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By Electronic Submission

Division of Dockets Management (HFA-305)
Food and Drug Administration
5630 Fishers Lane, rm. 1061
Rockville, MD 20852

**Comment on “Interpreting Sameness of Gene Therapy Products Under the Orphan Drug Regulations”
[Docket No. FDA-2019-D-5392]**

Dear Madam/Sir:

Pfizer Inc. (Pfizer) is submitting these comments in response to the Federal Register notice of January 30, 2020 (85 FR 5445 - 5446).

At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety, and value in the discovery, development, and manufacture of health care products. From specialized efforts in biosimilars and rare disease to oncology and vaccines, we are committed to developing medical solutions that will matter most to the people we serve.

Pfizer would like to thank the Agency for its release of this draft guidance and acknowledge the timeliness of the guidance. Pfizer has the following specific comments regarding the content of the guidance.

GENERAL COMMENTS:

A. Fostering innovation and enhancing public health

Pfizer acknowledges the importance of the Orphan Drug Act (the Act) in stimulating innovation and encouraging the development of treatments for patients with rare diseases. We fully support the Agency ensuring these important policy objectives are appropriately implemented. At the same time, we note the large amount of growth and transformative potential of gene therapies that is often spoken of by Peter Marks. To that end, Pfizer believes too narrow an interpretation of sameness may limit the potential contribution of AAV gene therapies to public health and discourage development of products that offer unique value.

B. Principal molecular structural features in sameness determination

Pfizer agrees that two gene therapy products that use different transgenes and/or utilize different virus classes should be different. We note, however, that for vectors from the same viral class, the Agency is proposing to evaluate sameness on a case-by-case

basis. Pfizer suggests that two serotypes from the same class, such as AAV2 and AAV5, be considered different. Such differentiation is supported by the science; evolutionary differences are captured by the parvovirus phylogenetic classification and are known to impact biological properties such as immunogenicity, tropism and infectivity. Pfizer would agree that variances within a phylogenetic classification should be interpreted on a case-by-case basis; for example, when differences exist or are engineered into a specific AAV serotype.

CONCLUSION:

We appreciate the opportunity to comment on this resource. Specific line-by-line and editorial comments are included in the tables below. If you have any questions about these comments, please contact Laura McKinley at laura.m.mckinley@pfizer.com.

Sincerely,

A handwritten signature in black ink that reads "Laura McKinley". The signature is written in a cursive, flowing style.

Laura McKinley, Ph.D.
Director, Regulatory Policy

