Kari Rosbeck, President & Chief Executive Officer

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MACPAC: Kari, you are self-muted. You can unmute yourself and make your comment.

MS. ROSBECK: Hi. Yes, thank you. My name is Kari Rosbeck. I'm the President and CEO of the Tuberous Sclerosis Alliance, a nonprofit dedicated to find a cure for tuberous sclerosis complex, or TSC, while improving the lives of those affected. TSC is a rare genetic disorder that causes tumor growth in all the body's vital organs. Symptoms can include seizures, kidney failure, brain and lung tumors, autism spectrum disorder, and severe learning disabilities. TSC is also the leading genetic cause of both epilepsy and autism.

TSC is a good example of how the accelerated approval pathway can work to get treatments to patients who do not have other safe, effective options. Subependymal giant cell astrocytomas, or SEGA, is a slow-growing tumor that can cause life-threatening complications by blocking cerebral spinal fluid, and remains a major clinical feature of TSC. Not all individuals are surgical candidates, leaving them with fatal complications.

Novartis' Afinitor, a cancer medicine, was originally introduced in 2009, and granted accelerated access approval for TSC indications in 2010 for SEGA, 2012 for renal angiomyolipomas, and 2018 as adjunctive therapy for partial-onset seizures. Novartis completed the FDA-required studies, submitted data to FDA, and received regular approval.

If the proposed additional rebate had been in place, I believe it is unlikely that they would have used the accelerated approval pathway and maybe even declined to study Afinitor for TSC. The outcome would be individuals would remain without treatment options. Applying the additional rebate to accelerated approval indications and rare diseases would be complex to administer and would penalize manufacturers who are doing everything right. Ultimately, it is the patients who would pay the price.

As the TSC community can attest, this FDA program is vital to providing treatments where previously there was a serious unmet medical need. We encourage you not to place disincentives on manufacturer use of this program and to keep the ultimate beneficiary in mind, the patients who desperately need these medications.

Thank you so much.

MACPAC CHAIR BELLA: Thank you, Kari.