

## Tennessee Rare Disease Advisory Committee (RDAC)

*Our mission is to improve the quality of life of individuals affected by rare diseases in Tennessee through collaboration, education, support and advocacy.*

Agenda March 27th, 2024

<b>8:00-8:05 CT</b>	Call to order and Welcome	Gillian W. Hooker, Interim RDAC Chair
<b>8:05-8:25</b>	Patient Story	Amanda Sinard, MPS1 Parent
<b>8:25-8:40</b>	Update: Rare Disease Legislation	Gillian W. Hooker, Interim RDAC Chair Terry Jo Bichell, RDAC Vice Chair
<b>8:40-8:55</b>	Updates in the Duchenne Muscular Dystrophy Treatment Space	Kathrin Kucharski, PharmD Sarepta Therapeutics
<b>8:55-9:10</b>	Bota-Vec Gene Therapy for XLRP	Leigh A. Denny, PharmD J&J Innovative Medicine
<b>9:10-9:20</b>	Cell and Gene Therapy (CGT) Access Model	Dr. Lametra Scott Breaking The Sickle Cell Cycle Foundation, Inc.
<b>9:20-9:25</b>	Pharmacy Advisory Committee Update	Reginald French, RDAC Member
	DUR Update	Tracey Lovett, RDAC Member
	Department of Intellectual and Developmental Disabilities	Terry Jo Bichell, RDAC Vice Chair
	Newborn Screening	Rizwan Hamid, RDAC Interim Secretary
<b>9:25-9:30 Closing</b>	Review of Action Items and Agenda Items for May Meeting	Gillian Hooker, RDAC Interim Chair