

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.

Minutes: July 28th, 2021

RDAC Members In Attendance: Scott Strome, Terry Jo Bichell, Chip Chambers, Megan Crow, Rizwan Hamid, Reginald French, Lora Underwood, Suzanne Jackowski, Clay Callison

Others In Attendance: Ray McIntyre, Kristen Hatcher, Todd Barber, Kim Black

	<i>Notes</i>	<i>Action Items</i>
Call to order and Welcome	Scott Stromel, RDAC Chair	
Rare Disease Patient Perspective	<p>Kristin Hatcher</p> <p>Diagnosed with alpha-1-antitrypsin She has lost family members to this conditions.</p> <p>Infants diagnosed can be treated with Prolastin and have a chance at a much better course.</p> <p>Her family experienced significant financial impact of the illness in the family.</p> <p>Her father was first diagnosed when she was mid 30's which led to her diagnosis, around the time her symptoms were becoming significant.</p> <p>By the time her father was diagnosed, there was significant lung damage and led to his early death.</p> <p>She is hopeful medications will keep her lungs healthier longer and lengthen her lifespan.</p> <p>Her dad is a ZZ - most severe form.</p> <p>Scott Strome - highlighted that the diagnosis is for many one of the biggest barrier. Asked about insurance coverage for medication.</p> <p>Prolastin is 10K/month, usually covered by insurance, but you need to go through steps first. Her mother stayed in a job she hated to maintain coverage - everytime she changed insurance, they had to requalify for medication and he was frequently hospitalized in the ICU. 8 year uphill battle with insurance.</p> <p>For Krisin, she wishes medicine could be made available more prophylactically, rather than waiting until she is sicker.</p>	

	<p>Also discussed the challenges of family member testing - some are afraid of the government getting their DNA, others are worried about costs. And their doctors are not offering genetic testing</p> <p>Chip Chambers - Asked about clinical trials. She said she does know of an upcoming trial at U of SC .</p> <p>Scott Strome - asked about genetic testing - she was tested at U of SC. once they found the specialists there.</p>	
<p>Survey Update</p>	<p>Kim Stephens, RDAC Member Terry Jo Bichell, RDAC Vice Chair</p> <p>Currently at 64 total responses to the survey. Would still like to draw in more responses, more geographic diversity from TN. 40 diseases represented.</p> <p>Note that the data in this survey will inform the TN UDN network idea.</p> <p>Rizwan suggested that he circulate to clinicians to forward to Vanderbilt patients - while they are waiting for doctor, they can be invited to complete the survey. He can bring this up in the August Genetics advisory committee meeting on Aug 19th. Folks from ETSU, Knoxville</p> <p>J. Woodruff, as a part of his rotation at UT w. Dr. Strome will provide additional support.</p> <p>For reference, the survey is on our website</p>	<p>All - Continue to share survey broadly</p> <p>R. Hamid will share with the genetics advisory committee</p> <p>S. Strome, J. Woodruff will help circulate at UT Knoxville.</p> <p>R. French will share with partners to recruit for survey.</p> <p>Continue to push the survey out through NORD rare action network list</p>
<p>TN Undiagnosed Disease Network update</p>	<p>Terry Jo Bichell, RDAC Member Rizwan Hamid, RDAC Member Scott Strome, RDAC Member</p> <p>Two areas of need were addressed in the subgroup meeting:</p> <ol style="list-style-type: none"> 1) There is a need to promote access to genomic testing to help patients get to right specialist from the get go. 2) Many patients and physicians don't know how to access rare disease specialists in the state <p>Need to discuss funding of access to genomic testing - via private insurance and public funds.</p> <p>Also need access to specialists/experts who can do a deep dive and connect the dots in the data to make the diagnosis.</p> <p>Drawing a vision of a network, following up on testing to connect patients to the right physicians- perhaps centers of excellence, having 3 or 4 centers in TN that receive funding from the state to support efforts.</p> <p>Could be justified with cost savings of cutting short a diagnostic odyssey. Drawing on data to show that additional referrals and</p>	<p>Subgroup will review one page working document developed by Rizwan</p> <p>We will work to schedule the next meeting, using the strawman document to set the agenda</p>

	<p>evaluations can be avoided when a diagnosis is made.</p> <p>Rare disease eval = 16-17K, vs cost of care >300K for those undiagnosed.</p> <p>Vision - work toward a scenario with public and private partnership from industry, academics, payers to build a network where people can be connected. Could use the health departments, could use the UT network.</p> <p>Circulated a one page strawman to the working group to evaluate.</p> <p>Rizwan Hamid cited the UDN as a trial over the last 6 years</p> <p>Nuts and bolts - good meeting of the working group</p> <p>Thinking through technical aspects of what would be involved.</p> <p>Concert/ G.Hooker has been involved with similar proposals in the past with payers and can support that part of the strategy.</p> <p>Two tiered - 1) Sequencing based on panels that we have available, 2) deeper diagnostics.</p> <p>Thinking through an argument to be made to the TN state legislature.</p>	
<p>Drug Utilization Review Committee Background & Opportunities</p>	<p>Ray McIntire, Director of Pharmacy Operations</p> <p>Provided brief overview of what DUR program in TennCare is about - started with a federal law- OBRA '90. It was the first law that required patient consultation.</p> <p>Also require creation of program to monitor drug utilization at the state level. DUR committee includes pharmacists and physicians, not more than 51%</p> <p>ProDUR - review that takes place before a prescription is filled. When a claim is submitted, there are ProDUR edits that are run - e.g. adverse events, early refill edits,</p> <p>Concurrent DUR - less common now- this was intended to happen as the prescription is filling</p> <p>RetroDUR- periodic review of pharmacy claims - looking at therapeutic appropriateness, adverse events, medically unnecessary care, fraud waste and abuse - there's a lot of this reviewed by the committee.</p> <p>Have made significant progress with hydrocodone, for example.</p> <p>Analyses of top prescribers, payments, utilization.</p> <p>OptumRx is TennCare's PBM. They are required to review 800 member profiles each month.</p> <p>They also do educational outreach. The PBM must produce and mail</p>	<p>Schedule follow up discussion with Dr. McIntyre And L. Underwood.</p>

	<p>educational letters based on topics that come from the DUR board. Eg. FDA warning about Ambien at a high dose or using Simvastatin and citalopram.</p> <p>Next DUR meeting is October 12, 2021 - RDAC members are welcome to attend and present.</p> <p>R. Hamid - asked about medications that metabolic patients need, that get classified as supplements, the frustrating part is that those medications get rejected - E.g. arginine for urea cycle disorders. Sometimes they hospitalize patients just to get them treatment.</p> <p>C. Callison - also struggling with CFTR patients</p>	
Pharmacy Advisory Committee Update & Planning for upcoming meeting	<p>Reginald French, RDAC Member Lora Underwood, RDAC Member</p>	Everyone review upcoming PAC agenda and share thoughts with R. French
Newborn Screening / Genetics Advisory Committee Updates	<p>Rizwan Hamid, RDAC Member</p> <p>Not discussed due to time constraints</p>	
Department of Intellectual and Developmental Disabilities	<p>Terry Jo Bichell, RDAC Vice Chair</p> <p>Will be meeting next month</p>	
Review of Action Items and Agenda Items for December Meeting	<p>Scott Strome, Chair, TN RDAC</p>	