



September 4, 2020

VIA ELECTRONIC DELIVERY

Committee on Equitable Allocation of Vaccine for the Novel Coronavirus
National Academy of Medicine
National Academies of Sciences, Engineering, and Medicine

RE: *Discussion Draft of the Preliminary Framework for Equitable Allocation COVID-19 Vaccine*,
commissioned by the National Institutes of Health and the Centers for Disease Control
and Prevention

Dear Committee Members,

On behalf of the estimated 30 million Americans living with rare diseases¹, the EveryLife Foundation for Rare Diseases is pleased to offer the following comments regarding the National Academy of Sciences' *Discussion Draft of the Preliminary Framework for Equitable Allocation of COVID-19 Vaccine*. The EveryLife Foundation for Rare Diseases is a 501(c)(3) nonprofit, 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.

The rare disease community includes more than 7,000 rare diseases¹ with varied causes, symptoms, progression, and prognosis, about 50% of whom are children². We commend you for your ongoing efforts to protect the public's health and urge you to continue to protect rare disease patients who are particularly vulnerable to the devastating effects of COVID-19.

Many with rare diseases are considered significantly immunocompromised and others have severe heart, lung, kidney or liver damage due to their rare disease. Patients and their families have largely put their lives on hold, making all possible sacrifices to reduce the likelihood that they would not contract COVID-19 due to their pre-existing health challenges.

Our community's hope for resuming activities of daily living rest on the development, approval and distribution of a safe and effective vaccine -- and on the implementation of guidelines practices that will enable a safe return to the community.

We appreciate the careful consideration that the National Academy of Sciences (NAS) taskforce has put into creating the recommendations in the proposed Framework for Equitable Allocation of a COVID-19 Vaccine. There are no simple answers and the honorable intentions of these

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recommendations are without question. However, we firmly believe that the recommendations for priority groups do not meet the needs of the rare disease community and will result in preventable suffering, morbidity and mortality. We offer the following 4 recommended Framework changes, as well as considerations for future deliberations, that will account for the unique factors facing the rare disease community.

Framework Recommendations:

A - Adults with rare diseases with conditions that may make them vulnerable to severe outcomes from COVID-19 should be listed as a priority group in Phase 1b. Further, the process for defining this group should be refined such that it incorporates the likelihood that one's disease makes them vulnerable based on similarities to documented higher risk conditions and their likelihood of increased exposure.

B - Paid Care providers for both adults and children with rare diseases that may make them vulnerable to severe outcomes from COVID-19 should be listed as a priority group in Phase 1b

C - Adults in households with children with rare diseases that may make them vulnerable to severe outcomes from COVID-19 should be listed as a priority group in Phase 2a

D - Upon regulatory approval of a vaccine that is safe and effective for children, those children with rare diseases that may make them vulnerable to severe outcomes from COVID-19 should be prioritized

Recommendation A:

Adults with rare diseases with conditions that may make them vulnerable to severe outcomes from COVID-19 should be listed as a priority group in Phase 1b. Further, the process for defining this group should be refined such that it incorporates the likelihood that one's disease makes them vulnerable based on similarities to documented higher risk conditions and their likelihood of increased exposure.

The Framework's current approach to Phase 1b includes "people of all ages with comorbid and underlying conditions that put them at significantly higher risk." However, the framework then defines this group to be those with two or more of the conditions recognized by the Centers for Disease Control and Prevention (CDC) as proven to put individuals at high risk. The data set that this recommendation is premised on cannot accurately represent the real dangers posed to rare disease patients. On March 8, 2020, the CDC advised that people at high risk of complications – a group including many people with rare diseases – begin taking needed

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medical precautions if staying at home for prolonged periods of time became necessary³. The rare disease community has since gone to extreme lengths to disrupt their lives, sacrifice financially, socially, and academically, forego necessary services, and remain isolated all in order to reduce the chances of coronavirus exposure. Furthermore, the conditions identified in the current recommendations have many parallels in pathophysiology and symptomology to rare diseases, thus it is reasonable to assume these individuals are also at higher risk of severe outcomes. Finally, the very nature of living with many rare diseases and the frequent healthcare services they need, results in these individuals having increased risks of exposure to COVID-19.

We strongly urge the task force to revise this section of the framework to acknowledge adults with rare diseases, **with conditions that may make them vulnerable to severe outcomes from COVID-19**, will be included within the population defined as having comorbid and underlying conditions that put them at significantly higher risk.

Recommendation B: Paid Care providers for both adults and children with rare diseases that may make them vulnerable to severe outcomes from COVID-19 should be listed as a priority group in Phase 1b

Individuals with rare diseases rely on a large network of traditional facility-based and home-based medical and non-medical support services. In addition to utilizing frequent facility-based health care services, people with rare diseases often rely on home based services from professionals such as nurses, physical, occupational, and speech therapists, home aids that assist with activities of daily living, and educational and behavioral support specialists. Many who support people with rare diseases in their homes visit multiple homes per day and are at greater risk of being exposed to COVID-19, as well as risk exposing their vulnerable patients who, in many cases, may be unvaccinated children.

While we fully agree that those healthcare workers operating in the highest risk settings must be prioritized as Phase 1a, the group of healthcare and support service providers that regularly treat vulnerable rare disease patients should be included in Phase 1b.

