Thank you for providing us with this opportunity to offer comments to the Committee today.

We are writing on behalf of the over 30 million Americans living with rare diseases and as co-chairs of the EveryLife Foundation's Newborn Screening and Diagnostics Working Group. The EveryLife Foundation is a non-profit, nonpartisan organization dedicated to empowering the rare disease patient community to advocate for impactful, science-driven legislation and policy that advances the equitable development of and access to lifesaving diagnoses, treatments, and cures.

Over the past year, the Advisory Committee has reviewed and updated its processes, including efforts focused on ensuring patient advocacy organizations have a better understanding of the RUSP nomination process and the role that patient advocacy organizations play in newborn screening and RUSP nominations. As the Advisory Committee and HRSA begin to review the nominations for the two open committee positions, we urge the Advisory Committee to include a patient advocacy organization representative as one of the two new members to be appointed, ensuring this constituency is represented on the Committee as it has been in the past.

An appropriately qualified patient advocate is an expert on their rare disease and can provide insight on the impact newborn screening can have on the rare disease community. Patient representatives can lend their experience, which often includes being a patient, parent, caregiver, scientist, and policy expert, to the nomination review process. Providing a distinct understanding of the significance of early diagnosis and treatment. Patient representatives lend insight into how families juggle the cost of treatment and how patient communities and providers can support families diagnosed through newborn screening. It is essential the Committee once again incorporate this perspective into the work that they do both during the RUSP nomination process and their work outside of the RUSP.

Continued inclusion of a patient advocacy organization representative as a committee member can build on the trust and understanding the Committee has worked to foster with these organizations. It can signal to the patient advocacy community that our voice remains important in not only the RUSP review process, but in discussions about how to improve the newborn screening system and prepare it for an influx of new disorders. Including an advocate's voice builds diversity and inclusion on the Committee, and can encourage further discussion and input by these patient advocates. The inclusion of a patient advocate can help to alleviate fears that problems outside of issues specific to a disease nomination may prevent a disorder from being added to the RUSP or that in the absence of an authentic advocate voice, non-patient advocates speak erroneously on the advocate perspective during Committee deliberations.

Patient advocacy organizations are a vital piece of the newborn screening system and must have meaningful input on Committee decisions that have the power to affect the entire newborn screening ecosystem. Representatives from patient advocacy organizations come from diverse backgrounds and can bring their own set of expertise to the Committee. Patient representatives can serve as a bridge between the patient advocacy community and the Committee, fostering more buy-in and support from even the most skeptical patient advocates. Like the Committee, patient advocacy organizations want to

ensure that we build a strong newborn screening system that can provide lifesaving diagnosis to newborns and that can withstand many of the challenges it faces now and in the future.

As the Committee and HRSA consider adding a patient advocacy organization representative, we encourage you to define what is meant when you consider a "patient advocacy organization." The National Health Council sets standards[1] for patient organizations interested in becoming members of the Council and we ask the Committee to consider these standards when defining a patient advocacy organization. These standards require organizations to be engaged in research, professional education, public education and health promotion, health services, community services, advocacy, or social action. When considering a patient advocacy organization representative, we encourage the Committee to define a patient advocacy organization as an organization engaged in one or more of these areas. We also ask the Committee to follow National Health Council standards and define a patient advocacy organization as an organization that has been active in the space for no less than three years.

We are grateful for the Committee's previous inclusion of a patient advocate as a Committee member and for all the work that is occurring within the newborn screening space and for all the updates the Committee is making to the RUSP nomination process. We are committed to working with the Committee to incorporate the patient voice more thoroughly by including a patient advocacy organization representative on the Committee.

Sincerely,

The EveryLife Foundation for Rare Diseases Community Congress Newborn Screening & Diagnostics Working Group

[1] https://www.nationalhealthcouncil.org/wp-content/uploads/2019/12/NHC_Files/Governance/Minimum_Standards_for_Voluntary_Health_Agencies.pdf