December 18, 2024

Dear SMA Community,

As you may have heard, Biohaven released the results from the RESILIENT SMA clinical trial on 25November2024. https://ir.biohaven.com/node/11026/pdf The results indicated that taldefgrobep alfa did not meet the study's primary endpoint; however, it did show clinically meaningful improvements at all timepoints on the Motor Function measurement-32 scale (MFM-32). Importantly, we observed a significant and clinically meaningful difference in a prespecified smaller group analysis that could be driven by imbalances between treatment arms in some genetic factors (SMN2 copy number, race). Biohaven is further analyzing the data to guide future plans.

What does this mean for the SMA community and what are Biohaven's next steps?

Given that this study is now in an Open-Label Extension (OLE) phase, with all participants on taldefgrobep, we will continue to provide study medication during this phase as we conduct further analyses and engage in discussion with Health Authorities. The OLE phase of the study will remain open. Biohaven plans to engage with the FDA about potential next steps with the study and what, if any, additional analyses and information may be helpful. Biohaven believes that the value of the study remaining open is to continue evaluating the efficacy signals that were observed in clinically relevant and biomarker-defined subgroups.

Biohaven has conducted calls with Investigators/study site personnel to discuss the study results and next steps. Study participants should please talk with their study doctor and team about any questions regarding continuation in the OLE phase of the study. Biohaven plans to keep you informed as we continue to evaluate and analyze all the trial data. We are grateful for the support of the SMA community, and we continue to persevere, with the hope to advance new treatments.

Kind regards,
The Biohaven SMA Team