

Senate State, Veterans, & Military Affairs

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SB24-203 Rx Drug Board Consider Rare Disease Adv Council

Typed Text of Testimony Submitted

Name, Position, Representing	Typed Text of Testimony
Kelly Wiberg Amend themselves	<p>I would like to thank the sponsors for the original bill SB 60 and for trying to protect the rare disease community. I would like to propose this bill be amended to "must consult with Rare Disease Advisory Council prior to selecting an orphan drug for review" and during the review process for any orphan drug that is selected for review. The patients of these drugs need protection from interruption in access and it needs to be determined whether any UPL is like benefit the patient. This is extremely important!!</p> <p>Thank you!</p>
Ryan Boone Amend themselves	<p>First question, Why is this bill in this committee?</p> <p>I request the bill be amended to: REQUIRE the PDAB(Board) to Consult with the Colorado rare disease advisory council (council). Not just consider input.</p> <p>"The purpose of the council is to study and make findings and recommendations to the general assembly, state agencies, and others, as appropriate, concerning the needs of individuals living in Colorado with rare diseases." Created in Section 25-1-1503</p> <p>The RDAC's purpose is to represent people like myself, who have a rare disease (Cystic Fibrosis in my instance). This is very important to myself and the rare disease community, not just here in Colorado, but nationwide, and worldwide. What is being done in Colorado will be used as an example in other places. We want protections in place to protect while the government makes these changes to help patients.</p> <p>Thank you,</p>

	<p>Ryan Boone</p>
<p>Jennifer Reinhardt Amend themselves</p>	<p>Hello,</p> <p>Our family went through the PDAB board review of Trikafta. Although the board found that the medicine Trikafta was not unaffordable- it was a very stressful few months for our family. It reminded me of when my child was dying, as losing access to the only drug saving her life would certainly cause her to die. If a drug is sole use for an orphan disease and is the only drug that saves someone’s life- I think it should be excluded from PDAB. The RDAC can guide the PDAB- however I believe we should require the RDAC to have one member who is actually on the medication being reviewed. This bill is not strong enough to protect families.</p> <p>Also it should be disclosed that PDAB does not give any of the hypothetical savings back to patients but does help insurance companies by not having to pay for the medicine if the drug maker leaves the state. The drug maker will likely leave the state as the PDAB could set a national medicaid best price that would impact all 50 states. Or instead of negotiating they could just leave Colorado.</p> <p>Until these issues are fixed - if a drug is deemed a sole use orphan drug, has no alternative, and is live saving, these factors should be looked at before having a full review and it should be excluded from PDAB for patients that already have access to the medications with low copays. A simple interview with a rare disease patient on the medicine that lives in Colorado and a google search could show this - it doesn’t take a million dollar PDAB review and months of worrying families that they will lose access to the drug to do this.</p> <p>Also it should be disclosed when people are testifying if they are sponsored by insurance and actually if they live in Colorado. The testimony is almost all insurance sponsored groups and patients without a medicine being looked at by PDAB.</p> <p>I am very sad we are having to go through this process in order to iron out the kinks- kinks such as “Will patients have access at all”- while testing the process out on vulnerable rare disease patients fighting for their lives. One should ask, who does PDAB benefit if not</p>

