

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.

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

Others In Attendance: Abby Trotter, Todd Barber

Call to order and Welcome	Scott Stromel, RDAC Chair	
Rare Disease Patient Perspective	<p>Chip Chambers, RDAC Member</p> <p>Shared the story of his 15 year diagnostic odyssey</p> <p>2 year old daughter - started in dermatology Concern for lymphoma - moved to heme path Moved to rheumatology Tried lots of drugs, had some success with a leprosy drug really just by chance She would have flares every couple of years At 13, they were on a max dose - and other things started happening Went to Mayo - did figure out that she had a mild immunodeficiency in addition to cutaneous vasculitis Moved to considering bone marrow transplant - two major centers offered to do transplant,</p> <p>10 year old son developed a nodule - debate over whether it's similar - determined that it was</p> <p>That moved them to exome sequencing</p> <p>Head of immunology at Mayo- posted the case to the clinical immunology blog. A man in Finland - said to check for ADA2 deficiency.</p> <p>Early 2014 - two articles in NEJM - described DADA2 - those articles published at same time as the samples came back from Finland with no detectable Adenosine Deaminase.</p> <p>Dan Kastner at NIH - following a cohort of patients</p> <p>In Israel- cohort of people with condition -"Georgian Pan" -</p> <p>TNF inhibitors hit the market - - they gave it to a boy in</p>	<p>Schedule a follow up meeting to discuss Center of Excellence idea and how RDAC can support this - TJ Bichell (Include R. Hamid, C.Chambers, S. Strome, A. Trotter, T. Barber, G. Hooker)</p>

	<p>the hospital with Georgian Pan and it worked.</p> <p>Even with the best access in the country - it was a long time coming to a diagnosis.</p> <p>Question about coverage for treatment - Humira/Enbrel - Still not FDA approved for this indication, denied by insurance companies. @ NIH- you get a 6 month supply of drug while you battle insurance company - in England - there is no coverage - India has biosimilars</p> <p>Question about carrier screening - Screening neonates - in children in the NICU - whole genome sequencing at birth - diagnosing a number of new conditions and intervening early - they would love to have DADA2 screened for prenatally or neonatally</p> <p>R. Hamid - suggested that we need broader policies for exome sequencing in TN</p> <p>Also need centers of excellence for rare and undiagnosed disease -</p> <p>S. Strome -we should combine efforts - find partners in East TN - potentially as well- like a virtual tumor board, but for rare disease</p> <p>Mentioned project with Regeneron - goal to sequence 100K children - enriched for populations under-represented in research</p> <p>S. Strome cited shortage of genetic counselors within institutions</p> <p>C. Chambers - if the state of TN were to do something like this- it would be ground breaking.</p> <p>A.Trotter is connected to other groups looking into biomarker testing that she could pull in.</p>	
<p>Press Release Response and Survey Update</p>	<p>Main thing - survey needs to be circulated more broadly</p> <p>Not currently a diverse population, only 54 total responses- showed break down by ethnicity and race</p> <p>Diverse geographical distribution, diverse age distribution, 36 different disorders represented - childhood neurodevelopmental disorders</p> <p>S. Strome will share a letter with the link to send out more broadly.</p> <p>Tj Bichell has started a draft white paper. Discussion of white paper and where we are going with these inputs - ideas from the RDAC, survey, other research, and learnings from the state about opportunities to support</p>	<p>All - circulate survey more broadly</p> <p>Tj Bichell - draft a letter for RDAC members to circulate more broadly</p> <p>All- consider target audience for white paper and what message we can bring</p>

	<p>organizations supporting patients with Rare Disease in TN</p> <p>T. Bichell mentioned Every Life Foundation document that will also inform the document</p> <p>S. Strome - wants to also have discussion of who is our audience and what are we trying to convey - What is the question and who is the audience</p> <p>Consider posting references on the TNRDAC website</p>	
<p>RDAC Communications (Slack, Email)</p>	<p>We will continue to use slack for targeted messaging, agenda will be circulated by email to all members.</p>	
<p>Pharmacy Advisory Committee Update & Planning</p>	<p>Last advisory committee on May 13th - one rare disease drug, Dojolvi that was discussed and approved for patients with VLCAD</p> <p>Next Meeting is August - if not anything on there related to treatment of rare disease.</p> <p>When it comes to new medications - when a new NDC pops into PBM system - identify a new drug - gets automatically reviewed.</p> <p>What does the PAC do with off-label use of drugs. Those drugs will require PA - for off-label use, will get denied on first attempt, then go to appeals - bring in case studies, etc - could be eligible for approval under appeal.</p> <p>TennCare webpage has listings of drugs and whether they require PA .</p> <p>Could we consider whether RDAC should have a role in PAs - most pharmacy committees might not be able to approve off-label drug as appropriate or not - Can RDAC support medical directors in areas of rare disease?</p> <p>Could we share our recommendations for approval of rare disease treatments? E.g. for Dojolvi, could we make a statement about recommendation.</p> <p>A.Trotter asked whether we could consider moving our meeting to give the RDAC more time to review the agenda to plan on whether we will speak at the meeting</p>	<p>L. Underwood will send finalized agenda with a list of drugs - all RDAC members will review list of drugs</p>
<p>Drug Utilization Review Committee Background & Opportunities</p>	<p>Dr. McIntire was unable to make this meeting and we will reschedule for July.</p> <p>Megan has asked if anyone can attend the DUR meeting on her behalf - Chip Chambers will attend on her behalf.</p>	<p>Add Dr. McIntire to the agenda for the July Call</p> <p>Chip Chambers will attend DUR meeting on Megan's behalf.</p>

<p>Newborn Screening / Genetics Advisory Committee Updates</p>	<p>Rizwan Hamid, RDAC Member</p> <p>Nothing new to report from previous meeting</p>	
<p>Department of Intellectual and Developmental Disabilities</p>	<p>Terry Jo Bichell, RDAC Vice Chair</p> <p>Brief report - They are discussing Katie Becket - more than 600 kids enrolled in part B, new accreditation for early intervention providers</p> <p>COVID - related activities</p> <p>They make live recommendations - add into minutes - realtime recommendations</p> <ul style="list-style-type: none"> - Make sure that families could get info about opening after COVID - DIDD would continue to support providers getting resources on COVID vaccines and protect equipment - Make sure that behavioral health is prominent and well funded. 	
<p>Review of Action Items and Agenda Items for December Meeting</p>	<p>Scott Strome, Chair, TN RDAC</p>	