November 15, 2022

The Honorable Nancy Pelosi
Speaker
United States Congress
H-232 The Capitol
Washington, DC 20515

The Honorable Kevin McCarthy
Minority Leader
United States Congress
H-204 The Capitol
Washington, DC 20515

The Honorable Chuck Schumer
Majority Leader
United States Senate
S-221 The Capitol
Washington, DC 20510

The Honorable Mitch McConnell
Minority Leader
United States Senate
S-230 The Capitol
Washington, DC 20510

Dear Speaker Pelosi and Leader McCarthy and Leader Schumer and Leader McConnell:

As you work to assemble your plans for year-end legislation, we write on behalf of all Americans impacted by rare disease to urge you to include as part of such legislation policies to support development of therapies to treat rare diseases and conditions. Specifically, we urge you to include provisions contained within the bipartisan **Speeding Therapy Access Today (STAT) Act (H.R. 1730, S. 670)**, including those that are reflected in the House-passed Food and Drug Administration (FDA) user fee legislation.

As we head to 2023 and what will be the 40th anniversary of the enactment of the landmark Orphan Drug Act that sparked a far greater focus on rare disease research and therapy development, we will be celebrating how much has been achieved while also noting how much more remains to be done. The policies summarized below are targeted and well-evidenced approaches that build upon current law and policy and address current and emerging needs. If included in year-end legislation, these policies will have a marked effect on rare disease communities. Specifically, we urge you to include provisions that will do the following:

- **Strengthen the Orphan Products Grant Program at FDA.** The House-passed Food and Drug Amendments of 2022 (H.R. 7667) includes as part of its reauthorization of this impactful program, language in Sec. 713 to expand its focus to support regulatory science activities in addition to the current focus on clinical trials and natural history studies. **We urge that this provision be included in any final legislation**.

- **Fully fund the Orphan Products Grant Program to its authorized level in addition to the $5 million funding for ALS as directed in the Act for ALS.** Taking this action would ensure the program can continue its broad rare disease focus while allocating $5 million from this program to support ALS-focused activities. The ALS-focused research funding was authorized by the Act for ALS, enacted with bi-partisan support in 2021, and never intended to supplant overall rare disease resources. The program receives many meritorious applications addressing diseases with no approved therapies that it cannot fund under existing funding levels. We believe fully funding the program and the newly authorized ALS-focused line of research will ensure that, as we approach the 40th anniversary of the Orphan Drug Act, the rare disease community has more hope, not less. As you consider the reauthorization of the Orphan Products Grant Program, we **urge you to also increase its authorization**

level by at least $10 million to account for the overwhelming need the program serves and to ensure that this meaningful commitment to ALS-focused activities does not detract from the other additional rare disease needs.

- **Authorize a voluntary process for rare disease drug manufacturers to engage with payors.** The House-passed bill includes as Sec. 810 a provision on pre-approval information exchanges between product manufacturers and payors. This provision also requires a Government Accountability Office (GAO) study on the types of communication between manufacturers and payors. **We urge Congress to include this provision in the final legislation and to expand upon it by authorizing a voluntary early payor communications program between manufacturers of rare disease therapies and payors**. Establishing this program would parallel a program established several years ago by the Center for Device and Radiological Health (CDRH) to foster earlier dialogue between medical device manufacturers and payors to "obtain payor input on clinical trial design or other plans for gathering clinical evidence needed to support positive coverage decisions."[1] Given current and anticipated issues around access policies for rare disease therapies, we believe establishing such a program focused on rare disease drugs would be most beneficial and help limit potential barriers to access that arise after a product has been approved. We urge Congress to include its authorization in the final legislation.

- **Pass policies that protect and strengthen the accelerated approval pathway.** The accelerated approval pathway is a vital tool in discovering and testing therapies for the 95% of rare diseases that still have no approved treatments. Congress has a clear opportunity to drive reforms that will strengthen the accelerated approval pathway by improving transparency and accountability. Both the Food and Drug Amendments of 2022 (H.R. 7667) and the Food and Drug Administration Safety and Landmark Advancements Act of 2022 (S. 4348) contain language to address these needed reforms. While there are some differences between accelerated approval provisions in these two bills, there is much alignment and we believe that both of your committees understand the reforms that are most needed.

We recognize the many issues that you will have to resolve before the end of 2022. However, given both the importance of these provisions to the rare disease community and the work done so far this Congress, we urge you to advance these provisions into law during the year-end session. Thank you for our attention to this request. If you have any questions, please contact Jamie Sullivan at jsullivan@everylifefoundation.org.

Sincerely,

Annie Kennedy
Chief of Policy, Advocacy, & Patient Engagement

Jamie Sullivan, MPH
Senior Director of Policy

CC: EveryLife Foundation Board of Directors
Julia Jenkins, Executive Director

[1] See: https://www.fda.gov/about-fda/cdrh-innovation/payor-communication-task-force