February 1, 2024

Representative Barbara Lee
Co-Chair
Congressional Sickle Cell Disease Caucus
U.S. House of Representatives
Washington, DC 20515

Representative Danny Davis
Co-Chair
Congressional Sickle Cell Disease Caucus
U.S. House of Representatives
Washington, DC 20515

Dear Rep. Lee and Rep. Davis,

We strongly urge the Congressional Sickle Cell Disease Caucus to support patient access to the recently approved gene therapies for sickle cell disease (SCD) by endorsing H.R. 2666, the MVP Act. The Food and Drug Administration's approval of Casgevy and Lyfgenia on December 8 marks a pivotal advancement in SCD treatment. These transformative interventions promise to elevate the standard of care for individuals grappling with this debilitating disorder. The MVP Act represents a critical initial step to helping "ensure meaningful access to these potentially curative SCD therapies on day one of their approval," as previously called for by the Congressional Black Caucus in a July 26, 2021, letter to U.S. Department of Health and Human Services (HHS) Secretary Xavier Becerra. Securing access of this nature is imperative for a community that has long been neglected, overlooked, and underserved.

Enactment of the bipartisan MVP Act, as passed by the House Committee on Energy and Commerce on May 24, will ensure patient access to rare disease gene therapies like Casgevy and Lyfgenia by enabling biopharmaceutical companies to offer value-based purchasing arrangements (VBPs) that allow for meaningful outcomes-based refunds during a specified warranty period. The HHS Office of Inspector General highlighted the historical reluctance of biopharmaceutical companies to offer VBPs due to concerns regarding the potential implications of such refunds on best price and average manufacturer price (AMP) and their consequential impact on the accuracy of Medicaid rebates and 340B discounts. Although the Centers for Medicare & Medicaid Services (CMS) attempted to address this issue through regulatory action, its efforts remained incomplete. The MVP Act aims to resolve this predicament by clarifying the CMS authority to establish a policy that allows for the reporting of the multiple best prices available under a VBP, as well as fill gaps in the regulation to ensure precision in the calculation of AMP and average sales price.

VBPs will instill accountability among biopharmaceutical companies, compelling them to stand by the performance of these groundbreaking medicines. In scenarios where a patient does not respond or stops responding to a treatment, VBPs would require a full or partial refund. This shared risk model is critical for gene therapies because of the need to balance potential patient benefit and future savings with unique patient responses and high cost. For a maintenance therapy treating SCD like Adakveo, in the event a patient stops responding, a physician would simply opt for an alternative like Oxbryta, which in turn, would protect the payor from paying for

a medicine that does not work for the individual patient. Because Casgevy and Lyfgenia are *single administration therapies*, VBPs are necessary for the payor to mitigate the financial risk. Thus, VBPs should serve as a viable substitute for step therapy and other payor coverage restrictions, expediting patient access to these crucial therapies. As such, we urge every member of the caucus to cosponsor H.R. 2666, the MVP Act.

Sincerely,

TaLana Hughes, MPH
Executive Director
Sickle Cell Disease Association of Illinois (SCDAI)

Teonna Woolford
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