BioMarin's BMN 255 Primary Hyperoxaluria Type1 Clinical Development Program: An Update for the PH1 Community.

About BioMarin

BioMarin is a global pharmaceutical company with 7 approved therapies and more than 20 years of experience in developing innovative medicines for rare genetic conditions. Each investigative medicine we pursue is guided by a fundamental understanding of the genetics and underlying biology of the condition it will address. We are committed to providing a big impact with a focus on rare disorders.

About the Biology of Primary Hyperoxaluria Type 1

Primary hyperoxaluria type1 (PH1) is rare and inherited. It is caused by a genetic variation leading to glyoxylate aminotranferase (AGT) enzyme that is either missing, low or not working properly. AGT is responsible for metabolizing (breaking down) a molecule called glyoxylate in the liver. When this molecule cannot be properly broken down by AGT, it is converted to oxalate, a molecule that the body cannot break down. Although oxalate can be removed from the body in urine, individuals with PH1 accumulate (or build up) oxalate more quickly than it can be removed. This causes a buildup in the body, particularly within the kidney, leading to kidney damage. The more damaged the kidneys are, the less oxalate they can remove. The less they can remove, the more it builds up and further damages the kidneys. If not eliminated properly, build up of oxalate can also lead to damage to bones, heart, retina and skin.

About BMN 255

BMN 255 is the name given to the investigational therapy BioMarin is researching for the potential treatment of PH1. BMN 255 is an investigational small molecule designed to be taken by mouth and aims to reduce oxalate build up. BMN 255 is designed to block the production of glyoxylate in the liver. BMN 255 is being researched to understand if is safe in humans and if it can block production of glyoxylate to reduce the amount of oxalate that is made, and if the excess can then be removed by the kidney. BMN 255 will also be tested to see if the reduction of oxalate accumulation can reduce kidney damage that occurs in those living with PH1.

BMN 255 is currently being tested in a Phase 1 study in healthy volunteers (those without PH1), aiming to select the appropriate dose. Selection of this dose is planned for the second half of 2022. As an investigational compound, BMN 255 has not been determined to be safe or effective or approved for use outside of a clinical trial.

For additional information:

- For inquiries or to provide feedback from advocacy organizations, please contact patientadvocacy@bmrn.com
- Contact BioMarin Medical Information at medinfo@bmrn.com