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Patient-Centric Design: Incorporating Patient Input Into a Clinical Study of Idiopathic Hypersomnia and Narcolepsy

DUET STUDY 407

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Introduction

- Jazz DUET (**D**evelop hypersomnia **U**nderstanding by **E**valuating low-sodium oxybate **T**reatment; NCT05875974) is a phase 4, prospective, multicenter, single-arm, open-label interventional study of low-sodium oxybate (LXB, Xywav®)[1-4]
  - LXB is approved by the US Food and Drug Administration (FDA) for the treatment of idiopathic hypersomnia in adults and the treatment of cataplexy or excessive daytime sleepiness (EDS) in patients 7 years of age or older with narcolepsy[1]
- DUET evaluates the association of LXB with sleep architecture and daytime and nighttime symptoms in adult participants with idiopathic hypersomnia and adult participants with narcolepsy (type 1 or type 2)
  - Multiple assessments, including overnight polysomnography (PSG), are included to generate data of interest to clinicians and patients
- DUET was intended to be as patient-centric as possible. A patient advisory board was convened to gain patient perspectives and feedback on aspects of the DUET study design and clinically meaningful outcomes that are of significant importance to patients

Objectives

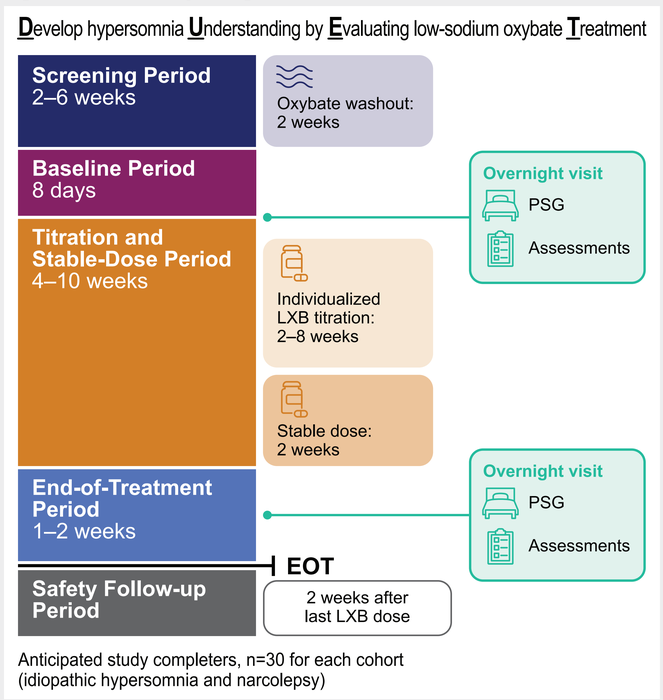
- Describe the input sought from a patient advisory board, and the incorporation of that input into the design of a patient-centered study
- Describe the patient-centric aspects of the DUET study design

Methods

- The advisory board consisted of 2 people with idiopathic hypersomnia and 4 people with narcolepsy
  - All 6 advisors had experience with patient advocacy, and at least 1 had participated in a clinical trial
- Patient advisors completed a premeeting survey and then attended a 3-hour advisory board meeting with the study sponsor
- Five main topics were discussed at the advisory board meeting:
  1. Feasibility of oxybate washout for participants entering the study on treatment
  2. Burden of assessments
  3. Burden of overnight visits
  4. Value of reporting individualized data back to participants
  5. Relevance of specific symptom evaluation to participants
- Advisors also ranked narcolepsy and idiopathic hypersomnia symptoms in order of importance
- DUET includes a screening period (including a washout for participants taking oxybate at study entry), a baseline period (off oxybate treatment), a titration period (for individualized LXB dosing adjustments based on participants' needs), a stable-dose period, an end-of-treatment period (taking an optimized stable dose of LXB), and a safety follow-up period
- Two overnight visits (the first on the day before beginning LXB treatment and the second after the stable-dose period) include *ad libitum* PSG as well as a battery of patient-reported outcome assessments (the evening before and morning after PSG)
- Further details on the DUET study design are reported in Abstract 655/Poster 283; additional details on the DUET idiopathic hypersomnia and narcolepsy patient populations are reported in Abstract 1332/Poster 432 and Abstract 1337/Poster 437, respectively

