

# 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 May 26th, 2021

RDAC Members in Attendance: Terry Jo Bichell, Suzanne Jackowski, Kim Stephens, Lora Underwood, Reginald French, Clay Callison

Other Attendees: Abby Trotter, Aaron Young, Brian Shank, Kate Segal, Todd Barber

	<i>Notes</i>	<i>Action Items</i>
<b>Call to order and Welcome</b>	<p><b>Terry Jo Bichell, RDAC Vice Chair</b> Dr. Bichell called the meeting to order as Dr. Strome is out this week.</p> <p>RDAC members introduced themselves.</p>	
<p><b>Rare Disease Patient Perspective</b></p> <p><b>Press Release Response and Survey Update</b></p>	<p><b>Chip Chambers, RDAC Member</b></p> <p>Dr. Chambers was out for this meeting, so Dr. Bichell offered a patient story regarding her own son who is diagnosed with Angelman Syndrome, a rare genetic neurodevelopmental disorder. Discussed challenges around getting a diagnosis, then setting out to find a treatment for what was then an untreatable disease. Recently life-changing treatments have been discovered and are now in clinical trials. There have been challenges finding medical caregivers, especially when he turned 18. It is very difficult to find primary care for adults on TennCare and especially for those with rare diseases. As a nurse and neuroscientist, Dr. Bichell has worked with pharma companies, academic research, and patient advocacy organizations and there are now several targeted treatments that are coming along. Her son graduated from high school yesterday at the age of 22 and will need lifelong care.</p> <p><b>Abby Trotter, LifeScience TN</b> <b>Kim Stephens, RDAC Member</b></p> <p>Kim mentioned 34 survey responses so far. Stated that the uptick of the survey responses came after the press release.</p>	<p><b>Consider second press release</b></p> <p><b>Consider adding language to the website that would indicate how people can attend the meeting and/or</b></p>

	<p>Abby Trotter gave a report on the press release. There will be a new release that is focused on the survey to help get more responses. We are also putting together an op ed.</p> <p>Abby encouraged the Council to get information out about the survey. She will be reaching out to Council members separately. Reginald will send the survey link out again.</p> <p>Create a form where people can come speak if they want - we have been receiving few of those.</p>	<p><b>request to speak</b></p>
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**2021 Planning Process for June Call**

**Terry Jo Bichell, RDAC Vice Chair**

**Gillian W. Hooker, RDAC Secretary**

Terry jo mentioned having joined a call with other RDACs and explored some of the other functions that RDACs are holding.

Some have legislators on their committees as a way to interact with legislation

Others serve an advisory/liaison role to state committees.

Also outline/discuss a potential report -

what we think are the most important aspects of describing rare disease in Tennessee - we could do it in that report  
**Plan for Strategy Discussion in August 2021**

<p><b>Pharmacy Advisory Committee Update &amp; Planning</b></p>	<p><b>Reginald French, RDAC Member</b></p> <p>Reginald French gave a report on the PAC: The drug Dojolvi was up for review. It is a treatment for Long-Chain Fatty Acid Oxidation Disorder (LC-FAOD), which is a rare disease. There are about 2,000 ppl with the disorder; and 200 more are diagnosed each year. The PAC approved the drug. Dojolvi provides short-chain fatty acids as an alternative source of energy in patients with FC-FAODs and who have diets that are restricted in fatty acids. It is a costly drug and TennCare will monitor the utilization of the drug through its Drug Utilization Review Committee. LC-FAOD causes metabolic challenges in patients with the liver and heart mostly affected. There is a range of severity of the disease. It can cause liver, heart and muscle problems; untreated persons are low energy and they can develop severe heart problems in the long term. Persons diagnosed often have highly regulated diets. It can lead to metabolic crisis and frequent hospitalizations. Diagnosis can happen through newborn screening.</p> <p>In the future, if the RDAC is going to present from a rare disease perspective, we'd need to get on the agenda or submit a letter on behalf of RDAC.</p> <p>The next PAC meeting is in August - if we know what drugs will be discussed in June, we can have a quick discussion then get it organized with any testimony that our members might be interested in.</p> <p>With PAC-we need to notify of public testimony within 72 hours of actual PAC date.</p>	<p><b>Put time on June call to review PAC agenda for August</b></p> <p><b>Ask R. Hamid to speak to Dojolvi as a use case for how the RDAC and support the PAC.</b></p>
<p><b>Drug Utilization Review Committee Update &amp; Plan</b></p>	<p><b>Megan Crow, RDAC Member</b></p> <p>Do the same thing for the DUR as the PAC</p> <p>The agenda doesn't come out as early for DUR as for PAC - they go over specific issues they are looking into</p> <p>It would be helpful to communicate with leaders of the DUR meeting to come to RDAC and give us an overview of what to expect.</p> <p>Regarding the DUR meeting- the last DUR meeting was in April - next would be in July.</p>	<p><b>Put time on June call to review DUR agenda for July</b></p> <p><b>L. Underwood will Dr. Ray McIntyre who leads DUR to attend RDAC meeting in June or July</b></p>

<p><b>Newborn Screening / Genetics Advisory Committee Updates</b></p>	<p><b>Rizwan Hamid, RDAC Member</b> Not present</p> <p><b>Kim Stephens raised a specific issue of adding MPS II to the newborn screening panel.</b> MPSII has been pushed to the larger federal list, if that happens, then there will be grounds to push to put it on the state screening list.</p>	<p><b>R. Hamid to present on next call.</b></p>
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