BioSpectrum the business of Bio & Health Sciences

FDA grants fast track designation to genzyme

28 April 2015 | News | By BioSpectrum Bureau

FDA grants fast track designation to genzyme

Fabry disease is a rare lysosomal storage disorder that results in abnormal tissue deposits of a particular fatty substance (called globotriaosylceramide, also referred to as GL-3 or Gb3) throughout the body. GZ/SAR402671 is a glucosylceramide synthase inhibitor that blocks the formation of glucosylceramide (GL-1), a key intermediate in the synthesis of GL-3.

FDA's Fast Track Drug Development Program is designed to facilitate frequent interactions with the FDA review team to expedite the clinical development and review of a New Drug Application (NDA) for medicines with the potential to treat serious or life-threatening conditions and address unmet medical needs for such diseases or conditions.

It also provides the opportunity to submit sections of an NDA on a rolling basis before a sponsor submits the complete application.

Genzyme is currently enrolling patients in its phase 2a trial of GZ/SAR402671, and plans to enroll nine treatment-naÃ¯ve male adult patients with Fabry disease in this international, multicenter study.

"Becoming a Fast Track Program is an important milestone and we appreciate this designation from FDA," said Genzyme's acting head of rare diseases, Dr Richard Peters. "We look forward to learning more about this small molecule, with the goal of providing more therapeutic options to the Fabry community as quickly as possible."