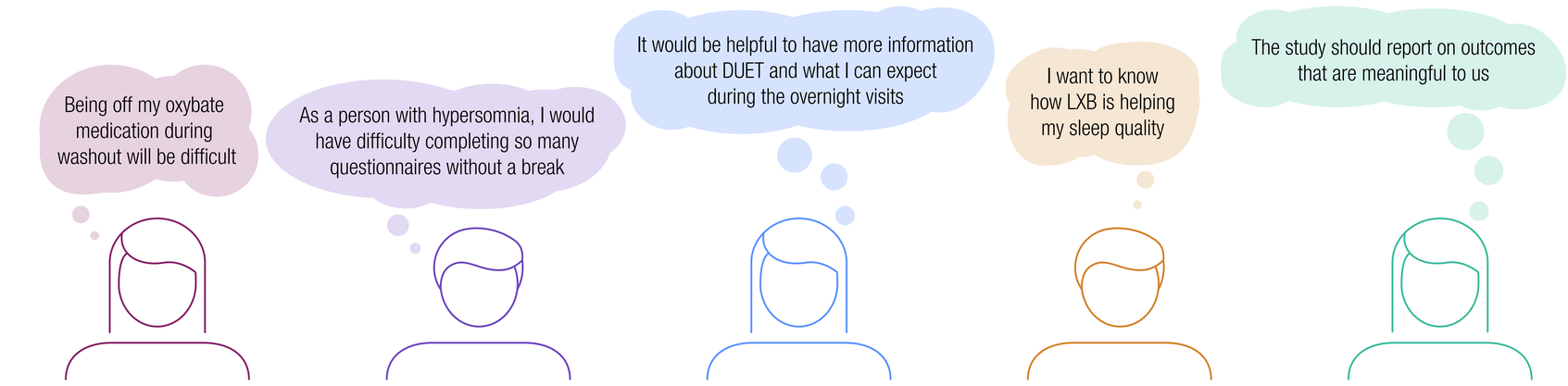
Results

Figure 1. DUET Study Design



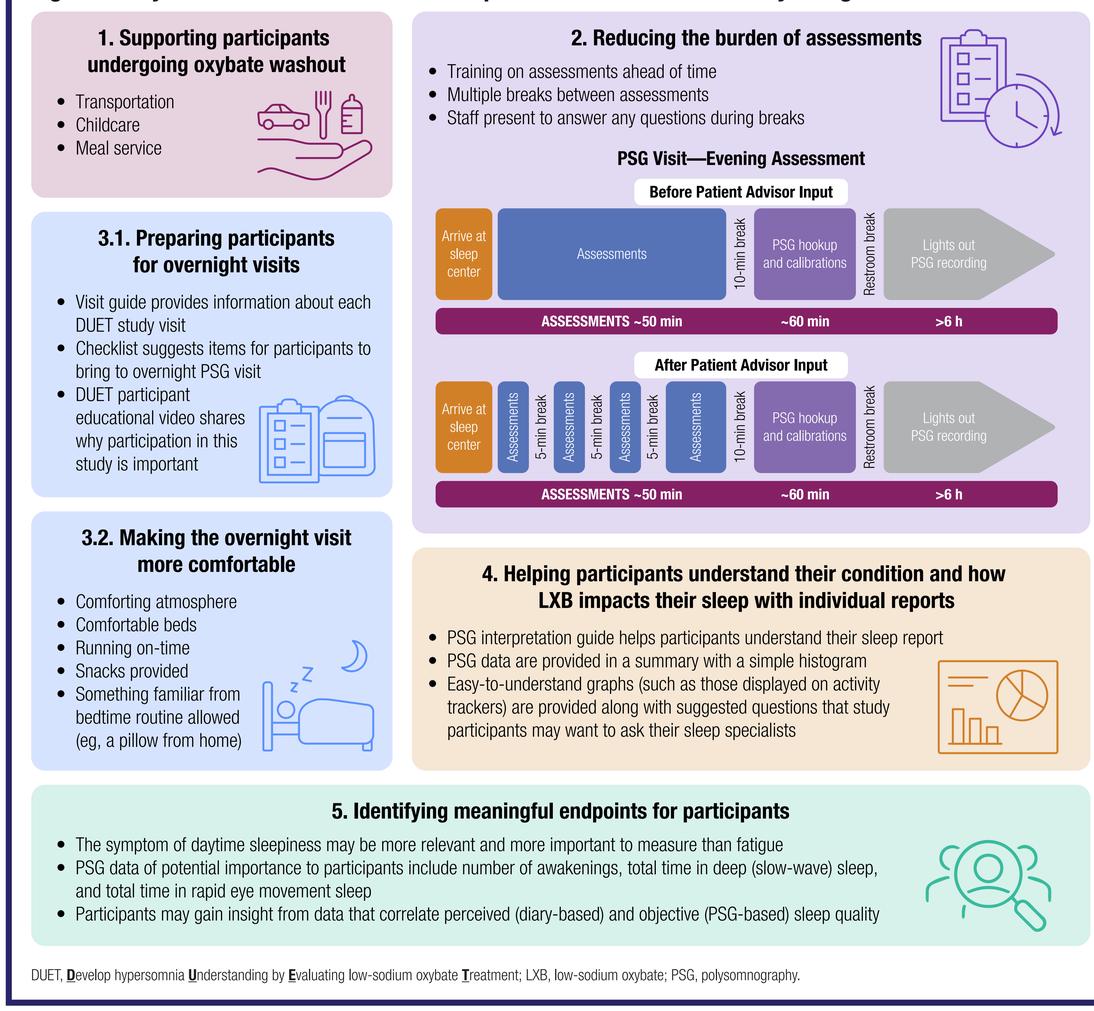
EOT, end of treatment; LXB, low-sodium oxybate; PSG, polysomnography.

