An R Shiny-based, Interactive Visualization Tool to Exploit Clinical Trial and Real World Data: Application to Acid Sphingomyelinase Deficiency and Olipudase Alfa ERT

Shayne Watson1,3, Chanchala Kaddi1, Jason Williams2, Edouard Ribes1, Jeffrey S. Barrett1, Karim Azer1

1Translational Informatics, TMED, Sanofi, Bridgewater, NJ; 2TMCP, Sanofi, Bridgewater, NJ; 3Drexel University, Philadelphia, PA

Objective: Acid Sphingomyelinase Deficiency (ASMD: Niemann-Pick disease types A and B), is a rare lysosomal storage disease resulting in sphingomyelin accumulation. Olipudase alfa, an ERT for ASMD, is currently under development. Natural history registries and clinical studies evaluating olipudase alfa encompass heterogeneous data types, including pharmacokinetic, pulmonary, and lab measurements. We have developed a tool to facilitate integrative data analysis, QSP model-based data assessment, and interdisciplinary communication.

Methods: The visualization tool was developed using R Shiny, a web-based program, which allows the tool to be hosted and distributed using an HPC environment. The R Shiny platform is ideal because of powerful R visualization packages and the ease of designing custom GUI layouts. A QSP simulator was also developed based on R Shiny, allowing for concurrent evaluation of model simulations and clinical data. Design criteria were selected through input from the clinical and modeling teams at Sanofi working on late-stage olipudase alfa development.

Results: The tool enables real-time interactive visualization of data across four clinical studies and two natural history registries. Key features include allowing the user to select studies, data types, and features of interest (Figure 1A), and visualization options, such as comparing data within or between studies (Figure 1B).

Figure 1: (A) The tool features customizable visualizations such as simultaneously viewing different data types, and examining subject-specific (orange) or collective (blue) data. (B) The tool enables rapid assessment of a large number of potential PD markers to determine those most likely to correlate with olipudase alfa concentrations.

Conclusion: The tool enables interactive and flexible visualization, including cross-comparisons of PK, PD, clinical response, and model simulations, thereby facilitating data interpretation and communication between clinical and modeling teams. Future development will focus on integrating the tool into an evolving data analysis, visualization, and simulation platform for lysosomal storage diseases.