August 24, 2022

Community update: Status of VIBRANT-HD, the study of branaplam/LMI070 in Huntington’s Disease

Dear Huntington’s Disease Community:

We are sharing this update to make sure you have the latest information regarding our ongoing clinical trial of branaplam in Huntington’s Disease. Novartis has decided to temporarily suspend dosing of study drug in the Phase 2b VIBRANT-HD study of branaplam in adults with HD. This decision was made because early signs of side effects were seen in some participants in the trial. The recommendation was made by an independent Data Monitoring Committee (DMC), following a regularly planned data review, which revealed findings that suggested possible side effects – specifically that branaplam might be causing peripheral neuropathy, which results from injury to the nerves located outside of the brain and spinal cord. The VIBRANT-HD steering committee, made up of medical professionals and community members, endorsed the decision to temporarily suspend dosing. We would like to emphasize that the study is moving forward with planned assessments. The study was specifically designed with robust monitoring to detect early safety signals and to allow us to make informed decisions for the trial. We will be gathering more data and information in the coming months to better understand these effects and decide on next steps.

We know this is difficult news for the entire HD community, especially for those who are part of the ongoing study. We wish to highlight how grateful we are to the people participating, and their families, and how important it is for those in the study to continue to be assessed. With this participation, the community will add to the body of knowledge about HD, help continue progress toward new treatments, and assist us in making the best decisions regarding the future development of branaplam. The level of engagement of everyone in the HD community is incredible and this provides us all with hope.

It is Novartis’ goal to provide updates when more information becomes available in the coming months. We will continue to work with HD patient organizations to ensure the community has the most up to date information. We understand that clinical trial news can affect everyone in the HD community, including people living with HD and their families, researchers, clinical scientists, and physicians, but we also know that there is great hope in the work being done by so many. We would like to thank the HD community for their input, participation, and continued interest in VIBRANT-HD.

Yours sincerely,

Novartis