldwide. She is co-founder of Tempero, a biotech start-up that aims to produce novel therapeutics in the autoimmunity/inflammation space. Dr. Mathis was elected to the US National Academy of Sciences in 2003, the German Academy in 2007, and the American Academy of Arts and Sciences in 2012. The lab works in the fields of T cell differentiation and autoimmunity.
Dr. Andrew Stewart has led a career devoted to patient care and basic and clinical research in endocrinology and diabetes research for over 30 years. He received his bachelor’s degree from Trinity College in Hartford, Connecticut and his M.D. from Columbia University, College of Physicians and Surgeons, New York, New York. He served as a fellow in Endocrinology and Metabolism at Yale University School of Medicine in Connecticut, and rose to the rank of tenured Professor at Yale. He moved to Pittsburgh in 1997 to become Chief of the Division of Endocrinology and Metabolism at the University of Pittsburgh School of Medicine. He recently moved to New York to serve as the Director of the Diabetes, Obesity and Metabolism Institute at Mount Sinai in November, 2012.

Dr. Stewart is a leading authority on human pancreatic beta cell replication and regeneration. His research focuses on understanding and developing novel means for inducing beta cell regeneration. His group was the first to demonstrate that growth factors could drive beta cell replication in vivo in mammals, and also improve glucose control in living animals. He was also the first to carefully define cell cycle control in the rodent and human beta cell, and is actively involved in understanding why human beta cells normally do not replicate. His team has been able to induce robust replication in human beta cells, and to reverse diabetes with engineered, rapidly replicating human beta cells in vivo. This work is the subject of invited symposia at both the American Diabetes Association (ADA), the European Islet Study Group (ISG), the European Association for the Study of Diabetes (EASD), the International Congress of Endocrinology, and the US National Institutes of Health.

Dr. Stewart has received numerous honors, including Councilor of both the Endocrine Society as well as the American Society for Bone and Mineral Research (ASBMR); Secretary-Treasurer of the Endocrine Society, and Chair of the Program Committee for the ADA. He has published more than 230 scientific papers, with many in journals of the highest quality, including the Proceedings of the National Academy of Sciences, the New England Journal of Medicine, and Science. Dr. Stewart is the continuous recipient of numerous research grants for the past 30 years. He currently has strong support from the NIH, the JDRF and other agencies. He has served on and chaired grant review panels for the NIH, the ADA and the JDRF. He was the 2008 recipient of the Endocrine Society’s Gerald Aurbach Award for outstanding scientific achievement. He served as the Chair of the American Diabetes Association Annual Meetings for 2010 and 2012.
Edward Damiano is an Associate Professor of Biomedical Engineering at Boston University. His expertise and training are in the areas of mechanical and biomedical engineering, applied mechanics, and applied mathematics. Ever since his 13-year-old son was diagnosed with type 1 diabetes at 11 months of age, he has been committed to creating and integrating closed-loop control technologies with a vision of building a bionic endocrine pancreas. This endeavor began with design and development work on mathematical algorithm strategies for blood glucose control, which he and his group began testing in his laboratory over five years ago in a swine model of type 1 diabetes. Working closely with the FDA, he and his group conducted all necessary animal experiments and performed all required software and hardware validation studies to fully qualify their system for clinical testing. Their first-generation device became the first academically sponsored investigational device exemption (IDE) ever to receive FDA approval for human testing. Their first-phase clinical trial testing this device in 24-hour experiments in adult subjects with type 1 diabetes was conducted in the Clinical Research Center (CRC) at the Massachusetts General Hospital (MGH), and was completed in 2009. They received IDE approval from the FDA to test their second-generation fully automated device in 48-hour experiments in a second-phase clinical trial, which began in July 2010 in the MGH CRC in children and adults with type 1 diabetes, and concluded in December 2012. They recently received IDE approval from the FDA to begin testing their third-generation system, which runs on a mobile-device platform and integrates an iPhone with their blood-glucose control algorithm, an insulin pump, and a continuous glucose monitor. They plan to begin conducting five-day experiments testing this platform in the out-patient setting in January 2013.

This is the dawning of the age of the bionic endocrine pancreas.
Jay S. Skyler, MD, MACP, is currently a Professor of Medicine, Pediatrics, & Psychology, in the Division of Endocrinology, Diabetes, & Metabolism, Department of Medicine, University of Miami Miller School of Medicine, Miami, Florida. He is Deputy Director for Clinical Research and Academic Programs at the Diabetes Research Institute, University of Miami, and is Adjunct Professor of Pediatrics at the Barbara Davis Center for Childhood Diabetes, University of Colorado.

Dr. Skyler’s research interest focuses on immune intervention and beta cell expansion or replacement. Since 2001, he has been Chairman of the NIH (NIDDK)-sponsored Type 1 Diabetes TrialNet, an international network conducting clinical trials to prevent type 1 diabetes or interdict the type 1 diabetes disease process. Type 1 Diabetes TrialNet is the successor of the Diabetes Prevention Trial — Type 1 Diabetes Study Group, of which Dr. Skyler served as Chairman throughout its existence from 1993 until it was replaced by TrialNet.

Dr. Skyler is a past President of the American Diabetes Association, the International Diabetes Immunotherapy Group, and the Southern Society for Clinical Investigation, and was a Vice-President of the International Diabetes Federation. He served as a member of the Endocrinology, Diabetes, and Metabolism Subspecialty Examining Board of the American Board of Internal Medicine, as Chairman of the Council of Subspecialty Societies of the American College of Physicians (ACP) and a member of the ACP Board of Regents.

Jay Skyler was founding Editor-in-Chief of Diabetes Care, and founding Scientific Editor of the International Diabetes Monitor. For many years, he was Western Hemisphere Regional Editor of Diabetes Research and Clinical Practice. He currently is Senior Editor of Diabetes Technology & Therapeutics. He has been author, editor, or co-editor of 21 books or monographs, and has written over 400 articles, book chapters, or editorials. He also has edited 14 special journal symposia.
Dr. Unanue’s laboratory research centers on the cellular and biochemical basis of recognition of protein antigens by the immune system and on the role of histocompatibility molecules (the molecules encoded in the Major Histocompatibility Complex-MHC) in antigen presentation. His laboratory has made two fundamental findings, that protein antigens are processed into peptides by antigen presenting cells (APC) before their recognition by T cells; and that MHC molecules are peptide-binding molecules that form the complex required for T cell recognition. These observations have contributed to a molecular understanding of T cell recognition and opened the field for a rational analysis of immunogenicity. His laboratory continues investigations on the cellular and biochemical basis of antigen processing and presentation focused now on the recognition of self proteins in autoimmune diabetes.

Emil Unanue was born and raised in Havana, Cuba and went to Medical School at the University of Havana. Upon coming to the US, he first did research training in immunopathology at Scripps Research Institute. His interest in cellular immunology was developed during a post doctoral research under Brigitte Askonas at the National Institute for Medical Research in London. He was in the faculty of Harvard Medical School from 1970 until 1985 at the Department of Pathology in the Medical School. In 1985 he moved, with his laboratory, to Washington University School of Medicine and chaired their Department of Pathology and Immunology until 2006. He now continues leading his research laboratory examining cellular aspects of autoimmunity.
Doug Melton was trained as a developmental biologist, taking a PhD degree at Trinity College, Cambridge University and the MRC Laboratory of Molecular Biology. Since that time, he has been on the faculty at Harvard and now holds the position of Xander University Professor as well as Investigator in the Howard Hughes Medical Institute. Melton is also the Co-Director and Co-Chair of the Harvard Stem Cell Institute and the Department of Stem Cell and Regenerative Biology.