



Our STN: BL 125610/0

**PROPOSED SUFFIXES UNACCEPTABLE
October 25, 2017**

Spark Therapeutics, Inc.
Attention: Jim Wang, Ph.D.
3737 Market Street, Suite 1300
Philadelphia, PA 19104

Re: **LUXTURNA (voretigene neparvovec – xxxx)**

Dear Dr. Wang:

Reference is made to Spark Therapeutics Inc. (Spark)'s October 11, 2017 request for review of proposed suffixes for *voretigene neparvovec*. You provided ten candidate suffixes, in order of preference, along with a consult review conducted by Addison Whitney. In your cover letter, you state that the names were developed and evaluated in accordance with the criteria established in the January 2017 FDA *Guidance for Industry – Nonproprietary Naming of Biological Products*. Specifically, Addison Whitney states that the proposed names were screened against:

- FDA-designated proper name suffixes and criteria;
- Currently marketed products (searching Mednet, Drugs@FDA, Drugs.com and Thomson Micromedex databases);
- Common medical abbreviations; and
- United States Patent and Trademark Office (USPTO) federal database.

The Center for Biologics Evaluation and Research's Office of Tissues and Advanced Therapies and Office of Compliance and Biologics Quality reviewed your proposed proper name suffixes and found all of them unacceptable. You represent that your suffixes are devoid of meaning, but we disagree and note that each one is meaningful in one or more of the following ways:

- Directly representating your product (e.g., (b) (4) is pronounced "eyes" and (b) (4) references the posterior retinal location known as *y*RPE, the RPE65 gene, or retinal pigment epithelium in general);
- Directly referencing Spark (e.g., (b) (4));
- Connoting your proper name, *voretigene neparvovec* (e.g., (b) (4)); and/or
- Using a medical abbreviation that is well known to the end users of your product, (e.g., (b) (4)).

Because your proposed suffixes were found unacceptable, you may provide us with additional proposed suffixes for consideration. Given the time constraints, we recommend that you provide proposed suffixes for review within the next 10 business days. Please be informed that, in the absence of acceptable alternate proposals, we intend to assign a randomly generated, pre-screened suffix for inclusion in the proper name if your product will be licensed within this review cycle.

The following are the FDA generated suffixes:

voretigene neparvovec-rzyl
voretigene neparvovec-fsjb
voretigene neparvovec-zklf

If you have any questions, please contact Nevitt Morris, Regulatory Project Manager on this file at (240) 402-8269.

Sincerely yours,

Ramani V. Sista, Ph.D.
Division Director
Regulatory Project Management
Office of Tissues and Advanced Therapies
Center for Biologics Evaluation and Research