The EveryLife Foundation for Rare Diseases is a 501(c)3 organization
Tax ID # 26-4614274

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January 12, 2021

President-elect Joseph Biden
1401 Constitution Ave., NW
Washington, DC 20230

Dear President-Elect Biden,

Congratulations on being elected as our nation's 46th President. We recognize you will have many health policy decisions to make in the coming months, especially as the COVID-19 public health emergency (PHE) continues. On behalf of the EveryLife Foundation for Rare Diseases and the estimated 30 million Americans living with a rare disease or disorder, we thank you for providing the opportunity to submit policy recommendations to be included for consideration as your health advisors set an agenda for 2021 and your first term. EveryLife's policy priorities are informed by the needs of our community and our shared mission of advancing the equitable development of and access to lifesaving diagnoses, treatments and cures. To inform our policy work, we convene the Community Congress, a forum for collaboration across stakeholders, representing over 150 individual rare disease patient advocacy organizations in addition to over 90 other healthcare and biotechnology organizations.

While exciting advances in science and medicine have propelled new rare disease therapies into reality, currently 93% of the over 7,000 rare diseases still have no FDA approved treatment. Further many of the transformational therapies that are approved then face access barriers that prohibit or delay life-changing treatments from reaching patients.

To address these barriers, we encourage the inclusion of the following priorities among those of your administration:

1. **Accelerate and Inform Rare Disease Therapy Development & Infrastructure**
2. **Access and Coverage for Early and Accurate Diagnoses and Care**
3. **Responding to the COVID-19 Pandemic**

The priorities laid out below are informed by ongoing efforts to document the significant unmet need of the rare disease community, including a landmark study on the economic health impact of rare disease that we will publish in early 2021 and an ongoing effort at the GAO to quantify the impact of rare diseases in the US set to be released in 2021 as well. We welcome an opportunity to meet

with members of your transition team and new administration in the coming weeks and months to present the compelling results from the National Burden of Rare Disease Study and to discuss the policy issues listed below.

Accelerate and Inform Rare Disease Therapy Development & Infrastructure:

Rare Disease FDA Center of Excellence- The 21st Century Cures Act authorized the FDA to establish intercenter institutes, also known as centers of excellence, to coordinate actions across disease areas to improve treatments for patients. The FDA established the first such center, focused on oncology, in 2017. Based on the robust therapeutic pipelines in rare disease and the experiences of the Oncology Center of Excellence, we believe there is an immediate need for the FDA to establish another center of excellence focused on rare diseases and disorders. The centers of excellence are intended to combine expertise from a range of areas to leverage the FDA's ability to facilitate the development of life-saving medical therapies. Developing therapies for small patient populations presents unique challenges, and a Center of Excellence for Rare Diseases could help to improve treatments for the patients who need them most. It is critical that we work to increase the number of safe, effective, and affordable treatments that are available for people with rare diseases and disorders. As such, we request you **use existing authority from the 21st Century Cures Act to promptly establish a Center of Excellence for Rare Diseases at the FDA.**

Rare Disease Advisory Committee - Despite the collective prevalence of rare diseases and the unique challenges faced by our community, there is currently no committee to advise the FDA and the Secretary on the myriad issues pertaining to the development of products to treat rare diseases. As such, the EveryLife Foundation recommends your administration **establish a rare disease and condition advisory committee within the proposed FDA Center of Excellence for Rare Diseases to advise the Secretary of HHS on policies to address barriers impeding the development of rare disease products.** This important committee could consider regulatory pathways, clinical trial design needs, qualifications of biomarkers and remote outcome measurement tools, drug repurposing, and other issues related to the drug review process. A committee comprised of members of the patient community as well as experts in the field of rare disease research, clinical trial design, and therapy development would be an invaluable tool to inform rare disease policies and the development of innovative approaches to therapy approval.

Cures 2.0 - The passage of the 21st Century Cures Act in 2016 demonstrated that science-based policy fixes to the development and regulatory process could significantly impact efforts to unlock treatments for rare disease patients. EveryLife and our rare disease community was proud to work closely with Congressional leaders as thoughtful innovations were transformed into policy solutions. 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 Act and realize the potential of existing opportunities to accelerate the development of therapies and access for Americans living with rare diseases as the Cures 2.0 legislative efforts continue this Congress.

