TSC Alliance, LGS Foundation & Dravet Syndrome Foundation

Joint Town Hall (Zoom)

Introduction to Staccato® alprazolam and Phase 3 study

UCB Pharma
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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 therapy¹

Staccato® delivery technology EMA and FDA approved in combination with loxapine¹²:
(Staccato loxapine, ADASUVE®)

Can be administered both by patient and caregiver²

Alprazolam³

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Image is for illustrative purposes only. Staccato® is a registered trademark of Alexza Pharmaceuticals, Inc. and Engage Therapeutics, Inc. (a wholly-owned subsidiary of UCB Pharma) is an authorized user of the Staccato® trademark. ADASUVE® is a registered trademark of Alexza Pharmaceuticals, Inc.
Key aspects of Phase 3 study\textsuperscript{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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\textsuperscript{1} UCB Pharma. Data on File. EP0162-protocol-concept approved 10 May 2021.
You can contact UCB

Contact UCB Cares

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