



### Supplemental Digital Content 3

#### Authors and affiliations:

Sarah P. Young<sup>a</sup>, PhD, Haoyue Zhang<sup>a</sup>, PhD, Deyanira Corzo<sup>b,c</sup>, MD, Beth L. Thurberg<sup>b</sup> MD  
PhD, Deeksha Bali<sup>a</sup>, PhD, Priya S. Kishnani<sup>a</sup>, MD, David S. Millington<sup>a</sup>, PhD

<sup>a</sup>Duke University Medical Center, <sup>b</sup>Genzyme Corporation, <sup>c</sup>Current affiliation: Millennium Pharmaceuticals Inc., Cambridge, MA

Funding for this study was provided by Genzyme Corporation, Cambridge, MA.