Figure 2. The Patient Voice: Feedback From the Patient Advisory Board



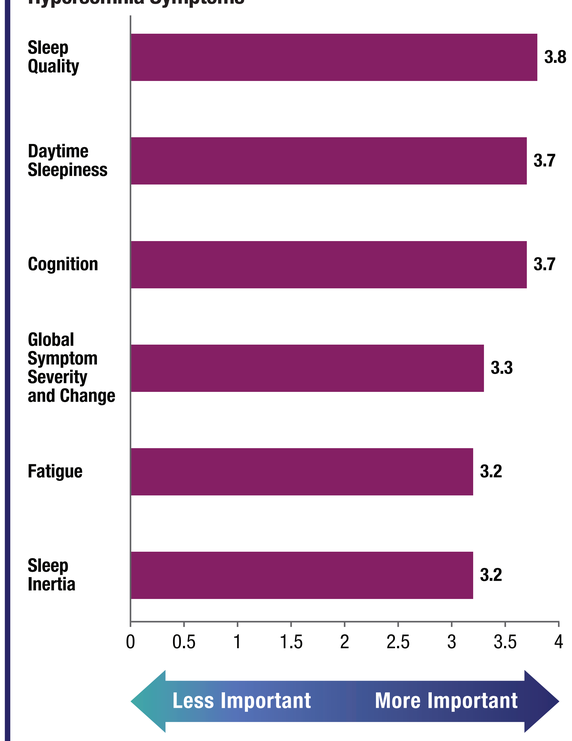
DUET, **D**evelop hypersomnia **U**nderstanding by **E**valuating low-sodium oxybate **T**reatment; LXB, low-sodium oxybate.

Figure 3. Key Patient-Centric Elements Incorporated Into Final DUET Study Design



DUET, **D**evelop hypersomnia **U**nderstanding by **E**valuating low-sodium oxybate **T**reatment; LXB, low-sodium oxybate; PSG, polysomnography.

Figure 4. Importance of Narcolepsy and Idiopathic Hypersomnia Symptoms



- Advisory board members were asked to rate (0 [not at all important] to 4 [very important]) the most important areas for DUET to explore
  - Sleep quality, daytime sleepiness, and cognition were rated the most important areas for DUET to investigate
    - In response, participants are provided reports on sleep quality (PSG). In addition, the endorsement of daytime sleepiness by patient advisors is in alignment with the study's primary endpoint of excessive daytime sleepiness as measured with the Epworth Sleepiness Scale

Conclusions

- The final DUET study design incorporated several overarching patient-centric elements as recommended by the patient advisory board
- One limitation of this approach is that the patient advisory board feedback was obtained from 6 individuals with idiopathic hypersomnia or narcolepsy, whose opinions may not reflect the opinions of the entire group of study participants
  - The study may have been skewed toward narcolepsy experience, as patient advisors with narcolepsy outnumbered those with idiopathic hypersomnia
- An additional limitation is the absence of a placebo group for comparison
- Implementation of these elements is anticipated to reduce participant burden, improve participant experience, enhance study recruitment and retention, and facilitate collection of comprehensive data that are meaningful to individuals with idiopathic hypersomnia or narcolepsy and their healthcare providers

References: 1. Xywav® (calcium, magnesium, potassium, and sodium oxybates) oral solution, CIII [prescribing information]. Palo Alto, CA: Jazz Pharmaceuticals, Inc. **2.** Szarfman A, et al. *N Engl J Med.* 1995;333(19):1291. **3.** US Food and Drug Administration. Clinical review for Binosto, NDA 202344. 2012. https://www.accessdata.fda.gov/drugsatfda_docs/nda/2012/202344Orig1s000MedR.pdf. **4.** US Food and Drug Administration. Quantitative labeling of sodium, potassium, and phosphorus for human over-the-counter and prescription drug products. Guidance for industry. 2022. https://www.fda.gov/regulatory-information/search-fda-guidance-documents/quantitative-labeling-sodium-potassium-and-phosphorus-human-over-counter-and-prescription-drug.

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Disclosures: DA Nichols, **S Akerman**, and **JK Alexander** are full-time employees of Jazz Pharmaceuticals who, in the course of this employment, have received stock options exercisable for, and other stock awards of, ordinary shares of Jazz Pharmaceuticals, plc. **EC Barker** has worked as an independent contractor and participant for medical advisory board meetings for Jazz Pharmaceuticals and has received speaker honoraria from Jazz Pharmaceuticals. **TL Steininger** is a former full-time employee and current contract worker for Jazz Pharmaceuticals who has previously received shares of Jazz Pharmaceuticals, plc.

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