Rare Diseases Clinical Research Network, National Institutes of Health- The EveryLife Foundation commends your long-standing commitment to advancing biomedical research, including your leadership in ensuring the 21st Century Cures Act became law in 2016. We urge your administration to continue this work by ensuring not only that the National Institutes of Health (NIH) but also **rare disease research specifically is robustly funded and supported** in the years to come. Further, we would like to bring to your attention the importance of NIH's Rare Diseases Clinical Research Network (RDCRN), which is an NIH-funded network of researchers, patients, and clinicians collaborating to better understand rare diseases and the all-important issues of diagnosis and treatment. The RDCRN is an initiative of the Office of Rare Diseases Research under the National Center for Advancing Translational Sciences (NCATS), however, other institutes and centers across NIH contribute to the network's success. As you know, rare disease issues span NIH and require the attention of every institute and center. We recommend that, under your leadership, **RDCRN expand its reach across NIH by engaging with and receiving support from every appropriate institute and center.**

The Undiagnosed Diseases Network (UDN) – One of the most arduous elements of any disease is the diagnostic odyssey; the time between the onset of symptoms to the confirmed diagnosis. In rare disease, that odyssey often takes many years, visits to dozens of specialists, costly and invasive diagnostic tests, and irreversible disease progression. But much more, rare disease patients often find no answers. The Undiagnosed Diseases Network (UDN) is a research study that was initially funded by the National Institutes of Health Common Fund. Its purpose is to bring together clinical and research experts from across the United States to solve the most challenging medical mysteries using advanced technologies. The UDN is made up of a Coordinating Center, Clinical Sites, and Core Facilities ("Cores"). The Coordinating Center, which coordinates the work of the UDN, is based at the Department of Biomedical Informatics at Harvard Medical School. The Clinical Sites, where UDN participants are evaluated, are in 12 cities across the United States. Since inception, more than 4713 people have applied to be served through the UDN, 1845 have been accepted, 1457 have been evaluated, and 434 have received confirmed diagnoses and been referred for expert care and treatment. Continued support for the UDN is a priority for the EveryLife Foundation and the rare disease community.

Orphan Products Clinical Trial Grants Program and Natural History Grants Program, Food and Drug Administration - The Office of Orphan Products Development (OOPD) within the FDA administers provisions of the Orphan Drug Act to promote development of therapies for rare disease patients. OOPD manages two vital and successful grant programs - the Orphan Product Clinical Trial Grants Program and the Natural History Grants Program. These programs help to bring therapies to patients that save lives and lower long-term healthcare costs. However, in recent years the programs' capacity to provide support to clinical trials that will lead to life-saving and life-improving therapies for patients with rare diseases has been significantly reduced. We urge your administration to recognize that these critical and life-saving research resources reflect the urgent and unmet need within the rare disease community.

Access and Coverage for Early and Accurate Diagnoses and Care:

Enhance Support for State Newborn Screening Programs & Other Innovative Genetic Testing Options- Newborn screening reaches each of the four million babies born in the U.S. every year, identifying serious, life-threatening conditions in approximately 1 in 300 newborns. Yet, newborn screening programs vary from state to state. That means that while a baby born with a disease such as Spinal Muscular Atrophy (SMA) in a state that includes SMA on the screening panel would be identified immediately at birth and offered life-saving treatment, that same baby could be born in a neighboring state where SMA is not screened for at birth would go undetected until the disease has irreversibly advanced and shortened their lifespan dramatically. Limited state resources are often the barrier to adding new conditions after they are thoroughly studied, reviewed, and approved by the federal oversight committee. Further, the federal authorization for the Advisory Committee on Heritable Disorders in Newborn and Children has been expired since 2019, starving the program of new resources and modernization efforts that could translate the remarkable advances in the discovery of new treatments for rare genetic disorders into more lives saved. As such, we urge your administration to **request substantial funding increases for the CDC to adequately support newborn screening programs, and we ask you to work with Congress to ensure that the Newborn Screening Saves Lives Act is finally reauthorized in 2021**. Newborn screening saves lives. But only when authorized, resourced, and implemented.

CMS-FDA Collaboration with Payers, Medicaid, and Medicare to Cover Rare Disease Therapies- The rare disease community is regularly challenged by payer coverage policies that narrow a broad indication in a therapy's label by limiting access to the population that participated in a product's clinical trial or trials. Examples include coverage policies that have sought to limit access to only those persons who meet age, functionality, and other criteria of the clinical trial(s) population. This approach overlooks the totality of scientific information that FDA uses to inform a product's label and, ultimately, undermines access. The impact is often felt most by vulnerable populations such as children, older adults, and those with comorbidities who are often excluded from initial registration clinical trials but have disease features very similar to the studied populations. The EveryLife Foundation recommends policies that will maximize the likelihood that Medicare, Medicaid/CHIP, and commercial coverage policies reflect the totality of medical and scientific information and the FDA's use of such data to inform label decisions.

