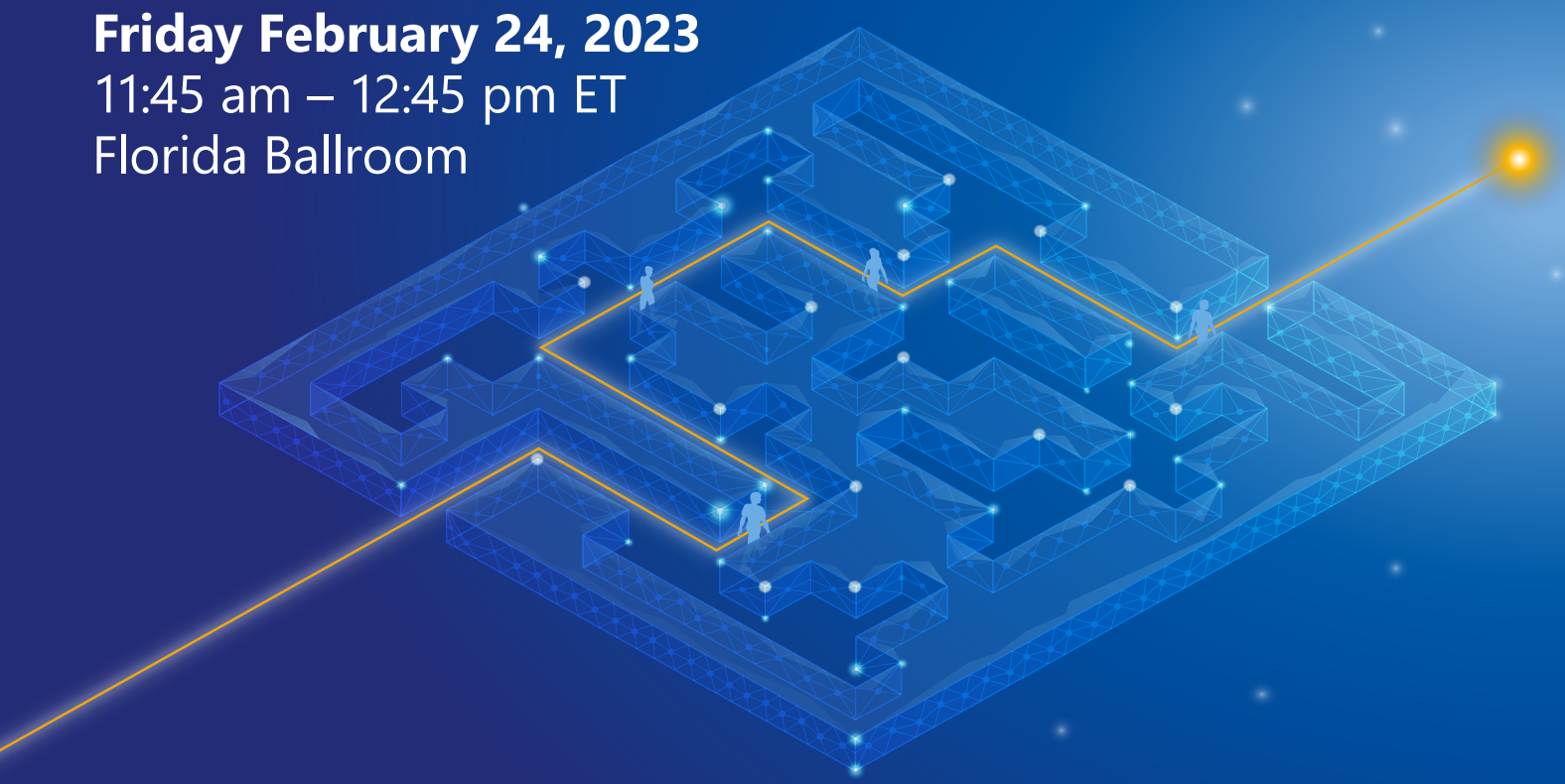
Invitation to attend an Industry Satellite Symposium sponsored by Amicus Therapeutics

Challenges in delivering ERT in lysosomal disorders: special considerations in LOPD

Friday February 24, 2023
11:45 am – 12:45 pm ET
Florida Ballroom



This thought-provoking symposium, hosted by **Dr Barry Byrne** (University of Florida) and **Professor Mark Sands** (Washington University School of Medicine), will discuss the most common clinical considerations associated with delivering enzyme replacement therapies (ERTs) in lysosomal disorders (LDs).

LDs result from deficient enzyme activity, leading to progressive substrate accumulation. Some LDs can be treated with infusion of an exogenous enzyme source, known as ERT. However, there are potential clinical and mechanistic considerations associated with infusion-based treatment. These include biodistribution and variable organ response,[1] the need for high and frequent dosing[1–3] that may impact on patients' quality of life,[4–6] and the potential for an immune response against the recombinant enzyme.[7,8]

In this symposium, Dr Byrne will provide an overview of enzyme dysfunction in LDs before delving into the clinical considerations associated with infusion-based treatments. A patient and a caregiver will also provide their valuable perspectives on living with and caring for a person with an LD. Using late-onset Pompe disease (LOPD) as a case study, Professor Sands will highlight the mechanistic considerations of delivering a recombinant human enzyme to target tissues.

The event will close with an **interactive Q&A**, moderated by Dr Byrne, giving you the opportunity to have your questions answered live by our expert panel.

We look forward to seeing you there.

Barry Byrne MD, PhD
Associate Professor of Pediatrics
Director of Powell Gene Therapy Center
University of Florida

Mark Sands PhD
Professor of Medicine
Department of Genetics
Washington University School of Medicine in St Louis

1. Desnick RJ *et al. Annu Rev Genomics Hum Genet* 2012;3:307–35; 2. de Fost M *et al. Blood* 2006;108:830–5; 3. Wenstrup RJ *et al. Bone Miner Res* 2007;22:119–26; 4. Hughes D *et al. Brit J Nurs* 2007;16:1386–9; 5. Ratko TA *et al.* Enzyme-Replacement Therapies for Lysosomal Storage Diseases. Rockville, MD: Agency for Healthcare Research and Quality (US) 2013. Available at: https://www.ncbi.nlm.nih.gov/books/NBK117223/ (accessed December 2022); 6. Patel N *et al.* The patient and clinician point of view: living with late-onset Pompe disease. WORLD*Symposium.* San Diego, CA, February 5–8, 2018; poster 298; 7. Brooks DA *et al. Trends Mol Med* 2003;450–3; 8. de Vries JM *et al. Genetic Med* 2017;19:90–7.

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