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The Honorable Diana DeGette
Member of Congress
2111 Rayburn HOB
Washington, DC 20515

The Honorable Fred Upton
Member of Congress
2183 Rayburn HOB
Washington, DC 20515

Dear Congresswoman Upton and Congressman Upton:

On behalf of the EveryLife Foundation for Rare Diseases, which is dedicated to empowering the rare disease patient community to advance laws and policies to improve lives of all people and families impacted by rare diseases and disorders, I write to commend your continued leadership on the Cures 2.0 legislation. Our Foundation was very pleased to work closely with both of you on multiple provisions included in the landmark 21st Century Cures Act, and we are excited and optimistic about this needed follow-on legislation.

As an advocate for the rare disease community, we are particularly pleased that the current version of the legislation includes multiple provisions that speak to the needs and challenges of our community. We applaud this recognition and urge that these policies be retained and strengthened as this legislation moves through the process. In the following pages, we will offer comments on specific provisions of the current legislation and offer some additional recommendations for further refinement.

Sec. 306: Establishment of Additional Intercenter Institutes at the Food and Drug Administration

We applaud you for including a provision to establish additional Intercenter Institutes or Centers of Excellence at the FDA and for specifying that one of these new centers should be focused on rare diseases and disorders. The EveryLife Foundation has long supported the establishment of a Rare Disease Center of Excellence, an objective that is also reflected in the bipartisan and bicameral **Speeding Therapy Access Today (STAT) Act – H.R. 1730 and S. 670**, Bilirakis/Butterfield and Klobuchar/Wicker). Rare disease experts have maintained that the scientific opportunity and need in rare disease are ripe to benefit from a Center of Excellence approach. As both of you know, rare disease therapies are handled by multiple centers and review divisions, making rare disease an ideal candidate to benefit from FDA-wide coordination.

A Center of Excellence in Rare Diseases would provide FDA with the rare disease resources and expertise that can be deployed as needed across centers and review divisions to support agency reviewer staff evaluating rare disease therapies. We also note and appreciate that the original Intercenter Institute authorization language included in Cures 2.0 is not overly prescriptive, providing FDA leaders with the ability to develop centers that reflect the needs of a center. Each of the existing institutes or centers has different attributes that respond to

the needs of the subject. We envision a rare disease center to possess some similarities as well as some important differences between existing centers and appreciate the flexibility included in the pending legislation.

As the Cures 2.0 legislation progresses, the EveryLife Foundation urges you to retain your commitment to this important provision and to consider additions that will strengthen it further. Specifically, we urge you to include additional elements of a rare disease center, as enumerated in the STAT Act, within this provision. These provisions would be consistent with the commitment exhibited already through the Centers of Excellence provision and would provide some additional direction to benefit the field. We encourage you to include the following additional rare disease provisions under this heading:

- **Establish a stakeholder engagement and guidance process to review labels for rare disease therapies.** This provision, similar in process to many convening and guidance activities included in the initial Cures law, would address concerns as to labels for rare disease products and do so in a way that engages all stakeholders. We urge its inclusion in an updated Cures 2.0 bill.

- **Create a voluntary rare disease third-party payor program**. This provision recognizes the value of early and voluntary interactions between payors and manufacturers and would authorize such a program focused on rare disease therapies. The hope is that earlier engagement of rare disease therapy manufacturers and payers will lead to coverage and payment policies that facilitate patient access to novel therapies.

- **Establish an FDA Rare Diseases and Conditions Advisory Committee.** Despite the prevalence of rare disease and disorders and the many uniquities associated with developing therapies to treat such conditions, FDA has no current standing advisory committee on this topic. We believe a standing rare disease and conditions advisory committee would provide FDA with the external expertise needed to inform and advise on both pending rare disease applications as well as current and emerging scientific, regulatory science and related issues in the sector.

- **Create the Accelerating Lifesaving Therapies in Treating Ultra-Rare Disease Entities (ALTITUDE) Program.** The ALTITUDE Program will make recommendations to address regulatory science and public policy challenges to the development of treatments for very small populations. The recommendations will focus on manufacturing standards, trial designs, regulatory flexibilities and manufacturing needs. ALTITUDE will also support decisions related to grants and contracts focused on therapies to treat very small populations.

In summary, we applaud you for including authorization of a Rare Disease Center of Excellence. We urge you to ensure this provision is retained as the legislation advances and to add these additional needed policies within this heading or otherwise.

Sec. 407: Expanding Access to Genetic Testing

The EveryLife Foundation is committed to shortening the diagnostic odyssey many rare disease patients and families must endure. We applaud the inclusion of multiple policies under Section 407, including guidance to state Medicaid programs as to ways to enhance necessary access to a range of genomic and genetic testing services and to establish a demonstration program in up to 15 states to facilitate such access. The tremendous promise and potential of precision medicine will go unrealized absent laws and policies that appropriately compensate for such services.

