Preparing to Implement a New Trreatment for Type 1 Diabetes – Stem cell transplant

**What is the Clinical Problem?**

Type 1 diabetes affects 1.6 million people in the U.S., including 187,000 children.

Type 1 diabetes is an autoimmune disease. It occurs when a person’s pancreas stops producing insulin, the hormone the controls blood-sugar levels

Type 1 diabetes develops when the insulin-producing pancreatic beta cells are mistakenly destroyed by the body’s immune system – genetic and environmental impact

Less than 33% of people with Type 1 diabetes in the U.S. are consistently achieving target blood-glucose control

Current therapy requires multiple injections of insulin daily, close monitoring of blood sugars and restricted diet

**What has the impact been?**

Type 1 diabetes affects 1.6 million people in the U.S., including 187,000 children

In the U.S. there are $16billion in Type 1 diabetes-associated healthcare expenditures and lost income annually

**What are the limitations of current management strategies?**

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**What is a possible solution?**  Stem-cell transplant

**How does the treatment work?**

Stem cells are produced by human embryos and in laboratories

Stem cells are manipulated to develop into insulin-producing pancreatic beta cells

Engineered cells are placed in humans and cells begin producing insulin in response to serum blood glucose levels

Medications to suppress recipients’ immune systems are administered

**Hypothetical clinical application**

Patient with Type 1 DM is referred to specialty transplant center for novel treatment

Patient has outpatient visit at specialty transplant center

* Undergoes laboratory evaluation to confirm eligibility for treatment – 1 hour
* Receives treatment (IV infusion over 30 minutes)
* Receives prescription for daily immunosuppressant medication

Patient completes follow-up visits for labs 2, 7, and 28 days after initial treatment

Patient completes routine blood test monitoring with primary care provider every 6 months

**Is the treatment safe and effective?**

* ***Stem cell transplants have been used in other clinical conditions (e.g. Parkinson’s), with some evidence of safety and efficacy***
* ***Current Type 1 diabetes treatment is being evaluated in a 17 person clinical safety study.***

***POTENTIAL IMPLEMENTATION CHALLENGES***

* There are still uncertainties in how to use the treatment (where should the cells be implanted, how should the immune system be suppressed or the cells be protected)
* Durability of treatment success unknown
* Cost/technical skills required to deliver
* Patient willingness to accept treatment – sample of reader comments from the NYT article
	+ “As a type 1 diabetic for over 20 years I would not touch this. It is exactly the same as a transplant which is dubious at best”
	+ “I have had Type 1 for 45 years and do believe this cure is needed”
	+ “Can you really call it a cure if you have to take immunosuppressant drugs? For someone like myself who has managed it well for forty-five years, the trade-off between maintaining my own health and potentially getting cancer would be too steep.”
	+ “My daughter is 23 years old and a type 1 diabetic since age 8. I am so thankful for this potential cure.”
* Political challenges for developing, producing and administering stem-cell derived therapies