Lifecycle and Promotion of a Custom Patient Registry Survey with an Industry Partner

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Background

- Creatine transporter deficiency (CTD) is a rare X-linked cerebral creatine deficiency syndrome (CCDS).[1]
- Individuals with CTD may show intellectual and developmental delays, expressive speech and language delays, autistic-like behavior, hyperactivity, seizures, gastrointestinal issues, and movement disorders.[2,3]
- There is currently **no treatment or cure for CTD**.

Objectives

- An **industry partner** wanted to learn about the oral medication preferences of CTD patients and caregivers to inform the development of their therapeutic, a possible treatment for CTD.
- **Goal:** Collaborate with an industry partner to **develop a custom oral medication survey** for our CreatineInfo Patient Registry and Natural History Study.

Methods

Data Sharing Agreement (5 months)

Created a **data sharing agreement** to establish legal protections for both ACD and the industry partner, defining project services, deadlines, deliverables, and payment schedule.

Question Development (2 months)

Developed survey questions in **collaboration with the industry partner**, to ensure that the goals of both parties were reflected in the survey questions.

Metabolic Dietician Consultation (1 month)

Consulted with **Krista Viau, PhD, RD (metabolic dietician)** to serve as our academic partner and provide guidance on the survey content.

Caregiver Feedback (1 month)

Held 2 meetings to gather **feedback from ACD's Family Advisory Board (FAB)**, to ensure patient and caregiver perspectives were represented, confirm the language was family-friendly, and add additional, important questions relevant to oral medication preferences.

NORD Survey Build (5 months)

Partnered with the **NORD patient registry team** to build and launch the IRB-approved survey on our CreatineInfo Patient Registry.

Recruitment Campaign (2 months)

Executed a **recruitment campaign** (13 social media posts, 1 email, 1 newsletter), with a target goal of 35 or more CTD responses.

Results

Project Outcomes:

1. Industry and non-profit data sharing agreement appropriate for future use
2. 52-question custom oral medication survey
3. Successful recruitment campaign with high participant engagement surpassing our target goal, with 37 CTD participants completing the survey in the first 6 weeks
4. 55-page report containing aggregate, de-identified data from the survey
5. Valuable insights into patient oral medications preferences to share with additional stakeholders in future drug development efforts (*see Figures 1 & 2*)
6. Industry partner support of patient registry costs resulting in a self-sustaining registry
7. Better understanding of the time requirements, and therefore appropriate cost to charge, for this type of endeavour.

Figure 1 What attributes of a medication are most important?

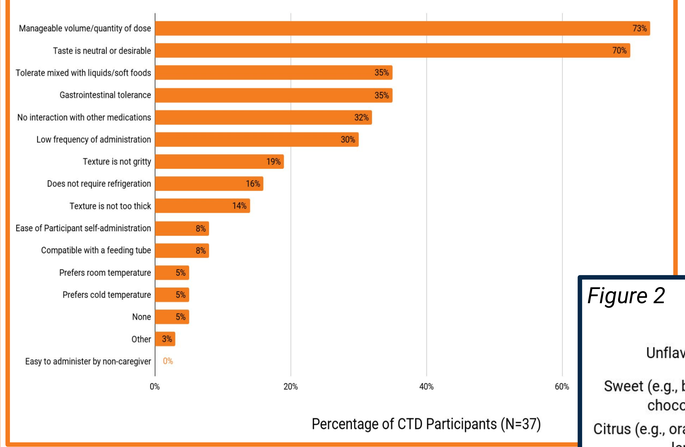

***Figure 1**: Percentage of CTD survey participants who included the medication attribute in their top 4 most important. Overwhelmingly, participants rated "Manageable volume or quantity of dose" and "Taste is neutral or desirable" as the top most rated attributes (N=37).*

Figure 2 What flavors do participants prefer?

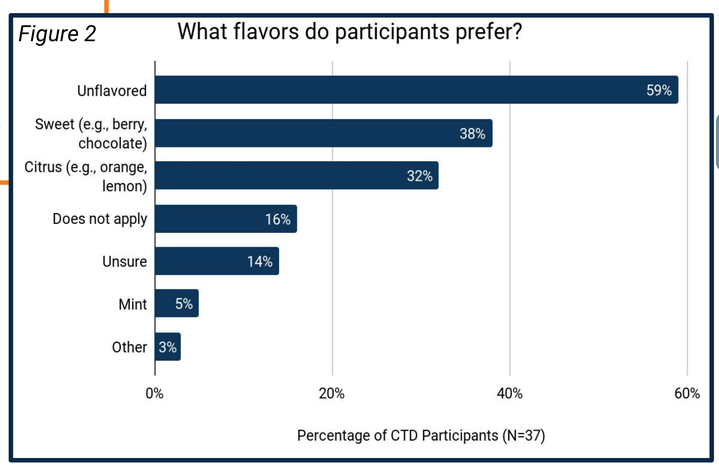

***Figure 2**: Percentage of CTD survey participants who selected each flavor. "Unflavored" was selected by the greatest percentage of participants, followed by "Sweet" (N=37).*

Conclusions

- Co-development of a custom patient registry survey can be a mutually beneficial endeavor for both patient advocacy groups and industry partners.
- Survey participants were incentivized to complete the survey by simply knowing that their data had meaningful implications.
- Survey objectives should be well-defined and agreed upon early in the process to avoid timely delays in the development of questions.

References & Contact

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2. Passi, G. R., Pandey, S., Devi, A. R. R., Konanki, R., Jain, A. R., Bhatnagar, S., ... & Jain, V. (2022). Cerebral creatine deficiency disorders–A clinical, genetic and follow up study from India. *Brain and Development*, *44*(4), 271-280.
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Let's Connect!
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