

# Type 1 Diabetes (T1D): Towards the Cure

## Organization

University of Missouri-Columbia

## Industry:

Bio-Science, Human Health

## Researchers:

Habib Zaghouni, Ph.D.

## Status of Intellectual Property:

Multiple patents issued and pending

## Publications:

Wan X, Guloglu FB, VanMorlan AM, Rowland LM, Zaghouni S, Cascio JA, Dhakal M, Hoeman CM, Zaghouni H. Recovery From Overt Type 1 Diabetes Ensues When Immune Tolerance and b-Cell Formation Are Coupled With Regeneration of Endothelial Cells in the Pancreatic Islets. J of Diab 62: 2879-2889, 2013.

## Next Steps:

- Licensing to established company or startup
- Clinical Trials



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## Customer Problem

Type 1 diabetes is a chronic disease in which the insulin-producing  $\beta$ -cells of the pancreatic islets are destroyed by inflammatory T lymphocytes of the immune system. This lack of insulin leads to high blood sugar levels and if not maintained regularly can lead to death. Sufferers inject regular amounts of insulin to maintain blood sugar levels. To date there is no known way to prevent type 1 diabetes. There is currently no cure for Diabetes.

## Potential Market Uses

Treatment of Type 1 Diabetes. Unpublished data suggests effects lasts far beyond treatment and can be effective regardless of how long the patient has been diabetic.

## Market Size

- Autoimmune disease that Affects:
  - ~20M - 40M people worldwide<sup>1</sup>
  - ~1.25M Americans (~40k new cases/year)<sup>2</sup>
- Global T1D market was ~\$25.5B in 2016<sup>3</sup>
- Current treatment consists of 2-4 insulin injections per day for life (with intense glucose monitoring; <1/3 in US achieve target [blood glucose])

## Innovation

Researchers at the University of Missouri have developed a new technology designed to treat type 1 diabetes through administration of a combination of Ig-GAD2 carrying a GAD peptide and donor bone marrow stem cells. Formation of new host derived  $\beta$ -cells and endothelial cells originating from the injected bone marrow stem cells were achieved through this combinatorial approach. Both the newly formed  $\beta$ -cells and endothelial cells were formed in the pancreas. The  $\beta$ -cells regulated insulin production while the bone marrow derived endothelial cells repaired the islet endothelial niche. This approach sustained formation of both  $\beta$ -cells and endothelial cells thus reversing type 1 diabetes. This technology has been demonstrated effective as both a preventative and a cure for Type I Diabetes in mouse models, and is ready to begin clinical trials.

## Stage of Development

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## Competitive Advantages Include:

- Combinatorial approach using Ig-GAD2, GAD peptide, and donor bone marrow stem cells
- Synthesis of host  $\beta$ -cells and endothelial cells derived from donor bone marrow stem cells
- Restoration of  $\beta$ -cells production and repair of islet endothelial niche

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## Current Competition:

- Current Treatments
  - Insulin manufacturers (Ely Lilly, Sanofi, Novo Nordisk, etc.)
  - Insulin pumps
- Novel Treatments under investigation
  - Antigen approaches to induce tolerance (e.g. Diamyd)
  - Modified PDL1 blood stem cells (Paolo Fiorina)
  - Islet cell transplantation (w/immune suppressants)
  - Insulin-producing implants (ViaCyte)
  - Others