Acute Treatment of Agitation Associated with Alzheimer's Dementia (AAD)

TRANQUILITY Program

April 10, 2024

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Forward-Looking Statements

This presentation includes “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995. BioXcel Therapeutics, Inc. (“BioXcel” or the “Company”) intends such forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act of 1933, as amended and Section 21E of the Securities Exchange Act of 1934, as amended. All statements contained in this presentation other than statements of historical fact should be considered forward-looking statements, including, without limitation, statements related to the safety, efficacy, and regulatory and clinical design or progress, potential regulatory submissions, approvals and timing thereof for BXCL501 as a potential acute treatment for AAD; developments and plans relating to the TRANQUILITY program; and the potential for the results from the Company’s completed, ongoing and proposed clinical trials to support regulatory approvals for its product candidates in both the care-facility and at-home settings. When used herein, words including “anticipate,” “believe,” “can,” “continue,” “could,” “designed,” “estimate,” “expect,” “forecast,” “goal,” “intend,” “may,” “might,” “plan,” “possible,” “potential,” “predict,” “project,” “should,” “target,” “will,” “would” and similar expressions are intended to identify forward-looking statements, though not all forward-looking statements use these words or expressions. In addition, any statements or information that refer to expectations, beliefs, plans, projections, objectives, performance or other characterizations of future events or circumstances, including any underlying assumptions, are forward-looking. All forward-looking statements are based upon the Company’s current expectations and various assumptions. The Company believes there is a reasonable basis for its expectations and beliefs, but they are inherently uncertain.

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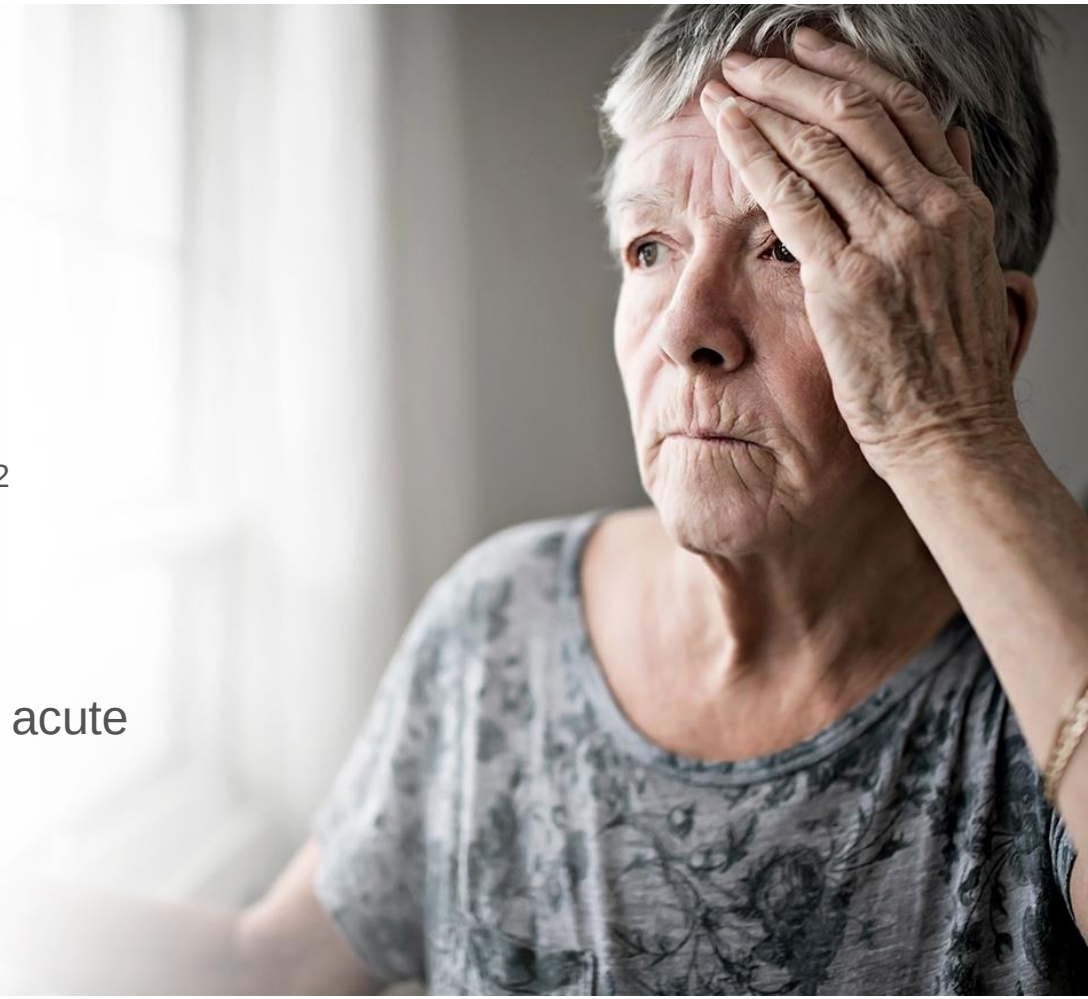
INDUSTRY AND OTHER DATA

