Biomarin commences BMN 701 Phase 1/2 trial for treatment of Pompe disease

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BioMarin Pharmaceutical Inc. announced today that it has initiated a Phase 1/2 trial for BMN 701, a novel fusion protein of insulin-like growth factor 2 and acid alpha glucosidase (IGF2-GAA) in development for the treatment of Pompe disease.

"We have a strong track record of quickly developing enzyme replacement therapies for unmet medical needs and expect to leverage our clinical and regulatory experience and manufacturing know-how in the development of BMN 701," said Jean-Jacques Bienaime, Chief Executive Officer of BioMarin. "There is a significant amount of interest in the medical community for a more effective treatment option for late-onset Pompe disease, and we believe, based on in vitro and in vivo nonclinical studies, that using our proprietary Glycosylation Independent Lysosomal Targeting, or GILT technology, BMN 701 has the potential to deliver more enzyme to lysosomes compared to traditional mannose-6-phosphate targeted approaches."

The Phase 1/2 trial is an open-label study to evaluate the safety, tolerability, pharmacokinetics, pharmacodynamic and clinical activity of BMN 701 administered as an intravenous infusion every two weeks at doses of 5 mg/kg, 10 mg/kg and 20 mg/kg. The company expects to enroll approximately 30 patients between the ages of 13 and 65 years old with late-onset Pompe disease for a treatment period of 24 weeks.

SOURCE BioMarin Pharmaceutical Inc.