

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.

Members In Attendance: Terry Jo Bichell, Gillian Hooker, Chip Chambers, Tracey Lovett

Others In Attendance Arthur Beudet (Luna Genetics), Ashley McMinn (Vanderbilt), Gene McCarty (Reata), Abby Trotter (Life Science TN), Holly Murphy (Acadia), John Fox (illumina), John Omick (Travere), Kemi Olabisi (Acadia), Kerri Engebrecht (NORD), Roya Mostafavi (Le Bonheur), Todd Barber (UT), Lora Underwood (TennCare)

Call to order and Welcome	Terry Jo Bichell, RDAC Vice-Chair	
An Update on Single Cell Genome Sequencing	<p>Art Beudet, Luna Genetics, diagnostic company based in Texas</p> <p>Novel prenatal test - non-invasive, cell-based testing. Different from NIPT in that they are isolating individual fetal cells - cleaner signal to background</p> <p>Validation studies - comparing to Amnio or CVS- so far, no false positives or negatives - mosaicism is possible</p> <p>Launching a non-invasive whole genome sequencing test that detects both inherited disease and de novo point mutations</p> <p>Have submitted for ARPA-H funding</p> <p>Opens possibility of In Utero treatment - e.g. Lysosomal Storage Diseases</p> <p>Goal is to get test down to less than \$1500 per test and offered to all women</p> <p>Optimal timing - as early as 9 weeks, can be done up to 15 weeks</p>	

	Will likely replace NIPT test and may replace some or most of newborn screening	
Update on TN Diagnostics Network Project	<p>Ashley McMinn, Project Manager Rizwan Hamid, RDAC Member</p> <p>A few updates to share with the group -</p> <ol style="list-style-type: none"> 1) Send out surveys to all potential contacts - state genetics advisory committee, TNGCA, NORD, Research Innovation Center at Vanderbilt - Thinking about Physician to physician consultation service - would they be willing to fund a project like this 2) Met with IT group at UT last week who will help develop secure RedCap portal - to use for referrals to project - now connected with individuals there 3) Getting help w/ folks from DADA2 to develop survey <p>Question about coverage of testing</p> <ul style="list-style-type: none"> - Should we collect information about preferences for who orders the tests? And or insurance considerations? <p>For a first pass, it's really becoming a referral network to specialist providers</p>	
Considerations for treatment coverage criteria in rare disease	<p>Holly Murphy Acadia Pharmaceuticals Inc</p> <p>And Kemi Olabisi</p> <p>Conference in Nashville - ARTEA Crossroads conference - 22 state medicaid programs represented</p> <p>Also had caregiver panels</p> <p>Came to RDAC to share frustrations that parents shared during the panel, it got her</p>	<p>Aug 10th is the upcoming PAC meeting</p> <p>If manufacturers would like to speak on their drugs, they must submit requests prior to the meeting</p>

	<p>thinking about things that could be done here TN in order to make things available in TN</p> <p>The road to Dx may be difficult, but once there is a dx, it can be really disappointing that there aren't actually treatments on the other side and/or those treatments aren't covered by insurance for them.</p> <p>Like offering you a balloon, and then taking it away</p> <p>Q: when a medication is approved - wouldn't that apply to anyone with that disorder, without clinical criteria - why should there be additional assessment required for coverage?</p> <p>Chip - weighed in on how companies make decisions on coverage - thinks the answer is better dialog between pharma, payer and FDA.</p> <p>Need to start thinking about value based care -</p> <p>How do we start this dialog?</p> <p>Start with medicaid - convince the state insurer - that they need to be meeting with companies that are producing the drug to confer on coverage criteria</p> <p>Falls back to the manufacturer to work with the medicaid program - drugs are very expensive - the rebates matter</p> <p>The FDA often limits treatment to people who are symptomatic</p> <p>Important resources within a state: The clinicians who treat this condition - how can we bridge between TennCare and the clinicians in the state - there are a number of different work streams</p> <p>TennCare has an industry day 1x per month for manufacturers are asked to present on their drug - can invite clinicians to that.</p>	
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RDAC Succession Planning	Terry Jo Bichell, RDAC Vice-Chair	
Brief Updates to circulated by email	Reginald French, RDAC Member	
Pharmacy Advisory Committee Update (see circulated notes)	Tracey Lovett, RDAC Member	
DUR Update	Terry Jo Bichell, RDAC Vice Chair	
Department of Intellectual and Developmental Disabilities		
Review of Action Items and Agenda Items for December Meeting	Scott Strome, RDAC Chair	