The value of an accurate diagnosis early on in the process is invaluable. In addition to providing patients and families with some greater certainty as to their own condition, it opens the door to appropriate treatment and care when such options exist. In cases in which a disease can be reversed, stopped, or managed, overall costs to all parties – including government payers – can be lessened. We are pleased to see this provision – including related studies and reports – included. As you move forward, we encourage its retention.

Sec. 501: Establishing the Advanced Research Projects Agency – Health (ARPA-H)

The EveryLife Foundation strongly supports creating the Advanced Research Project Agency – Health (ARPA-H) and has been actively involved in conversations about how to best achieve the goals envisioned through such an entity. As you and your colleagues work to refine your vision for an ARPA-H, we urge you to include the following attributes:

- Ensure APRA-H focuses on broad platforms and not disease-specific research projects.
- Be led by a director appointed by the President but not confirmed by the Senate.
- Be required to coordinate among other relevant agencies including the NIH, FDA, CDC and the National Science Foundation, including coordination with the FDA to support expeditious reviews of any medical products developed out of ARPA-H.
- Ensure ARPA-H has the ability to hire the needed scientific talent by exempting it from certain federal hiring laws and salary caps.
- Provide the entity with a full range of funding authorities including contracts, grants, cooperative agreements, prize competitions and other transactions, including the ability to end projects if milestones are not met to drive a "fail fast" mindset.
- Allow for ARPA-H to seek input from a broad base of stakeholders spanning the life sciences sector and including robust collaboration with industry.
- Recommend a headquarters location separate from the NIH or other existing agencies, just as DARPA is not located in the Pentagon.
- Provide APRA-H with an independent budget and consider authorizing a bypass budget so that agency recommendations go directly to Congress rather than through the Office of Management and Budget (OMB).
- Seek opportunities for investments into high/risk, high/reward programs

We applaud your inclusion of language that addresses many of these points. As you continue to advance this legislation, we urge that you continue to ensure the necessary details are included in any subsequent drafts to maximize the potential impact of ARPA-H.

Additional Rare Disease Provisions

Finally, I wish to applaud your recognition of the unique challenges and burdens to people and families impacted by rare diseases throughout the legislation. This recognition sends a clear message to our community that people impacted by rare diseases and conditions are not forgotten. In particularly, I wish to note our support for the following:

- **Sec. 103: Pandemic Preparedness Rare Disease Support Program:** The COVID-19 pandemic has laid bare the many challenges people impacted by rare diseases and conditions navigate on a

regular basis and that are accentuated during emergencies like this. We appreciate your recognition of this reality by creating a program to identify specific challenges for rare disease patients as well as solutions to address them, and we encourage that you consider authorizing a higher funding level to enable development of more plans to support more rare disease communities. Hopefully an initiative like this will leave our community and nation at large better prepared to respond to the needs of rare disease patients during any future public health emergencies.

- **Advancements in Clinical Trials (Secs. 204, 302, 304 and 305):** We are grateful that Cures 2.0 builds upon the original Cures' law through additional policies to ensure a patient-centric therapy development model that leverages modern technologies and supports access to therapies. To that end, we enthusiastically support provisions to continue driving use of patient experience data (204) and real-world evidence (304). We are also very supportive of policies to provide grants that support novel trial designs (302) and to establish a stronger relationship between FDA and CMS involving products given breakthrough therapy designation (305). As you work to develop subsequent iterations of this legislation, we urge you to maintain these provisions and also include legislation known as the bipartisan **BENEFIT Act (H.R. 4472, S. 373)**. This legislation will build upon advancements in the field of patient engagement by ensuring that patient experience data is part of the FDA's seminal risk-benefit framework. This action will help ensure that recent and ongoing gains are defined in statute.

Conclusion

Thank you for your attention to our rare disease community's priorities. The EveryLife Foundation was founded to help spur innovation for rare disease treatment through science driven public policy. The passage of the 21st Century Cures Act demonstrated that science-based policy fixes to the development and regulatory process could significantly impact efforts to unlock treatments for patients. We look forward to now continuing to work alongside you to build upon the successes of the passage and implementation of the landmark 21st Century Cures legislation – and realize the potential of existing opportunities to accelerate the development of therapies and access for Americans living with rare diseases through Cures 2.0 and ARPA-H. For additional information, please contact Jamie Sullivan, Director of Public Policy, EveryLife Foundation for Rare Diseases at jsullivan@everylifefoundation.org or by phone at 202-445-4009.

Sincerely,

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Annie Kennedy
Chief of Policy & Advocacy
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