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Evidence Report/Technology Assessment
Number 98

Islet Transplantation in Patients with Type 1 Diabetes Mellitus

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Preface

The Agency for Healthcare Research and Quality (AHRQ), through its Evidence-Based Practice Centers (EPCs), sponsors the development of evidence reports and technology assessments to assist public- and private-sector organizations in their efforts to improve the quality of health care in the United States. This report on islet transplantation in Type 1 diabetes mellitus was requested by PacifiCare. The reports and assessments provide organizations with comprehensive, science-based information on common, costly medical conditions and new health care technologies. The EPCs systematically review the relevant scientific literature on topics assigned to them by AHRQ and conduct additional analyses when appropriate prior to developing their reports and assessments.

To bring the broadest range of experts into the development of evidence reports and health technology assessments, AHRQ encourages the EPCs to form partnerships and enter into collaborations with other medical and research organizations. The EPCs work with these partner organizations to ensure that the evidence reports and technology assessments they produce will become building blocks for health care quality improvement projects throughout the Nation. The reports undergo peer review prior to their release.

AHRQ expects that the EPC evidence reports and technology assessments will inform individual health plans, providers, and purchasers as well as the health care system as a whole by providing important information to help improve health care quality.

We welcome written comments on this evidence report. They may be sent to: Director, Center for Outcomes and Evidence, Agency for Healthcare Research and Quality, 540 Gaither Road, Rockville, MD 20850.

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The authors of this report are responsible for its content. Statements in the report should not be construed as endorsement by the Agency for Healthcare Research and Quality or the U.S. Department of Health and Human Services of a particular drug, device, test, treatment, or other clinical service.

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Structured Abstract

Context: Pancreas transplantation is used selectively for labile type 1 diabetes to achieve physiologic insulin regulation. Infusing pancreatic islets into the liver via catheter ("islet transplant") may offer similar benefit with less surgical risk.

Objectives: Systematic evidence review on the outcomes of islet transplantation, particularly using the Edmonton or a subsequently developed islet transplant protocol.

Data Sources: MEDLINE searched through October 2003. Primary evidence from published papers and registries, supplemented with evidence from recent meeting abstracts and presentations.

Study Selection: Selected studies were prospective trials of allogeneic islet transplant for treatment of type 1 diabetes that reported glycemic outcomes and/or adverse events at least 3 months post-procedure, and used the Edmonton or a subsequently developed islet transplant protocol.

Data Extraction: A single reviewer selected studies and abstracted data. A second reviewer fact-checked the evidence tables.

Data Synthesis: Twelve published articles reporting efficacy and adverse outcomes, and two others reporting only adverse outcomes, constituted the available primary evidence. Supplemental sources provided preliminary results of studies in progress. Outcomes of interest were summarized in tables and synthesized across studies.

Conclusions: Evidence on outcomes of islet transplant is limited by small patient numbers, short followup, and lack of standardized reporting. (These issues are being addressed by the NIH-funded Collaborative Islet Transplant Registry.) Of 37 patients from three centers, 28 (76 percent) maintained insulin independence at 1 year (published evidence); similarly, 50 to 90 percent of 104 patients from four centers were insulin independent (supplemental evidence). Serious adverse events, including portal vein thrombosis and hemorrhage, occur infrequently. Data are lacking on long-term durability of the procedure, effects on diabetic complications, or long-term consequences of immunosuppression. Evidence is insufficient for comparison with whole-organ pancreas transplant.

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Appendixes

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Appendixes are provided electronically at http://www.ahrq.gov/clinic/epcindex.htm