Persistent Treatment Burden and Glycemic Instability in Congenital Hyperinsulinism: Insights from the HI Global Registry

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INTRODUCTION AND METHODS

- **Congenital hyperinsulinism (HI)** is the most common cause of persistent hypoglycemia in newborns and children and can lead to neurological injury.
- Current HI medications are often limited by adverse effects and inadequate glycemic control.

We aimed to characterize treatment burden among individuals with HI in the HI Global Registry and to contextualize these findings with patient-reported experiences.

N = 192 individuals reported current use of 1+ HI medication

Adverse events were summarized by treatment group

Glycemic stability was assessed through CGM data from 23 individuals

Qualitative data were used to contextualize findings

The HI Global Registry (HIGR) is the only international patient-powered registry for people with HI.

13 surveys*
** Reported by the participant or a caregiver*

1 physician survey

Glucose Data

> 600 registrants

8 Languages

Electronic Health Records*
** Currently available for USA participants only*

RESULTS

BURDEN OF ADVERSE EFFECTS

Most common adverse effects reported by treatment

Diazoxide (n = 159)

No adverse effects	14%
Hirsutism	83%
Loss of appetite	41%
Stomach pain	25%

Octreotide (n = 27)

No adverse effects	46%
Changes in stool	25%
Gallstone/ gallbladder sludge	21%
Stomach pain	17%

Lanreotide (n = 19)

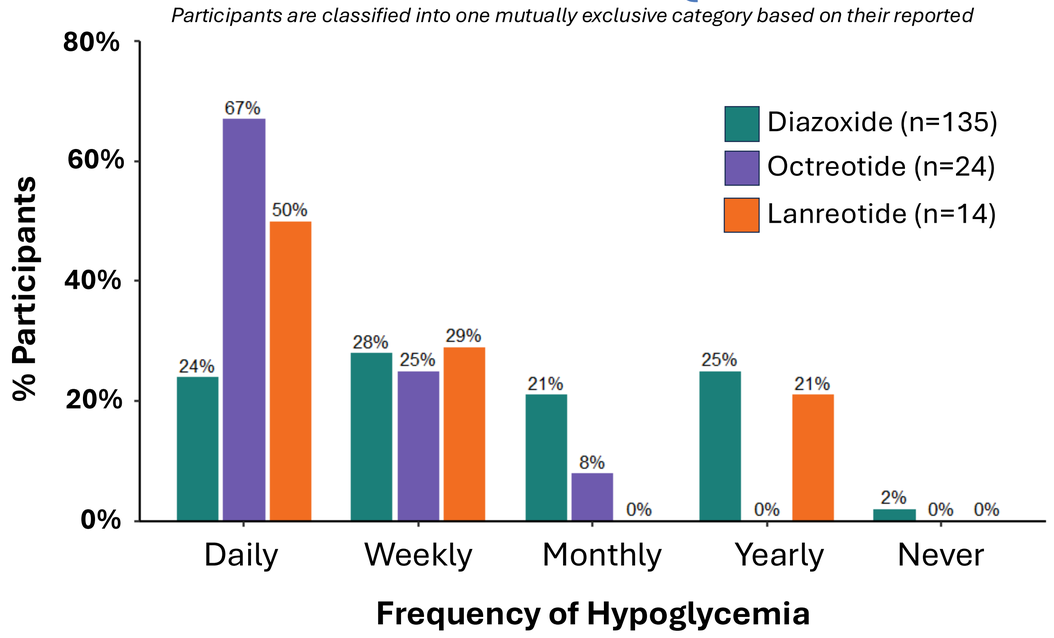
No adverse effects	42%
Changes in stool	53%
Gallstone/ gallbladder sludge	21%
Nausea or stomach pain	21%

Percentages may exceed 100% as participants may be taking more than one medication, and participants could report multiple side effects

Of 192 people in HIGR taking medication for HI, 83% reported ≥1 side effect of their current medication

PATIENT-REPORTED HYPOGLYCEMIA FREQUENCY

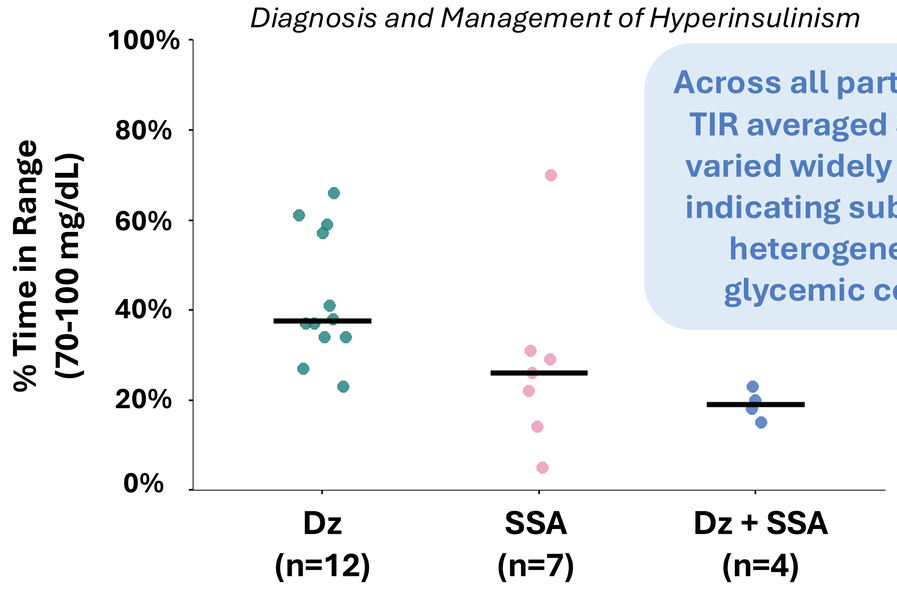
Participants are classified into one mutually exclusive category based on their reported



30% of individuals with HI taking medication reported experiencing hypoglycemia at least once per day

CGM-REPORTED TIME IN RANGE

Time in range reflecting guidance in the International Guidelines for the Diagnosis and Management of Hyperinsulinism



Dz = currently taking only diazoxide
SSA = currently taking only a somatostatin analog (octreotide/lanreotide)
Dz + SSA = currently taking diazoxide in conjunction with a SSA
Median is represented by the bar

Across all participants, TIR averaged 34% but varied widely (5-70%), indicating substantial heterogeneity in glycemic control

FAMILY PERSPECTIVES: THE LIVED BURDEN OF TREATMENT

"The diazoxide made her immediately throw up. She didn't like it, but we could coax her into taking it, but it went into her stomach, and it just came back, right back out."-*Caregiver to 6 year old child*

"There is currently no medication working. We as a family regulate the symptoms by constantly (day and night) paying attention to the diet to avoid hypoglycemia."-*Caregiver to a 3 year old, no longer taking medication*

"The lanreotide injection is very painful, and it causes [CHILD] a lot of anxiety when the time comes to get her injection. She also knows that it makes her feel quite unwell with stomach upset and diarrhea for a few days after, which impacts her life. It would be great to see oral treatment options that don't have all the side effects that come with lanreotide and diazoxide."-*Caregiver* to a 15 year old

CONCLUSIONS

- Current HI treatments are associated with significant adverse effects and do not consistently achieve glycemic control.
- There is a significant and urgent need for therapies that effectively control blood glucose levels without compromising quality of life.

ACKNOWLEDGEMENTS

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More about HIGR