NOVARTIS

Community Update: FDA Accepts Delpacibart Zotadirsen (del-zota) Biologics License Application for Priority Review in DMD44

September 8, 2026

Dear DMD Community,

We are pleased to announce that the U.S. Food and Drug Administration (FDA) has notified us that the Biologics License Application (BLA) of delpacibart zotadirsen (also known as "del-zota") is sufficiently complete to permit a substantive review. The application has been granted Priority review and will be assessed under the Accelerated Approval pathway.

The Accelerated Approval pathway may allow treatments for serious or life-threatening conditions that address an unmet medical need to be approved based on a surrogate endpoint, while confirmatory studies continue to verify the anticipated clinical benefit

This BLA includes data from the Phase 1/2 EXPLORE44® and EXPLORE44-OLE™ clinical trials.
This application seeks FDA's approval of del-zota as a treatment option for people living with Duchenne muscular dystrophy (DMD) who have a genetic variant that may be treated through exon 44 skipping (DMD44).

A global phase 3 trial (SAFARI44™) is initiated as a confirmatory trial and is intended to further evaluate the long-term safety of del-zota and help confirm its clinical benefit. The study will be conducted outside the United States. You can learn more about the phase 3 trial by visiting the study's page on clinicaltrials.gov.

This important milestone would not have been possible without the participation and dedication of the individuals and families who took part in the EXPLORE44® clinical development program. We extend our sincere gratitude to all trial participants, their caregivers and families, advocacy organizations, investigators, and study teams. Your commitment has helped advance the development of a potential new treatment option for the DMD44 community. We will share further updates as available.

If you have questions about del-zota or the EXPLORE44® or EXPLORE44-OLE™ clinical trials, please speak with your healthcare provider.
Sincerely,
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