To the NBIADA and NBIA community,

First and foremost, thank you for your everlasting advocacy on behalf of all patients and caregivers living with PKAN, and for welcoming our organization to spend time with you and your patient community over the last several years. You have enabled us to genuinely recognize the significant unmet need this community faces with no approved treatment option.

Last week, we were incredibly disappointed and disheartened to report that the Phase 3 FORT Study of fosmetpantotenate in PKAN did not achieve a successful outcome. In our update, we shared that the FORT Study did not achieve its primary or secondary endpoints in the trial, and fosmetpantotenate did not show a benefit when compared to placebo.

After seeing the initial results, our medical and clinical teams worked quickly to understand if there were any age groups, differences in stage of PKAN (for example, classical vs. atypical), or signs that extending treatment for a longer period may increase the chances of responding to fosmetpantotenate. Unfortunately, there was no evidence to suggest that fosmetpantotenate was having a positive impact in any of these additional analyses.

Given these results, we made the exceptionally difficult decision to discontinue further development of fosmetpantotenate. As you might imagine, we are still working through the details of the program, but we anticipate communicating additional decisions to the study sites in the coming weeks.

Our number one priority is working closely with all of the study investigators to establish the appropriate next steps for each of the patients that have been receiving fosmetpantotenate. Consistent with that priority, we will ensure that updates are promptly provided to patients and their families as we make additional decisions regarding the open-label portion of the study. Your respective study sites will have the most up-to-date information as it becomes available, so we encourage you to regularly reach out to them with any questions.

This is a difficult time for everyone who had confidence and hope in the fosmetpantotenate program. The NBIA community has Retrophin’s full commitment to openness; in the future we will be sharing additional results from further analyses of the data. Despite the unsuccessful outcome of the FORT Study, we believe there is valuable knowledge to be shared and we are hopeful that this research may help further the field’s understanding of PKAN and contribute to others’ work in developing potential treatments for this disorder.

Without this community our FORT Study and potential future studies by others would not be possible. Thank you again to NBIADA for your unwavering commitment to making the fosmetpantotenate program possible, and for your continuing advocacy on behalf of all patients and families living with PKAN and other NBIA disorders.

My best,

Eric Dube, CEO, Retrophin