Assessment of Safety and Efficacy of Extended Release Cornstarch Therapy in Glycogen Storage Disease Type Ia

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INTRODUCTION:

Glycogen storage disease (GSD) type I is caused by deficiency of glucose-6-phosphatase resulting in severe hypoglycemia, hepatomegaly, hyperlactatemia, and hyperlipidemia. Cornstarch has been the main treatment for GSD since 1982. In 2012, a new extended-release cornstarch was released, but there have been no studies on the long-term efficacy and safety of this product.

HYPOTHESIS:

A new modified starch, Glycosade® (Vitaflo® (Int'l) Ltd.), will allow patients with GSD to have longer uninterrupted sleep and improve quality of life without sacrificing metabolic control.

METHODS:

A prospective cohort study was performed using all patients that tried overnight Glycosade at University of Florida. A total of 69 subjects with GSD Ia (31 M, 38 F; average age 18.0 years, age range 5-59 years) attempted fasting with Glycosade between 2012 and 2013. For inclusion in follow-up studies, one nighttime dosage of Glycosade extended-release cornstarch needed to be consumed at least 3 nights per week for at least 3 months. Long-term laboratory data are available for 27 of the patients who have transitioned to Glycosade.

Figure 1: Duration of Fasting on Uncooked Cornstarch and Glycosade

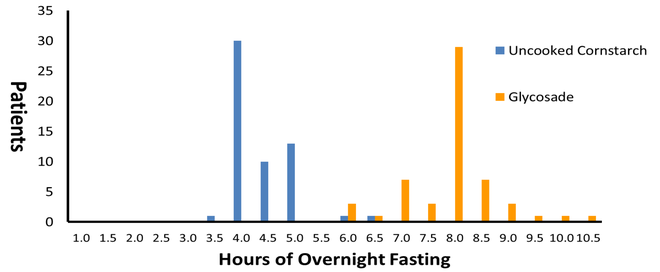
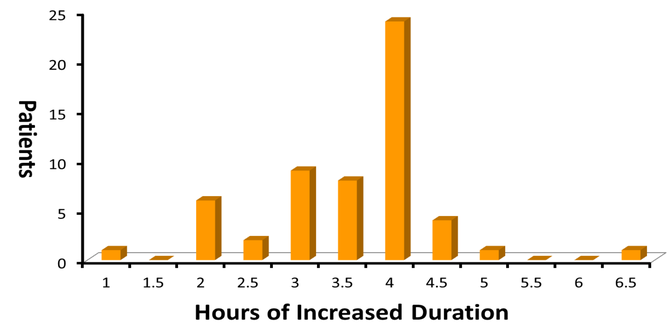


Figure 2: Duration of Fasting Increase on Glycosade Compared with Uncooked Cornstarch



	Pre-Glycosade Mean ± SD (n=27)	3 Month Post-Glycosade Mean ± SD (n=19)	6 Month Post-Glycosade Mean ± SD (n=22)	12 Month Post-Glycosade Mean ± SD (n=4)
AST (U/L)	30.1 ± 11.5	36.6 ± 19.6	25.4 ± 7.0	27.0 ± 18.7
ALT (U/L)	27.4 ± 17.4	36.4 ± 34.0	25.0 ± 13.1	25.8 ± 17.3
Cholesterol (mg/dL)	186.5 ± 38.2	199.0 ± 46.2	197.6 ± 55.1	186.8 ± 35.3
Triglycerides (mg/dL)	282.1 ± 124.8	291.6 ± 131.1	282.3 ± 157.8	215.8 ± 51.9
Uric acid (mg/dL)	6.4 ± 1.7	5.8 ± 1.4	6.2 ± 1.7	6.4 ± 0.9
Total protein (g/dL)	7.3 ± 0.4	7.5 ± 0.5	7.3 ± 0.4	7.3 ± 0.4
Albumin (g/dL)	4.3 ± 0.3	4.5 ± 0.3	4.4 ± 0.3	4.2 ± 0.3

RESULTS:

- Dosing for overnight Glycosade is comparable to total uncooked cornstarch for the same period. Median dose in adults was 135 grams (range 120-150 grams).
- 58 of 69 patients (84.1%) GSD type Ia experienced improved short term metabolic control and increased duration of fasting on a Glycosade challenge.
- The success rate at extending fasting was 95% for females but 71% for males. No factors could be identified to predict who would benefit from the extended release preparation.
- All markers of long term metabolic control remained stable on the new therapy.
- No episodes of severe hypoglycemia or hospitalizations have been required for subjects treated with the new therapy.
- 1 patient developed rapidly growing adenomas on Glycosade therapy, but it is unclear if there is an association with the therapy.

CONCLUSION :

Glycosade has allowed patients with glycogen storage disease type Ia to maintain normoglycemia for an extended period of time and improved quality of life without sacrificing metabolic control.

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