Your administration can take the following steps to **help ensure early, informed coordination between FDA and payers with the goal of improving patients' access to care**:

- Establish a rare disease or disorder therapy payor advisory program to bring together multiple payers – governmental and commercial— on a voluntary basis to help inform coverage recommendations pertaining to rare disease products with a focus on coverage policies that match the full indicated population.
- Direct CMS to issue guidance—including coverage recommendations—to state Medicaid and CHIP plans as soon as possible following an FDA approval of a therapy to treat a rare disease or condition

Responding to the COVID-19 Pandemic

Many with rare diseases are considered significantly immunocompromised and others have severe heart, lung, kidney or liver damage due to their rare disease. During the ongoing COVID-19 pandemic, patients and their families have largely put their lives on hold, making all possible sacrifices – including foregoing care, sacrificing financially, socially, academically, and professionally - to reduce the chances of coronavirus exposure. To inform our COVID-19 related work, we convened a working group of rare disease patient advocacy organizations and partners from the biotechnology industry. Since the start of the pandemic, the collaboration has identified real-time impacts on rare disease patients and families and considered the longer-term implications of policy changes during the pandemic. The authorization of two novel vaccines for SARS-CoV-2 offers hope to the community, however there are **concerns unique to the rare disease community that we urge your administration to proactively address. We also urge you to consider the lessons learned during the pandemic that support the long-term implementation of policies put in place to mitigate the risk from COVID-19** and to act where you can, and to work with Congress where necessary to implement these changes beyond the public health emergency.

Rare Disease Community Concerns Around the Vaccine – We conducted a survey of nearly 1500 rare disease patients and family members to identify the likelihood that they would accept the vaccine upon authorization or full FDA approval and to consider the factors that are most important to their decision making. Early results indicate people with rare diseases have significant concerns and questions that must be answered before they are willing to consider the vaccine. Less than 8% of patients said that they were 'very likely' to be vaccinated and 37% were 'very unlikely' under an emergency use authorization. Free text responses revealed some of the community's most pressing questions about the vaccine. The questions focused on safety considering there were very few, if any, patients that look like them in the clinical trials; efficacy for immune compromised patients; impact of the vaccine on innovative gene therapies in the future, and the safety of vaccine administration considering most have avoided public interactions. We look forward to sharing the full results from this survey with you as the analysis is finalized in the coming months. **We urge you to work across agencies and with industry to implement robust real-world evidence collection and public facing communication that will account for these and other questions of the rare disease community.**

CMS Flexibilities for Home Health and Telehealth- In the early weeks and months of the COVID-19 PHE policymakers took important steps to ensure patients could access essential care while the nation grappled with controlling the spread of the virus. Specific time-limited regulatory flexibilities have removed significant barriers to care and improved access for the 30 million Americans living with a rare disease or condition. However, these flexibilities are at risk of going away when the PHE ends. The EveryLife Foundation urges your administration to recognize how these flexibilities have benefited members of the rare disease community and consider which policies should be kept in place even after the PHE ends. Specifically, we urge you to **work with Congress, as necessary, to ensure patients—especially those with a rare disease or other underlying condition—continue to have access to home health and telehealth services** even as the PHE and accompanying regulatory flexibilities eventually

expire. These flexibilities include CMS's updated definition of "homebound," which has allowed patients to receive the Medicare home health benefit during the PHE as well as policies that temporarily removed arbitrary restrictions on Medicare patients' access to telehealth services.

Thank you for keeping the rare disease community in mind and giving these policy recommendations every consideration as you and your team begin to prioritize health-related activities in the early days of your administration. The EveryLife Foundation for Rare Diseases stands ready to work with you on the above-mentioned areas as well as any health programs and policies that touch the rare disease community.

Please know our organization will be a resource to you in the coming weeks, months, and years. We welcome the opportunity to meet with you about the priorities of the rare disease community or to help facilitate engagement of our community organization partners in administration policy priorities. Please contact Annie Kennedy, Chief of Policy and Advocacy at akennedy@everyLifefoundation.org

Sincerely,

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Executive Director
EveryLife Foundation for Rare Diseases

CC: Annie Kennedy, Chief of Policy & Advocacy
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