

RARE → READY

A GENETIC CONDITION COALITION



Hemophilia Foundation
of Southern California
FOR ALL BLEEDING DISORDERS



February 24, 2025

The Honorable Pamela Beidle, Chair
Senate Committee on Finance
Miller Senate Office Building, 3 East Wing
11 Bladen St., Annapolis, MD 21401 – 1991

Dear Senate President Ferguson, Chair Beidle, Vice Chair Hayes, and members of the committee:

The Rare & Ready Coalition would like to express our opposition to SB 357, a bill to expand the scope of the Maryland Prescription Drug Affordability Board (PDAB) and urge consideration of an amendment that would protect rare disease patient access.

The Rare & Ready Coalition represents non-profit organizations working to ensure rare disease patients get timely access to the care they need and deserve. We are alarmed by the devastating impact the existing PDAB and its potential expansion will have on patient access to life-saving rare disease therapies.

We strongly urge the Committee to consider the unique circumstances of rare disease patients and therapies as it considers this legislation. You must protect access for patients living with a rare disease, who have no treatment alternatives. It's critical you consider the real-world experiences of those living with or caring for someone with a rare disease.

Patients living with rare, genetic diseases have limited treatment options, with 95 percent of such conditions lacking any FDA-approved therapies. State efforts to create PDABs, while intended to make drugs more affordable for health plans, can deter access to critical medical innovations. PDABs do not lower patient copayments, reduce premiums, create health system transparency, or increase access to care for rare patients and may prevent insurers and pharmacies from purchasing medications exceeding government-set prices, taking away treatment options for patients. The implications are most profound for patients living with rare diseases, the majority of which are children. PDAB reimbursement caps reduce rare disease research, limit new treatments, and restrict patient access to medicines.

The Rare & Ready Coalition urges the Committee to support the following amendment to ensure continued access for patients to life-changing rare disease medicines:

Amendment:

The process for setting upper payment limits shall:

- (1) Prohibit the application of an upper payment limit for a prescription drug product that is on the federal Food and Drug Administration prescription drug shortage list; and
- (2) **Prohibit the performance of a cost review in § 21-2C-09 of this subtitle and the application of an upper payment limit for a prescription drug product that is designated as a drug for a rare disease or condition by the Federal Food and Drug Administration Pursuant to 21 U.S.C. Sec. 360bb or a licensed biological product that is derived from human whole blood or plasma, as indicated on the approved product labeling.**
- [(2)] (3) Require the Board to: (i) Monitor the availability of any prescription drug product for which it sets an upper payment limit; and (ii) If there becomes a shortage of the prescription drug product in the State, reconsider or suspend the upper payment limit.]**

For these reasons, we respectfully ask that SB 357 be amended to protect rare disease patient access. We urge the committee to hear directly from rare disease patients and the parents of children battling these conditions before making any decisions. We welcome the opportunity to connect you to those who are directly impacted—please reach out to the coalition administrator at kari.lato@rx4good.com to schedule a meeting with your constituents.

Sincerely,

Rare & Ready Coalition Members