	<p>the patients? The answer may lie in insurance savings and state budget savings at the cost of patient lives.</p> <p>For this reason- I think the bill should be amended to have stronger protections. The PDAB should be modified to meet the sales pitch of "helping patients" by amending the goal of the PDAB to lower patient copays and increase patient access.</p>
<p>Siri Vaeth For Cystic Fibrosis Research Institute</p>	<p>Dear Members of the Senate State Affairs Committee.</p> <p>On behalf of the Cystic Fibrosis Research Institute (CFRI) and the Colorado residents impacted by cystic fibrosis that we serve, I write to express our cautious support for SB 24-203, which mandates that Colorado’s Prescription Drug Affordability Review Board consider input from the Rare Disease Advisory Council. Our support is cautious, as we had strongly hoped that there would be a definitive carve-out for rare disease drugs, so that these therapies would not be selected for review.</p> <p>We still hope that legislation will advance with this carve out, but in the meantime, we are encouraged by this requirement for members of the PDAB to consider input from members of the Rare Disease Advisory Committee. During last year’s PDAB hearings regarding Trikafta, it was concerning that there seemed little understanding or recognition by Board members of the complexity of rare disease drug development. With only 5% of rare diseases currently having an FDA approved therapy, we remain concerned that the inclusion of rare disease drugs in Colorado PDAB reviews will discourage drug development for the remaining 95% of rare diseases.</p> <p>We hope that should SB 24-203 advance, there will be oversight to ensure that RDAC input is valued and incorporated in a meaningful way, and will not merely serve as window-dressing to the process.</p> <p>Thank you, Siri Vaeth, MSW Cystic Fibrosis Research Institute</p>
<p>Amanda Boone</p>	<p>I want to say thank you to the sponsors on our previous bill who have worked tirelessly to protect rare disease patients. We know this has</p>

<p>Amend themselves</p>	<p>not been an easy task. We also thank the sponsors of this bill and any other legislators who support protections for us.</p> <p>I support the RDAC having a voice in matters regarding the PDAB and patients with rare diseases. I have cystic fibrosis, and I truly wish that the RDAC would have been involved from day one with PDAB when Trikafta was on the list of potential drugs to review. I trust that the RDAC has the expertise to represent my needs in a meaningful way. This is why an RDAC was created in Colorado, and I think it would be wise for other legislators to consult them as well when these type of issues come up.</p> <p>I would like to ask for the following amendment:</p> <p>Please change the verbiage to, "The PDAB MUST consult with the RDAC...." instead of "should."</p> <p>Thank you for your consideration. This would give the patients who worry about our access being compromised more comfort then how the bill is currently written.</p>
<p>Heather Kluck Amend themselves</p>	<p>I've testified in person regarding SB24-60, shown you pictures of my miracle child who wasn't supposed to survive birth let alone walk or talk, but does it all & is a Special Olympics multi-medalist at 12 years old...</p> <p>My daughter needs to start an orphan drug, Sabril (Vigabatrin) for her specific type of drug resistant epilepsy that can cause death. Sabril is one of the first 300 medications up for the PDAB's review. It's THE ONLY MEDICATION for her specific form of epilepsy. We haven't started her on it yet because we're afraid it will be taken away, and a 3 month prior notice is not enough time to wean her off of it. Weaning that fast can also cause death. Sabril/Vigabatrin is also THE ONLY non-steroid FRONTLINE MEDICATION for the treatment of a catastrophic form of infant epilepsy (infantile spasms)!! Every seizure a baby has with this disorder can cause permanent brain damage, loss of skills & actually can cause developmental disabilities!! 1 in 26 people in their lifetime will develop epilepsy (not just have 1 seizure, but develop a full seizure disorder). At any moment you or your loved one could develop epilepsy & your life-saving medication</p>

	<p>could be next-up for review. Would you be comfortable without a 2nd or 3rd educated party looking out for your best interest?</p> <p>This new bill is not strong or protective as it stands & I ask that you add an amendment to this bill that the PDAB "MUST" consult with the Rare Disease Advisory Council. Please lean on the experts for your decisions that could literally be the difference between life & death.</p> <p>We don't know what the results of the PDAB will be initially, hopefully it will all be positive, but until then, please Colorado legislators, don't make those relying on life-saving medications your experiments. Our most vulnerable population suffers enough, please don't make it worse.</p> <p>Best regards,</p> <p>Heather Kluck Certified Nursing Assistant- MGA Homecare Executive Board of Directors/Secretary- The Arc Pikes Peak Region Special Olympics Colorado Coach- Pikes Peak Wolves</p>
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