Unless otherwise indicated, information contained in this presentation concerning our industry and the markets in which BioXcel Therapeutics operates, including its general expectations, market position and market opportunity, is based on its management’s estimates and research, as well as industry and general publications and research, surveys and studies conducted by third parties. While BioXcel Therapeutics believes the information from these third-party publications, research, surveys and studies is reliable, it does not guarantee the accuracy or completeness of such information, and BioXcel Therapeutics has not independently verified this information. Management’s estimates are derived from publicly available information, their knowledge of the company's industry and their assumptions based on such information and knowledge, which they believe to be reasonable. This data involves a number of assumptions and limitations which are necessarily subject to a high degree of uncertainty and risk due to a variety of factors, including those described in BioXcel Therapeutics' periodic reports filed with the SEC under the captions “Forward Looking Statements,” "Risk Factor Summary" and “Risk Factors.” These and other factors could cause BioXcel Therapeutics' future performance and market expectations to differ materially from its assumptions and estimates.

AAD is Debilitating for Patients and a Burden for Caregivers

Agitation cited as a top driver in deciding to move a patient from home setting to residential care facility[1]

- Nearly 7 million Alzheimer's dementia patients in the U.S., with approximately 50% suffering from agitation.[2]
- AD-related agitation typically worsens over time[2]
  - Both the number and severity of agitation episodes increase[2]
  - Often places significant burden on caregivers[1,2]
- No FDA-approved therapeutic options for an as-needed (PRN) acute treatment of agitation in Alzheimer's patients[3]



1. Data on File InVibe Patient and Caregiver Research (n=75) December 2022
2. Alzheimer's Association. 2023 Alzheimer's Disease Facts and Figures. Accessed November 14, 2023. https://www.alz.org/media/Documents/alzheimers-facts-and-figures.pdf.; Halpern R, Seare J, et al. Using electronic health records to estimate the prevalence of agitation in Alzheimer disease/dementia.. Int J Geriatri Psychiatry. 2019; 34: 420-431.
3. Joint Meeting of the Psychopharmacologic and the Peripheral and Central Nervous System Drugs Advisory Committee Meeting April 14th, 2023

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TRANQUILITY Program Offers Potential Path to sNDA

- Plan to discuss details of requirement for long-term safety data at future meeting with FDA**
- Company has developed preliminary TRANQUILITY At-Home trial design and is re-evaluating initiation timing

* Trial protocol under development, design may be subject to change.
** Per ICH guidelines, the Company may be required to collect 6-month safety data from at least 300 patients and 1-year safety data from at least 100 patients prior to submitting any sNDA

