In Favor [FAV] HB 0302: Public Health - Rare Disease Advisory Council

Honorable Delegate Joseline A. Pena-Melnyk
Room 241
House Office Building
Annapolis, Maryland 21401

Re: Written testimony of Jennifer Payne for 2/14/2023 hearing at 1pm.

Dear Chair Pena-Melnyk, Vice Chair Kelly, and Members of the House Health and Government Operations Committee,

My name is Jennifer Payne, and I am a constituent of District 12. As an ambassador for the greater Maryland rare disease community, I thank you for the opportunity to submit my testimony in support of HB 302. Not only it is a critical imperative for Maryland leadership to engage - *as partners with rare disease patients*, for shaping policy development relevant to the unmet needs and concerns on equitable, quality, and affordable access to specialty treatment, but it is a moral imperative to identify and eliminate the inappropriate and discriminatory barriers preventing individuals diagnosed in the State of Maryland from obtaining the continuity and quality of care across all stages of life.

Every baby born in the United States is screened for the early identification of phenylketonuria]PKU] to prevent severe disability. Hence, this is my story, being among the first diagnosed with PKU [1973] in the early days of Maryland's newborn screening program. And, 50 years later [as 2023 marks my milestone birthday], I can tell you I have been a lifelong, proactive champion on medical nutrition equity for inclusion on the whole health equation - to the level of the courts. My whole health, mentally, physically, and spiritually has been sacrificed at the expense of having [and continuously fighting] singlehandedly - over the last 7 years and ongoing, - for ending discriminatory provision of health care services by insurers using PKU - and my age - as arbitrary and exclusionary criteria prohibiting access and coverage on the treatment I need to survive and thrive; – even into adulthood. My PKU story of prevention has become a story of social injustice and discrimination.

And, I am not alone. Many in the rare disease community share such common struggles and obstacles that are often 100% preventable and socially unjustifiable, and result in unnecessary suffering and disease progression. I wholeheartedly welcome support for the establishment of an RDAC to serve as a health advocate for all Marylanders, like myself, in need of representation and a special conduit to amplify vulnerable voices in health matters that have direct, and more often than not, negative implications and risk. Now is the time for us all to partner and be change ambassadors in this cultural shift that embraces diversity, equity, inclusion, and accessibility in the ways we live and the ways we care – for rare disease as part of the whole health equation. We must ensure the necessary improvements to Maryland's life saving newborn screening program stay strong. We must educate, as I have done with University of Maryland medical students - the future doctors of America - to think out of the box and equip them, the greater public, and all stakeholders – with the requisite tools to recognize, diagnose, facilitate, and optimize treatment specific to rare diseases. We can establish a published list or compendia to allow for transparency, accessibility, and education in the process. Knowledge is

power, and patients are often the experts in their own rare diseases. Will you listen? We need novel reimbursement payment models that allow coverage for specialty treatment accordingly – and equitably – and with inclusion keeping pace with innovative technology platforms. Rare disease patients only ask for a fair shake of such readily available resources, not to be targets as demonstrated by my example. The RDAC serves to empower rare disease patients as partners – from the point of the research bench (informing future studies), to diagnoses, to getting the treatment into the hands of those that need it most and at the right time - for life. Until there is a cure, for change ambassadors and partners with RDAC, the story [as with my PKU story] does not end at diagnosis.

Thank you,

/s/

Jennifer Payne, PharmD, MAPP

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