RARE ACCESS ACTION PROJECT

I am the Executive Director of the Rare Access Action Project, a non-profit based that advocates on behalf of patient access issues. I also belong to a family that has been affected by rare diseases, and because of that I have spent much of my career in the life sciences advocating for access to rare disease therapies and supporting families dealing with a variety of rare diseases. Because of my experiences, I am passionate about advocating for access to rare disease therapies.

Only 5% of rare diseases have a treatment approved by the Food and Drug Administration (FDA) and for one-third of individuals with a rare disease, it can take between one and five years to receive a proper diagnosis. Half of all patients diagnosed with a rare disease are children, and as many as 3 in 10 children with a rare disease will not live to see their 5th birthday. For the few fortunate to have a treatment, patients face many barriers to these orphan therapies across our health care system in addition to facing a patient journey filled with misdiagnosis and lack of treatment options.

As you can imagine, having seen both the legislative efforts and the implementation surrounding PDABs in other states, we are very concerned. Since the first PDAB was created in 2019 by the Maryland legislature, PDAB legislation has been passed in 8 states (Colorado, Maine, Michigan, Minnesota, New Hampshire, Ohio, Oregon, and Washington). One of the states, Ohio, does not have an upper payment limit requirement. However, since 2019, the experiment in government drug price controls has amassed a dubious record of success. Maryland, the oldest of the PDABs, has yet to become operational, and others have struggled to fulfill their mission. The pharmaceutical ecosystem is far more complex than proponents have portrayed it, and PDAB legislation patches on a state bureaucracy over the ecosystem with little understanding of pharmaceutical pricing policies and implications of that flawed PDAB patch.

Through our work with PDAB, we have found that dysfunctional PDABs are spending state resources and not providing benefits to consumers through increased access and lower out of pocket costs and premiums.

Pharmaceutical products are purchased across the United States with customers from Maryland involved in an interstate marketplace that includes a network of federal and state discount and rebate programs (Medicaid best price, 340b pricing, VA, and FSS as well as the calculations and penalties that are attached to them). Further, many commercial plans and PBMs already negotiate discounts, and some payers have already begun to implement value-based pricing. States such as Arizona are exploring risk pooling for rare disease therapies, as well as reinsurance. Price controls from PDABs could jeopardize efforts to innovate value and payment in the pharmaceutical ecosystem.

Last year, the initial PDAB list of drugs in Colorado disproportionately included rare therapies, most used by fewer than 100 Coloradans. State price setting for those medicines could unleash unintended consequences on patients who simply cannot afford to have their current treatments interrupted.

Also consider, rare disease patients increasingly participate in Center of Excellence care. Universities and larger hospital systems have become hubs for both care and treatment. Drugs are purchased and administered for some rare patients outside of Maryland. These are real challenges that will face Maryland rare patients who may be on a therapy purchased outside the state and provided by institutions beyond the purview of the Maryland PDAB. And payment for those medicines will create risk for those providers, potentially choking off access to that necessary treatment.

The policymakers involved with PDAB across the country have heard from patients that **they pay copays each month for their treatment, the amount set by their plan**. And for those without coverage there are programs to ensure patient access to the therapies. Many life sciences companies offer access assistance through patient assistance and free drug programs that reduce the cost to patients throughout the year. And non-profit foundations offer copay and other support. So to clarify, patients pay high copays through their plans. A PDAB will not change the benefit structure of the plans.

Consider the experience we already have with price setting challenges, on which proponents of PDAB claim they have modeled this form of price controls. The incentives to develop and bring to market therapies for rare and ultra-rare populations were intended to overcome economic hurdles that shift investment costs to larger, blockbuster therapies. The Inflation Reduction Act (IRA), which was passed in 2022, intended a similar exercise of federal government price settling. Even though an exemption for rare therapies was included in the bill, the implementation is problematic because it eroded the incentives of the seminal Orphan Drug Act. Now we are seeing the closure of rare programs, investment resources fleeing to other therapeutic areas, and market uncertainty caused by federal policymaking.[1][2][3]

Targeting rare therapies will not bring meaningful savings to Marylanders. Proponents have shown us no evidence that PDABs will lower copays, encourage lower premiums, create transparency, or not damage access that Marylanders with rare disease have through their current insurance. To-date they have become costly new, experimental bureaucracy. So, the question becomes, will an upper payment limit for a medicine that could disrupt patient access really bring savings to patients, and provide those savings in a transparent fashion? Because PDABs are largely experimental, with a strategy hoping to pick up the pieces of the pharmaceutical market once the damage has been done is unacceptable. Rare patients cannot afford this indulgence.

We urge you to navigate this issue with a commitment to ensuring that no patient is left fearful of losing current access to the treatments that offer them a chance at a better tomorrow. We believe there are solutions that can enhance affordability across healthcare. But proponents of PDAB are focused on an experimental program that has generated zero savings and offers no solution to patient costs since the first PDAB was created in 2019. We urge you to vote no on HB 340.

[1] Chambers JD, Clifford KA, Enright DE, Neumann PJ. Follow-On Indications for Orphan Drugs Related to the Inflation Reduction Act. JAMA Netw Open. 2023 Aug 1;6(8):e2329006. doi: 10.1001/jamanetworkopen.2023.29006. PMID: 37581890; PMCID: PMC10427936.

[2] HOW THE INFLATION REDUCTION ACT IS IMPACTING RARE DISEASE PATIENTS https://cahc.net/how-the-inflation-reduction-act-is-impacting-rare-disease-patients/

[3] Inflation Reduction Act: Unintended consequences for rare disease, orphan drugs https://bio.news/bio-convention/inflation-reduction-act-unintended-consequences-orphan-drugs-rare-disease-bio-patient-advocacy-summit/

Michael Eging
Rare Access Action Project
(202) 631-5752
mike@rareaccessactionproject.com
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