TSC Alliance, LGS Foundation & Dravet Syndrome Foundation

Joint Town Hall (Zoom)

Introduction to Staccato® alprazolam and Phase 3 study

UCB Pharma

Robert Roebling, MD

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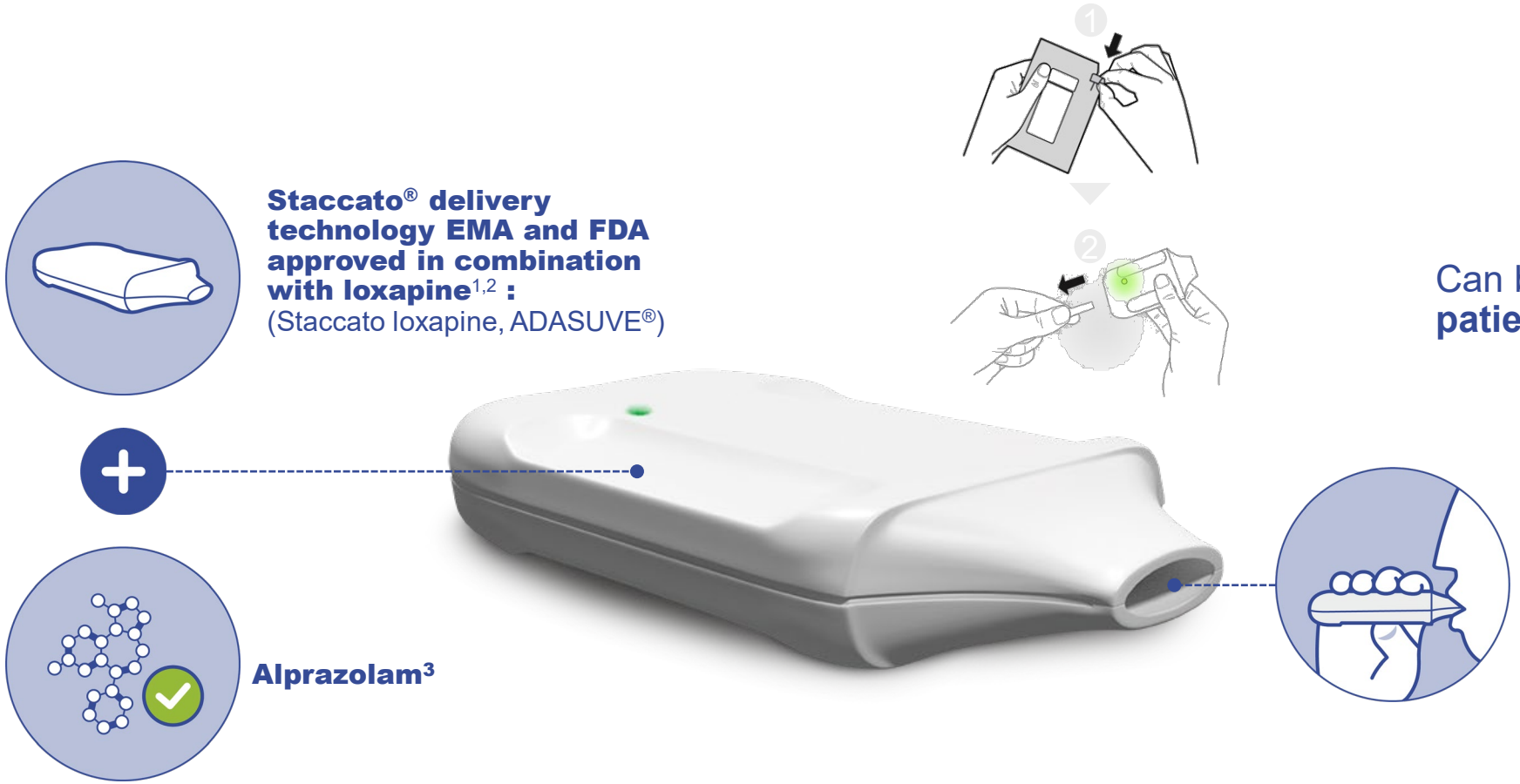
Inspired by patients. Driven by science.

Disclaimer and background

- Staccato® alprazolam is an investigational product. The safety and efficacy has not been established and it is not approved by the Food and Drug Administration, or by any health authority worldwide
- All contributors to these slides are full-time employees of UCB Pharma
- Alexza Pharmaceuticals has developed the Staccato device technology to deliver a molecule via normal inhalation in adults
- UCB Pharma has acquired Engage Therapeutics (which has conducted the Phase IIB) and is continuing the clinical development of Staccato alprazolam
- Phase III has started in 2021

Staccato® alprazolam is a breath-activated investigational therapy[1]



Can be administered both by **patient and caregiver**[2]

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EMA, European Medicines Agency; FDA Food and Drug Administration.

1. Alexza Pharmaceuticals. Staccato® One Breath Technology. Available at: http://staccatoobt.com (accessed November 2020); 2. UCB Pharma. Data on file. Engage Therapeutics_April 2020 Confidential Corporate Presentation; 3. French JA, et al. *Epilepsia.* 2019;60:1602-1609.

Key aspects of Phase 3 study[1]

- **Patients 12 years of age or older, with history of (focal) seizures typically lasting 3 minutes or more can be enrolled. Several other conditions have to be met.**
- **During (at most) 12-week main study, a single seizure is treated. Half the patients receive placebo (device with no active substance). Patient and caregiver are trained in the clinic.**
- **Study occurs at home. A caregiver needs to be present e.g. to measure time to end of seizure.**
- **Standard rescue medication can be used if seizure does not stop.**
- **After main study, study participants can enter into "open label" study where everyone will receive active drug (not placebo) for up to 4 years.**

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1. UCB Pharma. Data on File. EP0162-protocol-concept approved 10 May 2021.

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Phone Number: +1-844-599-CARE (2273)

Email Address: UCBCares@ucb.com