

References

I-178

1. Buton BK, Balwani M, Feillet F, et al. A Phase 3 Trial of Sebelipase Alfa in Lysosomal Acid Lipase Deficiency. *N Engl J Med.* 2015;1010-20.
2. Erwin AL. The role of sebelipase alfa in the treatment of lysosomal acid lipase deficiency. *Ther Adv Gastroenterol.* 2017;553-562.
3. MICROMEDEX®SOLUTIONS Compendia.2020. Sebelipase alfa.
4. Clinical Pharmacology Compendia. [database online]. Tampa FL: Gold Standard, Inc. Sebelipase alfa.2020.
5. KANUMA (sebelipase alfa) Intravenous Infusion [package insert]. Alexion Pharmaceuticals Inc. New Haven, CT, 01/2015. Revised 12/2015.
6. Jones SA, Rojas-Caro S, Quinn AG, Friedman M, Marulkar S, Ezgu F, et al. Survival in infants treated with sebelipase alfa for lysosomal acid lipase deficiency: an open-label, multicenter, dose-escalation study. *Orphanet Journal of Rare Diseases.* 2017; 12(25):1-12.
7. MCG™ Care Guidelines, 22nd edition, 2018, Home Infusion Therapy, CMT: CMT-0009(SR).
8. Jones, S. A., Valayannopoulos, V., Schneider, E., et al. Rapid progression and mortality of lysosomal acid lipase deficiency presenting in infants. *Genet Med.* 2016 18(5), 452-458.
9. Patterson, C., So, S., DeAngelis, M., Ghent, E., Southmayd, D., & Carpenter, C. Physical activity experiences in children post–liver transplant: Developing a foundation for rehabilitation interventions. *Pediatr Transplant.* 2018. 22(4), e13179.