Recommendation C: Adults in households with children with rare diseases that may make them vulnerable to severe outcomes from COVID-19 should be listed as a priority group in Phase 2a

We are aware that no existing COVID-19 studies have enrolled children with rare diseases and understand that safety and efficacy data related to the vaccination of pediatric patients with



rare diseases may not be imminent. That said, the ability to protect children with rare diseases through the availability of vaccinations to adult household members is a priority. This is an effort to create a protective ‘household herd immunity’ for vulnerable children with rare diseases.

Recommendation D: - Upon regulatory approval of a vaccine that is safe and effective for children, those children with rare diseases that may make them vulnerable to severe outcomes from COVID-19 should be prioritized.

Additional Considerations:

1. Rare Disease Community Representation:

We recognize that the proposed Discussion Draft is the first of many steps in the process to create an equitable allocation system. As the Committee continues its work, and as additional groups at the federal, state and local level form to address implementation and distribution of an approved vaccine, we recommend the inclusion of both rare disease patient community and rare disease clinical experts as key stakeholders.

2. Loss of Life Year vs Loss of Life

The Discussion Draft proposes that the Framework utilize standards of fairness when determining principles for vaccine allocation and referenced “loss of life vs life years lost” as a potential consideration in the decision matrix (Discussion Draft, pg 40). The Discussion Draft referenced these considerations in terms of factors related to age, and further stated, *“A related debate about age concerns the loss of life years versus the loss of life. Older adults in their eighties, for instance, generally lose fewer life years if they die than children or young adults who die.... Resolving these conflicts depends on evidence about the relative effectiveness of different vaccine strategies at particular stages in the pandemic give available supplies of vaccine...”*

While we support an evidence-based approach, we caution the Committee against utilizing discriminatory metrics that devalue the quality of life of individuals with rare diseases and disabilities.⁴

3. Process & Transparency:

The Discussion Draft states that *“Procedural fairness is also crucial. This means that decisions about allocation, distribution, and access to vaccine should incorporate input from affected groups, especially those disproportionately affected.... Ideally affected individuals and communities should be able to appeal decisions, and in doing so, the committee believes that*

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the transparency of its principles will help adjudicate those subsequent debates.” (Discussion Draft, 875-881)

The national rare disease patient community is among those at substantially high-risk whose priorities have yet to be well understood and incorporated into the Framework. A defined and transparent appeals process that includes experts with knowledge of rare disease issues will be essential to meeting procedural fairness obligations.

4. Ensuring Access to Safe Vaccination Administration

While we recognize that the purpose of the Framework is not to create specific guidelines for how the vaccine will be administered to the public, we urge you to include concrete suggestions that will help local decision makers keep the priority populations that you have identified safe while receiving the vaccine. Rare disease patients and families have exercised great caution to avoid COVID-19 exposure and many have unique mobility needs that might necessitate in-home or drive-through vaccination. It is important that any implementation planning account for the unique needs of rare disease and other priority populations so that a vaccine will be accessible, and they will not be disadvantaged by barriers to public vaccine center access.

5. Evolving and Continuing the Research and Review Process

As recognized in the Discussion Draft, the guidelines and their implementation will require adjustment as the available evidence evolves, and will also be dependent on the specifics of the approved vaccine to be administered. The Committee should be clear in their recommendations that above all, the priority populations defined in the Framework, including patients and families with rare diseases, will be depending on access to a vaccine as soon as, but not before, it is proven to be safe and efficacious. We also urge you to recognize that the first approval is not the end of the process. In order to inform the evolution of the Framework’s recommendations and individual’s own benefit and risk considerations, it is vital to support and participate in ongoing clinical research. The rare disease community has a long history of proactive involvement in research and in healthcare decision making and once again stands ready to actively engage in the process long after the first vaccines administered so that vital research on the vaccines real-world effects can be collected and used to inform ongoing prioritization and decision making.

Conclusion

The EveryLife Foundation applauds the Committee on Equitable Allocation of Vaccine for the Novel Coronavirus for your ongoing efforts to protect the public’s health and urge you to continue to protect rare disease patients who are particularly vulnerable to the devastating

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effects of COVID-19. We offer our comments with the hope of achieving greater clarity and definition that is of high importance to the rare disease patient community. The EveryLife Foundation would be happy to serve as a resource or to facilitate engagement with rare disease community partners; please contact Jamie Sullivan, Director of Policy at jsullivan@everylifefoundation.org.

Sincerely,

A handwritten signature in black ink, appearing to read "Julia Jenkins".

Julia Jenkins
Executive Director
EveryLife Foundation for Rare Diseases

A handwritten signature in black ink, appearing to read "Annie Kennedy".

Annie Kennedy
Chief of Policy & Advocacy
EveryLife Foundation for Rare Diseases

A handwritten signature in black ink, appearing to read "Jamie Sullivan".

Jamie Sullivan
Director of Policy
EveryLife Foundation for Rare Diseases

CC: Mark Dant, Board Chairman
Frank J. Sasinowski, Board Vice-Chairman
Amrit Ray, MD, Board of Directors
Richard Finkel, MD, Board of Directors

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