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PTC Therapeutics Announces Topline Results of CardinALS Trial of Utreloxastat in ALS Patients

- Study failed to meet primary and secondary efficacy endpoints -

PTC Therapeutics announced that the global Phase 2 placebo-controlled CardinALS study did not meet its primary endpoint of slowing disease progression on the composite ALSFRS-R and mortality analysis. While there was modest numerical benefit recorded on the primary endpoint and correlation of favorable clinical effect with lowering of plasma neurofilament light chain (NfL), a biomarker of neuronal damage, statistical significance was not achieved (p= 0.52). In addition, significance was not achieved on the secondary efficacy endpoints.

"We wish to thank all of the patients, their families and physicians who participated in the CardinALS trial," said Matthew B. Klein, M.D., Chief Executive Officer, PTC Therapeutics. "We are of course disappointed that we were not able to demonstrate treatment efficacy and provide a potential therapy that could address the significant unmet medical need of ALS patients."

Utreloxastat was demonstrated to be safe and well tolerated in the CardinALS trial. However, due to the lack of efficacy and biomarker signal, further development is not planned at this time.

Source: **Press release PTC Therapeutics**

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