Dear Cure SMA,

Thank you for your questions regarding Biogen's announcement on Sept. 23, 2025, that the U.S. Food and Drug Administration (FDA) issued a Complete Response Letter (CRL) for our application for a potential new dose regimen of nusinersen.

We understand and share the community's disappointment regarding this news. We know the community is eagerly awaiting updates, and we remain dedicated to people living with SMA in the U.S.

We are deeply grateful to the study participants, their families, and the international network of study doctors and clinical staff whose dedication has made the nusinersen clinical development program – the longest in spinal muscular atrophy (SMA) to date – possible. We will share additional information as available.

The following responses are being provided to address your questions and are aligned with the press release which you can find here.

Questions & Answers

- *What is a CRL?*
  - A CRL is a message sent from the FDA to a company to indicate that a new drug application is not ready for approval.

- *What additional information has the FDA asked for?*
  - The FDA requested an update to the technical information included in the Chemistry Manufacturing and Controls module of our application for the new dose regimen of nusinersen.
  - The FDA has identified options for resolution, and Biogen is working diligently to resubmit the application based upon readily available information.

- *Is there any impact on the currently approved dose?*
  - This does not impact use or availability of the currently approved dose, which can continue to be used according to the approved product label.

- *Did the CRL list any concerns with the clinical data in the application?*
  - The letter did not cite any issues with the clinical data in the application of the new dose regimen.

- *Does this impact ongoing clinical studies and those participating in the studies?*
  - No. There are no changes to any ongoing clinical studies with nusinersen.

- *How long will it take the FDA to review the resubmission?*
  - We cannot speculate on the FDA's review timeline at this time. The FDA has identified options for resolution, and Biogen is working diligently to resubmit the application based upon readily available information.

Sincerely,
Biogen