Therapeutic Update

Mallinckrodt Pharmaceuticals

Mallinckrodt presented an update on Study VTS301 (the Phase 2b/3 study) outlining the results of the 52-week double-blind phase of the study at several recent scientific and patient advocacy conferences (NNPDF, NPUK, SSIEM, and Michael, Marcia and Christa Parseghian Scientific Conference).

On July 31, 2019, Mallinckrodt was notified by the regulatory agencies in France and the United Kingdom that VTS301 Part C (the open label long-term treatment arm of the study) was suspended following a preliminary determination by those agencies of an unfavorable benefit / risk balance. The German regulatory agency is still evaluating the risk/benefit to determine if patients can continue to be treated. Mallinckrodt disagrees with this determination, and based on our knowledge today, continues to believe there is a positive benefit / risk balance. Importantly, no new safety concerns have been identified in Study VTS301, including the long-term treatment arm or in any other sponsored clinical study or expanded access program.

In order to provide additional explanation about the study results and the action taken by the regulatory agencies, Mallinckrodt will provide a video during the month of October to be shared with the NPC community. In the meantime, VTS301 Part C is ongoing in the US, Germany, Turkey, Singapore and Australia.