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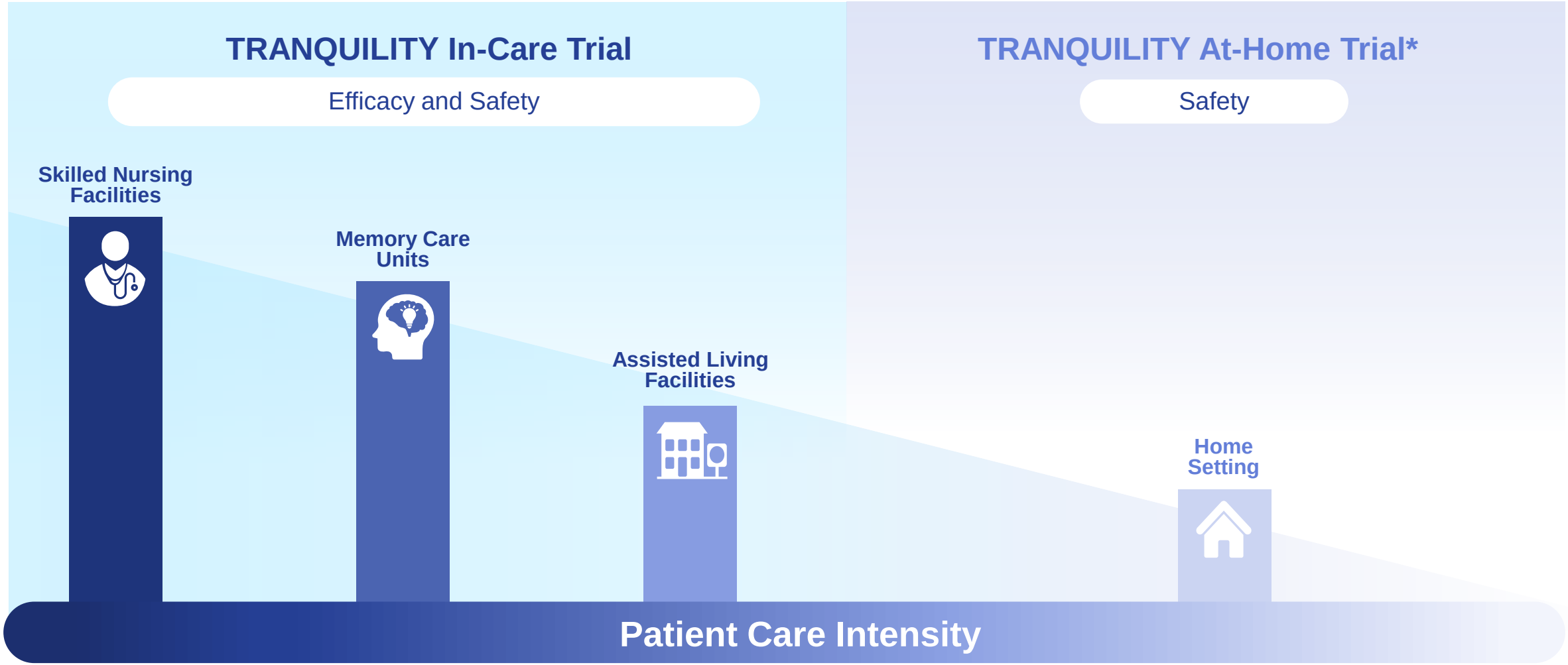
Evaluating BXCL501 for AAD in High to Low Care Settings

Clinical trial strategy designed to maximize potential commercial opportunity across patient locations



*Trial design may be subject to change

Skilled-Nursing Facilities: medical setting for patients with advanced health conditions who receive 24/7 skilled nursing care and medical monitoring
Memory Care Units: medical setting for patients with dementia who receive specialized care for symptom management
Assisted Living Facilities: residential setting for elderly patients who are largely independent but need help with ADLs (bathing, dressing, and other non-medical type assistance)

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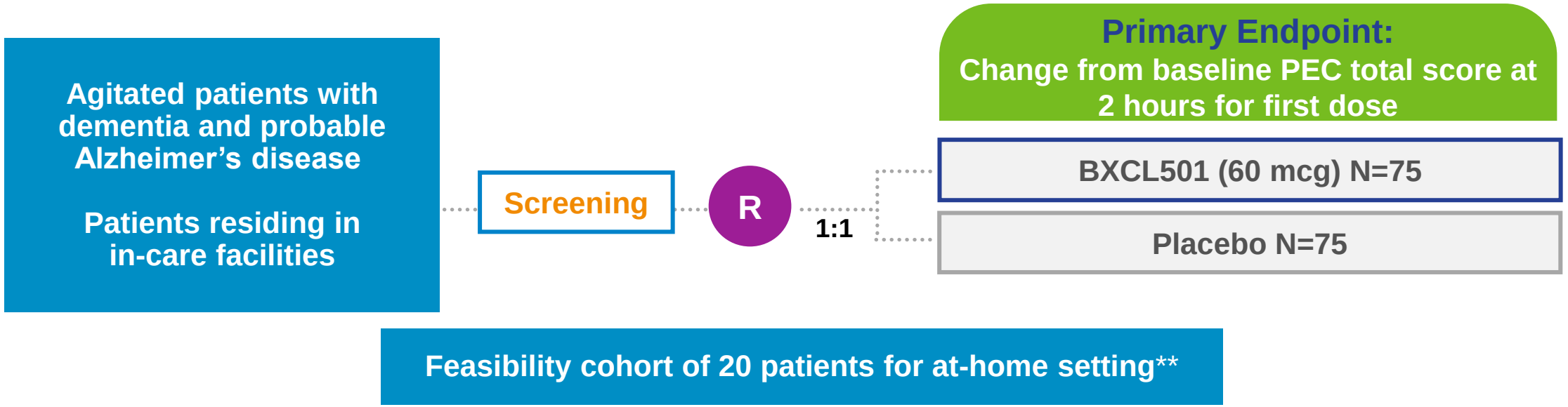
TRANQUILITY In-Care Study Design*



