Areas of Research Focus:
The PDRC at WU/SLCH is currently focused on four underserved areas of pediatric diabetes research.

**T2DM:** WU continues to participate in the TODAY study which has resulted in improved understanding of the aggressive nature of adolescent type 2 diabetes (T2DM). Mouse models explore the influence of prenatal/perinatal exposure on predisposition to T2DM. Investigators actively participate in clinical trials of medications to improve glycemic control in adolescent T2DM. Novel clinical strategies for improving diabetes self-management are being developed and studied.

**Monogenic Diabetes:** Mouse models of KATP channel defects pioneered the understanding of the genetic cause of sulfonylurea responsive neonatal diabetes. This has led to the development of a sulfonylurea screening protocol to identify antibody negative type 1 diabetic patients who potentially have a monogenic form of diabetes. Whole exome sequencing of these subjects is ongoing.

**Wolfram Syndrome:** Past work identified causative genetic defect in Wolfram syndrome. An international registry and annual natural history research clinic have been established to better characterize Wolfram Syndrome. Using mouse models, patient samples, clinical markers of disease progression and potential therapies are being investigated.

**CFRD:** WU is a leader in cystic fibrosis and pediatric lung transplant care. Advances in CF care has resulted in cystic fibrosis related diabetes (CFRD) becoming a common co-morbidity. Collaborative efforts between the Divisions of Pediatric Pulmonology and Pediatric Endocrinology seek to better understand the CF specific pathogenesis and complications of diabetes in order to improve clinical outcomes.

---

**Research Centers at Washington University**

- Diabetes Research Center (DRC)
- Center for Investigation of Membrane Excitability Diseases (CIMED)
- Diabetic Cardiovascular Disease Center (DCDC)
- Center for Diabetes Translational Research (CDTR)
- Nutritional and Obesity Research Center (NORC)
- The Genome Institute (TGI)
- Genome Technology Access Center (GTAC)

**Director:** Paul Hruz, M.D., Ph.D.  
**Website:** pdrd.wustl.edu  
**Email:** hruz_p@kids.wustl.edu  
**Phone:** (314) 286-2725
Overview
The Pediatric Diabetes Research Consortium (PDRC) at Washington University/St. Louis Children's Hospital (WU/SLCH) strives to improve the lives of all children with diabetes and their families. The PDRC fosters awareness and application of broadly based pediatric diabetes research spanning basic science and clinical departments to address the unique challenges of this life-altering disease in children. The PDRC supports diverse and robust basic, translational and clinical research activities relevant to childhood diabetes throughout Washington University.

The program includes a talented pool of highly accomplished faculty from a wide range of scientific disciplines with a highly collaborative spirit found throughout the Washington University School of Medicine. Dedicated support staff, readily accessible core facilities, and established research centers of excellence strengthen the PDRC. The research activities performed by PDRC investigators provide direct and tangible benefit to the comprehensive clinical pediatric diabetes treatment program at St Louis Children's Hospital and the Pediatric Endocrinology Fellowship Program within the Washington University Department of Pediatrics.

History
Washington University has a longstanding history of leadership in pediatric diabetes. Independent researchers at Washington University simultaneously with those at Eli Lilly discovered a method for large-scale production of insulin by isoelectric precipitation in 1922. That same year, Washington University/St. Louis Children’s Hospital physicians became the first in the USA to administer insulin to a child with diabetes.

The Consortium pioneered the clinical use of insulin pumps. The late Dr. Julio Santiago spearheaded the design and implementation of the DCCT which changed diabetes management promoting intensive treatment to reduce the risk of long-term diabetes complications.

Under Dr. Neil White's leadership, the Consortium has a robust and sustained participation in multi-center clinical trials. Dr. Phillip Cryer has greatly expanded the understanding of mechanisms and consequences of hypoglycemia.

Dr. Ana Maria Arbelaez continues to develop our knowledge of the effects of hypoglycemia on brain structure and function. Stemming from the discovery of the gene for Wolfram Syndrome (Diabetes Insipidus, Diabetes Mellitus, Optic Atrophy, Deafness; DIDMOAD), Drs. Alan Permutt, Tamara Hershey, Fumihiko Urano and Bess Marshall established an international registry and annual research clinic for patients with Wolfram Syndrome.

This esteemed history of leadership has contributed greatly to pediatric diabetes, paving the way for current and future investigators in the PDRC to continue to improve the lives of children with diabetes.

Research Environment
The PDRC functions within Washington University School of Medicine. The School of Medicine’s institutional culture promotes strong partnerships between clinical and basic science departments that fosters collaboration. This allows the PDRC to leverage the intellectual and physical resources of Washington University Medical Center, to make significant contributions to diabetes research. The university includes a broad array of resources including:

- Institute of Clinical and Translational Sciences (ICTS)
- Children’s Discovery Institute (CDI)
- Established and Subsidized Core Facilities for Diabetes Related Research
- Premier Medical Scientist Training Program
- Robust Pediatric Endocrinology Fellowship Training Program, WU/SLCH consortium
- Highly Accessible Registry of Patient Research Volunteers
- NIH funded Diabetes Research Center (DRC)

These resources have made the PDRC ideally-positioned to lead the transformation of clinical and translational research discoveries into improvements in the health of children.