

October 12, 2026

Dear Brighter Hope Foundation Community,

I'm writing to you today to share an update on our commitment to making the findings from the BMN 401 program available to the broader scientific community, as a follow-up to our letter earlier this summer. We recently shared data from the ENERGY 3 study at the American Society for Bone and Mineral Research (ASBMR) Annual Meeting in Boston. The data were presented by Dr. Weber over the weekend, showing that while BMN 401 increased plasma inorganic pyrophosphate (PPi) and was generally well tolerated, treatment did not lead to improvements in clinical or skeletal outcomes evaluated in the study after 52 weeks of treatment compared to conventional therapy. Below is an overview of the ENERGY 3 data that were presented, and we continue to work toward publishing more of the data in a peer-reviewed journal.

- Results were included from 27 children ages 1 to 12 with ENPP1 deficiency who were assigned at random to two arms of the study: 19 children received BMN 401 treatment (study medicine), and 8 children did not receive the study medicine and instead were treated with the current standard of care. The median age at enrollment was 9.5 years; 48.1% were male and 51.9% were female.
- While there was a statistically significant change from baseline through week 52 in plasma PPi levels between children receiving study drug versus those who did not, the score for Radiographic Global Impression of Change (RGI-C, a measure of whether rickets resolves or worsens over time) showed no significant improvement.
- Rickets Severity Score (RSS) and growth Z-scores (height/body length and weight) did not show improvement in children treated with BMN 401 compared to the children who did not receive study drug.
- BMN 401 was generally well tolerated, with injection-site reactions representing the most common side effect.

We recognize how important it is to share what was learned transparently. This poster presentation is the first of a series of publications planned to share the data from the BMN 401 program. Our hope is that these results will contribute to the scientific community's understanding of ENPP1 deficiency and may help inform future research.

We remain committed to sharing the data responsibly through peer-reviewed scientific forums and to keeping the community informed about future data presentations and scientific publications. If you have any questions, please reach out to the medical information team at BioMarin (medinfo@bmrn.com).

We are deeply grateful to every child and family who participated in the BMN 401 program, as well as to the investigators, healthcare providers and the Brighter Hope Foundation. Thank you for your understanding and for the trust you have placed in us as we work to honor our commitment to transparency and contribute what we have learned to future ENPP1 deficiency research.

Thank you,

A handwritten signature in black ink that reads "Marni Kottle".

Marni Kottle
Chief Corporate Affairs & Patient Advocacy Officer