Feasibility cohort of 20 patients for at-home setting**

- **Design:** Randomized, double-blind, placebo-controlled, parallel group trial
- **Power:** Over 80% power
- **Inclusion Criteria**
  - Patients with probable AD (mild, moderate, or severe, MMSE ≤ 25), who experience agitation, and residing in skilled nursing facilities, memory care units, or assisted living facilities
  - Patients with episodes of agitation in the month prior to enrollment
  - PEC total score ≥14 prior to randomization
- **Primary Endpoint:** Change from baseline of PEC total score at 2 hours for **first dose**
- **Study Duration:** 12 weeks with assessment of continued efficacy (up to 3 PECs)

*For illustrative purposes only: protocol under development and trial design may be subject to change. The FDA has not provided feedback on this trial.
** Represents a separate cohort of 20 patients who reside at home in addition to the 150 patients who are in care facilities

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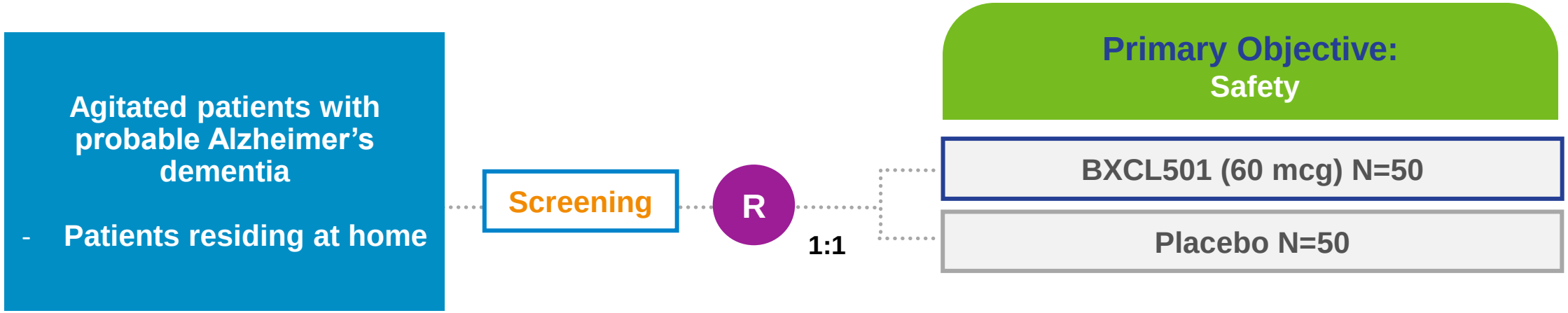
Preliminary TRANQUILITY At-Home Study Design*



- **Study Design:** Randomized, double-blind, placebo-controlled, parallel group trial
- **Primary Objective:** Safety and tolerability of BXCL501 60 mcg
- **Inclusion Criteria**
  - Patients with mild, moderate, or severe probable AD who experience agitation, MMSE ≤ 25
  - Patients with **not more than three episodes of agitation per week** in the month prior to enrollment
  - Patients with caregivers
- **Treatment**
  - BXCL501 60 mcg or placebo administered for agitation in at-home setting

* For illustrative purposes only. Protocol under development and trial design may be subject to change. The FDA has not provided feedback on this trial.

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Thank you!