July 30, 2026

Dear Brighter Hope Foundation Community,

Earlier this year, I had the opportunity to meet some of you at the Family Conference in Orlando. It was an honor to hear your stories and to gain a deeper understanding of what it means for children and adults to live with ENPP1 deficiency. I left the conference with a greater appreciation for the resilience of this community and for the significant need for a medicine to treat the underlying cause of this lifelong condition.

At the time of the Orlando conference, all of us still had great hopes for the BMN 401 program and the ENERGY 3 data. As you know, in May we shared initial results from that study, which demonstrated that the medicine failed to meet one of its two co-primary endpoints. These results left us without a path to regulatory approval for BMN 401. Since then, the BioMarin team has worked to better understand the data. I am writing today to share that after evaluating the data further and assessing other ongoing BMN 401 studies, we have made the decision to discontinue the BMN 401 program. We will be moving to shut down all ongoing trials, including the Expanded Access Program. I want to acknowledge on behalf of BioMarin that we recognize this is extraordinarily difficult news for the Brighter Hope Foundation community.

Our highest priority is ensuring that every person receiving BMN 401 and their caregivers are provided with the information they need to transition off the medicine. BioMarin will work closely with investigators and the care teams of participants in ENERGY, ENERGY 2, ENERGY 3, ADAPT, the Expanded Access Program and the PROPEL registry to support this transition. The specific details of study closure will be discussed directly with each participant and family by the participant's treating physician, who can tailor the transition to meet individual needs.

While our work on the BMN 401 program is coming to an end, we remain committed to communicating with transparency, and to listening and supporting the community through this transition. We are also committed to sharing the key learnings and data generated through this program in a peer-reviewed scientific setting to inform and enable future research for people living with ENPP1 deficiency and GACI-related conditions. We will share more details once the data have been accepted for presentation or publication.

Finally, we will keep the community informed through our ongoing communication with the Brighter Hope Foundation, including at the upcoming Paris family conference. In the meantime, for anything urgent related to a participant's care, please reach out to your treating physician. For other questions about the program or this transition, please contact the medical information team at BioMarin (medinfo@bmrn.com).

To every child, adult, family member, investigator, healthcare provider and the Brighter Hope Foundation, thank you for participating in this program. This isn't the outcome any of us hoped for, and we are grateful to all of you for your support.

Thank you,

Marni Kottle
Chief Corporate Affairs & Patient Advocacy Officer