Statement for the Record for Senate Finance Committee, September 17, 2024, Full Committee Hearing
"Lower Health Care Costs for Americans: Understanding the Benefits of the Inflation Reduction Act"

Tigerlily Foundation (Tigerlily) is a national women's health and oncology organization providing education, awareness, advocacy and hands-on support to young women before, during and after cancer. Our vision also includes ending disparities of age, stage and color, in our lifetime. Through our programs, we seek to educate and empower women of all backgrounds, including those at heightened risk, those facing health disparities, and those with less access to care.

Approximately 30 million Americans are living with a rare disease.[1] Yet, of the estimated 10,000 rare diseases, 95 percent lack an FDA-approved treatment specific to their condition. Millions of Americans with a rare disease need the hope that continued research and development into new treatments brings.

In addition to supporting those with primary diagnoses of rare cancers such as ovarian cancer (which has an astounding 98 designations but only 6 approvals[2]), cervical cancer (which has 9 designations and 0 approvals[3]) and fallopian tube cancer (with only 4 designations and 1 approval[4]), all of which urgently need available treatments to save lives, Tigerlily is deeply concerned about the common side effects of cancer treatments that manifest as rare diseases and affect those with non-rare cancers as well, including neuropathy (only has one designation for chemotherapy-induced neuropathy[5]), kidney disease[6], and liver disease[7]. These conditions have very limited or no FDA-approved treatments, creating an urgent need for continued research and therapy development. We must also recognize that within the rare disease space, there are significant racial disparities, with Black and Brown patients facing higher mortality rates and poorer outcomes. Without sustained and targeted investment in research, these gaps will only continue to widen, further exacerbating health inequities for marginalized communities. It is critical that we address these disparities and ensure that all patients, regardless of disease type or race, have access to effective treatments and the potential for improved outcomes.

The ORPHAN Cures Act legislation would correct the overly narrow orphan drug exclusion in the MDPNP by (1) ensuring that eligibility for the Orphan Drug Exclusion allows products to remain excluded from negotiation as long as their FDA approved uses are exclusively for rare diseases, and (2) clarifies that negotiation eligibility starts upon a product's first non-rare approval. Both changes are critical to ensuring that individuals with rare diseases – including ultra rare – can continue to benefit from scientific research in their disease states.

Tigerlily thanks Members of the Committee for exploring this important policy issue and its impacts.
We continue to support the ORPHAN Cures Act and would be glad to serve as support to the Committee through this process to ensure that our nation's health care is more inclusive and responsive to the needs of millions of Americans with rare diseases.

1 https://www.nih.gov/about-nih/what-we-do/nih-turning-discovery-into-health/promise-precision-medicine/rare-diseases.
2. https://www.accessdata.fda.gov/scripts/opdlisting/oopd/listResult.cfm
3. https://www.accessdata.fda.gov/scripts/opdlisting/oopd/listResult.cfm
4. https://www.accessdata.fda.gov/scripts/opdlisting/oopd/listResult.cfm
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